Table of Contents

UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
Washington, DC 20549

FORM 10-K

(Mark One)

☒ **ANNUAL REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**

For the fiscal year ended December 31 , 2023

OR

☐ **TRANSITION REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**

For the transition period from to

Commission File Number: 001-37625

Voyager Therapeutics, Inc.
(Exact Name of Registrant as Specified in Its Charter)

Delaware	**46-3003182**
(State or Other Jurisdiction of Incorporation or Organization)	**(IRS Employer Identification No.)**
75 Hayden Avenue , **Lexington , Massachusetts**	**02421**
(Address of Principal Executive Offices)	**(Zip Code)**

(857) 259-5340
(Registrant's Telephone Number, Including Area Code)

Securities registered pursuant to Section 12(b) of the Act:

Title of each class	Trading Symbol(s)	Name of each exchange on which registered
Common Stock, $0.001 par value	VYGR	Nasdaq Global Select Market

Indicate by check mark if the registrant is a well-known seasoned issuer, as defined in Rule 405 of the Securities Act. **Yes** ☐ **No** ☒

Indicate by check mark if the registrant is not required to file reports pursuant to Section 13 or Section 15(d) of the Act.**Yes** ☐ **No** ☒

Indicate by check mark whether the registrant: (1) has filed all reports required to be filed by Section 13 or 15(d) of the Securities Exchange Act of 1934 during the preceding 12 months (or for such shorter period that the registrant was required to file such reports), and (2) has been subject to such filing requirements for the past 90 days. **Yes** ☒ **No** ☐

Indicate by check mark whether the registrant has submitted electronically every Interactive Data File required to be submitted pursuant to Rule 405 of Regulation S-T (§ 232.405 of this chapter) during the preceding 12 months (or for such shorter period that the registrant was required to submit such files). **Yes** ☒ **No** ☐

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, a smaller reporting company, or an emerging growth company. See the definitions of "large accelerated filer," "accelerated filer," "smaller reporting company," and "emerging growth company" in Rule 12b-2 of the Exchange Act.

Large accelerated filer	☐	Accelerated filer	☐
Non-accelerated filer	☒	Smaller reporting company	☒
		Emerging growth company	☐

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act. ☐

Indicate by check mark whether the registrant has filed a report on and attestation to its management's assessment of the effectiveness of its internal control over financial reporting under Section 404(b) of the Sarbanes-Oxley Act (15 U.S.C. 7262(b)) by the registered public accounting firm that prepared or issued its audit report. ☒

If securities are registered pursuant to Section 12(b) of the Act, indicate by check mark whether the financial statements of the registrant included in the filing reflect the correction of an error to previously issued financial statements. ☐

Indicate by check mark whether any of those error corrections are restatements that required a recovery analysis of incentive-based compensation received by any of the registrant's executive officers during the relevant recovery period pursuant to § 240.10D-1(b). ☐

Indicate by check mark whether the registrant is a shell company (as defined in Rule 12b-2 of the Act). **Yes** ☐ **No** ☒

The aggregate market value of Common Stock held by non-affiliates of the registrant computed by reference to the price of the registrant's Common Stock as of June 30, 2023, the last business day of the registrant's most recently completed second fiscal quarter, was approximately $ 488.8 million (based on the last reported sale price on the Nasdaq Global Select Market as of such date).

As of February 21, 2024 there were 54,300,627 shares of the registrant's common stock, par value $0.001 per share, outstanding.

DOCUMENTS INCORPORATED BY REFERENCE

Portions of the registrant's definitive Proxy Statement relating to its 2024 Annual Meeting of Stockholders are incorporated by reference into Part III of this Annual Report on Form 10-K where indicated. Such Proxy Statement is expected to be filed with the U.S. Securities and Exchange Commission not later than 120 days after the end of the fiscal year to which this report relates.

Table of Contents

		Page
PART I.		
Item 1.	Business	7
Item 1A.	Risk Factors	49
Item 1B.	Unresolved Staff Comments	111
Item 1C.	Cybersecurity	111
Item 2.	Properties	112
Item 3.	Legal Proceedings	112
Item 4.	Mine Safety Disclosures	112
PART II.		
Item 5.	Market for Registrant's Common Equity, Related Stockholder Matters and Issuer Purchases of Equity Securities	112
Item 6.	[Reserved]	113
Item 7.	Management's Discussion and Analysis of Financial Condition and Results of Operations	113
Item 7A.	Quantitative and Qualitative Disclosures about Market Risk	123
Item 8.	Financial Statements and Supplementary Data	124
Item 9.	Changes in and Disagreements with Accountants on Accounting and Financial Disclosure	124
Item 9A.	Controls and Procedures	124
Item 9B.	Other Information	125
PART III.		
Item 10.	Directors, Executive Officers and Corporate Governance	126
Item 11.	Executive Compensation	126
Item 12.	Security Ownership of Certain Beneficial Owners and Management and Related Stockholder Matters	126
Item 13.	Certain Relationships and Related Transactions, and Director Independence	126
Item 14.	Principal Accountant Fees and Services	126
PART IV.		
Item 15.	Exhibits and Financial Statement Schedules	126
Item 16.	Form 10-K Summary	127
Signatures		

FORWARD-LOOKING STATEMENTS

This Annual Report on Form 10-K contains forward-looking statements that involve substantial risks and uncertainties. All statements other than statements of historical facts contained in this Annual Report on Form 10-K, including statements regarding our strategy, future operations, future financial position, future revenue, projected costs, prospects, plans, objectives of management and expected market growth, are forward-looking statements. These statements involve known and unknown risks, uncertainties and other important factors that may cause our actual results, performance, or achievements to be materially different from any future results, performance or achievements expressed or implied by the forward-looking statements.

The words “anticipate,” “believe,” “estimate,” “expect,” “intend,” “may,” “might,” “plan,” “predict,” “project,” “target,” “potential,” “contemplate,” “anticipate,” “goals,” “will,” “would,” “could,” “should,” “continue,” and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. These forward-looking statements include, among other things, statements about:

- our plans to develop and commercialize our product candidates based on adeno-associated virus, or AAV, gene therapy and our proprietary antibodies;
- our ability to continue to develop our proprietary gene therapy platform technologies, including our TRACER™ (Tropism Redirection of AAV by Cell-type-specific Expression of RNA) discovery platform and our vectorized antibody platform, our proprietary antibody program, and our gene therapy and vectorized antibody programs;
- our ability to identify and optimize product candidates and proprietary AAV capsids;
- our strategic collaborations and licensing agreements with, and funding from, our collaboration partners Neurocrine Biosciences, Inc. and Novartis Pharma AG, or Novartis, and our licensee Alexion, AstraZeneca Rare Disease (successor-in-interest to former licensee Pfizer Inc.);
- our ongoing and planned preclinical development efforts, related timelines and studies;
- our ability to enter into future collaborations, strategic alliances, or option and license arrangements;
- the timing of and our ability to submit applications and obtain and maintain regulatory approvals for our product candidates, including the ability to submit investigational new drug, or IND, applications for our programs;
- our estimates regarding revenue, expenses, contingent liabilities, future revenues, existing cash resources, capital requirements and cash runway;
- our intellectual property position and our ability to obtain, maintain and enforce intellectual property protection for our proprietary assets;
- our estimates regarding the size of the potential markets for our product candidates and our ability to serve those markets;
- our need for additional funding and our plans and ability to raise additional capital, including through equity offerings, debt financings, collaborations, strategic alliances, and option and license arrangements;
- our competitive position and the success of competing products that are or might become available for the indications that we are pursuing;
- the impact of government laws and regulations including in the United States, the European Union, and other important geographies such as Japan; and

- our ability to control costs and prioritize our product candidate pipeline and platform development objectives successfully in connection with our strategic initiatives.

These forward-looking statements are only predictions and we may not actually achieve the plans, intentions or expectations disclosed in our forward-looking statements. You should not place undue reliance on our forward-looking statements. Actual results or events could differ materially from the plans, intentions and expectations disclosed in the forward-looking statements we make. We have based these forward-looking statements largely on our current expectations and projections about future events and trends that we believe may affect our business, financial condition and operating results. We have included important factors in the cautionary statements included in this Annual Report on Form 10-K, particularly in “Part I, Item 1A - Risk Factors” that could cause actual future results or events to differ materially from the forward-looking statements that we make. Our forward-looking statements do not reflect the potential impact of any future acquisitions, mergers, dispositions, strategic collaborations, licenses, joint ventures or investments we may make.

You should read this Annual Report on Form 10-K and the documents that we have filed as exhibits to the Annual Report on Form 10-K with the understanding that our actual future results may be materially different from what we expect. We do not assume any obligation to update any forward-looking statements whether as a result of new information, future events or otherwise, except as required by applicable law.

We obtained the statistical and other industry and market data in this Annual Report on Form 10-K and the documents we have filed as exhibits to the Annual Report on Form 10-K from our own internal estimates and research, as well as from industry and general publications and research, surveys, studies and trials conducted by third parties. Some data is also based on our good faith estimates, which are derived from management’s knowledge of the industry and independent sources. This data involves a number of assumptions and limitations, and you are cautioned not to give undue weight to such estimates. In addition, while we believe the market opportunity information included in this Annual Report on Form 10-K and the documents we have filed as exhibits to the Annual Report on Form 10-K is reliable and is based upon reasonable assumptions, such data involves risks and uncertainties and are subject to change based on various factors, including those discussed under “Risk Factors” and in the documents we have filed as exhibits to the Annual Report on Form 10-K. In addition, statements that “we believe” and similar statements reflect our beliefs and opinions on the relevant subject. These statements are based upon information available to us as of the date of this Annual Report on Form 10-K, and while we believe such information forms a reasonable basis for such statements, such information may be limited or incomplete, and our statements should not be read to indicate that we have conducted an exhaustive inquiry into, or review of, all potentially available relevant information. These statements are inherently uncertain and investors are cautioned not to unduly rely upon these statements.

We own various U.S. federal trademark registrations and applications and unregistered trademarks, including our corporate logo. This Annual Report on Form 10-K and the documents filed as exhibits to the Annual Report on Form 10-K contain references to trademarks, service marks and trade names referred to in this Annual Report on Form 10-K and the information incorporated herein, including logos, artwork, and other visual displays, that may appear without the ® or TM symbols, but such references are not intended to indicate, in any way, that we will not assert, to the fullest extent under applicable law, our rights or the rights of the applicable licensor to these trademarks, service marks or trade names. We do not intend our use or display of other companies’ trade names, service marks or trademarks to imply a relationship with, or endorsement or sponsorship of us by, any other companies. All trademarks, service marks and trade names included or incorporated by reference into this Annual Report on Form 10-K and the documents filed as exhibits to the Annual Report on Form 10-K are the property of their respective owners.

RISK FACTOR SUMMARY

Investment in our securities involves risk and uncertainties that you should be aware of when evaluating our business. The following is a summary of what we believe to be the principal risks facing our business, as more fully described under " Part I, Item 1A - Risk Factors" and elsewhere in this Annual Report on Form 10-K. The risks and uncertainties described below are not the only risks and uncertainties we face. Additional risks and uncertainties not presently known to us or that we presently deem less significant may also impair our business operations.

- We have a history of incurring significant losses and anticipate that we will continue to incur losses for the foreseeable future and may never achieve or maintain consistent profitability.
- We will need to raise additional funding, which may not be available on acceptable terms, or at all. Failure to obtain this necessary capital when needed may force us to delay, limit or terminate certain of our product development efforts or other operations.
- Our AAV gene therapy and other biological therapy product candidates are based on a proprietary technology and, in several disease areas, unvalidated treatment approaches, which makes it difficult and potentially infeasible to predict the duration and cost of development of, and subsequently obtaining regulatory approval for, our product candidates.
- Regulatory requirements governing biological and gene therapy products have changed frequently and may continue to change in the future. Such requirements may lengthen the regulatory review process, require us to modify current studies or perform additional studies or increase our development costs, which in turn may force us to delay, limit, or terminate certain of our programs.
- We are early in our development efforts. All of our active product candidates are currently in preclinical development or are advancing into the clinic. We may encounter substantial delays or difficulties in commencement, enrollment or completion of our preclinical studies or clinical trials, or we may fail to demonstrate safety and efficacy to the satisfaction of applicable regulatory authorities, any of which could prevent us from commercializing our current and future product candidates on a timely basis, if at all.
- Our product candidates may cause undesirable side effects or have other properties that could delay or prevent their regulatory approval, limit their commercial potential or result in significant negative consequences following any potential marketing approval.
- We face significant competition in an environment of rapid technological change and the possibility that our competitors may achieve regulatory approval before us or develop therapies that are more advanced or effective than ours, which may harm our business and financial condition, and our ability to successfully market or commercialize our product candidates.
- To date, all of our revenue has been derived from our ongoing collaborations and licensing agreements with Neurocrine, Novartis, Alexion and Sangamo and from our prior collaborations with Sanofi Genzyme Corporation, AbbVie Biotechnology Ltd and AbbVie Ireland Unlimited Company. If any ongoing or future collaboration, option and license, or license agreements were to be terminated, our business financial condition, results of operations and prospects could be harmed.
- Our gene therapies are novel, complex and difficult to manufacture. We could experience manufacturing problems that result in delays in the development or commercialization of our product candidates or otherwise harm our business.
- Our future success depends on our ability to retain key members of our management and research and development teams, and to attract, retain and motivate qualified personnel.
- Our gene therapy and vectorized antibody approaches utilize vectors derived from viruses that are selectively engineered, which may be perceived as unsafe or may result in unforeseen adverse events.

Negative public opinion and increased regulatory scrutiny of gene therapy may damage public perception of the safety of our gene therapy product candidates and adversely affect our ability to conduct our business or obtain regulatory approvals for our gene therapy product candidates.

- If we are unable to obtain and maintain patent protection for our products and technology, or if the scope of the patent protection obtained is not sufficiently broad, our competitors could develop and commercialize products and technology similar or identical to ours, and our ability to successfully commercialize our products and technology may be adversely affected.

PART I

ITEM 1. BUSINESS

We are a biotechnology company whose mission is to leverage the power of human genetics to modify the course of and ultimately cure neurological diseases. Our pipeline includes programs for Alzheimer's disease, or AD; amyotrophic lateral sclerosis, or ALS; Parkinson's disease; and multiple other diseases of the central nervous system. Many of our programs are derived from our TRACER™ (Tropism Redirection of AAV by Cell-type-specific Expression of RNA) adeno-associated virus, or AAV, capsid discovery platform, which we have used to generate novel capsids, or TRACER Capsids, and identify associated receptors to potentially enable high brain penetration with genetic medicines following intravenous dosing. Some of our programs are wholly-owned, and some are advancing with licensees and collaborators including Alexion, AstraZeneca Rare Disease, or Alexion; Novartis Pharma AG, or Novartis; Neurocrine Biosciences, Inc., or Neurocrine; and Sangamo Therapeutics, Inc., or Sangamo.

We focus on leveraging our expertise in capsid discovery and neuropharmacology to address the delivery hurdles that have constrained the genetic medicine and neurology disciplines, with the goal of either halting or slowing disease progression or reducing symptom severity, and therefore providing clinically meaningful impact to patients. We are advancing our own proprietary pipeline of drug candidates for neurological diseases, with a focus on AD. Our wholly-owned prioritized pipeline programs include an anti-tau antibody for AD; a superoxide dismutase 1, or SOD1, silencing gene therapy for ALS; and a tau silencing gene therapy for AD. We identified a lead development candidate for our anti-tau antibody program in the first quarter of 2023 and expect to submit an investigational new drug, or IND, application to the U.S. Food and Drug Administration, or the FDA, for this program in the first half of 2024. We believe this trial could result in the potential to generate proof-of-concept data for slowing the spread of pathological tau via tau positron emission tomography, or PET, imaging in 2026. We identified a lead development candidate for the SOD1 silencing gene therapy program in the fourth quarter of 2023, and we expect to submit the IND application for this program in mid-2025. We promoted our tau silencing gene therapy program to a prioritized program in the first quarter of 2024, based on preclinical data demonstrating robust reductions in tau messenger RNA, or mRNA, in a murine model, and we anticipate submission of an IND in 2026. Our proprietary pipeline also includes an early research initiative to develop a gene therapy for the treatment of AD. This program seeks to combine a vectorized anti-amyloid antibody with a TRACER Capsid.

We are also working with our collaboration partners on multiple programs. In January 2019 and January 2023, we entered into collaboration and license agreements with Neurocrine. Under our agreements with Neurocrine, we are actively advancing two later preclinical stage programs: a glucocerebrosidase 1, or GBA1, gene therapy program for Parkinson's disease and other GBA1-mediated diseases, and a frataxin, or FXN, gene therapy program for Friedreich's ataxia, or the FA Program. Pursuant to such agreements, we are also working with Neurocrine on five early-stage programs for the research, development, manufacture and commercialization of gene therapies designed to address central nervous system diseases or conditions associated with rare genetic targets. We have also entered into agreements with licensees including Novartis, Alexion, and Sangamo, to license or to provide options to receive exclusive licenses to certain TRACER Capsids. As described further below, in December 2023, we entered into a license and collaboration agreement with Novartis to provide Novartis certain rights regarding the development of potential gene therapy product candidates for the treatment of spinal muscular atrophy and to collaborate with Novartis to develop gene therapy product candidates for the treatment of Huntington's disease. The joint steering committee with Neurocrine selected a development candidate for the FA Program, during the first quarter of 2024, and expects to advance into first-in-human clinical trials in 2025. We anticipate that our collaborative partners and licensees will submit at least one additional IND application for a partnered program and initiate clinical development for the associated program by the end of 2025.

All of the gene therapies in our wholly-owned and collaborative pipeline leverage novel capsids derived from our TRACER™ Capsid discovery platform. TRACER is a broadly applicable, RNA-based screening platform that enables rapid discovery of AAV capsids with robust penetration of the blood-brain barrier and enhanced central nervous system, or CNS, tropism in multiple species, including non-human primates, or NHPs.

Vision, Mission, and Strategy

Our vision is a world in which transformative treatments and cures are available to the millions afflicted with neurological diseases. Our mission is to create disease-modifying neurogenetic medicines by identifying validated targets, advancing multiple therapeutic modalities, and delivering to the right areas within the central nervous system.

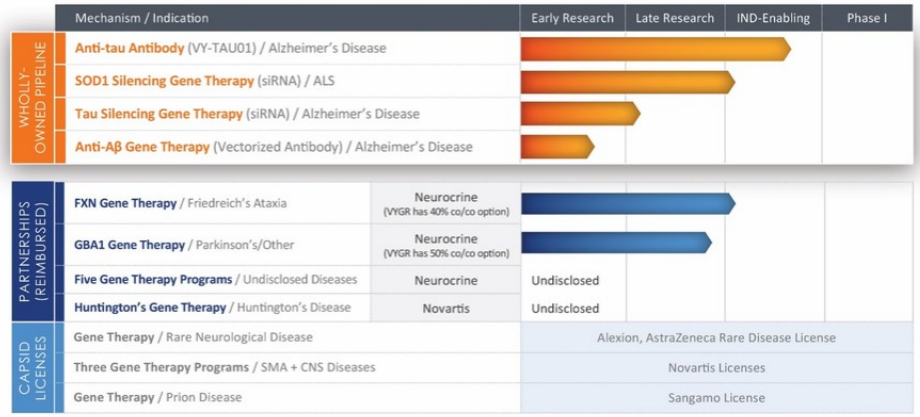
Our strategy is to define and become a leader in the field of neurogenetic medicine. We intend to achieve our broader vision, mission, and strategic imperatives through the following strategic initiatives:

- Advancing our prioritized CNS pipeline by achieving preclinical and clinical milestones and establishing proof-of-biology and proof-of-concept.
- Fueling our future by initiating and advancing research programs in gene therapy and other therapeutic approaches to neurogenetic medicine.
- Maximizing value for all stakeholders by advancing our wholly-owned and partnered assets intended to transform the treatment of neurological disease.

Overview of Our Pipeline

We have leveraged our TRACER discovery platform and other gene therapy platforms, our expertise with proprietary antibodies, vectorized small interfering RNA, or siRNA, knockdown, gene delivery and our vectorized antibody platform to assemble a pipeline of proprietary antibody, AAV gene therapy and other genetic medicine programs for the treatment of neurological diseases. We have prioritized pipeline programs for our development based on the following criteria: high unmet medical need, target validation, efficient path to human proof of biology, robust preclinical pharmacology, and strong commercial potential. Depending on the disease, we are seeking to develop AAV gene therapies that will use a gene replacement, gene silencing or vectorized antibody approach, and antibodies that will use a passive administration approach.

Our pipeline of programs, all of which are in preclinical development, is summarized in the table below:



Our Platforms

We have expertise in both viral and non-viral approaches to neurogenetic medicine.

Our TRACER Capsid discovery platform is a broadly applicable, RNA-based screening platform that enables rapid discovery of AAV capsids with robust penetration of the blood-brain barrier, or BBB, and enhanced CNS, tropism observed in multiple animal species, including NHPs. TRACER allows us to identify proprietary AAV capsids, the outer viral protein shells that enclose genetic material that makes up the vector payload. TRACER employs directed evolution to facilitate the selection of AAV capsids with enhanced tissue delivery characteristics, such as more effective delivery across the BBB and cell-specific transduction. The TRACER discovery platform is a broadly applicable, functional RNA-based AAV capsid discovery platform that allows for rapid in vivo evolution of AAV capsids with cell-specific transduction properties in multiple species, including NHPs. We believe that these TRACER Capsids have the potential to significantly enhance the activity and safety of our single dose gene therapy product candidates, which we expect to be delivered with systemic infusions, as compared with conventional capsids. We have leveraged the TRACER discovery platform to generate multiple families of TRACER Capsids with robust CNS tropism following intravenous delivery. We have presented data at scientific conferences demonstrating strong transduction to multiple areas within the brain and activity across multiple animal species. We have entered into agreements with licensees including Alexion, Novartis, and Sangamo, to license or to provide options to receive exclusive licenses to certain of our TRACER Capsids to develop and commercialize AAV gene therapy candidates in specified indications.

Vector Engineering and Optimization

The key components of an AAV vector include: (a) the capsid; (b) the therapeutic gene, or transgene; and (c) payload control elements, including the promoter or other DNA sequences that modulate the expression of the transgene. We have advanced or intend to advance our multiple preclinical programs towards selection of lead clinical candidates using AAV vectors that we believe are best suited for each of our programs either through use of our existing capsids, through exercising a non-exclusive worldwide commercial license to capsid sequences covered by third parties, or by engineering or optimizing TRACER Capsids. We have also built, or intend to build, capabilities to design, screen, and advance genetic sequences within our AAV vectors, including transgenes and payload control elements, to create optimized therapeutic candidates for each of our preclinical programs.

Non-Viral Delivery

We have additional expertise in the discovery and development of monoclonal antibodies as well as in receptor-mediated non-viral delivery to the CNS. We have discovered multiple antibodies as part of the anti-tau antibody program and other research programs. Some of these programs have been advanced in a vectorized setting, such as our anti-amyloid program, while others have been advanced in a non-vectorized setting, such as the anti-tau antibody program.

Separately, we have identified receptors for some of our TRACER Capsids as well as ligands for a particular receptor and are conducting experiments to evaluate the potential to leverage our receptors to shuttle non-viral genetic medicines across the BBB.

Wholly-Owned Programs

Anti-Tau Antibody (VY-TAU01) for the Treatment of Alzheimer's Disease

Disease Overview

AD is a progressive neurodegenerative disease estimated to affect 6 million people in the United States and up to 416 million people globally. The disease causes memory loss and may escalate to decreased independence, communication challenges, behavioral disorders such as paranoia and anxiety, and lack of physical control. In 2023, the total cost of caring for people living with Alzheimer's and other dementias in the United States is estimated at $345 billion.

Our Treatment Approach

We have maintained a long-standing focus on developing proprietary and complimentary approaches to disrupt the progression of tau pathology believed to be central to AD and other tauopathies. Reduction of toxic tau aggregates

may slow disease progression and cognitive decline in these diseases. We selected VY-TAU01 as our lead humanized anti-tau antibody candidate to advance against AD. We believe VY-TAU01 is differentiated from other anti-tau antibodies based on the epitope, or the part of a foreign protein or antigen that is capable of generating an immune response, it targets, which is located in the C-terminal rather than the N-terminal, mid-domain, or microtubule binding region of the tau protein.

Preclinical Studies

At the Alzheimer's Association International Conference in August 2022, we presented data for our proprietary anti-tau antibodies, targeting the mid-domain and C-terminus with high affinity and showing favorable biophysical characteristics and strong activity in preclinical studies in mouse models. In the P301S seeding-propagation tauopathy mouse model, our C-terminal targeting anti-tau antibody blocked the seeding/propagation of filamentous tau and demonstrated substantial reduction of induced tau pathology. In March 2023, we presented data at the Alzheimer's and Parkinson's Diseases, or AD/PD, 2023 Conference highlighting the differentiating characteristics resulting in the selection of lead candidate VY-TAU01. In March 2024, we will present data at the AD/PD 2024 Conference demonstrating VY-TAU01 was well-tolerated, and its serum pharmacokinetic profile was as expected in an NHP study.

Program Status

In January 2023, we selected VY-TAU01 as our lead humanized anti-tau antibody candidate to advance against AD. In April 2023, we received pre-IND written feedback from the FDA for VY-TAU01. Process development and manufacturing at a contract manufacturer have been initiated, and we initiated good laboratory practice, or GLP, toxicology studies in the third quarter of 2023 to enable an IND submission.

These GLP toxicology studies are progressing, and we plan to submit an IND application for VY-TAU01 to the FDA in the first half of 2024. Following clearance of the IND, the planned Phase 1 clinical trial is designed to assess the safety of VY-TAU01 in a single ascending dose, or SAD, study in healthy subjects, and is expected to be initiated in 2024. A multiple ascending dose, or MAD, study in subjects with mild cognitive impairment or early AD is expected to be initiated in 2025. The MAD study has the potential to generate proof-of-concept data for slowing the spread of pathological tau via tau PET imaging in 2026.

SOD1 Silencing Gene Therapy Program for the Treatment of ALS

Disease Overview

We are developing a gene therapy leveraging a BBB-penetrant, CNS-tropic TRACER Capsid to treat ALS caused by the SOD1 mutation via a gene silencing approach. ALS is a progressive neurodegenerative disease in which the motor neurons atrophy and die, resulting in loss of the ability to speak, move, eat and, eventually, breathe. SOD1 ALS is typically fatal within approximately two to five years of symptom onset. The disease is estimated to affect approximately 20,000 people in the United States. Multiple genes have been implicated in ALS; mutations in the SOD1 gene are estimated to occur in approximately 2-3% of ALS cases, or up to 600 people in the United States. SOD1 mutations in ALS patients are thought to cause a toxic gain-of-function that leads to the degeneration of motor neurons along the entire length of the spinal cord, the brainstem, and the upper motor neurons in the cerebral cortex.

Our Treatment Approach

We believe that a therapeutic delivering a vectorized highly potent siRNA construct via intravenous administration of an AAV gene therapy may enable broad CNS knockdown of SOD1. This could potentially slow the decline of functional ability in ALS patients with the SOD1 mutation. We have selected a potent, specific vectorized siRNA transgene targeting SOD1, delivered using a novel TRACER Capsid. We believe that a Phase 1 clinical trial to demonstrate reductions in SOD1 in the cerebrospinal fluid and in neurofilament light chain in the plasma will provide evidence of target engagement and the attenuation of motor neuron loss, respectively.

Preclinical Studies

At the American Society of Gene & Cell Therapy 25th Annual Meeting in May 2022, or the ASGCT 2022 Meeting, we presented preclinical data demonstrating robust SOD1 knockdown in all levels of the spinal cord and significant improvements in motor performance, body weight, and survival in an SOD1-ALS mouse model following intravenous delivery of a vectorized siRNA using a mouse BBB-penetrant capsid. When we announced the selection of a development candidate in the fourth quarter of 2023, we disclosed that, in an NHP study, the candidate demonstrated 73% reduction of SOD1 in cervical spinal cord motor neurons following a single intravenous dose in cynomolgus macaques. The candidate also demonstrated robust knockdown of SOD1 across all levels of the spinal cord and motor cortex. Further, the candidate demonstrated an ability to transduce both neurons and astrocytes, two cell types thought to play an important role in ALS.

Program Status

We have identified a potent and specific vectorized siRNA transgene that resulted in substantially extended lifespan and motor function when delivered using a BBB-penetrant capsid in a mouse model and on December 6, 2023, we announced the selection of a lead development candidate for our SOD1 program. We plan to submit an IND application to the FDA in mid-2025 for our lead development candidate and to initiate a Phase 1 clinical trial in subjects with SOD1 ALS for the program as soon as possible thereafter. We expect to evaluate the safety and biological activity of its SOD1 ALS product candidate in this Phase 1 trial.

Tau Silencing Gene Therapy Program for the Treatment of AD

Disease Overview

AD is a progressive neurodegenerative disease estimated to affect 6 million people in the United States and up to 416 million people globally. The disease causes memory loss and may escalate to decreased independence, communication challenges, behavioral disorders such as paranoia and anxiety, and lack of physical control. In 2023, the total cost of caring for people living with Alzheimer's and other dementias in the United States is estimated at $345 billion.

Our Treatment Approach

We have maintained a long-standing focus on developing proprietary and complimentary approaches to disrupt the progression of tau pathology believed to be central to AD and other tauopathies. Reduction of toxic tau aggregates may slow disease progression and cognitive decline in these diseases. In addition to our aforementioned anti-tau antibody program, we are advancing a gene therapy that leverages an intravenously delivered TRACER Capsid containing a vectorized siRNA, specifically targeting tau mRNA.

Preclinical Studies

In March 2024, we will present data at the AD/PD 2024 Conference demonstrating that a single intravenous administration of our tau silencing gene therapy in mice expressing human tau resulted in broad AAV distribution across multiple brain regions and dose-dependent reductions in tau mRNA levels of up to 90%, which were associated with robust reductions in human tau protein levels across the brain.

Program Status

In the first quarter of 2024, we promoted the tau silencing gene therapy program to a prioritized program, and we promoted it to the late-research stage of our wholly-owned pipeline, based on its demonstration on in vivo proof-of-concept and expected advancement to IND within two to three years. We are evaluating the optimal combination of payload and capsid for this program, to enable selection of a development candidate. We expect to file an IND in 2026.

Vectorized Anti-Amyloid Antibody Early Research Program for the Treatment of AD

In August 2023, we announced an early research initiative investigating a gene therapy targeting anti-amyloid for the treatment of AD. The program combines a vectorized anti-amyloid antibody with an intravenously delivered TRACER Capsid.

Collaboration Programs

Friedreich's Ataxia Program: VY-FXN01 (2019 Neurocrine Collaboration)

Disease Overview

Friedreich's ataxia is a debilitating neurodegenerative disease resulting in poor coordination of legs and arms, progressive loss of the ability to walk, generalized weakness, loss of sensation, scoliosis, diabetes and cardiomyopathy as well as impaired vision, hearing and speech. The typical age of onset is 10 to 12 years, and life expectancy is severely reduced with patients generally dying of neurological and cardiac complications between the ages of 35 and 45. According to the Friedreich's Ataxia Research Alliance, there are approximately 6,400 patients living with the disease in the United States. While one treatment for Friedreich's ataxia has recently been approved by the FDA, we believe there remains a significant unmet need.

Friedreich's ataxia patients have mutations of the FXN gene that reduce production of the frataxin protein, resulting in the degeneration of sensory pathways and a variety of debilitating symptoms. Friedreich's ataxia is an autosomal recessive disorder, meaning that a person must obtain a defective copy of the FXN gene from both parents in order to develop the condition. One healthy copy of the FXN gene, or 50% of normal frataxin protein levels, is sufficient to prevent the disease phenotype. We therefore believe that restoring FXN protein levels to at least 50% of normal levels by AAV gene therapy might lead to a successful therapy.

Our Treatment Approach

We are seeking to develop an AAV gene therapy approach that we believe will deliver a functional version of the FXN gene to the sensory pathways through intravenous injection. We think this approach has the potential to improve balance, ability to walk, sensory capability, coordination, strength and functional capacity of Friedreich's ataxia patients. Most Friedreich's ataxia patients produce low levels of the frataxin protein, which although insufficient to prevent the disease, exposes the patient's immune system to frataxin. This reduces the likelihood that the FXN protein expressed by AAV gene therapy will trigger a harmful immune response.

Preclinical Studies

We initially conducted preclinical studies in NHPs and achieved high FXN expression levels within the target sensory ganglia, or clusters of neurons, along the spinal region following intrathecal injection. More recently, we conducted preclinical studies in NHPs with intravenous injection and achieved target FXN expression levels within sensory ganglia and the heart. The levels of FXN expression observed in the brain using an AAV vector were, on average, greater than FXN levels present in control normal human brain tissue. FXN expression was also observed in the cerebellar dentate nucleus, another area of the CNS that is often affected in Friedreich's ataxia, and that is often considered difficult to target therapeutically.

Our Program Status

Under the collaboration and license agreement with Neurocrine entered into in January 2019, or the 2019 Neurocrine Collaboration Agreement, we are developing VY-FXN01 for the treatment of Friedreich's ataxia. VY-FXN01 is currently in preclinical development. In February 2024, the joint steering committee with Neurocrine selected a development candidate combining an FXN gene replacement payload with a novel TRACER Capsid for its FA Program and expects to advance into first-in-human clinical trials in 2025.

GBA1 Gene Replacement Program for the Treatment of Parkinson's Disease (2023 Neurocrine Collaboration)

Disease Overview

We are developing a gene therapy leveraging a BBB-penetrant, CNS-tropic TRACER Capsid to treat diseases linked to GBA1 mutations via a gene replacement approach. Our lead indication for this gene therapy is Parkinson's disease with GBA1 mutations. Mutations in GBA1, the gene encoding the lysosomal glucocerebrosidase enzyme, or Gcase, are the most common genetic risk factor for synucleinopathies such as Parkinson's disease. Parkinson's disease is among the most common neurodegenerative diseases, affecting about one million patients in the United States and more than 10.0 million patients worldwide. Up to 10% of Parkinson's disease patients have a GBA1 mutation, and these mutations increase the risk of Parkinson's disease by approximately 20-fold. GBA1 mutations can decrease the activity of Gcase, leading to the accumulation of Gcase substrates which is linked to alpha-synuclein aggregates, which are thought to be toxic to neurons.

Our Treatment Approach

We believe that restoring Gcase activity may attenuate disease progression and potentially slow neurodegeneration. We anticipate delivering GBA1 via intravenous administration of an AAV gene therapy to enable widespread distribution to multiple affected brain regions and to avoid the need for more invasive approaches. We believe that the measurement of the Gcase substrates such as glucosylsphingosine as cerebrospinal fluid biomarkers may facilitate efficient clinical demonstration of proof-of-biology. Such substrates of the Gcase enzyme are elevated in the cerebrospinal fluid of Parkinson's disease patients who harbor the GBA1 mutation, and we expect that substrate levels would be normalized if our gene therapy restores Gcase enzyme expression in the brain. This gene therapy may also have potential utility in idiopathic Parkinson's disease, where there is evidence of loss of Gcase activity in the substantia nigra in Parkinson's disease patients even in the absence of GBA1 mutations as well as evidence of lysosomal dysfunction in general.

Preclinical Studies

At the ASGCT 2022 Meeting, we presented preclinical data demonstrating CNS target engagement and delivery of therapeutically relevant levels of Gcase in a GBA1 loss of function mouse model, as well as sustained expression for three or more months following intravenous administration. At the AD/PD 2023 Conference, we presented new data from additional mouse efficacy studies showing that three potential development candidates each demonstrated significant improvement in several efficacy biomarkers. We presented data at the ASGCT 2023 Meeting summarizing the mouse findings and additional data from an NHP study showing that the administration of a reporter transgene via a single, intravenous dose using two novel BBB-penetrant AAV capsids demonstrated substantially improved biodistribution and gene expression compared to conventional AAV9 in the putamen and substantia nigra, two areas of the brain that are affected in Parkinson's disease.

Program Status

Under the collaboration and license agreement with Neurocrine entered into in January 2023, or the 2023 Neurocrine Collaboration Agreement, we are developing gene therapy products directed to the gene that encodes GBA1 for the treatment of Parkinson's disease and other diseases associated with GBA1, or the GBA1 Program. The GBA1 Program is currently in preclinical development. We and Neurocrine are in the process of identifying a lead candidate that will be comprised of a TRACER Capsid, promoter, and transgene. If we and Neurocrine successfully identify a lead development candidate for this program, we plan to complete IND enabling studies to evaluate its safety and efficacy.

HD Program (2023 Novartis Collaboration Agreement)

Disease Overview

Huntington's disease is a fatal, inherited neurodegenerative disease that results in the progressive decline of motor and cognitive functions and a range of behavioral and psychiatric disturbances. Huntington's disease is caused by mutations in the huntingtin, or HTT, gene. Huntington's disease is an autosomal dominant disorder, which means that an

individual is at risk of inheriting the disease if only one parent is affected. While the exact function of the HTT gene in healthy individuals is unknown, it is essential for normal development before birth. Mutations in the HTT gene ultimately lead to the production of abnormal intracellular huntingtin protein aggregates and expansions in the gene in neurons that may cause neuronal cell death.

Program Status

On December 28, 2023, we entered into a license and collaboration agreement with Novartis, or the 2023 Novartis Collaboration Agreement. Under the 2023 Novartis Collaboration Agreement, we and Novartis have agreed to collaborate to develop AAV gene therapy products and product candidates intended for the treatment of Huntington's disease, which we refer to as the Novartis HD Program. The Novartis HD Program is currently in preclinical development. From and after the first IND application filing for the Novartis HD Program, we and Novartis have agreed that Novartis will assume sole responsibility for the development and commercialization of gene therapy products and product candidates under the Novartis HD Program, including all further preclinical and clinical development and any commercialization of the Novartis HD Program products and product candidates.

Collaboration Programs and Licensing Agreements

2023 Novartis Collaboration Agreement

On December 28, 2023, or the 2023 Novartis Collaboration Agreement Effective Date, we entered into the 2023 Novartis Collaboration Agreement, with Novartis to (a) provide rights to Novartis with respect to certain TRACER Capsids for use in the research, development, and commercialization by Novartis of AAV gene therapy products and product candidates, comprising such TRACER Capsids and payloads intended for the treatment of spinal muscular atrophy, or the Novartis SMA Program, and (b) collaborate to develop AAV gene therapy products and product candidates under the Novartis HD Program, in each case, leveraging TRACER Capsids and other intellectual property controlled by us.

Novartis SMA Program and Novartis HD Program Licenses

Under the terms of the 2023 Novartis Collaboration Agreement, we granted to Novartis and its affiliates:

- a non-exclusive, non-transferable, non-sublicensable (except in limited circumstances for contractors), worldwide, royalty-free right and license under any patents or know-how controlled by us and related to the TRACER Capsids to evaluate the same for use in the development of a product or product candidate under the Novartis SMA Program, or a Novartis SMA Program Product, comprising such a TRACER Capsid and a payload selected by Novartis during the period beginning on the 2023 Novartis Collaboration Agreement Effective Date and ending on the third anniversary of the 2023 Novartis Collaboration Agreement Effective Date;

- an exclusive (even as to us), sublicensable, non-transferable, worldwide, royalty-bearing right and license under any patents or know-how controlled by us and relating to the selected TRACER Capsids to exploit the same as incorporated into a Novartis SMA Program Product for all human and veterinary diagnostic, prophylactic and therapeutic uses during the 2023 Novartis Collaboration Term (as defined below); and

- an exclusive (even as to us), non-transferable, sublicensable, worldwide, royalty-bearing right and license under any patents and know-how controlled by us and relating to the development of a product or product candidate under the Novartis HD Program, or a Novartis HD Program Product to exploit the same for all human and veterinary diagnostic, prophylactic and therapeutic uses during the 2023 Novartis Collaboration Term.

Governance

We and Novartis have agreed to manage the Novartis HD Program through a joint steering committee until dissolved after the first IND application filing for a Novartis HD Program Product. We and Novartis have further agreed

that day-to-day activities of both the Novartis SMA Program and the Novartis HD Program shall be managed through designees from each of us and Novartis, acting as alliance managers.

Development, Regulatory Approval, Commercialization and Diligence.

Under the 2023 Novartis Collaboration Agreement, Novartis is solely responsible for, and has sole decision-making authority with respect to, at its own expense, the exploitation of a Novartis SMA Program Product.

With respect to the Novartis HD Program, the parties have agreed to conduct research and pre-clinical development of Novartis HD Program Products pursuant to a research plan, with Novartis reimbursing us for our activities thereunder in accordance with the agreed-to budget. From and after the first IND application filing for the Novartis HD Program, the parties have agreed that Novartis will assume sole responsibility for the development and commercialization of Novartis HD Program Products, including all further preclinical and clinical development and any commercialization of the Novartis HD Program products and product candidates.

With respect to each of the Novartis SMA Program Products and Novartis HD Program Products, Novartis is obligated to use commercially reasonable efforts to develop and obtain regulatory approval for at least one of each such product in the United States and in certain other international markets specified in the 2023 Novartis Collaboration Agreement.

Intellectual Property

Under the terms of the 2023 Novartis Collaboration Agreement, each party owns the entire right, title, and interest in and to all patents or know-how controlled by such party and existing as of or before the 2023 Novartis Collaboration Agreement Effective Date, or invented, authored, discovered, developed, created or acquired solely by or on behalf of such party after the 2023 Novartis Collaboration Effective Date outside of its activities under the 2023 Novartis Collaboration Agreement.

We and Novartis have further agreed that all know-how created by either or both parties in the performance of the activities as undertaken pursuant to the performance of the Novartis HD Program plan or in the course of development, manufacture and commercialization of Novartis HD Program Products and all patent rights covering such know-how, or collectively, the 2023 Novartis Arising IP, is to be owned as follows: (i) we solely own all 2023 Novartis Arising IP comprised of know-how or other intellectual property rights related to any TRACER Capsid, including the use or manufacture of any TRACER Capsid, and that is created jointly by our representatives and representatives of Novartis or created solely by representatives of Novartis through the use of our confidential information; and (ii) with respect to all other 2023 Novartis Arising IP, (A) we solely own all such 2023 Novartis Arising IP created solely by our representatives, (B) Novartis solely owns all such 2023 Novartis Arising IP created solely by Novartis representatives; and (C) the parties jointly own all such 2023 Novartis Arising IP created jointly by representatives of both Novartis and us.

Exclusivity

Subject to certain limitations and exceptions, we have agreed during the 2023 Novartis Collaboration Term not to (i) conduct any wholly-owned program or program on behalf of a third party that is directed to the development or commercialization of any capsids for use in any therapeutic product containing a capsid in combination with a payload designed to have therapeutic effect on the gene agreed between the parties as the target of the Novartis SMA Program when packaged into a capsid and delivered to the appropriate cells; (ii) develop or commercialize any competing Novartis HD Program Product intended to have a therapeutic effect on genes agreed between the parties as the targets of the Novartis HD Program; or (iii) grant any third party any right, license, option, covenant not to assert or similar right, under any patents or know-how controlled by us or our affiliates (excluding an acquiring entity) as of the 2023 Novartis Collaboration Agreement Effective Date or during the 2023 Novartis Collaboration Term, that would enable a third party to do any of the foregoing.

Termination

Unless earlier terminated, with respect to any licensed product(s) under the 2023 Novartis Collaboration Agreement, on a country-by-country basis, the 2023 Novartis Collaboration Agreement expires upon the expiration of the last-to-expire royalty term with respect to such licensed product in such country in the territory, or the 2023 Novartis Collaboration Term. Subject to a cure period, either party may terminate the 2023 Novartis Collaboration Agreement, in whole or in part, subject to specified conditions, in the event of the other party's uncured material breach. Novartis may also terminate the 2023 Novartis Collaboration Agreement, in whole or in part, subject to specified conditions, for our insolvency, for the occurrence of a violation of global trade control laws, or for our non-compliance with certain anti-bribery or anti-corruption covenants. Novartis may terminate the 2023 Novartis Collaboration Agreement, in whole or in part, for any or no reason upon ninety days' written notice to us. In the event that Novartis has the right to terminate the 2023 Novartis Collaboration Agreement as a result of an uncured material breach by us that materially impairs the ability of Novartis to exploit one or more licensed products, Novartis may, in lieu of such termination, elect for the 2023 Novartis Collaboration Agreement to remain in full force and effect, and all milestone payments and royalties that would have otherwise been payable by Novartis under such licenses had the 2023 Novartis Collaboration Agreement not been breached would be substantially reduced.

Financial Terms

Under the 2023 Novartis Collaboration Agreement, Novartis paid us an upfront payment of $80.0 million. We are eligible to receive specified development, regulatory, and commercialization milestone payments of up to an aggregate of $200.0 million for the Novartis SMA Program and up to an aggregate of $225.0 million for the Novartis HD Program, in each case for the first corresponding product to achieve the corresponding milestone. We are also eligible to receive (a) specified sales milestone payments of up to an aggregate of $400.0 million for the Novartis SMA Program and up to an aggregate of $375.0 million for the Novartis HD Program and (b) tiered, escalating royalties in the high single-digit to low double-digit percentages of annual net sales of the Novartis SMA Program Products and the Novartis HD Program Products. The royalties are subject to potential customary reductions, including patent claim expiration, payments for certain third-party licenses, and biosimilar market penetration, subject to specified limits.

2023 Novartis Stock Purchase Agreement

We and Novartis also entered into a stock purchase agreement on December 28, 2023, or the 2023 Novartis Stock Purchase Agreement, for the sale and issuance of 2,145,002 shares of our common stock, or the Novartis Shares, to Novartis at a price of $9.324 per share, for an aggregate purchase price of approximately $20.0 million. In accordance with the terms and conditions of the 2023 Stock Purchase Agreement, we issued and sold the Novartis Shares to Novartis on January 3, 2024, or the 2023 Novartis Investment Closing Date.

2023 Novartis Investor Agreement

We and Novartis also entered into an investor agreement on December 28, 2023, or the 2023 Novartis Investor Agreement, which became effective as of the 2023 Novartis Investment Closing Date, providing for standstill and lock-up restrictions.

Pursuant to the terms of the 2023 Novartis Investor Agreement, Novartis has agreed not to, without the prior written approval of us and subject to specified conditions, directly or indirectly acquire shares of our outstanding common stock, publicly seek or propose a tender or exchange offer or merger between the parties, solicit proxies or consents to vote any voting securities that we have issued, or undertake other specified actions related to the potential acquisition of additional equity interests in us, or the Novartis Standstill Restrictions. Further, Novartis has also agreed not to, and to cause its affiliates not to, sell or transfer any of the Novartis Shares without our prior approval, subject to specified conditions, or the Novartis Lock-Up Restrictions.

Each of the Novartis Standstill Restrictions and the Novartis Lock-Up Restrictions terminate upon the earliest to occur of: (i) the expiration or earlier termination of the 2023 Novartis Collaboration Agreement; (ii) the date that is the third anniversary of the 2023 Novartis Investment Closing Date; (iii) our liquidation or dissolution; and (iv) the

deregistration of our common stock. The Novartis Lock-Up Restrictions also terminate on a change of control of us or the date on which Novartis and its affiliates beneficially own less than three percent of our common stock on an outstanding basis.

2022 Novartis Option and License Agreement

Summary of Agreement

On March 4, 2022, or the 2022 Novartis Option and License Effective Date, we entered into an option and license agreement with Novartis, or the 2022 Novartis Option and License Agreement. Pursuant to the 2022 Novartis Option and License Agreement, we granted Novartis options, or the Novartis License Options, to license TRACER Capsids, or the Novartis Licensed Capsids, for exclusive use with certain targets to develop and commercialize AAV gene therapy candidates comprised of Novartis Licensed Capsids and payloads directed to such targets, or the Novartis Payloads.

Research and License Option

During the period commencing on the 2022 Novartis Option and License Effective Date and ending on the first anniversary thereof or, in the event Novartis has exercised a Novartis License Option, the third anniversary thereof, on a target-by-target basis, or the Novartis Research Term, we have granted Novartis a non-exclusive research license to evaluate our TRACER Capsids for potential use, in combination with Novartis Payloads, in programs targeting three specified genes, or the Initial Novartis Targets. Upon the payment of additional fees, Novartis may also assess our TRACER Capsids for use with up to two other targets, or the Additional Novartis Targets, subject to certain conditions including that such target is not part of, or reasonably competitive with, our current development programs. We refer to the Initial Novartis Targets and the Additional Novartis Targets collectively as the Novartis Targets. During the Novartis Research Term, as applicable, we may, at our sole discretion and expense, conduct further research activities to identify additional TRACER Capsids. If we elect to do so, we have agreed to disclose performance characteristics of such new TRACER Capsids to Novartis on a rolling basis.

During the Novartis Research Term, Novartis may exercise up to three Novartis License Options—or up to five Novartis License Options if Novartis is evaluating the Additional Novartis Targets—in the aggregate, provided that Novartis may only exercise one Novartis License Option for each Novartis Target. Upon the exercise of any Novartis License Option, we have granted Novartis a target-exclusive, worldwide license, with the right to sublicense, under certain of our intellectual property, the rights to develop and commercialize the applicable Novartis Licensed Capsid as incorporated into products containing the corresponding Novartis Payload, or the Novartis Licensed Products. Upon the exercise of a Novartis License Option, we have agreed to provide certain additional know-how to enable Novartis to exploit the Novartis Licensed Capsid and the corresponding Novartis Payload for use in a Novartis Licensed Product. Novartis may, during the applicable Novartis Research Term but following the exercise of a Novartis License Option, conduct additional evaluation of our capsid candidates and has the right to substitute any other TRACER Capsid for a Novartis Licensed Capsid.

Governance

Subject to our disclosure obligations described above, we and Novartis have agreed to conduct our respective research and evaluation activities independently, with communications being managed by two alliance managers comprised of a designee from each of the parties.

Development, Regulatory Approval, and Commercialization

Under the 2022 Novartis Option and License Agreement, Novartis is solely responsible for, and has sole decision-making authority with respect to, development and commercialization of the Novartis Licensed Products. Novartis is required to use commercially reasonable efforts to develop and obtain regulatory approval for at least one Novartis Licensed Product for each Novartis Target for which it has exercised a Novartis License Option in (a) the United States and (b) at least three of the following countries: the United Kingdom, France, Germany, Italy, Spain

and Japan, each of which we refer to as a Novartis Major Market Country, subject to certain limitations. Novartis is also required to use commercially reasonable efforts to commercialize each Novartis Licensed Product in the United States and at least three Novartis Major Market Countries where Novartis or its designated affiliates or sublicensees has received regulatory approval for such Novartis Licensed Product, subject to certain limitations.

During the Novartis Research Term, we have agreed to provide plasmids to Novartis for the production of TRACER Capsids for evaluation upon request. We have also granted Novartis a non-exclusive license, effective upon an exercise of a Novartis License Option and in addition to its options for target-exclusive licenses under certain of our intellectual property described above, on a Novartis Licensed Capsid-by-Novartis Licensed Capsid basis, under certain of our know-how to exploit the applicable Novartis Licensed Capsid as incorporated into Novartis Licensed Products containing the corresponding Novartis Payload.

Financial

Under the terms of the 2022 Novartis Option and License Agreement, Novartis paid us an upfront payment of $54.0 million. Effective as of March 1, 2023, Novartis exercised its Novartis License Options to license TRACER Capsids for use in gene therapy programs against two undisclosed Initial Novartis Targets. With Novartis' option exercise on two Initial Novartis Targets, we received a $25.0 million option exercise payment in April 2023, and are eligible to receive associated potential development, regulatory, and commercial milestone payments, as well as mid- to high-single-digit tiered royalties based on net sales of the Novartis Licensed Products incorporating the Novartis Licensed Capsids. The two Initial Novartis Targets licensed are distinct from targets in our wholly-owned and partnered pipeline. In addition, during the research term, Novartis retains the right to expand the agreement to include options to license capsids for up to two Additional Novartis Targets, subject to their availability, for a fee of $18.0 million per Additional Novartis Target. Under such an expansion, we would be eligible to receive a $12.5 million license option exercise fee for each Additional Novartis Target exercised, as well as future potential milestone payments per Additional Novartis Target and tiered mid- to high-single digit royalties on the Novartis Licensed Products incorporating the Novartis Licensed Capsids.

Novartis elected not to license a capsid for one Initial Novartis Target under the 2022 Novartis Option and License Agreement prior to the expiration of the applicable Novartis License Option. As a result, the non-exclusive research license that we granted to Novartis in connection with this Initial Novartis Target has terminated, the Novartis Research Term for this Initial Novartis Target has expired, and we are no longer eligible to receive development, regulatory, and commercial milestone payments or royalties in connection with this Initial Novartis Target. All capsid rights with respect to that Initial Novartis Target have returned to us.

Intellectual Property

Under the terms of the 2022 Novartis Option and License Agreement, each party owns the entire right, title, and interest in and to all patents or know-how controlled by such party and existing as of or before the 2022 Novartis Option and License Effective Date, or invented, developed, created, generated or acquired solely by or on behalf of such party after the 2022 Novartis Option and License Effective Date. Subject to certain specified exceptions, any patents and know-how that are invented or otherwise developed jointly by or on behalf of the parties during the term of the 2022 Novartis Option and License Agreement and in the course of the parties' activities under the 2022 Novartis Option and License Agreement will follow inventorship under U.S. patent law.

Exclusivity

Subject to certain limitations and exceptions, we have agreed (a) during the Novartis Research Term, not to conduct any internal program or program on behalf of a third party that is directed to the development or commercialization of any of our capsids, or grant any third party or affiliate any right or license under our rights in such capsids, to exploit any therapeutic product containing a capsid in combination with a payload designed to have therapeutic effect on any of the Novartis Targets; and (b) after Novartis' exercise of Novartis License Options, not to grant any third party or affiliate any right or license under our patents to exploit any Novartis Licensed Capsid for the applicable Novartis Target.

Termination

Unless earlier terminated, the 2022 Novartis Option and License Agreement expires on the expiration of the last-to-expire royalty term with respect to all Novartis Licensed Products in all countries. Subject to a cure period, either party may terminate the 2022 Novartis Option and License Agreement, in whole or in part, subject to specified conditions, in the event of the other party's uncured material breach. Novartis may also terminate the 2022 Novartis Option and License Agreement, in whole or in part, subject to specified conditions, for our insolvency, the occurrence of a violation of global trade control laws, or for our non-compliance with certain anti-bribery or anti-corruption covenants. Novartis may terminate the 2022 Novartis Option and License Agreement, in whole or in part, for any or no reason upon ninety days' written notice to us.

Upon certain terminations for cause by Novartis, the licenses granted by us to Novartis under the 2022 Novartis Option and License Agreement shall become irrevocable and perpetual, and all milestone payments and royalties that would have otherwise been payable by Novartis under such licenses had the 2022 Novartis Option and License Agreement remained in effect would be substantially reduced.

2023 Neurocrine Collaboration Agreement

Summary of Agreement

On January 8, 2023, we entered into the 2023 Neurocrine Collaboration Agreement for the research, development, manufacture and commercialization of the 2023 Neurocrine Programs.

Collaboration and License

Under the 2023 Neurocrine Collaboration Agreement, we and Neurocrine have agreed to collaborate on the conduct of the 2023 Neurocrine Programs. The 2023 Neurocrine Collaboration Agreement became effective upon the expiration of all applicable waiting periods under the Hart-Scott-Rodino Antitrust Improvements Act of 1976, as amended, which occurred on February 21, 2023, or the Neurocrine Effective Date. Under the terms of the 2023 Neurocrine Collaboration Agreement, subject to the rights retained by us thereunder, we granted to Neurocrine, as of the Neurocrine Effective Date, an exclusive, royalty-bearing, sublicensable, worldwide license, under certain of our intellectual property rights, to research, develop, manufacture and commercialize gene therapy products, or the 2023 Collaboration Products, arising under the 2023 Neurocrine Programs.

Pursuant to mutually-agreed development plans, during the period beginning on the Neurocrine Effective Date and ending on the third anniversary of the Neurocrine Effective Date, which period may be extended upon mutual written agreement of us and Neurocrine, or the 2023 Discovery Period, and as overseen by the Joint Steering Committee, or JSC, that oversees our ongoing collaboration with Neurocrine, we are responsible for identifying capsids meeting target criteria, producing development candidates, and conducting other non-clinical activities regarding the 2023 Collaboration Products. Neurocrine has agreed to be responsible for all costs we incur in conducting preclinical development activities for each 2023 Neurocrine Program, in accordance with JSC-agreed upon workplans and budgets. If we breach our development responsibilities or, in certain circumstances, upon a change of control, Neurocrine has the right, but not the obligation, to assume the conduct of our activities under such 2023 Neurocrine Program.

We have been granted the option, or a 2023 Co-Co Option, to co-develop and co-commercialize 2023 Collaboration Products in the GBA1 Program in the United States upon the occurrence of a specified event, or a 2023 Co-Co Trigger Event. Should we elect to exercise our 2023 Co-Co Option, we and Neurocrine agree to enter into a cost- and profit-sharing arrangement, or a 2023 Co-Co Agreement, whereby we and Neurocrine agree to jointly develop and commercialize 2023 Collaboration Products in the GBA1 Program, or 2023 Co-Co Products, in the United States and share equally in the GBA1 Program's costs, profits and losses in the United States, with each party entitled to or responsible for 50% of profits and losses with respect to each 2023 Co-Co Product in the United States, subject to specified exceptions. The parties have agreed that the 2023 Co-Co Agreement will provide us the right to terminate the 2023 Co-Co Agreement for any reason upon prior written notice to Neurocrine and provide Neurocrine the right to

terminate or amend the 2023 Co-Co Agreement upon a change of control under certain circumstances. In the event we exercise our 2023 Co-Co Option, the parties have also agreed that Neurocrine is entitled to receive (in addition to its 50% share of profits) 50% of our share of profits until our obligation to repay 50% of all development costs incurred by Neurocrine in connection with the GBA1 Program prior to such exercise have been paid off out of such 50% of our share of profits. The 2023 Co-Co Trigger Event is the date on which we receive topline data from the first Phase 1 clinical trial for a product candidate being developed pursuant to the GBA1 Program.

Governance

Our research and development activities under the 2023 Collaboration Agreement are to be conducted pursuant to plans agreed to by the parties, on a 2023 Neurocrine Program-by-2023 Neurocrine Program basis, and overseen by the JSC, which is composed of an equal number of representatives from each of us and Neurocrine. The JSC may delegate matters within its authority to subcommittees of the JSC. In addition, the 2023 Collaboration Agreement establishes working groups to handle specified matters on a subject matter-by-subject matter basis. If a working group or subcommittee cannot agree on a matter within its purview within a specified time, such matter is to be referred sequentially to the JSC and then the executive officers of the parties. If the executive officers are not able to resolve the matter, then (a) with respect to the GBA1 Program, subject to specified exceptions, (x) Neurocrine has the right to resolve such matter prior to our exercise of our 2023 Co-Co Option for the GBA1 Program or in the event we elect not to exercise our 2023 Co-Co Option, and (y) following the exercise by us of our 2023 Co-Co Option for the GBA1 Program, depending on the subject of such matter, either Neurocrine, in certain instances, or the parties jointly or the JSC, in other instances, would have the right to resolve such matter, and (b) with respect to the 2023 Discovery Programs, subject to specified exceptions, Neurocrine has the right to decide any unresolved matters relating to a 2023 Discovery Program that are within the JSC's authority.

Candidate Selection

Either party may notify the JSC of any gene therapy product candidate that includes a Voyager capsid and a payload that is being developed under a 2023 Neurocrine Program, or a Collaboration Candidate, that it desires to nominate as a development candidate. In such event, the JSC shall determine whether such nominated Collaboration Candidate meets certain development criteria. There will be a maximum of four potential development candidates for which development is being performed under any 2023 Neurocrine Program at any given time during the 2023 Discovery Period. If a Collaboration Candidate fails to meet criteria established by the JSC and is removed from consideration to become a development candidate or is named a development candidate, then a new Collaboration Candidate may be nominated to be a potential development candidate to replace the Collaboration Candidate that has failed or succeeded such that not more than four potential development candidates per program are under consideration at any one time during the 2023 Discovery Period.

Manufacturing

The parties have agreed that the applicable development plans shall specify the allocation between us and Neurocrine of responsibilities for the manufacturing of Collaboration Candidates associated with the applicable 2023 Neurocrine Program during the 2023 Discovery Period. In accordance with the 2023 Collaboration Agreement, the parties have also agreed that, if we conduct any portion of the manufacturing of a Collaboration Candidate, the applicable development plan shall include an obligation for us to assist with the technology transfer of such manufacturing responsibilities to Neurocrine or a third-party contract manufacturing organization, as reasonably requested by Neurocrine, on terms to be mutually-agreed by us and Neurocrine. Following the end of the 2023 Discovery Period, Neurocrine shall be responsible for the manufacturing of all Collaboration Candidates and products.

Financial Terms

Under the terms of the 2023 Neurocrine Collaboration Agreement, Neurocrine paid us an upfront payment of approximately $136.0 million and approximately $39.0 million as consideration for an equity purchase of 4,395,588 shares of our common stock in February 2023. The 2023 Collaboration Agreement provides for aggregate development milestone payments from Neurocrine to us for 2023 Collaboration Products under (a) the GBA1 Program of up to $985.0

million; and (b) each of the three 2023 Discovery Programs of up to $175.0 million for each 2023 Discovery Program. We may be entitled to receive aggregate commercial milestone payments for up to two 2023 Collaboration Products under the GBA1 Program of up to $950.0 million per 2023 Collaboration Product and for one 2023 Collaboration Product under each 2023 Discovery Program of up to $275.0 million per 2023 Discovery Program.

Neurocrine has also agreed to pay us tiered royalties, based on future net sales of the 2023 Collaboration Products. Such royalty percentages, for net sales in and outside the United States, range from (a) for the GBA1 Program, the low double-digits to twenty and the high single-digits to mid-teens, respectively, and (b) for each 2023 Discovery Program, high single-digits to mid-teens and mid-single digits to low double-digits, respectively. On a country-by-country and 2023 Neurocrine Program-by-2023 Neurocrine Program basis, the parties have agreed royalty payments would commence on the first commercial sale of a 2023 Collaboration Product in such country and terminate upon the latest of (a) the expiration, invalidation or the abandonment of the last patent covering the composition of the 2023 Collaboration Product or its approved method of use in such country, (b) ten years from the first commercial sale of the 2023 Collaboration Product in such country and (c) the expiration of regulatory exclusivity in such country, or the 2023 Royalty Term. Royalty payments may be reduced by up to 50% in specified circumstances, including expiration of patent rights related to a 2023 Collaboration Product, approval of biosimilar products in a given country, or required payment of licensing fees to third parties related to the development and commercialization of any 2023 Collaboration Product. Additionally, the licenses granted to Neurocrine shall automatically convert to a fully-paid, perpetual, irrevocable royalty-free license on a country-by-country and 2023 Collaboration Product-by-2023 Collaboration Product basis upon the expiration of the 2023 Royalty Term applicable to the 2023 Collaboration Product in such country.

Intellectual Property

Under the terms of the 2023 Neurocrine Collaboration Agreement, each party owns all right, title and interest in and to all patent rights or know-how controlled by such party and existing as of or before the Neurocrine Effective Date or created or acquired solely by or on behalf of such party (including through its or its affiliate's representatives) after the Neurocrine Effective Date outside of its activities under the 2023 Neurocrine Collaboration Agreement. The parties have further agreed that all know-how created by either or both parties in the performance of the activities as undertaken pursuant to a development plan during the 2023 Discovery Period or in the course of development, manufacture and commercialization of Collaboration Candidates or products and all patent rights covering such know-how, or collectively the 2023 Neurocrine Arising IP, is to be owned as follows: (a) we solely own all 2023 Neurocrine Arising IP created jointly by representatives of us and Neurocrine that constitutes capsid know-how and capsid patent rights, and 2023 Neurocrine Arising IP created solely by representatives of Neurocrine through the use of our confidential information, including unpublished sequence information for our capsids; and (b) with respect to all other 2023 Neurocrine Arising IP, (x) we solely own all such 2023 Neurocrine Arising IP created solely by our representatives, (y) Neurocrine solely owns all such 2023 Neurocrine Arising IP created solely by Neurocrine representatives; and (z) the parties jointly own all such 2023 Neurocrine Arising IP created jointly by representatives of both Neurocrine and us. 2023 Neurocrine Arising IP owned by us is included in the license granted from us to Neurocrine described above.

Exclusivity

During the term of the 2023 Neurocrine Collaboration Agreement, neither party nor any of its respective affiliates is permitted to directly or indirectly develop, manufacture or commercialize any other gene therapy product directed to a target under any 2023 Neurocrine Program, or grant any affiliate or third party a license or sublicense to enable any third-party to do so, subject to specified exceptions, including the parties' conduct of certain basic research, provided that Neurocrine or its affiliates may develop competitive products that do not contain an adeno-associated virus as the viral vector.

Termination

Unless earlier terminated, the 2023 Neurocrine Collaboration Agreement expires on the later of (a) the expiration of the last to expire 2023 Royalty Term with respect to all 2023 Collaboration Products worldwide or (b) the expiration or termination of any 2023 Co-Co Agreement. Neurocrine may terminate the 2023 Neurocrine Collaboration Agreement in its entirety or on a 2023 Neurocrine Program-by-2023 Neurocrine Program and/or country-by-country

basis by providing at least (a) 180-day advance notice if such notice is provided prior to the first commercial sale of any 2023 Collaboration Product to which the termination applies or (b) one-year advance notice if such notice is provided after the first commercial sale of any product to which the termination applies. Neurocrine may terminate the 2023 Neurocrine Collaboration Agreement with respect to a given 2023 Collaboration Product by providing written notice of termination to us within thirty days after complete readout of any clinical trial if the results of such clinical trial fail to meet the pre-specified primary endpoint(s) set forth in the applicable protocol or if there is a safety finding during the clinical trial relating to such 2023 Collaboration Product that either (a) is substantially irreversible or not monitorable in patients or (b) results in Neurocrine's decision to designate such 2023 Collaboration Product as a terminated product under the 2023 Collaboration Agreement.

We may terminate the 2023 Neurocrine Collaboration Agreement with respect to a particular patent right of ours, if Neurocrine challenges the validity or enforceability of such patent right. Subject to a cure period, either party may terminate the 2023 Neurocrine Collaboration Agreement in the event of a material breach in whole or in part, subject to specified conditions.

2023 Neurocrine Stock Purchase Agreement

In connection with the execution of the 2023 Neurocrine Collaboration Agreement, we and Neurocrine also entered into a stock purchase agreement on January 8, 2023 for the sale and issuance of 4,395,588 shares of common stock to Neurocrine at a price of $8.88 per share, for an aggregate purchase price of approximately $39.0 million. In accordance with the terms and conditions of the stock purchase agreement, we issued and sold these shares to Neurocrine on February 23, 2023.

2023 Neurocrine Amended and Restated Investors Rights Agreement

In connection with the execution of the 2023 Neurocrine Collaboration Agreement, we and Neurocrine also amended and restated our existing investor agreement on January 8, 2023, or the 2023 Neurocrine Amended and Restated Investor Agreement, providing for standstill and lock-up restrictions and a voting agreement with respect to our shares owned by Neurocrine. Pursuant to the 2023 Neurocrine Amended and Restated Investor Agreement, we caused Jude Onyia, Ph.D., Chief Scientific Officer of Neurocrine, to be appointed to our board of directors as a Class III director on February 23, 2023. We have agreed that we shall cause Dr. Onyia, or another individual designated by Neurocrine, to be nominated for election to our board of directors when Dr. Onyia's initial term is scheduled to expire. Under the 2023 Neurocrine Amended and Restated Investor Agreement, Neurocrine's right to designate an individual to serve as a director on our board of directors and our agreement to nominate such individual for election to our board of directors is subject to specified conditions and shall terminate upon the earliest of (a) Neurocrine holding less than 10% of our outstanding common stock; (b) a change of control of us or Neurocrine; (c) a liquidation or dissolution of us; and (d) the date that is ten years from the closing date of the 2023 Neurocrine Amended and Restated Investor Agreement.

Pursuant to the terms of the 2023 Neurocrine Amended and Restated Investor Agreement, Neurocrine has agreed not to, without the prior written approval of us and subject to specified conditions, directly or indirectly acquire shares of our outstanding common stock, seek or propose a tender or exchange offer or merger between the parties, solicit proxies or consents with respect to any matter, or undertake other specified actions related to the potential acquisition of additional equity interests in us, or the Neurocrine Standstill Restrictions. Further, Neurocrine has also agreed not to, and to cause its affiliates not to, sell or transfer any of our shares without our prior written approval subject to specified conditions, or the Neurocrine Lock-Up Restrictions.

In addition, pursuant to the terms of the 2023 Neurocrine Amended and Restated Investor Agreement, Neurocrine has agreed that any of our shares it owns are subject to a voting agreement such that, subject to specified conditions and excluding specified extraordinary matters, Neurocrine has agreed to, and has agreed to cause its permitted transferees to, vote in accordance with the recommendation of our board of directors and has granted us an irrevocable proxy with respect to the foregoing, or the Neurocrine Voting Agreement.

Each of the Neurocrine Standstill Restrictions, the Neurocrine Lock-Up Restrictions, and the Neurocrine Voting Agreement terminate upon the earliest to occur of: (i) the date that is the third anniversary of the effective date of the

2023 Neurocrine Amended and Restated Investor Agreement and (ii) our liquidation or dissolution. The Neurocrine Standstill Restrictions and Neurocrine Lock-Up Restrictions also terminate upon the deregistration of our common stock, if earlier. The Neurocrine Lock-Up Restrictions and Neurocrine Voting Agreement also terminate on a change of control of us or the date on which Neurocrine and its affiliates beneficially own less than three percent of our common stock on an outstanding basis. The Neurocrine Standstill Restrictions and Neurocrine Voting Agreement also terminate upon the later of (x) the expiration or termination of the 2019 Neurocrine Collaboration Agreement and (y) the expiration or termination of the 2023 Neurocrine Collaboration Agreement.

2019 Neurocrine Collaboration Agreement

In January 2019, we entered into the 2019 Neurocrine Collaboration Agreement for the research, development and commercialization of certain of our AAV gene therapy products. Under the 2019 Neurocrine Collaboration Agreement, we agreed to collaborate on the conduct of four collaboration programs, which we refer to collectively as the 2019 Neurocrine Programs: the NBIb-1817 (VY-AADC) program for the treatment of Parkinson's disease, or the VY-AADC Program; the FA Program, which together with the VY-AADC Program, we refer to as the Legacy Programs; and other undisclosed programs, or the 2019 Discovery Programs.

Collaboration and Licenses

Under the terms of the 2019 Neurocrine Collaboration Agreement, subject to the rights retained by us thereunder, we agreed to collaborate with Neurocrine on, and to grant, exclusive, royalty-bearing, non-transferable, sublicensable licenses to certain of our intellectual property rights, for all human and veterinary diagnostic, prophylactic, and therapeutic uses, for the research, development, and commercialization of gene therapy products, which we refer to as the 2019 Collaboration Products, under (a) the VY-AADC Program on a worldwide basis; (b) the FA Program, in the United States and, all countries in the world in which the 2019 Neurocrine Collaboration Agreement remains in effect with respect to the FA Program; and (c) each of the 2019 Discovery Programs, on a worldwide basis. Licenses related to the VY-AADC Program terminated in August 2021.

As a result of the June 2019 Sanofi Genzyme Termination Agreement, we gained worldwide rights to the Huntington's disease program for VY-HTT01 and ex-U.S. rights to the FA program. We subsequently transferred the ex-U.S. rights to the FA Program to Neurocrine pursuant to the 2019 Neurocrine Collaboration Agreement. To facilitate our transfer of the ex-U.S. rights to the FA Program to Neurocrine, we and Neurocrine amended the 2019 Neurocrine Collaboration Agreement and we received a $5.0 million payment from Neurocrine.

Pursuant to development plans to be agreed by the parties, which are overseen by the JSC, we have operational responsibility, subject to certain exceptions, for the conduct of each 2019 Neurocrine Program prior to the occurrence of a specified event for each 2019 Neurocrine Program, or a 2019 Transition Event, and are required to use commercially reasonable efforts to develop the 2019 Collaboration Products. Neurocrine has agreed to be responsible for all costs incurred by us in conducting these activities for each 2019 Neurocrine Program, in accordance with an agreed budget. If we breach our development responsibilities or in certain circumstances upon a change in control of us, Neurocrine has the right but not the obligation to assume the activities under such 2019 Neurocrine Program.

Upon the occurrence of a 2019 Transition Event for each 2019 Neurocrine Program, Neurocrine agreed to assume responsibility for development, manufacturing and commercialization activities for such 2019 Neurocrine Program from us and to pay milestones and royalties on future net sales as described further below. For each Legacy Program, we were granted the option, or a 2019 Co-Co Option, to co-develop and co-commercialize such 2019 Neurocrine Program upon the occurrence of a specified event, or a 2019 Co-Co Trigger Event. We agreed, were we to exercise a 2019 Co-Co Option, to enter into a cost- and profit-sharing arrangement with Neurocrine, or a 2019 Co-Co Agreement, and (a) jointly develop and commercialize 2019 Collaboration Products for such 2019 Neurocrine Program, or 2019 Co-Co Products, (b) share in its costs, profits and losses, and (c) forfeit certain milestones and royalties on net sales in the United States during the effective period of the applicable 2019 Co-Co Agreement. The 2019 Co-Co Option has expired, and the 2019 Transition Event and the 2019 Co-Co Trigger Event are no longer applicable, with respect to the VY-AADC Program in light of the termination of the 2019 Neurocrine Collaboration Agreement with respect to the program. The remaining 2019 Transition Events are (a) with respect to the FA Program, our receipt of topline data for

the initial Phase 1 clinical trial for an FA Program product candidate; and (b) with respect to each 2019 Discovery Program, the preparation by us and the approval by Neurocrine of an IND application to be filed with the FDA by Neurocrine for the first development candidate in such 2019 Discovery Program. The 2019 Co-Co Trigger Event for the FA Program is the achievement of milestones or metrics specified in the applicable development plan, as determined by the JSC.

Under the 2019 Neurocrine Collaboration Agreement, subject to exceptions specified, we and Neurocrine agreed that profits and losses under our 2019 Co-Co Option would be allocated (a) 50% to Neurocrine and 50% to us for a 2019 Collaboration Product from the VY-AADC Program and (b) 60% to Neurocrine and 40% to us for a 2019 Collaboration Product from the FA Program; provided, however, that Neurocrine would have the right to elect, within a specified period following the acceptance for filing of a biologics license application, or BLA, from the FDA, to pay a $35.0 million rate-shifting fee to us to change the allocation for the VY-AADC Program to 55% to Neurocrine and 45% to us. The parties agreed that each 2019 Co-Co Agreement would provide us the right to terminate for any reason upon prior written notice to Neurocrine and Neurocrine the right to terminate in certain circumstances upon our change of control.

Governance

Our research and development activities under the 2019 Neurocrine Collaboration Agreement are to be conducted pursuant to plans agreed to by the parties, on a program-by-program basis, and overseen by the JSC, which is composed of an equal number of representatives from the parties. The JSC may delegate matters within its authority to subcommittees of the JSC. In addition, the 2019 Neurocrine Collaboration Agreement establishes working groups to handle specified matters on a subject matter-by-subject matter basis. If a working group or subcommittee cannot agree on a matter within its purview within a specified time, such matter is to be referred sequentially to the JSC and then the executive officers of the parties. If the executive officers are not able to resolve the matter, then (a) with respect to each Legacy Program, subject to specified exceptions, (x) Neurocrine has the right to resolve such matter prior to our exercise of our 2019 Co-Co Option with regard to such 2019 Co-Co Product or if such 2019 Co-Co Option expires or goes unexercised and (y) following the timely exercise by us of our 2019 Co-Co Option, depending on the subject of such matter, either Neurocrine, in certain instances, or the parties jointly or the JSC, in other instances, would have the right to resolve such matter, and (b) with respect to 2019 Discovery Programs, subject to specified exceptions, Neurocrine has the right to resolve such matter.

Candidate Selection

The parties have committed to agree on a list of up to eight target genes, or Targets, from which Neurocrine has the right to nominate Targets for the two 2019 Discovery Programs. The Targets nominated for the 2019 Discovery Programs must be approved by a consensus of the JSC or the executive officers.

Manufacturing

Prior to the 2019 Transition Event for a 2019 Neurocrine Program, we are responsible for the manufacture of any 2019 Collaboration Products for the 2019 Neurocrine Program. Following the Transition Event, the parties shall negotiate the manufacturing and supply responsibilities, subject to the terms of any applicable 2019 Co-Co Agreement.

Financial Terms

Under the terms of the 2019 Neurocrine Collaboration Agreement, Neurocrine has paid us an upfront payment of $115.0 million. In connection with the 2019 Neurocrine Collaboration Agreement, Neurocrine also paid us $50.0 million as consideration for an equity purchase of 4,179,728 shares of our common stock. The 2019 Neurocrine Collaboration Agreement provides for aggregate development milestone payments from Neurocrine to us for 2019 Collaboration Products under (a) the FA Program of up to $195.0 million, and (b) each of the two 2019 Discovery Programs of up to $130.0 million per 2019 Discovery Program. We may be entitled to receive aggregate commercial milestone payments for each 2019 Collaboration Product of up to $275.0 million, subject to an aggregate cap on commercial milestone payments across all 2019 Neurocrine Programs of $1.1 billion. We are no longer eligible to

receive milestone or royalty payments for the VY-AADC Program in light of the partial termination of the 2019 Neurocrine Collaboration Agreement with respect to the VY-AADC Program.

Neurocrine has also agreed to pay us royalties, based on future net sales of the 2019 Collaboration Products. Such royalty percentages, for net sales in and outside the United States, as applicable, range (a) for the FA Program, from the low-teens to high-teens and high-single digits to mid-teens, respectively; and (b) for each 2019 Discovery Program, from the high-single digits to mid-teens and mid-single digits to low-teens, respectively. On a country-by-country and program-by-program basis, royalty payments would commence on the first commercial sale of a 2019 Collaboration Product and terminate on the later of (x) the expiration of the last patent covering the 2019 Collaboration Product or its method of use in such country, (y) 10 years from the first commercial sale of the 2019 Collaboration Product in such country and (z) the expiration of regulatory exclusivity in such country, or the 2019 Royalty Term. Royalty payments may be reduced by up to 50% in specified circumstances, including expiration of patents rights related to a 2019 Collaboration Product, approval of biosimilar products in a given country or required payment of licensing fees to third parties related to the development and commercialization of any 2019 Collaboration Product. Additionally, the licenses granted to Neurocrine shall automatically convert to fully paid-up, non-royalty bearing, perpetual, irrevocable, exclusive licenses on a country-by-country and product-by-product basis upon the expiration of the 2019 Royalty Term applicable to such 2019 Collaboration Product in such country.

Intellectual Property

Under the terms of the 2019 Neurocrine Collaboration Agreement and subject to specified exceptions therein, each party owns the entire right, title and interest in and to all intellectual property rights made solely by its employees or agents in the course of the collaboration. The parties jointly own all rights, title and interest in and to all intellectual property rights made or invented jointly by employees or agents of both parties.

Exclusivity

During the term of the 2019 Neurocrine Collaboration Agreement, neither party nor any of its respective affiliates is permitted to directly or indirectly exploit any AAV-based gene therapy products directed to a Target to which a 2019 Collaboration Product is directed, subject to specified exceptions, including the parties' conduct of basic research activities.

Termination

Unless earlier terminated, the 2019 Neurocrine Collaboration Agreement expires on the later of (a) the expiration of the last to expire 2019 Royalty Term with respect to a 2019 Collaboration Product in all countries in the relevant territory or (b) the expiration or termination of all 2019 Co-Co Agreements. Neurocrine may terminate the 2019 Neurocrine Collaboration Agreement in its entirety or on a program-by-program or country-by-country basis by providing at least (x) 180-day advance notice if such notice is provided prior to the first commercial sale of the 2019 Collaboration Product to which the termination applies or (y) one-year advance notice if such notice is provided after the first commercial sale of the 2019 Collaboration Product to which the termination applies. We may terminate the 2019 Neurocrine Collaboration Agreement, subject to specified conditions, if Neurocrine challenges the validity or enforceability of certain of our intellectual property rights. Subject to a cure period, either party may terminate the 2019 Neurocrine Collaboration Agreement in the event of a material breach by the other party in whole or in part, subject to specified conditions.

Upon termination in certain cases, Neurocrine has agreed to grant to us licenses to certain Neurocrine intellectual property, subject to a negotiation between the parties to establish royalty rates for use of such intellectual property. In the event of a breach by us with respect to a 2019 Neurocrine Program, if such termination were to occur after a 2019 Transition Event, then (a) if a 2019 Co-Co Agreement is in effect with respect to such program, Neurocrine can terminate the 2019 Co-Co Agreement for such program and we would no longer have co-development and co-commercialization rights with respect to the 2019 Collaboration Product and (b) subject to any license agreements, Neurocrine would no longer have any obligations with respect to any 2019 Collaboration Products resulting from such program.

On February 2, 2021, Neurocrine notified us that it had elected to terminate the 2019 Neurocrine Collaboration Agreement solely with regards to the VY-AADC Program, effective as of the Neurocrine VY-AADC Program Termination Effective Date. The 2019 Neurocrine Collaboration Agreement remains in full force and effect for each other program thereunder. As a result of the termination, as of the Neurocrine VY-AADC Program Termination Effective Date, the license granted by us to Neurocrine thereunder regarding the VY-AADC Program expired and we regained worldwide intellectual property rights regarding the VY-AADC Program.

Alexion Option and License Agreement (Formerly Pfizer Option and License Agreement)

Summary of Agreement

On October 1, 2021, or the Pfizer Effective Date, we entered into an option and license agreement, or the Pfizer Agreement, with Pfizer, Inc., or Pfizer, pursuant to which we granted Pfizer the Pfizer License Options to certain TRACER Capsids to develop and commercialize certain AAV gene therapy candidates comprised of a capsid and specified Pfizer transgenes, or the Pfizer Transgenes. Under the terms of the Pfizer Agreement, during an initial research term that ended as of October 1, 2022, or the Pfizer Research Term, Pfizer had the right to evaluate the potential use of the capsids in combination with up to two Pfizer Transgenes to help treat respective CNS and cardiovascular diseases.

Research and License Option

During the Pfizer Research Term, we agreed to provide Pfizer with certain quantities of materials encoding specified existing capsids for Pfizer's evaluation. Further, during the Pfizer Research Term, we agreed to disclose to Pfizer, on a rolling basis, the performance characteristics identified during the Pfizer Research Term for all such capsid candidates. Pfizer had the right, in its sole discretion, to select any capsid candidate for evaluation to determine its interest in exercising a Pfizer License Option with respect to such capsid candidate. Pfizer had the right to exercise up to two Pfizer License Options, provided that it could exercise only one Pfizer License Option for each Pfizer Transgene.

Effective as of September 30, 2022, Pfizer exercised its Pfizer License Option with respect to a capsid for the specified Pfizer Transgene for potential treatment of a rare neurological disease. Pfizer did not exercise its option to license a capsid for the potential treatment of a cardiovascular disease. As result, Pfizer's right to exercise a Pfizer License Option for a cardiovascular disease has terminated in accordance with the terms of the Pfizer Agreement and all rights to capsids for that cardiovascular disease have reverted to us. Pfizer's exercise of a Pfizer License Option extended the Pfizer Research Term to October 1, 2024, during which period we may, at our sole discretion and expense, conduct additional research activities to identify additional proprietary capsids that may be useful for AAV gene therapies for the treatment of the rare neurological disease associated with the exercise of the applicable Pfizer License Option.

Pursuant to the exercise of the Pfizer License Option, we granted Pfizer an exclusive, worldwide license, with the right to sublicense, under certain of our intellectual property, the rights to develop and commercialize rare neurological disease products utilizing the capsid candidate and incorporating the corresponding Pfizer Transgene, or the Pfizer Licensed CNS Products.

On July 28, 2023, Alexion entered into a definitive purchase and license agreement for preclinical gene therapy assets and enabling technologies from Pfizer. Effective upon the closing of the transaction on September 20, 2023, Alexion acquired all of Pfizer's rights under the Pfizer Agreement, which we now refer to as the Alexion Agreement, and became the successor-in-interest to Pfizer thereunder. The acquisition does not impact the material terms of the option and license agreement. Until October 1, 2024, while we are not obligated to conduct additional research activities to identify additional proprietary capsids that may be useful for AAV gene therapies for the treatment of rare neurological diseases, we have agreed to continue to disclose to Alexion, on a rolling basis, the performance characteristics identified for all such capsid candidates, if and when available, until the expiration of the Pfizer Research Term, which we now refer to as the Alexion Research Term. Alexion may, during the Alexion Research Term, conduct additional evaluations of such capsid candidates and has the right to substitute any other capsid candidate for the capsid Pfizer elected to license when it exercised the Pfizer License Option.

Development, Regulatory Approval and Commercialization

Under the Alexion Agreement, Alexion is solely responsible for, and has sole decision-making authority with respect to, development and commercialization of the Pfizer Licensed CNS Products, which we now refer to as the Alexion Licensed Products. Alexion is required to use commercially reasonable efforts to develop and obtain regulatory approval for at least one Alexion Licensed CNS Product for which Pfizer exercised its Pfizer License Option in (a) the United States and (b) at least one of the following countries: the United Kingdom, France, Germany, Italy, Spain and Japan, each of which we refer to as an Alexion Major Market Country, subject to certain limitations. Alexion is also required to use commercially reasonable efforts to commercialize each Alexion Licensed CNS Product in the United States and at least one Alexion Major Market Country where Alexion or its designated affiliates or sublicensees has received regulatory approval for such Alexion Licensed CNS Product, subject to certain limitations.

Intellectual Property

Under the terms of the Alexion Agreement, each of the parties owns the entire right, title, and interest in and to all patents or know-how controlled by such party and existing as of or before the effective date of the Alexion Agreement, or invented, developed, created, generated or acquired solely by or on behalf of such party after such effective date.

Exclusivity

Subject to certain specified exceptions, any patents and know-how that are invented or otherwise developed jointly by or on behalf of the parties during the term of the Alexion Agreement and in the course of our and Alexion's activities under the Alexion Agreement will follow inventorship under U.S. patent law. Subject to certain limitations and exceptions, we have agreed (a) during the Alexion Research Term, not to conduct any internal program or program on behalf of a third party that is directed to development or commercialization of any capsid candidates, or grant any third party or affiliate any right or license under our rights in such capsid candidates to exploit any therapeutic product, in combination with any Pfizer Transgene, which we now refer to as an Alexion Transgene, in any indication for therapeutic, diagnostic and prophylactic human and veterinary use; and (b) not to grant any third party or affiliate any right or license under our patents to exploit any licensed capsid in combination with any Alexion Transgene.

Financial

Under the terms of the Alexion Agreement, Pfizer paid us an upfront payment of $30.0 million in October 2021. Following the exercise of the Pfizer License Option, Pfizer paid us a fee of $10.0 million. We are also eligible to receive specified development, regulatory, and commercialization milestone payments of up to an aggregate of $115.0 million for the first corresponding Alexion Licensed CNS Product to achieve the corresponding milestone. On an Alexion Licensed CNS Product-by-Alexion Licensed CNS Product basis, we are also eligible to receive (a) specified sales milestone payments of up to an aggregate of $175.0 million per Alexion Licensed CNS Product and (b) tiered, escalating royalties in the mid- to high-single-digit percentages of annual net sales of each Alexion Licensed CNS Product. The royalties are subject to potential reductions in customary circumstances including patent claim expiration, payments for certain third-party licenses, and biosimilar market penetration, subject to specified limits.

Termination

Unless earlier terminated, the Alexion Agreement expires on the expiration of the last-to-expire royalty term with respect to all Alexion Licensed CNS Products in all countries. Subject to a cure period, either party may terminate the Alexion Agreement, in whole or in part, subject to specified conditions, in the event of the other party's uncured material breach. Alexion may also terminate the Alexion Agreement, in whole or in part, subject to specified conditions, for our insolvency, the occurrence of a violation of global trade control laws, or for our noncompliance with certain anti-bribery or anti-corruption covenants. Alexion may also terminate the Alexion Agreement, in whole or in part, for any or no reason upon ninety days' written notice to us.

Upon certain terminations for cause by Alexion, the license that we have granted to Alexion under the Alexion Agreement shall become irrevocable and perpetual, and all milestone payments and royalties that would have otherwise been payable by Alexion under such license had the Alexion Agreement remained in effect would be substantially reduced.

License Agreement with Sangamo

On June 28, 2023, we entered into a definitive license agreement for a potential treatment of prion disease with Sangamo. Using their proprietary epigenetic regulation platform, Sangamo has developed zinc finger transcriptional regulators which they believe can specifically and potently block expression of the prion protein, the pathogenic driver of prion disease. We are eligible to earn certain license fees, royalties on potential commercial sales of any products using our capsid, and, in the event the prion program is out licensed by Sangamo, a portion of all licensing revenues received with respect to this program.

License Agreement with Touchlight IP Limited

On November 3, 2022, we and Touchlight IP Limited, or Touchlight, entered into a license agreement, or the Touchlight License Agreement, to authorize historical use by us of a certain DNA preparation process, or the Subject DNA Preparation Process, and to authorize the prospective exploitation of TRACER Capsids created with the use of the Subject DNA Preparation Process.

The terms of the Touchlight License Agreement include a one-time, non-refundable technology access fee of $5.0 million, paid to Touchlight during the fourth quarter of 2022.

The terms of the Touchlight License Agreement also include future milestone payments and low single-digit royalties payable to Touchlight by us if we or our program collaborators or licensees choose to utilize in a therapeutic product certain TRACER Capsids that were created with the historical use of the Subject DNA Preparation Process. Additionally, we are obligated to pay low single-digit royalties to Touchlight on future payments we receive in connection with licensing of certain TRACER Capsids that were created with the historical use of the Subject DNA Preparation Process, excluding the licensing of or collaboration on any of our therapeutic programs.

Competition

The biopharmaceutical industry is characterized by intense and dynamic competition to develop new technologies and proprietary therapies. Any product candidates that we successfully develop into products and commercialize may compete with existing therapies and new therapies that may become available in the future. While we believe that our gene therapy platform, product programs, product candidates and scientific expertise in the fields of gene therapy and neuroscience provide us with competitive advantages, we face potential competition from various sources, including larger and better-funded pharmaceutical, specialty pharmaceutical and biotechnology companies, as well as from academic institutions, governmental agencies and public and private research institutions.

We expect that our TRACER discovery platform and preclinical programs will compete with a variety of therapies in development, including:

- Our anti-tau antibody and tau silencing gene therapy programs for AD will potentially compete with tau antibodies being developed by Lundbeck Inc., Merck & Co. Inc. in collaboration with Teijin Limited, Roche Genentech Inc. in collaboration with AC Immune SA, Eisai Co., Ltd., Janssen Pharmaceuticals, Inc., UCB S.A., Bristol Myers Squibb Company in collaboration with Prothera Inc., along with several other companies, as well as an antisense oligonucleotide program being developed by Ionis in collaboration with Biogen;

- Our program for a monogenic form of ALS will potentially compete with Tofersen being developed by Biogen, in collaboration with Ionis, and gene therapies being developed by Novartis Gene Therapies, Inc. and uniQure, Inc.; and

- Our TRACER discovery platform will potentially compete with a variety of companies developing AAV capsids, including: 4D Molecular Therapeutics, Inc., Affinia Therapeutics Inc., Apertura Gene Therapy, LLC, Capsida Biotherapeutics, Inc., Capsigen, Inc., Dyno Therapeutics, Inc., Kate Therapeutics, Inc., and Shape Therapeutics Inc.

We are aware of several companies focused on developing AAV gene therapies in various indications, including Abeona Therapeutics, Inc., Adverum Biotechnologies, Inc., Akouos, Inc. (acquired by Eli Lilly and Company, or Eli Lilly), Alcyone Therapeutics, Inc., Amicus Therapeutics, Inc., Asklepios BioPharmaceutical, Inc. (acquired by Bayer), Astellas Gene Therapies, Inc., Beacon Therapeutics Holdings Limited, Biogen, Inc., or Biogen, BioMarin Pharmaceuticals, Inc., Encoded Therapeutics, Inc., GenSight Biologics S.A., Homology Medicines, Inc., LEXEO Therapeutics, Inc., LogicBio Therapeutics, Inc. (acquired by AstraZeneca), MeiraGTx Ltd., Neurogene, Inc., Novartis Gene Therapies, Inc. (formerly AveXis, Inc.), Passage Bio, Inc., Pfizer, Inc., Prevail Therapeutics, Inc. (acquired by Eli Lilly), REGENXBio Inc., Sarepta Therapeutics, Inc., Solid Biosciences, Inc., Spark Therapeutics, Inc. (acquired by Roche Genentech Inc.), Taysha Gene Therapies, Inc. and uniQure, Inc., as well as several companies addressing other methods for modifying genes and regulating gene expression. Any advances in gene therapy technology made by a competitor may be used to develop therapies that could compete against any of our product candidates.

Many of our competitors, either alone or with their strategic partners, have substantially greater financial, technical and human resources than we do and significantly greater experience in the discovery and development of product candidates, obtaining FDA and other regulatory approvals of product candidates and commercializing those product candidates. Accordingly, our competitors may be more successful than us in obtaining approval for product candidates and achieving widespread market acceptance. Our competitors' product candidates may be more effective, or more effectively marketed and sold, than any product candidate we may commercialize and may render our treatments obsolete or non-competitive before we can recover the expenses of developing and commercializing any of our product candidates.

Mergers and acquisitions in the biotechnology and pharmaceutical industries may result in even more resources being concentrated among a smaller number of our competitors. These competitors also compete with us in recruiting and retaining qualified scientific and management personnel and establishing clinical trial sites and subject registration for clinical trials, as well as in acquiring technologies complementary to, or necessary for, our programs. Smaller or early-stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies.

We anticipate that we will face intense and increasing competition as new product candidates enter the market and advanced technologies become available. We expect any product candidates that we develop and commercialize to compete on the basis of, among other things, efficacy, safety, convenience of administration and delivery, price, and the availability of reimbursement from government and other third-party payers.

Our commercial opportunity could be reduced or eliminated if our competitors develop and commercialize products that are safer, more effective, have fewer or less severe side effects, are more convenient or are less expensive than any products that we may develop. Our competitors also may obtain FDA or other regulatory approval for their product candidates more rapidly than we may obtain approval for ours, which could result in our competitors establishing a strong market position before we are able to enter the market.

Manufacturing

The manufacture of gene therapy products is technically complex, and necessitates substantial expertise and capital investment. Production difficulties caused by unforeseen events may delay the availability of material for our clinical studies. To meet the requirements of our current and planned future trials we have developed a proprietary HEK 293 transient transfection manufacturing process that is scalable for large scale AAV manufacturing – both for preclinical research activities and clinical and commercial manufacturing. We continuously innovate and develop advanced manufacturing technologies to enable high yields and product quality, and we manufacture preclinical AAV material for large animal studies at our onsite, state-of-the-art chemistry, manufacturing, and controls, or CMC,

development facility. Our new manufacturing process is being transferred to our contract manufacturing organizations to enable clinical current good manufacturing practice, or, cGMP manufacturing.

We presently contract with third parties for the manufacturing of our program materials. We currently have no plans to build our own clinical or commercial scale cGMP manufacturing capabilities. The use of contract manufacturing and reliance on collaboration partners is relatively cost-efficient and we believe that it eliminates the need for our direct investment in manufacturing facilities and additional staff early in development. Although we expect to rely on contract manufacturers, we have personnel with manufacturing and quality experience to oversee our contract manufacturers.

Intellectual Property

Overview

We strive to protect the proprietary technology, inventions, and know-how to enhance improvements that are commercially important to the development of our business, including seeking, maintaining, and defending patent rights, whether developed internally or licensed from third parties. We also rely on trade secrets and know-how relating to our proprietary technology platform, on continuing technological innovation and on in-licensing opportunities to develop, improve and maintain the strength of our position in the field of neurogenetic medicines and gene therapy that may be important for the development of our business. We additionally may rely on regulatory protection afforded through data exclusivity, market exclusivity and patent term extensions where available.

Our commercial success may depend in part on our ability to: obtain and maintain patent and other protections for commercially important technology, inventions and know-how related to our business; defend and enforce our patents; preserve the confidentiality of our trade secrets and know-how; and operate without infringing the valid and enforceable patents and intellectual property rights of third parties. Our ability to stop third parties from making, having made, using, selling, offering to sell or importing our products may depend on the extent to which we have rights under valid and enforceable licenses and patents that cover these activities. In some cases, these rights may need to be enforced by third-party licensors. With respect to both licensed and company-owned intellectual property, we cannot be sure that patents will be granted with respect to any of our pending patent applications or with respect to any patent applications filed by us in the future, nor can we be sure that any of our existing patents or any patents that may be granted to us in the future will be commercially useful in protecting our commercial products and methods of using or manufacturing the same.

We own at least 422 pending patent applications and at least 79 patents have issued in the United States and foreign jurisdictions. We co-own at least 42 pending patent applications and at least 12 patents have issued from these co-owned families in the United States and foreign jurisdictions. At least ten patent applications have been filed and are pending in the United States and foreign jurisdictions by or on behalf of universities which have granted us exclusive license rights to the technology. To date, at least 29 patents have issued to our licensors which have granted us exclusive license rights to the technology. To date, at least 153 patents have issued to our licensors which have granted us non-exclusive license rights to the technology with at least 58 applications pending. Our policy is to file patent applications to protect technology, inventions and improvements to inventions that are commercially important to the development of our business. We seek U.S. and international patent protection for a variety of technologies, including: AAV-based biological products and constructs, antibodies, non-viral therapeutic modalities, methods of delivering said AAV-based biological products and constructs, antibodies and non-viral modalities, methods of treating diseases of interest, as well as methods of engineering and manufacturing of the same. We also intend to seek patent protection or rely upon know-how and trade secret rights to protect other technologies that may be used to discover and validate targets and that may be used to identify and develop novel biological products. We seek protection, in part, through confidentiality and proprietary information agreements. We are a party to various other license agreements that give us rights to use specific technologies in our research and development.

Company-Owned Intellectual Property

Tauopathies

We own seven pending patent families directed to antibodies to tau and vectorized forms thereof with 38 pending patent applications. Patents that grant from these families are generally expected to commence expiration in 2037, with some later filed applications commencing expiration in 2040, 2041, 2042, and 2043 all of which are subject to possible patent term extensions. We own one pending patent family to RNA inhibitors for treating tauopathies with three pending patent applications. Patents that grant from this family are generally expected to commence expiration in 2043, subject to possible patent term extensions.

ALS

We own five pending patent families and have 12 issued patents and 35 patent applications directed to targeting SOD1 for the treatment of ALS. We co-own a sixth patent family with one granted patent and seven pending patent applications directed to pharmaceutical compositions and methods for the treatment of ALS to protect our intellectual property arising from a funded grant from The Amyotrophic Lateral Sclerosis Association. Patents that grant from these patent families are generally expected to commence expiration in 2035, with some applications expiring in 2038, 2039, and 2040, all of which are subject to possible patent term extensions.

Friedreich's Ataxia

We own three pending patent families with one granted patent and 21 patent applications and we co-own one pending patent family with eight patent applications directed to AAVs encoding frataxin constructs for the treatment of Friedreich's ataxia. Patents that grant from these patent families are generally expected to commence expiration in 2036, with some later filed applications commencing expiration in 2038, 2039, and 2040, all of which are subject to possible patent term extensions.

GBA1 Gene Therapy

We own three pending patent families with 17 pending patent applications directed to AAVs encoding GBA1 for the treatment of Parkinson's disease, Gaucher disease, and dementia with Lewy Bodies. Patents that grant from this patent family are expected to commence expiration in 2041, 2043, and 2044, subject to possible patent term extensions.

Huntington's Disease

We own five pending patent families with eight issued patents and 24 patent applications directed to pharmaceutical compositions and methods for targeting HTT for the treatment of Huntington's disease. Patents from this family are generally expected to commence expiration in 2037, with some applications expiring in 2038, 2040, and 2044 all of which are subject to possible patent term extensions.

Parkinson's Disease

We own three pending patent families with five issued patents and at least 23 patent applications directed to AAV constructs encoding the gene AADC for therapeutic uses. Patents that grant from these patent families are generally expected to commence expiration in 2035, 2038, and 2039, subject to possible patent term extensions.

Capsids

We own two patent families pending in the United States and foreign jurisdictions that are directed to the TRACER discovery platform for selection of AAV capsids with BBB crossing and cell-specific transduction properties. In these two pending patent families directed to the TRACER discovery platform, there are 11 applications pending, and are generally expected to commence expiration in 2039 and 2041, respectively, subject to possible patent term extensions. We also own seven pending patent families comprising 45 pending U.S. and foreign applications and one

granted U.S. patent, as well as ten pending provisional applications directed to capsid variants identified using the TRACER discovery platform showing improved properties over their wild-type AAV counterparts. Patents that grant from these patent families and pending provisional applications are generally expected to commence expiration in 2041, 2042, 2043, 2044, and 2045, subject to possible patent term extensions. We own at least 49 pending provisional applications and five pending non-provisional applications directed to constructs containing TRACER Capsids in combination with specific payloads for treatment of CNS and other indications. Patents that grant from these pending provisional and non-provisional applications are generally expected to commence expiration in 2042, 2043, and 2044, subject to possible patent term extensions. We own one pending patent family with three pending non-provisional patent applications, as well as one pending provisional application directed to ligands to capsid receptors. Patents that grant from these pending provisional and non-provisional applications are generally expected to commence expiration in 2044 and 2045, subject to possible patent term extensions.

We also own four patent families pending in the United States and foreign jurisdictions directed to capsid variants generated using other methodologies. In these four pending patent families, there are three granted patents and 18 pending patent applications. Patents that grant from these patent families are generally expected to commence expiration in 2038 and 2039, subject to possible patent term extensions. We also co-own three patent families directed to other capsid variants. In these three pending patent families there are five pending applications. Patents that grant from these patent families are generally expected to commence expiration in 2039 and 2040, subject to possible patent term extensions.

Vectorized Antibodies

We own four patent families with two issued patents and eight pending patent applications directed to vectorized antibodies and related platforms. Patents that grant from these patent families are generally expected to commence expiration in 2037, with some later filed applications commencing expiration in 2040, all of which are subject to possible patent term extensions.

Regulatable Expression

We own one pending patent family with three pending patent application directed to regulatable expression control of AAV transgenes. Patents that grant from this patent family are generally expected to commence expiration in 2036, subject to possible patent term extensions.

Vector and Genome Engineering

We own three patent families with 43 issued patents (including 15 patents in European countries) and 41 patent applications directed to engineering of the vector genome. Patents that grant from these patent families are generally expected to commence expiration in 2035, 2037, and 2038, which are all subject to possible patent term extensions.

We own one patent family with one patent application directed to genome engineering. Patents that grant from this patent family are generally expected to commence expiration in 2040, subject to possible patent term extensions.

Production; Chemistry, Manufacturing, and Controls

We own 21 pending patent families with two granted patents and at least 64 pending patent applications directed to AAV production and CMC. Patents that grant from the earliest filed patent families are generally expected to commence expiration in 2035 and patents that grant from the latest filed patent families are generally expected to commence expiration in 2042, all of which are subject to possible patent term extensions. We co-own one pending patent family with nine granted patents and ten pending patent applications directed to AAV production and CMC. Patents that grant from this patent family are generally expected to commence expiration in 2037, subject to possible patent term extensions.

Other

We own one pending patent family with one pending patent application directed to AAVs encoding HER2 antibodies for treating metastatic HER2 positive cancers. Patents that grant from these patent families are generally expected to commence expiration in 2042, subject to possible patent term extensions.

We own one pending patent family with one patent application directed to cannula delivery system and methods of use. Patents that grant from this patent family are generally expected to commence expiration in 2039, subject to possible patent term extensions.

We co-own two pending patent families directed to trajectory array delivery devices, including the variable trajectory array guide, or V-TAG®, device and methods of use. The first pending patent family has one granted patent and six pending patent applications, and the second pending patent family has one granted patent and six pending patent applications. Patents that grant from these patent families are generally expected to commence expiration in 2037 and 2038, subject to possible patent term extensions.

Licensed Intellectual Property

We have obtained exclusive licenses and non-exclusive licenses to patents directed to both compositions of matter and methods of use.

We have licensed six families of patents and patent applications, in the field of gene therapy for human diseases, directed to RNAi constructs as vector payloads, their design and use in the treatment of neurological disorders from the University of Massachusetts. Three of the six families of patents and applications are exclusively licensed and comprise 14 granted patents and seven applications in the United States and other territories. Three of the six families of patents and applications are non-exclusively licensed and comprise 56 granted patents and one pending application in the United States and other territories. Patents from these six families have been granted in the United States, Canada, Europe, Israel, Japan, Korea and Australia. Nationalization for some members has taken place in Germany, Spain, France, Great Britain, Italy, and Netherlands. Patents that grant from these patent families are generally expected to expire between 2024 and 2036, subject to possible patent term extensions.

We have exclusively licensed one family of patents and patent applications directed to AAV capsids from the University of Massachusetts. In this pending patent family, there are 15 granted patents and three pending patent applications. Patents that grant from this patent family are generally expected to commence expiration in 2031, subject to possible patent term extensions.

We have non-exclusively licensed two pending patent families from Ablexis, LLC. These families of patents and patent applications are pending and/or granted in the United States and other territories and comprise 50 granted patents and 6 applications. Patents have been granted in Australia, Canada, Europe, Korea, New Zealand and the United States. Nationalization for some members has taken place in Austria, Belgium, Denmark, France, Germany, Ireland, Italy, Netherlands, Poland, Spain, Switzerland, and United Kingdom. Patents that grant from these patent families are generally expected to expire between 2029 and 2031, subject to possible patent term extensions.

We have non-exclusively licensed two pending patent families directed to AAV capsids from the California Institute of Technology. These families of patents and patent applications are pending in the United States and internationally and comprise 47 granted patents and ten applications. Patents have been granted in the United States. Patents that grant from these patent families are generally expected to commence expiration in 2034 and 2036, subject to possible patent term extensions.

We have non-exclusively licensed three pending patent families directed to microRNA detargeting from the University of Pennsylvania. These families of patent applications are pending in the United States and internationally and comprise 41 applications. Patents that grant from these patent families are generally expected to commence expiration in 2039, 2041, and 2042, subject to possible patent term extensions.

Trademark Protection

We own trademark registrations in the United States for the marks VOYAGER THERAPEUTICS, the VOYAGER THERAPEUTICS Logo, and VOYAGER (with design elements), and registrations in the European Union and United Kingdon for VOYAGER (with design elements) for "pharmaceutical research and development in the field of gene therapy." We also own registrations in the United States, the European Union, and United Kingdom for VOYAGER (with design elements), and pending applications in the United States for VOYAGER and VOYAGER (with design elements) for goods including, among other, biological preparations for gene therapy for the treatment of various diseases. We also own pending applications for VOYAGER and VOYAGER (with design elements) in the United States, and registrations for VOYAGER (with design elements) in the European Union and United Kingdom, for goods and services including, among others, "medical services provided for clinical trials."

We also own U.S. trademark registrations for the mark V-TAG and the V-TAG Logo, for "medical system comprised of a surgical device for guiding, locating or placing a diagnostic device or therapeutic device, namely, stents, probes, needles, leads, grafts, pumps, syringes, catheters, and implants during a medical procedure and related software sold as a unit, none of the aforesaid for use in cardiac ablation; MRI-compatible medical system comprised of an MRI-compatible surgical device for guiding, locating or placing a diagnostic device or therapeutic device, namely, stents, probes, needles, leads, grafts, pumps, syringes, catheters, and implants during a MRI-guided procedure and related software sold as a unit, none of the aforesaid for use in cardiac ablation," as well as trademark registrations in the European Union and United Kingdom for V-TAG for similar trademark classes.

We also own registrations in the United States and United Kingdom, for the mark TRACER for services including, among others, "research and development of platform technologies for genetic delivery of therapies and pharmaceutical via adeno-associated virus (AAV) capsids."

We plan to register trademarks in connection with our biological products.

Protection of Confidential Information, Know-how and Trade Secrets

Finally, we may rely, in some circumstances, on confidential information, know-how and trade secrets to protect our technology. We seek to protect our proprietary technology and processes, in part, by entering into confidentiality agreements with our employees, consultants, scientific advisors and contractors. We also seek to preserve the integrity and confidentiality of our data, know-how and trade secrets by maintaining physical security of our premises and physical and electronic security of our information technology systems. While we have confidence in these individuals, organizations and systems, agreements or security measures may be breached, and we may not have adequate remedies for any breach. In addition, our confidential information, know-how and trade secrets may otherwise become known or be independently discovered by competitors. To the extent that our consultants, contractors or collaborators use intellectual property owned by others in their work for us, disputes may arise as to the rights in related or resulting know-how and inventions.

Government Regulation

The research, development, testing, manufacture, quality control, packaging, labeling, storage, record-keeping, distribution, import, export, promotion, advertising, marketing, sale, pricing and reimbursement of biologic products are extensively regulated by governmental authorities in the United States and other countries. The processes for obtaining regulatory approvals in the United States and in foreign countries and jurisdictions, along with compliance with applicable statutes and regulations and other regulatory requirements, both pre-approval and post-approval, require the expenditure of substantial time and financial resources. The regulatory requirements applicable to biological product development, approval and marketing are subject to change, and regulations and administrative guidance often are revised or reinterpreted by the agencies in ways that may have a significant impact on our business.

U.S. Government Regulation

U.S. Biological Products Development Process

In the United States, the FDA approves and regulates gene therapy and antibody products as biological products, or biologics. These products are licensed for marketing under the Public Health Service Act, or the PHSA, and regulated under the Federal Food, Drug, and Cosmetic Act, or FDCA. A company, institution, or organization which takes responsibility for the initiation and management of a clinical development program for such products, and for their regulatory approval, is typically referred to as a sponsor.

The process required by the FDA before a biological product may be marketed in the United States generally involves the following:

- completion of nonclinical laboratory tests and animal studies according to the FDA's GLPs and applicable requirements for the humane use of laboratory animals or other applicable regulations;
- preparation of clinical trial material in accordance with cGMPs;
- design of a clinical protocol and submission to the FDA of an application for an IND, which must become effective before human clinical trials may begin;
- approval by an independent institutional review board, or IRB, or ethics committee representing each clinical trial site before each clinical trial may be initiated;
- performance of adequate and well-controlled human clinical trials according to the FDA's good clinical practices, or GCPs, and any additional requirements for the protection of human research subjects and their health information, to establish the safety, purity, potency, and efficacy, of the proposed biological product for its intended use;
- submission to the FDA of a BLA for marketing approval that includes substantive evidence of safety, purity, and potency from results of nonclinical testing and clinical studies, including payment of application user fees;
- satisfactory completion of an FDA inspection prior to BLA approval of the manufacturing facility or facilities where the biological product is produced to assess compliance with cGMP, to assure that the facilities, methods and controls are adequate to preserve the biological product's identity, strength, quality and purity;
- potential FDA inspection of the nonclinical and clinical study sites that generated the data in support of the BLA;
- potential FDA Advisory Committee meeting to elicit expert input on critical issues;
- FDA review and approval, or licensure, of the BLA; and
- compliance with any post approval requirements, including the potential requirement to implement a Risk Evaluation and Mitigation Strategy, or REMS, and the potential requirement to conduct post approval studies.

Preclinical Studies

Before a sponsor begins testing a product candidate with potential therapeutic value in humans, the product candidate enters the preclinical testing stage. Preclinical tests include laboratory evaluations of product chemistry, formulation and stability, as well as other studies to evaluate, among other things, the toxicity of the product candidate.

These studies are generally referred to as IND-enabling studies. The conduct of the preclinical tests and formulation of the compounds for testing must comply with federal regulations and requirements, including GLP regulations and standards and the United States Department of Agriculture's Animal Welfare Act, if applicable. The results of the preclinical tests, together with manufacturing information and analytical data, are submitted to the FDA as part of an IND. Some long-term preclinical testing, such as animal tests of reproductive adverse events and carcinogenicity, and long-term toxicity studies, may continue after the IND is submitted.

The IND and IRB Processes

An IND is an exemption from the FDCA that allows an unapproved product candidate to be shipped in interstate commerce for use in an investigational clinical trial and a request for FDA authorization to administer such investigational product to humans. An IND must be secured prior to interstate shipment and administration of any product candidate that is not the subject of an approved new drug application, or NDA, or BLA. In support of a request for an IND, sponsors must submit a protocol for each clinical trial and any subsequent protocol amendments must be submitted to the FDA as part of the IND. An IND automatically becomes effective 30 days after receipt by the FDA, unless before that time the FDA raises concerns or questions related to one or more proposed clinical trials or studies and places the trial on a clinical hold. The FDA may also place a hold or partial hold on a clinical study based on chemistry, manufacturing, and controls issues involving the investigational product. In either case, the IND sponsor and the FDA must resolve any outstanding concerns before the clinical trial may proceed. As a result, submission of an IND may not result in the FDA allowing clinical trials to commence.

Following commencement of a clinical trial under an IND, the FDA may also place a clinical hold or partial clinical hold on that trial. A clinical hold is an order issued by the FDA to the sponsor to delay a proposed clinical investigation or to suspend an ongoing investigation. A partial clinical hold is a delay or suspension of only part of the clinical work requested under the IND. For example, a partial clinical hold might state that a specific protocol or part of a protocol may not proceed, while other parts of a protocol or other protocols may do so. No more than 30 days after the imposition of a clinical hold or partial clinical hold, the FDA will provide the sponsor a written explanation of the basis for the hold. Following the issuance of a clinical hold or partial clinical hold, a clinical investigation may only resume once the FDA has notified the sponsor that the investigation may proceed. The FDA will base that determination on information provided by the sponsor correcting the deficiencies previously cited or otherwise satisfying the FDA that the investigation can proceed or recommence. Occasionally, clinical holds are imposed due to manufacturing issues that may present safety issues for the clinical study subjects.

An IRB representing each institution participating in the clinical trial must also review and approve the plan for any clinical trial before it commences at that institution, and the IRB must conduct continuing review and reapprove the study at least annually. The IRB, which must operate in compliance with FDA regulations, must review and approve, among other things, the study protocol and informed consent information to be provided to study subjects before the study can commence at the institution and must monitor the trial until completed. An IRB can suspend or terminate approval of a clinical trial at its institution, or an institution it represents, if the clinical trial is not being conducted in accordance with the IRB's requirements or if the product candidate has been associated with unexpected serious harm to patients.

Additionally, some trials are overseen by an independent group of qualified experts organized by the trial sponsor, known as a data monitoring committee, or DMC. This group provides an independent recommendation as to whether or not a trial may move forward at designated checkpoints based on review of available data from the study, to which only the DMC maintains access. Suspension or termination of development during any phase of a clinical trial can occur if the DMC recommends stopping a trial due to safety or efficacy.

Human Clinical Trials

Clinical trials involve the administration of the investigational product candidate to human subjects under the supervision of a qualified investigator in accordance with GCP requirements which include, among other things, the requirement that all research subjects provide their informed consent in writing before they participate in any clinical trial. Clinical trials are conducted under written clinical trial protocols detailing, among other things, the objectives of the trial, inclusion and exclusion criteria, the parameters to be used in monitoring safety and the effectiveness criteria to be

evaluated. Each protocol, and any subsequent material amendment to the protocol, must be submitted to the FDA as part of the IND, and progress reports detailing the status of the clinical trials must be submitted to the FDA annually.

Human clinical trials are typically conducted in three sequential phases, but the phases may overlap or be combined. Additional studies may also be required after approval.

Phase 1 clinical trials are initially conducted in a limited population, which may be healthy volunteers or subjects with the target disease, to test the product candidate for safety, including adverse effects, dose tolerance, absorption, metabolism, distribution, excretion and pharmacodynamics in healthy humans or in patients. During Phase 1 clinical trials, information about the product candidate's pharmacokinetics and pharmacological effects may be obtained.

Phase 2 clinical trials are generally conducted in a patient population to identify possible adverse effects and safety risks, evaluate the efficacy of the product candidate for specific targeted indications and determine dose tolerance and optimal dosage. Multiple Phase 2 clinical trials may be conducted by the sponsor to obtain information prior to beginning larger and more costly Phase 3 clinical trials. Phase 2 clinical trials are typically well-controlled and closely monitored.

Phase 3 clinical trials proceed if the prior clinical trials demonstrate that the product candidate is potentially effective and has an acceptable safety profile. Phase 3 clinical trials are undertaken in the proposed patient population to, provide evidence of clinical efficacy and safety. A well-controlled, statistically robust Phase 3 clinical trial is designed to deliver the data that regulatory authorities will use to decide whether or not to approve, and, if approved, how to appropriately label a new biologic product. Such Phase 3 clinical trials are referred to as "pivotal" trials.

A clinical trial may combine the elements of more than one phase and the FDA often requires more than one Phase 3 trial to support marketing approval of a product candidate. A company's designation of a clinical trial as being of a particular phase is not necessarily indicative that the study will be sufficient to satisfy the FDA requirements of that phase because this determination cannot be made until the protocol and data have been submitted to and reviewed by the FDA. Moreover, as noted above, a pivotal trial is a clinical trial that is believed to satisfy FDA requirements for the evaluation of a product candidate's safety and efficacy such that it can be used, alone or with other pivotal or non-pivotal trials, to support regulatory approval. Generally, pivotal trials are Phase 3 trials, but they may be Phase 2 trials if the design provides a well-controlled and reliable assessment of clinical benefit, particularly in an area of unmet medical need or rare patient population.

In December 2022, with the passage of the Food and Drug Omnibus Reform Act, or FDORA, Congress required sponsors to develop and submit a diversity action plan for each Phase 3 clinical trial or any other "pivotal study" of a new drug or biological product. These plans are meant to encourage the enrollment of more diverse patient populations in late-stage clinical trials of FDA-regulated products. Specifically, action plans must include the sponsor's goals for enrollment, the underlying rationale for those goals, and an explanation of how the sponsor intends to meet them.

In some cases, the FDA may approve an NDA or BLA for a product candidate but require the sponsor to conduct additional clinical trials to further assess the product candidate's safety and effectiveness after approval. Such post-approval trials, typically referred to as post-marketing studies or clinical trials, may be conducted after initial marketing approval. These trials are used to either gain additional experience from the treatment of a larger number of patients in the intended treatment group or to evaluate a specific outcome of interest (safety or efficacy). In certain instances, the FDA may mandate the performance of post-marketing studies or clinical trials, such as to verify clinical benefit in the case of products approved under accelerated approval regulations. Failure to exhibit due diligence with regard to conducting mandatory post-marketing studies or clinical trials could result in withdrawal of FDA approval for products.

In June 2023, the FDA issued draft guidance with updated recommendations for GCPs aimed at modernizing the design and conduct of clinical trials. The updates are intended to help pave the way for more efficient clinical trials to facilitate the development of medical products. The draft guidance is adopted from the International Council for Harmonisation's recently updated E6(R3) draft guideline that was developed to enable the incorporation of rapidly

developing technological and methodological innovations into the clinical trial enterprise. In addition, the FDA issued draft guidance outlining recommendations for the implementation of decentralized clinical trials.

Finally, sponsors of clinical trials are required to register and disclose certain clinical trial information on a public registry (clinicaltrials.gov) maintained by the U.S. National Institutes of Health, or NIH. In particular, information related to the product, patient population, phase of investigation, study sites and investigators and other aspects of the clinical trial is made public as part of the registration of the clinical trial. The NIH's Final Rule on registration and reporting requirements for clinical trials became effective in 2017. Although the FDA has historically not enforced these reporting requirements due to a long delay by the Department of Health and Human Services, or HHS, in issuing final implementing regulations, the FDA has issued several pre-notices for voluntary corrective action and several notices of non-compliance during the past two years. While these notices of non-compliance did not result in civil monetary penalties, the failure to submit clinical trial information to clinicaltrials.gov, as required, is a prohibited act under the FDCA with violations subject to potential civil monetary penalties of up to $10,000 for each day the violation continues.

Clinical Studies Outside of the United States in Support of FDA Approval

In connection with the clinical development of our programs, we may conduct trials at sites outside of the United States. When a foreign clinical study is conducted under an IND, all IND requirements must be met unless waived. When a foreign clinical study is not conducted under an IND, the sponsor must ensure that the study complies with certain regulatory requirements of the FDA in order to use the study as support for an IND or application for marketing approval. Specifically, the studies must be conducted in accordance with GCPs, including undergoing review and receiving approval by an independent ethics committee and seeking and receiving informed consent from subjects. GCP requirements encompass both ethical and data integrity standards for clinical studies. The FDA's regulations are intended to help ensure the protection of human subjects enrolled in non-IND foreign clinical studies, as well as the quality and integrity of the resulting data. They further help ensure that non-IND foreign studies are conducted in a manner comparable to that required for IND studies.

The acceptance by the FDA of study data from clinical trials conducted outside the United States in support of United States approval may be subject to certain conditions or may not be accepted at all. In cases where data from foreign clinical trials are intended to serve as the sole basis for marketing approval in the United States, the FDA will generally not approve the application on the basis of foreign data alone unless (i) the data are applicable to the U.S. population and U.S. medical practice; (ii) the trials were performed by clinical investigators of recognized competence and pursuant to GCP regulations; and (iii) the data may be considered valid without the need for an on-site inspection by the FDA, or if the FDA considers such inspection to be necessary, the FDA is able to validate the data through an on-site inspection or other appropriate means.

In addition, even where the foreign study data are not intended to serve as the sole basis for approval, the FDA will not accept the data as support for an application for marketing approval unless the study is well-designed and well-conducted in accordance with GCP requirements and the FDA is able to validate the data from the study through an onsite inspection if deemed necessary. Many foreign regulatory authorities have similar approval requirements. In addition, such foreign trials are subject to the applicable local laws of the foreign jurisdictions where the trials are conducted.

Interactions with the FDA During the Clinical Development Program

Following the clearance of an IND and the commencement of clinical trials, a sponsor is given the opportunity to meet with the FDA at certain points in the clinical development program. There are five types of meetings that occur between sponsors and the FDA. Type A meetings are those that are necessary for an otherwise stalled product development program to proceed or to address an important safety issue. Type B meetings include pre-IND and pre-NDA meetings as well as end of phase meetings such as end of Phase 2, or EOP2, meetings. A Type C meeting is any meeting other than a Type A or Type B meeting regarding the development and review of a product. A Type D meeting is focused on a narrow set of issues and should not require input from more than three disciplines or divisions. Finally, INTERACT meetings are intended for novel products and development programs that present unique challenges in the early development of an investigational product.

The FDA has indicated that its responses, as conveyed in meeting minutes and advice letters, only constitute mere recommendations and/or advice made to a sponsor and, as such, sponsors are not bound by such recommendations and/or advice. Nonetheless, from a practical perspective, a sponsor's failure to follow the FDA's recommendations for design of a clinical program may put the program at significant risk of failure.

Gene Therapy Products

We expect that the procedures and standards applied to gene therapy products will be applied to any product candidates we may develop. The FDA has defined a gene therapy product as one that seeks to modify or manipulate the expression of a gene or to alter the biological properties of living cells for therapeutic use. The products may be used to modify cells in vivo or transferred to cells ex vivo prior to administration to the recipient. Within the FDA, the Center for Biologics Evaluation and Research, or CBER, regulates gene therapy products. Within CBER, the review of gene therapy and related products is consolidated in the Office of Therapeutic Products, or OTP.

The FDA has issued various guidance documents regarding gene therapies. Although the FDA has indicated that these and other guidance documents it previously issued are not legally binding, the guidance documents provide the FDA's current thinking on, among other things: the proper preclinical assessment of gene therapies; the chemistry, manufacturing and control information that should be included in an IND application; the proper design of tests to measure product potency in support of an IND or BLA application; and measures to observe for potential delayed adverse effects in participants who have received investigational gene therapies with the duration of follow-up based on the potential for risk of such effects. For AAV vectors specifically, the FDA typically recommends that sponsors continue to monitor participants for potential gene therapy-related adverse events for up to a five-year period.

Manufacturing and Other Regulatory Requirements

Concurrently with clinical trials, sponsors usually complete additional animal safety studies, develop additional information about the chemistry and physical characteristics of the product candidate and finalize a process for manufacturing commercial quantities of the product candidate in accordance with cGMP requirements. The manufacturing process must be capable of consistently producing quality batches of the product candidate and, among other criteria, the sponsor must develop methods for testing the identity, strength, quality, and purity of the finished product. Additionally, appropriate packaging must be selected and tested, and stability studies must be conducted to demonstrate that the product candidate does not undergo unacceptable deterioration over its shelf life.

Specifically, the FDA's regulations require that pharmaceutical products be manufactured in specific approved facilities and in accordance with cGMPs. The cGMP regulations include requirements relating to organization of personnel, buildings and facilities, equipment, control of components and product containers and closures, production and process controls, packaging and labeling controls, holding and distribution, laboratory controls, records and reports and returned or salvaged products. Manufacturers and other entities involved in the manufacture and distribution of approved pharmaceuticals are required to register their establishments with the FDA and some state agencies and are subject to periodic unannounced inspections by the FDA for compliance with cGMPs and other requirements. The PREVENT Pandemics Act, which was enacted in December 2022, clarifies that foreign drug manufacturing establishments are subject to registration and listing requirements even if a drug or biologic undergoes further manufacture, preparation, propagation, compounding, or processing at a separate establishment outside of the United States prior to being imported or offered for import into the United States. Inspections must follow a "risk-based schedule" that may result in certain establishments being inspected more frequently. Manufacturers may also have to provide, on request, electronic or physical records regarding their establishments. Delaying, denying, limiting, or refusing inspection by the FDA may lead to a product being deemed to be adulterated. Changes to the manufacturing process, specifications or container closure system for an approved product are strictly regulated and often require prior FDA approval before being implemented. The FDA's regulations also require, among other things, the investigation and correction of any deviations from cGMP and the imposition of reporting and documentation requirements upon the sponsor and any third-party manufacturers involved in producing the approved product.

Pediatric Studies

Under the Pediatric Research Equity Act of 2003, or PREA, a BLA or supplement thereto must contain data that are adequate to assess the safety and effectiveness of the product for the claimed indications in all relevant pediatric subpopulations and to support dosing and administration for each pediatric subpopulation for which the product is safe and effective. The sponsor must submit an initial pediatric study plan within 60 days of an EOP2 meeting or as may be agreed between the sponsor and the FDA. Sponsors must also submit pediatric study plans prior to the assessment data. Those plans must contain an outline of the proposed pediatric study or studies the sponsor plans to conduct, including study objectives and design, any deferral or waiver requests, and other information required by regulation. The sponsor, the FDA, and the FDA's internal review committee must then review the information submitted, consult with each other, and agree upon a final plan. The FDA or the sponsor may request an amendment to the plan at any time.

The FDA may, on its own initiative or at the request of the sponsor, grant deferrals for submission of some or all pediatric data until after approval of the product for use in adults, or full or partial waivers from the pediatric data requirements. A deferral may be granted for several reasons, including a finding that the product or therapeutic candidate is ready for approval for use in adults before pediatric trials are complete or that additional safety or effectiveness data needs to be collected before the pediatric trials begin. The FDA is required to send a PREA non-compliance letter to sponsors who have failed to submit their pediatric assessments required under PREA, have failed to seek or obtain a deferral or deferral extension or have failed to request approval for a required pediatric formulation. Unless otherwise required by regulation, the pediatric data requirements generally do not apply to products with orphan designation, although the FDA has taken steps to limit what is considers abuse of this statutory exemption in the PREA by announcing that is does not intend to grant any additional orphan drug designations for rare pediatric subpopulations of what is otherwise a common disease. In May 2023, the FDA issued new draft guidance that further describes the pediatric study requirements under PREA.

Submission of a BLA to the FDA

FDA approval is required before any new biologic product can be marketed in the United States. Thus, assuming successful completion of all required preclinical and human testing in accordance with all applicable regulatory requirements, detailed product information is submitted to the FDA in the form of a BLA. Under the Prescription Drug User Fee Act, or PDUFA, each BLA must be accompanied by a significant user fee unless an exception or waiver applies, such as the first application filed by a small business or BLAs for product candidates designated as orphan drugs, unless the product candidate includes an indication that is not for a rare disease or condition. For federal fiscal year 2024, the application user fee is $4,048,695 for an application requiring clinical data, and the sponsor of a licensed BLA is subject to an annual program fee, which for fiscal year 2024 is more than $416,734.

The FDA conducts a preliminary review of all applications within 60 days of receipt and must inform the sponsor at that time or before whether an application is sufficiently complete to permit substantive review. In pertinent part, FDA's regulations state that an application "shall not be considered as filed until all pertinent information and data have been received" by the FDA. In the event the FDA determines that an application does not satisfy this standard, it will issue a Refuse to File, or RTF, determination to the sponsor. In this event, the BLA must be resubmitted.

If the submission is accepted for filing, the FDA's goal is to review the BLA, within ten months for a standard review, or, if the BLA relates to an unmet medical need in the treatment of a serious or life-threatening condition, perform a priority review, within six months. The review process may be extended by the FDA for three additional months to consider new information or in the case of a clarification provided by the sponsor to address an outstanding deficiency identified by the FDA following the original submission. Despite these review goals, it is not uncommon for FDA review of an application to extend beyond the PDUFA target action date.

The FDA may refer applications for novel biological products or biological products that present difficult questions of safety or efficacy to an advisory committee, typically a panel that includes clinicians and other experts, for review, evaluation and a recommendation as to whether the application should be approved and under what conditions. The FDA is not bound by the recommendations of an advisory committee, but it considers such recommendations carefully when making decisions. During the biological product approval process, the FDA also will determine whether a

REMS is necessary to assure the safe use of the biological product. If the FDA concludes a REMS is needed, the sponsor of the BLA must submit a proposed REMS; the FDA will not approve the BLA without a REMS, if required.

In connection with its review of a BLA, the FDA will inspect the facilities at which the product is manufactured. The FDA will not approve the product unless it determines that the manufacturing processes and facilities are in compliance with cGMP requirements and adequate to assure consistent production of the product within required specifications. Additionally, the FDA will typically inspect one or more clinical trial sites to assure that the clinical trials were conducted in compliance with IND study requirements and GCP requirements. With passage of FDORA, Congress clarified the FDA's authority to conduct inspections by expressly permitting inspection of facilities involved in the preparation, conduct, or analysis of clinical and non-clinical studies submitted to the FDA as well as other persons holding study records or involved in the study process. To assure cGMP and GCP compliance, a sponsor must incur significant expenditure of time, money and effort in the areas of training, record keeping, production, and quality control.

The FDA's Decision on a BLA

The FDA reviews an application to determine, among other things, whether the product is safe, pure, and potent. To reach this determination, the FDA must determine that the investigational product is effective and that its expected benefits outweigh its potential risks to patients. This "benefit-risk" assessment is informed by the extensive body of evidence about the product's safety, purity and potency in the BLA. This assessment is also informed by other factors, including: the severity of the underlying condition and how well patients' medical needs are addressed by currently available therapies; uncertainty about how the premarket clinical trial evidence will extrapolate to real-world use of the product in the post-market setting; and whether risk management tools are necessary to manage specific risks.

The FDA typically requires a robust safety database and two adequate and well-controlled clinical investigations to establish the efficacy of a new product. Under certain circumstances, however, FDA has indicated that a single trial with certain characteristics and additional information may satisfy this standard. The FDA issued draft guidance in September 2023 that outlines considerations for relying on confirmatory evidence in lieu of a second clinical trial to demonstrate efficacy.

After evaluating the application and all related information, including the advisory committee recommendations, if any, and inspection reports of manufacturing facilities and clinical trial sites, the FDA will issue either a Complete Response Letter, or CRL, or an approval letter. A CRL indicates that the review cycle of the application is complete, and the application will not be approved in its present form. A CRL generally outlines the deficiencies in the submission and may require substantial additional testing or information in order for the FDA to reconsider the application. The CRL may require additional clinical or other data, additional pivotal Phase 3 clinical trial(s) and/or other significant and time-consuming requirements related to clinical trials, preclinical studies or manufacturing. If a CRL is issued, the sponsor will have one year to respond to the deficiencies identified by the FDA, at which time the FDA can deem the application withdrawn or, in its discretion, grant the sponsor an additional six-month extension to respond. For those seeking to challenge the FDA's CRL decision, the FDA has indicated that sponsors may request a formal hearing on the CRL or they may file a request for reconsideration or a request for a formal dispute resolution. During the product approval process, the FDA also will determine whether a REMS is necessary to assure the safe use of the product. If the FDA concludes a REMS is needed, the sponsor of the BLA must submit a proposed REMS; the FDA will not approve the BLA without a REMS, if required.

An approval letter, on the other hand, authorizes commercial marketing of the product with specific prescribing information for specific indications. That is, the approval will be limited to the conditions of use (e.g., patient population, indication) described in the FDA-approved labeling. Further, depending on the specific risk(s) to be addressed, the FDA may require that contraindications, warnings or precautions be included in the product labeling, require that post-approval trials, including post-marketing studies or clinical trials, be conducted to further assess a product's safety after approval, require testing and surveillance programs to monitor the product after commercialization or impose other conditions, including distribution and use restrictions or other risk management mechanisms under a REMS which can materially affect the potential market and profitability of the product. The FDA may prevent or limit further marketing of a product based on the results of post-marketing studies, clinical trials, or surveillance programs. After approval, some

types of changes to the approved product, such as adding new indications, manufacturing changes and additional labeling claims, are subject to further testing requirements and FDA review and approval.

Post-Approval Requirements

Biologic products manufactured or distributed pursuant to regulatory approvals are subject to pervasive and continuing regulation by the regulatory authorities, including, among other things, requirements relating to formal commitments for post approval clinical trials and studies, manufacturing, recordkeeping, periodic reporting, product sampling and distribution, marketing, labeling, advertising and promotion and reporting of adverse experiences with the product. After approval, most changes to the approved product, such as adding new indications or other labeling claims, are subject to prior regulatory authority review and approval.

Manufacturers are subject to periodic unannounced inspections by regulatory authorities and country or state agencies for compliance with cGMP and other requirements. Changes to the manufacturing process are strictly regulated, and, depending on the significance of the change, may require prior regulatory approval before being implemented. Regulations also require investigation and correction of any deviations from cGMP and impose reporting and documentation requirements upon us and any third-party manufacturers that we may decide to use. Accordingly, manufacturers must continue to expend time, money and effort in the area of production and quality control to maintain compliance with cGMP and other aspects of regulatory compliance.

Further, although physicians may prescribe legally available products for unapproved uses or patient populations, which are commonly referred to as “off-label uses,” manufacturers may not market or promote such uses. The FDA and other agencies actively enforce the laws and regulations prohibiting the promotion of off-label uses, and a company that is found to have improperly promoted off-label uses may be subject to significant liability. In September 2021, the FDA published final regulations which describe the types of evidence that the FDA will consider in determining the intended use of a biologic. If a company is found to have promoted off-label uses, it may become subject to administrative and judicial enforcement by the FDA, the Department of Justice, or the Office of the Inspector General of the HHS, as well as state authorities.

It may be permissible, under very specific, narrow conditions, for a manufacturer to engage in nonpromotional, non-misleading communication regarding off-label information, such as distributing scientific or medical journal information. Moreover, with passage of the Pre-Approval Information Exchange Act, or PIE Act, in December 2022, sponsors of products that have not been approved may proactively communicate to payors certain information about products in development to help expedite patient access upon product approval. Previously, such communications were permitted under FDA guidance, but the new legislation explicitly provides protection to sponsors who convey certain information about products in development to payors, including unapproved uses of approved products. In addition, in October 2023, the FDA published draft guidance outlining its non-binding policies governing the distribution of scientific information on unapproved uses to healthcare providers. This draft guidance calls for such communications to be truthful, non-misleading, factual, and unbiased and include all information necessary for healthcare providers to interpret the strengths and weaknesses and validity and utility of the information about the unapproved use.

Expedited Review Programs

The FDA is authorized to expedite the review of applications in several ways. None of these expedited programs, however, changes the standards for approval but each may help expedite the development or approval process governing product candidates.

- *Fast Track Designation*. Candidate products are eligible for Fast Track designation if they are intended to treat a serious or life-threatening condition and demonstrate the potential to address unmet medical needs for the condition. Fast Track designation applies to the combination of the product candidate and the specific indication for which it is being studied. In addition to other benefits, such as the ability to have greater interactions with the FDA, the FDA may initiate review of sections of a Fast Track application before the application is complete, a process known as rolling review.

- *Breakthrough therapy designation*. To qualify for the breakthrough therapy program, product candidates must be intended to treat a serious or life-threatening disease or condition and preliminary clinical evidence must indicate that such product candidates may demonstrate substantial improvement on one or more clinically significant endpoints over existing therapies. The FDA will seek to ensure the sponsor of a breakthrough therapy product candidate receives intensive guidance on an efficient development program, intensive involvement of senior managers and experienced staff on a proactive, collaborative and cross-disciplinary review and rolling review.

- *Priority review*. A product candidate is eligible for priority review if it treats a serious condition and, if approved, it would be a significant improvement in the safety or effectiveness of the treatment, diagnosis or prevention compared to marketed products. FDA aims to complete its review of priority review applications within six months as opposed to ten months for standard review.

- *Accelerated approval*. Biologic products studied for their safety and effectiveness in treating serious or life-threatening illnesses and that provide meaningful therapeutic benefit over existing treatments may receive accelerated approval. Accelerated approval means that a product candidate may be approved on the basis of adequate and well controlled clinical trials establishing that the product candidate has an effect on a surrogate endpoint that is reasonably likely to predict a clinical benefit, or on the basis of an effect on a clinical endpoint other than survival or irreversible morbidity or mortality or other clinical benefit, taking into account the severity, rarity and prevalence of the condition and the availability or lack of alternative treatments. As a condition of approval, the FDA may require that a sponsor of a biologic product candidate receiving accelerated approval perform adequate and well controlled post-marketing studies or clinical trials. In addition, the FDA currently requires as a condition for accelerated approval pre-approval of promotional materials.

- With the passage of FDORA in December 2022, Congress modified certain provisions governing accelerated approval of drug and biologic products. Specifically, the new legislation authorized the FDA to: require a sponsor to have its confirmatory clinical trial underway before accelerated approval is awarded, require a sponsor of a product granted accelerated approval to submit progress reports on its post-approval studies to the FDA every six months until the study is completed; and use expedited procedures to withdraw accelerated approval of an NDA or BLA after the confirmatory trial fails to verify the product candidate's clinical benefit. Further, FDORA requires the FDA to publish on its website "the rationale for why a post-approval study is not appropriate or necessary" whenever it decides not to require such a study upon granting accelerated approval. In March 2023, the FDA issued draft guidance that outlines its current thinking and approach to accelerated approval for designing, conducting, and analyzing data for trials intended to support accelerated approvals of oncology therapeutics.

- *Regenerative advanced therapy*. With the passage of the 21st Century Cures Act, or the Cures Act, in December 2016, Congress authorized the FDA to accelerate review and approval of products designated as regenerative advanced therapies. A product is eligible for this designation if it is a regenerative medicine therapy that is intended to treat, modify, reverse or cure a serious or life-threatening disease or condition and preliminary clinical evidence indicates that the product candidate has the potential to address unmet medical needs for such disease or condition. The benefits of a regenerative advanced therapy designation include early interactions with the FDA to expedite development and review, benefits available to breakthrough therapies, potential eligibility for priority review and accelerated approval based on surrogate or intermediate endpoints.

U.S. Orphan Drug Designation and Exclusivity

A product may qualify for orphan drug designation, or ODD, under the Orphan Drug Act, if it is intended to treat a rare disease or condition, which is generally a disease or condition that affects fewer than 200,000 individuals in the United States, or more than 200,000 individuals in the United States and for which there is no reasonable expectation that the cost of developing and making a drug or biological product available in the United States for this type of disease or condition will be recovered from sales of the product as stipulated in the ODD. ODD must be requested before

submitting a BLA. After the FDA grants ODD, the identity of the therapeutic agent and its potential orphan use are disclosed publicly by the FDA. ODD entitles a party to financial incentives such as opportunities for grant funding towards clinical study costs, tax advantages, and user-fee waivers. ODD does not convey any advantage in or shorten the duration of the regulatory review and approval process.

If a product that has ODD receives the first FDA approval for the disease or condition for which it has such designation, the product is entitled to orphan product exclusivity, which means that the FDA may not approve any other applications to market the same product for the same indication for seven years, except in limited circumstances, such as not being able to supply the product for patients or showing clinical superiority to the product with orphan exclusivity. Competitors, however, may receive approval of a different therapy for the indication for which the orphan product has exclusivity or obtain approval for the same product but for a different indication for which the orphan product has exclusivity. Orphan-drug exclusivity also could block the approval of one of our products for seven years if a competitor obtains approval of the same therapy as defined by the FDA.

In September 2021, the Court of Appeals for the Eleventh Circuit held that, for the purpose of determining the scope of market exclusivity, the term “same disease or condition” in the statute means the designated “rare disease or condition” and could not be interpreted by the FDA to mean the “indication or use.” Thus, the court concluded, orphan drug exclusivity applies to the entire designated disease or condition rather than the “indication or use.” Although there have been legislative proposals to overrule this decision, they have not been enacted into law. On January 23, 2023, the FDA announced that, in matters beyond the scope of that court order, the FDA will continue to apply its existing regulations tying orphan-drug exclusivity to the uses or indications for which the orphan drug was approved.

Priority Review Vouchers

A priority review voucher, or PRV, is a voucher that the FDA issues to a sponsor of a rare pediatric disease or tropical disease product application at the time of the marketing application approval. Vouchers are transferable to other sponsors that may apply it to their NDAs or BLAs. A PRV entitles the holder to designate a single human drug application submitted under Section 505(b)(1) of the FDCA or Section 351 of the PHSA as qualifying for a priority review. An FDA priority review may expedite the review process of a marketing application reducing the review time from ten months after formal acceptance of the file to six months after formal acceptance of the file. Applying the PRV to a marketing application does not ensure the FDA’s approval of the marketing application and all requirements supporting the safety and efficacy of the product must be met. Orphan drug products are eligible for Rare Pediatric Disease Designation if greater than 50% of patients living with the disease are under age 18.

Biosimilars and Exclusivity

When a biological product is licensed for marketing by FDA with approval of a BLA, the product may be entitled to certain types of market and data exclusivity barring FDA from approving competing products for certain periods of time. In March 2010, the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act of 2010, or collectively the ACA, was enacted in the United States and included the Biologics Price Competition and Innovation Act of 2009, or the BPCIA. The BPCIA amended the PHSA to create an abbreviated approval pathway for biological products that are biosimilar to or interchangeable with an FDA-licensed reference biological product. To date, the FDA has approved both biosimilar products and interchangeable biosimilar products. In December 2022, Congress clarified through FDORA that the FDA may approve multiple first interchangeable biosimilar biological products so long as the products are all approved on the first day on which such a product is approved as interchangeable with the reference product.

A reference biologic is granted twelve years of exclusivity from the time of first licensure of the reference product. Approval of a 351(k) application may not be made effective until twelve years after the date of first licensure of the reference product, which under the statute excludes the date of licensure of supplements and certain other applications. Additionally, a 351(k) application for a biosimilar or interchangeable biological product cannot be submitted for review until four years after the date on which the reference product was first licensed under section 351(a) of the PHSA. Even if a product is considered to be a reference product eligible for exclusivity, however, another company could market a competing version of that product if the FDA approves a full BLA for such product containing

the sponsor's own preclinical data and data from adequate and well controlled clinical trials to demonstrate the safety, purity, and potency of their product. There have been recent government proposals to reduce the twelve-year reference product exclusivity period, but none has been enacted to date. At the same time, since passage of the BPCIA, many states have passed laws or amendments to laws, which address pharmacy practices involving biosimilar products.

Pediatric Exclusivity

Pediatric exclusivity is a type of non-patent marketing exclusivity in the United States and, if granted, provides for the attachment of an additional six months of exclusivity. For biologic products, the six-month period may be attached to any existing regulatory exclusivities but not to any patent terms. The conditions for pediatric exclusivity include the FDA's determination that information relating to the use of a new product in the pediatric population may produce health benefits in that population, the FDA making a written request for pediatric clinical trials, and the sponsor agreeing to perform, and reporting on, the requested clinical trials within the statutory timeframe. This six-month exclusivity may be granted if an NDA sponsor submits pediatric data that fairly respond to a written request from the FDA for such data. The data does not need to show the product to be effective in the pediatric population studied; rather, if the clinical trial is deemed to fairly respond to the FDA's request, the additional protection is granted. If reports of requested pediatric studies are submitted to and accepted by the FDA within the statutory time limits, whatever statutory or regulatory periods of exclusivity that cover the product are extended by six months.

U.S. Patent Term Restoration

Depending upon the timing, duration and specifics of the FDA approval of the use of our product candidates, some of our U.S. patents may be eligible for limited patent term extension under the Drug Price Competition and Patent Term Restoration Act of 1984, commonly referred to as the Hatch-Waxman Amendments. The Hatch-Waxman Amendments permit a patent restoration term of up to five years as compensation for patent term lost during product development and the FDA regulatory review process. However, patent term restoration cannot extend the remaining term of a patent beyond a total of 14 years from the product's approval date. The patent term restoration period is generally one-half the time between the effective date of an IND and the submission date of a BLA plus the time between the submission date of a BLA and the approval of that application, less any time the sponsor failed to act with due diligence. Only one patent applicable to an approved biological product is eligible for the extension and the application for the extension must be submitted prior to the expiration of the patent. The U.S. Patent and Trademark Office, in consultation with the FDA, reviews and approves the application for any patent term extension or restoration. In the future, we may intend to apply for restoration of patent term for one of our currently owned or licensed patents to add patent life beyond its current expiration date, depending on the expected length of the clinical trials and other factors involved in the filing of the relevant BLA.

Other Healthcare Laws

Although we currently do not have any products on the market, we will be subject to additional healthcare regulation and enforcement by the federal government and by authorities in the states in which we conduct our business, if and when our product candidates are approved by the FDA and subject to federal healthcare reimbursement. Such laws include, without limitation, state and federal anti-kickback, fraud and abuse, false claims, privacy and security and physician sunshine laws and regulations. In addition, the U.S. Foreign Corrupt Practices Act, to which we are subject, prohibits corporations and individuals from engaging in certain activities to obtain or retain business or to influence a person working in an official capacity. It is illegal to pay, offer to pay or authorize the payment of anything of value to any foreign government official, government staff member, political party or political candidate in an attempt to obtain or retain business or to otherwise influence a person working in an official capacity. If our operations are found to be in violation of any of such laws or any other governmental regulations that apply to us, we may be subject to penalties, including, without limitation, administrative, civil and criminal penalties, damages, fines, disgorgement, the curtailment or restructuring of our operations, exclusion from participation in federal and state healthcare programs and imprisonment, any of which could adversely affect our ability to operate our business and our financial results.

Healthcare Reform

A primary trend in the U.S. healthcare industry and elsewhere is cost containment. There have been a number of federal and state proposals during the last few years regarding the pricing of biologic products, limiting coverage and reimbursement for medical products and other changes to the healthcare system in the United States. In March 2010, the United States Congress enacted the ACA, which, among other things, includes changes to the coverage and payment for pharmaceutical products under government healthcare programs. The ACA is intended to broaden access to health insurance, reduce or constrain the growth of healthcare spending, enhance remedies against fraud and abuse, add transparency requirements for the healthcare and health insurance industries, impose taxes and fees on the health industry and impose additional health policy reforms. Since enactment of the ACA, there have been, and continue to be, numerous legal challenges and Congressional actions to repeal and replace provisions of the law. Litigation and legislation over the ACA are likely to continue, with unpredictable and uncertain results.

In March 2010, the United States Congress enacted the ACA, which, among other things, includes changes to the coverage and payment for products under government healthcare programs. Other legislative changes have been proposed and adopted in the United States since the ACA was enacted. For example, in August 2011, the Budget Control Act of 2011, among other things, created measures for spending reductions by Congress. A Joint Select Committee on Deficit Reduction, tasked with recommending a targeted deficit reduction of at least $1.2 trillion for the years 2012 through 2021, was unable to reach required goals, thereby triggering the legislation's automatic reduction to several government programs. This includes aggregate reductions of Medicare payments to providers of up to 2% per fiscal year, which will remain in effect through 2031 pursuant to the Coronavirus Aid, Relief and Economic Security Act, or CARES Act.

The American Taxpayer Relief Act of 2012, which was enacted in January 2013, among other things, further reduced Medicare payments to several providers, including hospitals, imaging centers, and cancer treatment centers, and increased the statute of limitations period for the government to recover overpayments to providers from three to five years. These laws may result in additional reductions in Medicare and other healthcare funding and otherwise affect the prices we may obtain for any of our product candidates for which we may obtain regulatory approval or the frequency with which any such product candidate is prescribed or used. Indeed, under current legislation, the actual reductions in Medicare payments may vary up to 4%.

Since enactment of the ACA, there have been, and continue to be, numerous legal challenges and Congressional actions to repeal and replace provisions of the law. For example, with enactment of the Tax Cuts and Jobs Act of 2017, or the TCJA, which was signed by President Trump on December 22, 2017, Congress repealed the "individual mandate." The repeal of this provision, which requires most Americans to carry a minimal level of health insurance, became effective in 2019. Further, on June 17, 2021, the United States Supreme Court dismissed a challenge to the ACA after finding that the plaintiffs do not have standing to challenge the constitutionality of the ACA. Litigation and legislation over the ACA are likely to continue, with unpredictable and uncertain results.

Although the Trump administration took executive actions to undermine or delay implementation of the ACA, those actions were rescinded with issuance of an Executive Order on January 28, 2021 by President Biden, which directs federal agencies to reconsider rules and other policies that limit Americans' access to health care, and consider actions that will protect and strengthen that access. Under this Executive Order, federal agencies are directed to re-examine: policies that undermine protections for people with pre-existing conditions, including complications related to COVID-19; demonstrations and waivers under Medicaid and the ACA that may reduce coverage or undermine the programs, including work requirements; policies that undermine the Health Insurance Marketplace or other markets for health insurance; policies that make it more difficult to enroll in Medicaid and the ACA; and policies that reduce affordability of coverage or financial assistance, including for dependents.

Pharmaceutical Price Reform

The prices of prescription pharmaceuticals have also been the subject of considerable discussion in the United States. There have been several recent United States congressional inquiries, as well as proposed and enacted state and federal legislation designed to, among other things, bring more transparency to pharmaceutical pricing, review the

relationship between pricing and manufacturer patient programs, and reduce the costs of pharmaceuticals under Medicare and Medicaid. In 2020, President Trump issued several executive orders intended to lower the costs of prescription products and certain provisions in these orders have been incorporated into regulations. These regulations include an interim final rule implementing a most favored nation model for prices that would tie Medicare Part B payments for certain physician-administered pharmaceuticals to the lowest price paid in other economically advanced countries, effective January 1, 2021. That rule, however, has been subject to a nationwide preliminary injunction and, on December 29, 2021, the Centers for Medicare & Medicaid Services, or CMS, issued a final rule to rescind it. With issuance of this rule, CMS stated that it will explore all options to incorporate value into payments for Medicare Part B pharmaceuticals and improve beneficiaries' access to evidence-based care.

In addition, the HHS and the FDA published a final rule allowing states and other entities to develop a Section 804 Importation Program, or SIP, to import certain prescription drugs from Canada into the United States. That regulation was challenged in a lawsuit by the Pharmaceutical Research and Manufacturers of America, or PhRMA, but the case was dismissed by a federal district court in February 2023 after the court found that PhRMA did not have standing to sue HHS. Nine states (Colorado, Florida, Maine, New Hampshire, New Mexico, North Dakota, Texas, Vermont, and Wisconsin) have passed laws allowing for the importation of drugs from Canada. Certain of these states have submitted Section 804 Importation Program proposals and are awaiting FDA approval. On January 5, 2024, the FDA approved Florida's plan for Canadian drug importation.

Further, on November 20, 2020, HHS finalized a regulation removing safe harbor protection for price reductions from pharmaceutical manufacturers to plan sponsors under Part D, either directly or through pharmacy benefit managers, unless the price reduction is required by law. The final rule would also eliminate the current safe harbor for Medicare drug rebates and create new safe harbors for beneficiary point-of-sale discounts and pharmacy benefit manager service fees. The Inflation Reduction Act of 2022, or IRA, further delayed implementation of this rule to January 1, 2032.

On July 9, 2021, President Biden signed Executive Order 14063, which focuses on, among other things, the price of pharmaceuticals. To address these costs, the executive order directs HHS to create a plan within 45 days to combat "excessive pricing of prescription drugs and enhance domestic pharmaceutical supply chains, to reduce the prices paid by the federal government for such drugs, and to address the recurrent problem of price gouging." Thereafter, on September 9, 2021, HHS released its plan to reduce drug prices. The key features of that plan are to: (a) make drug prices more affordable and equitable for all consumers and throughout the health care system by supporting drug price negotiations with manufacturers; (b) improve and promote competition throughout the prescription drug industry by supporting market changes that strengthen supply chains, promote biosimilars and generic drugs, and increase transparency; and (c) foster scientific innovation to promote better healthcare and improve health by supporting public and private research and making sure that market incentives promote discovery of valuable and accessible new treatments.

On August 16, 2022, the IRA was signed into law by President Biden. The new legislation has implications for Medicare Part D, which is a program available to individuals who are entitled to Medicare Part A or enrolled in Medicare Part B to give them the option of paying a monthly premium for outpatient prescription drug coverage. Among other things, the IRA requires manufacturers of certain drugs to engage in price negotiations with Medicare (beginning in 2026), with prices that can be negotiated subject to a cap; imposes rebates under Medicare Part B and Medicare Part D to penalize price increases that outpace inflation (first due in 2023); and replaces the Part D coverage gap discount program with a new discounting program (beginning in 2025). The IRA permits the Secretary of the HHS to implement many of these provisions through guidance, as opposed to regulation, for the initial years.

Specifically, with respect to price negotiations, Congress authorized Medicare to negotiate lower prices for certain costly single-source drug and biologic products that do not have competing generics or biosimilars and are reimbursed under Medicare Part B and Part D. CMS may negotiate prices for ten high-cost drugs paid for by Medicare Part D starting in 2026, followed by 15 Part D drugs in 2027, 15 Part B or Part D drugs in 2028, and 20 Part B or Part D drugs in 2029 and beyond. This provision applies to drug products that have been approved for at least 9 years and biologics that have been licensed for 13 years, but it does not apply to drugs and biologics that have been approved for a single rare disease or condition. Further, the legislation subjects drug manufacturers to civil monetary penalties and a

potential excise tax for failing to comply with the legislation by offering a price that is not equal to or less than the negotiated “maximum fair price” under the law or for taking price increases that exceed inflation. The legislation also requires manufacturers to pay rebates for drugs in Medicare Part D whose price increases exceed inflation. The new law also caps Medicare out-of-pocket drug costs at an estimated $4,000 a year in 2024 and, thereafter beginning in 2025, at $2,000 a year.

On June 6, 2023, Merck & Co. filed a lawsuit against HHS and CMS asserting that, among other things, the IRA’s Drug Price Negotiation Program for Medicare constitutes an uncompensated taking in violation of the Fifth Amendment of the Constitution. Subsequently, a number of other parties, including the U.S. Chamber of Commerce, Bristol Myers Squibb Company, the Pharmaceutical Research and Manufacturers of America, Astellas, Novo Nordisk, Janssen Pharmaceuticals, Novartis, AstraZeneca and Boehringer Ingelheim, also filed lawsuits in various courts with similar constitutional claims against HHS and CMS. Litigation involving these and other provisions of the IRA will continue with unpredictable and uncertain results.

At the state level, individual states are increasingly aggressive in passing legislation and implementing regulations designed to control pharmaceutical and biological product pricing, including price or patient reimbursement constraints, discounts, restrictions on certain product access and marketing cost disclosure and transparency measures, and, in some cases, designed to encourage importation from other countries and bulk purchasing. In addition, regional health care authorities and individual hospitals are increasingly using bidding procedures to determine what pharmaceutical products and which suppliers will be included in their prescription product and other health care programs. These measures could reduce the ultimate demand for our products, once approved, or put pressure on our product pricing. We expect that additional state and federal healthcare reform measures will be adopted in the future, any of which could limit the amounts that federal and state governments will pay for healthcare products and services, which could result in reduced demand for our product candidates or additional pricing pressures.

Additional Regulation

In addition to the foregoing, state and federal laws regarding environmental protection and hazardous substances, including the Occupational Safety and Health Act, the Resource Conservancy and Recovery Act and the Toxic Substances Control Act, affect our business. These and other laws govern our use, handling and disposal of various biological, chemical and radioactive substances used in, and wastes generated by, our operations. If our operations result in contamination of the environment or expose individuals to hazardous substances, we could be liable for damages and governmental fines. We believe that we are in material compliance with applicable environmental laws and that continued compliance therewith will not have a material adverse effect on our business. We cannot predict, however, how changes in these laws may affect our future operations.

Government Regulation Outside of the United States

In addition to regulations in the United States, we will be subject to a variety of regulations in other jurisdictions governing, among other things, clinical trials and any commercial sales and distribution of our products. Because biologically sourced raw materials are subject to unique contamination risks, their use may be restricted in some countries.

Whether or not we obtain FDA approval for a product, we must obtain the requisite approvals from regulatory authorities in foreign countries prior to the commencement of clinical trials or marketing of the product in those countries. Certain countries outside of the United States have a similar process that requires the submission of a clinical trial application much like the IND prior to the commencement of human clinical trials in these countries.

If we fail to comply with applicable foreign regulatory requirements, we may be subject to, among other things, fines, suspension or withdrawal of regulatory approvals, product recalls, seizure of products, operating restrictions and criminal prosecution.

Our Corporate Information

We were incorporated under the laws of Delaware in June 2013. Our principal executive offices are located at 75 Hayden Avenue, Lexington, MA. Other operations, including laboratory space, are located at 64 Sidney Street, Cambridge, MA. We lease our office and laboratory space, which consist of approximately 26,148 square feet located in Cambridge, Massachusetts and 93,449 square feet located in Lexington, MA. Our lease in Cambridge expires in 2026 and our lease in Lexington expires in 2031.

Employees and Human Capital Resources

As of December 31, 2023, we employed 162 full-time employees in the United States, including 126 in research and development positions and 36 in general and administrative positions. Approximately 53 of our employees have either an MD or PhD degree. We have never had a work stoppage, and none of our employees is represented by a labor organization or under any collective-bargaining arrangements. We consider our employee relations to be positive.

Our human capital resources objectives include, as applicable, identifying, recruiting, retaining, incentivizing and integrating our existing and additional employees. The principal purposes of our equity incentive plans are to attract, retain and motivate our employees and directors and selected consultants through the granting of stock-based compensation awards.

Available Information

Our Internet address is http://www.voyagertherapeutics.com. We make available, free of charge, on or through our website our annual reports on Form 10-K, quarterly reports on Form 10-Q, current reports on Form 8-K, proxy statements and any amendments to those reports filed or furnished pursuant to Section 13(a) or 15(d) of the Securities and Exchange Act as soon as reasonably practicable after such material is electronically filed with or furnished to the Securities and Exchange Commission, or the SEC. The information on our website is not part of this Annual Report for the year ended December 31, 2023.

ITEM 1A. RISK FACTORS

The following risk factors and other information in this Annual Report on Form 10-K, including our financial statements and related notes thereto, should be carefully considered. The risks and uncertainties described below are not the only ones we face. Additional risks and uncertainties not presently known to us or that we presently deem less significant may also impair our business operations. Please see the discussion under the caption "Forward-Looking Statements" in this Annual Report on Form 10-K for a discussion of some of the forward-looking statements that are qualified by these risk factors. If any of the following risks occur, our business, financial condition, results of operations and future growth prospects could be materially and adversely affected.

Risks Related to Our Financial Position and Need for Capital

We have a history of incurring significant losses and anticipate that we will continue to incur losses for the foreseeable future and may never achieve or maintain consistent profitability.

We are an early-stage biotechnology company and have not yet generated revenues from the sales of our product candidates. All of our product candidates are in the early stages of development. Investment in biotechnology companies is highly speculative because it entails substantial upfront capital expenditures and significant risk that any product candidates will fail to be safe and efficacious, obtain regulatory approval or become commercially viable. We have not yet demonstrated the ability to complete any clinical trials of our product candidates, obtain marketing approvals, manufacture a commercial-scale product or conduct sales and marketing activities necessary for successful commercialization. We continue to incur significant expenses related to research and development, and other operations in order to commercialize our product candidates. We have a history of incurring significant operating losses. We had net income of $132.3 million and net losses of $46.4 million for the years ended December 31, 2023 and December 31, 2022, respectively. As of December 31, 2023, we had an accumulated deficit of $261.2 million.

We historically have financed our operations primarily through private placements of our redeemable convertible preferred stock, public offerings and private placements of our common stock, and strategic collaborations, including our prior collaborations with Sanofi Genzyme Corporation, or Sanofi Genzyme, AbbVie Biotechnology Ltd and AbbVie Ireland Unlimited Company, and our ongoing collaborations with Neurocrine Biosciences, Inc., or Neurocrine, and Novartis Pharma AG, or Novartis; our option and license agreement, or the Alexion Agreement, with Alexion, AstraZeneca Rare Disease, or Alexion; and our option and license agreement, or the 2022 Novartis Option and License Agreement, with Novartis. We refer to our ongoing collaborations with Neurocrine collectively as the Neurocrine Collaborations.

To date, we have devoted substantially all of our financial resources to building our gene therapy platform, selecting product programs, conducting research and development, including preclinical development of our product candidates, building our intellectual property portfolio, building our team, and establishing strategic collaborations. We expect that it could be several years before we have a commercialized product, if ever. We expect to continue to incur significant expenses and increasing operating losses for the foreseeable future. We also anticipate the cost of goods and services and the levels of compensation paid to employees will increase due to inflationary conditions existing in the general economy. The net losses we incur may fluctuate significantly from quarter to quarter.

We anticipate that our expenses will increase substantially if, and as, we:

- conduct preclinical development activities and initiate investigational new drug, or IND, application-enabling studies and clinical trials in connection with our anti-tau antibody program and our SOD1 ALS gene therapy program;
- continue investing in our proprietary antibody program, gene therapy and vectorized antibody platforms and programs, and other research and development initiatives;
- increase our investment in and support for TRACER™ (Tropism Redirection of AAV by Cell Type-Specific Expression of RNA), our proprietary discovery platform to facilitate the selection of adeno-associated virus, or AAV, capsids and expand our investment to discover TRACER Capsids with broad tropism in central nervous system, or CNS and other tissues with cell-specific transduction properties for particular therapeutic applications;
- increase our investment in the discovery and development of modalities for receptor-mediated non-viral delivery of therapeutic payloads to the CNS;
- conduct joint research and development under our strategic collaborations for the research, development, and commercialization of certain of our pipeline programs, including our FXN gene therapy program for Friedreich's ataxia, or the FA Program, pursuant to our collaboration and license agreement with Neurocrine entered into in January 2019, or the 2019 Neurocrine Collaboration Agreement; our glucocerebrosidase 1, or GBA1, gene therapy program for Parkinson's disease and other GBA1-mediated diseases, or the GBA1 Program, pursuant to our collaboration and license agreement with Neurocrine entered into on January 8, 2023, or the 2023 Neurocrine Collaboration Agreement; and our Huntington's disease program, or the Novartis HD Program pursuant to our license and collaboration agreement with Novartis entered into on December 28, 2023, or the 2023 Novartis Collaboration Agreement;
- initiate additional preclinical studies and clinical trials for, and continue research and development of, our other programs;
- continue our process research and development activities, as well as establish our research-grade manufacturing capabilities;
- identify additional diseases for treatment with our AAV gene therapies and develop additional programs or product candidates;

- seek marketing and regulatory approvals for any of our product candidates that successfully complete clinical development;
- maintain, expand, protect and enforce our intellectual property portfolio;
- identify, acquire or in-license other product candidates and technologies;
- expand our operational, financial and management systems and personnel, including personnel to support our clinical development, manufacturing and commercialization efforts;
- continue our clinical trial insurance coverage as we expand our clinical trials and increase our product liability insurance once we engage in commercialization efforts; and
- continue to operate as a public company.

Because of the numerous risks and uncertainties associated with pharmaceutical product development, we are unable to accurately predict the timing or amount of increased expenses. Our expenses will increase if:

- we are required by the U.S. Food and Drug Administration, or the FDA, or the European Medicines Agency, or EMA, or other regulatory agencies to redesign or modify trials or studies or to perform trials or studies in addition to those currently expected;
- there are any delays in the receipt of regulatory clearance to begin our planned clinical programs; or
- there are any delays in enrollment of patients in or completing our clinical trials or the development of our product candidates.

To become and remain profitable, we must develop and commercialize, alone or with our collaborators, product candidates with significant market potential, which will require us to be successful in a range of challenging activities. These activities include completing preclinical studies and clinical trials of our product candidates; obtaining marketing approval for these product candidates; contracting with third parties with expertise in current good manufacturing practices, or cGMPs, to manufacture our product candidates at clinical and commercial scale; marketing and selling those products that are approved; satisfying any post-marketing requirements and achieving an adequate level of market acceptance of and obtaining and maintaining adequate coverage and reimbursement from third-party payors for such products; and protecting our rights to our intellectual property portfolio. We may never succeed in any or all of these activities and, even if we do, we may never generate revenues that are significant or large enough to achieve profitability. If we do achieve profitability, we may not be able to sustain or increase profitability on a quarterly or annual basis. Our failure to become and remain profitable would decrease the value of our company and could impair our ability to raise capital, maintain our research and development efforts, expand our business or continue our operations. A decline in the value of our company also could cause our stockholders to lose all or part of their investment.

We may not be able to generate sufficient revenue from the commercialization of our product candidates and may never be consistently profitable.

Our ability to generate revenue and achieve profitability depends on our ability, alone or with our collaboration partners, to successfully complete the development of, and obtain the regulatory approvals necessary to commercialize, our current and future product candidates. All of our product candidates are in the early stages of development. We do not anticipate generating revenues from product sales for at least the next several years, and we may never succeed in doing so. Our ability to generate future revenues from product sales depends heavily on our and our collaborators' and licensors' success in:

- completing preclinical and clinical development of our product candidates or product candidates incorporating our licensed capsids or other technologies and identifying new product candidates;

- seeking and obtaining regulatory and marketing approvals for product candidates for which we or they complete clinical trials;
- launching and commercializing product candidates for which we or they obtain regulatory and marketing approval by establishing a sales, marketing and distribution infrastructure or, alternatively, collaborating with a commercialization partner;
- obtaining and maintaining adequate coverage and reimbursement by government and third-party payors for our product candidates if and when approved;
- maintaining and enhancing a sustainable, scalable, reproducible and transferable manufacturing process for our vectors and product candidates;
- establishing and maintaining supply and manufacturing relationships with third parties that have the financial, operating and technical capabilities to provide adequate products and services, in both amount and quality, to support clinical development and the market demand for our or their product candidates, if and when approved;
- obtaining an adequate level of market acceptance of our or their product candidates as a viable treatment option;
- addressing any competing technological and market developments;
- implementing additional internal systems and infrastructure, as needed;
- negotiating favorable terms in any collaboration, option, licensing, or other arrangements into which we may enter and performing our obligations in such collaborations;
- obtaining, maintaining, protecting, enforcing and expanding our portfolio of intellectual property rights, including patents, trade secrets and know-how;
- avoiding and defending against third-party claims of interference or infringement; and
- attracting, hiring and retaining qualified personnel.

Even if one or more of the product candidates that we develop is approved for commercial sale, we anticipate incurring significant costs associated with commercializing any approved product candidate. Our expenses could increase beyond expectations if we are required by the FDA, EMA, or other regulatory authorities to redesign or modify preclinical studies or clinical trials or to perform preclinical studies or clinical trials in addition to those that we currently anticipate. Even if we are able to generate revenues from the sale of any approved products, we may not become profitable and may need to obtain additional funding to continue operations.

We will need to raise additional funding, which may not be available on acceptable terms, or at all. Failure to obtain this necessary capital when needed may force us to delay, limit or terminate certain of our product development efforts or other operations.

We expect our expenses to increase over time in connection with our ongoing and planned activities, particularly as we continue the research and development of, continue or initiate clinical trials of, and seek marketing approval for, our product candidates. In addition, if we obtain marketing approval for any of our product candidates, we expect to incur significant expenses related to product sales, medical affairs, marketing, manufacturing and distribution. We also continue to incur costs associated with operating as a public company. Accordingly, we will need to obtain substantial additional funding in connection with our continuing operations. If we are unable to raise capital or enter into business development transactions when needed or on acceptable terms, we could be forced to delay, reduce or eliminate certain of our research and development programs or any future commercialization efforts.

Our operations have consumed significant amounts of cash since inception. As of December 31, 2023, our cash, cash equivalents, and marketable securities were $230.9 million. Based upon our current operating plan, we expect that our existing cash, cash equivalents, and marketable securities at December 31, 2023, together with the $80.0 million upfront payment received in January 2024 in connection with the 2023 Novartis Collaboration Agreement, the $20.0 million in proceeds from Novartis' stock purchase, and the $93.5 million in net proceeds received from our public offering in January 2024, along with amounts expected to be received as reimbursement for development costs under our collaboration and license agreements with Neurocrine and Novartis, certain near term milestones, and interest income, to be sufficient to meet our planned operating expenses and capital expenditure requirements into 2027 .

Our future capital requirements will depend on many factors, including:

- the scope, progress, results, and costs of product discovery, preclinical studies and clinical trials for our product candidates;
- the scope, progress, results, costs, prioritization, and number of our research and development programs;
- the progress and status of our strategic collaborations and option and license agreements and any similar arrangement we may enter into in the future, including any research and development costs for which we are responsible, and our receipt of any future milestone payments and royalties from our collaboration partners or licensors;
- the extent to which we are obligated to reimburse preclinical development and clinical trial costs, or the achievement of milestones or occurrence of other developments that trigger milestone and royalty payments, under any collaboration or license agreements to which we might become a party, such as our license agreement with Touchlight IP Limited, or Touchlight, which we refer to as the Touchlight License Agreement;
- the costs, timing and outcome of regulatory review of our product candidates;
- our ability to establish and maintain collaboration, distribution, or other marketing arrangements for our product candidates on favorable terms, if at all;
- the costs and timing of preparing, filing and prosecuting patent applications, maintaining and enforcing our intellectual property rights and defending intellectual property-related claims;
- the extent to which we acquire or in-license other product candidates and technologies, including any intellectual property associated with such candidates or technologies, acquire or invest in other businesses, or out-license our product candidates, capsids or other technologies;
- the costs of advancing our manufacturing capabilities and securing manufacturing arrangements for pre-commercial and commercial production;
- the level of product sales by us or our collaborators from any product candidates for which we obtain marketing approval in the future;
- the costs of operating as a public company and maintaining adequate product, clinical trial, and directors' and officers' liability insurance coverage; and
- the costs of establishing or contracting for sales, manufacturing, marketing, distribution, and other commercialization capabilities if we obtain regulatory approvals to market our product candidates .

Identifying potential product candidates and conducting preclinical studies and clinical trials is a time-consuming, expensive, and uncertain process that takes years to complete. We may never generate the necessary data or results required to maintain the financial support of our collaborators or obtain marketing approval and achieve product

sales. In the event we are unable to achieve milestones necessary to demonstrate progress on those programs, a current or future collaboration partner or licensor may be unwilling to fund these programs at the desired levels or at all, which could require us to fund these programs to a greater extent than we have expected, to decline to pursue certain program objectives or to discontinue one or more of the programs. Our ability to develop a product candidate for any of our lead gene therapy or other biological therapy programs may take longer than we anticipate, or may not happen at all, and could require funding at a level higher than we expect. Our product revenues, if any, and any commercial milestone payments or royalty payments under our collaboration or option and license agreements will be derived from sales of products that may not be commercially available for many years, if at all. In addition, our product candidates, if approved, may not achieve commercial success. Accordingly, we will need to continue to rely on additional financing and business development to achieve our business objectives. Adequate additional financing or business development transactions may not be available to us on acceptable terms, or at all.

Raising additional capital may cause dilution to our stockholders, restrict our operations or require us to relinquish rights to our technologies or product candidates.

Until such time, if ever, as we can generate product revenues sufficient to achieve consistent profitability, we expect to finance our cash needs through a combination of equity offerings, debt financings, collaborations, strategic alliances, and option and license arrangements. We do not have any committed external source of funds other than the amounts we are entitled to receive from our collaboration partners Neurocrine and Novartis for the reimbursement of certain research and development expenses, the achievement of specified regulatory and commercial milestones, and royalty payments under the 2019 Neurocrine Collaboration Agreement, the 2023 Neurocrine Collaboration Agreement, and the 2023 Novartis Collaboration Agreement and the amounts we are entitled to receive from our licensees Alexion and Novartis for the achievement of specified development, regulatory, and commercialization milestones and royalty payments under the applicable option and license agreements. To the extent that we raise additional capital through the sale of equity or equity-linked securities, including convertible debt, our stockholders' ownership interests will be diluted. The amount of stockholder dilution will be affected by the size of each securities offering and the offering price for the securities sold. The offering price will likely reflect the prevailing market price for our securities, with dilution increasing as the prevailing market price for our securities decreases. The terms of these securities may include liquidation or other preferences that adversely affect our existing stockholders' rights as holders of our common stock. For example, we completed a private placement of 2,145,002 shares of our common stock to Novartis and an underwritten public offering of 7,777,778 shares of our common stock and pre-funded warrants to purchase up to 3,333,333 shares of our common stock in January 2024. Debt financing and preferred equity financing, if available, may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, obtaining additional capital, acquiring or divesting businesses, making capital expenditures or declaring dividends. In addition, we may seek additional capital due to favorable market conditions or strategic considerations, even if we believe we have sufficient funds for our current or future operating plans. Our issuance of additional securities, whether equity or debt, or the possibility of such issuance, may cause the market price of our common stock to decline. Further, our existing stockholders may not agree with the terms of such financings.

If we raise additional funds through collaborations, strategic alliances, or option and license arrangements with third parties, we may have to relinquish valuable rights to our technologies, future revenue streams, research programs or product candidates or to grant licenses on terms that may not be favorable to us. If we are unable to raise additional funds through equity or debt financings when needed, we may be required to delay, limit, reduce or terminate our product development or future commercialization efforts or grant rights to develop and market products or product candidates that we would otherwise prefer to develop and market ourselves. Such collaborations, alliances, or option and license arrangements could therefore cause the market price of common stock to decline.

The early stage of our development efforts may make it difficult for our stockholders to evaluate the success of our business to date and to assess our future viability.

Our operating history to date has been limited to building our team, business planning, raising capital, establishing our intellectual property portfolio, determining which neurological diseases to pursue, advancing our product candidates including delivery and manufacturing and conducting preclinical studies and early-phase clinical trials. Consequently, any predictions about our future success or viability may not be as accurate as they could be if we

had an operating history that included the late stage of clinical development, completion of clinical development, or commercialization of one or more product candidates. All of our active product candidates are currently in preclinical development.

In addition, we may encounter unforeseen expenses, difficulties, complications, delays and other known and unknown factors such as the regulatory setbacks that previously occurred in prior clinical programs such as those put on hold by the FDA. These and other events that are part of our operating history may impact our ability to operate our business and to raise capital. All of our product candidates are in the early stages of development. To achieve our current goals, we will need to transition in the future from a company with a research and development focus to a company capable of supporting commercial activities. We may not be successful in such a transition.

We expect our financial condition and operating results to continue to fluctuate significantly from quarter-to-quarter and year-to-year due to a variety of factors, many of which are beyond our control as we advance our programs into the clinical stage. Accordingly, our stockholders should not rely upon the results of any quarterly or annual periods as indications of future operating performance.

Risks Related to the Development and Regulatory Approval of Our Product Candidates

Our AAV gene therapy and other biological therapy product candidates are based on a proprietary technology and, in several disease areas, unvalidated treatment approaches, which makes it difficult and potentially infeasible to predict the duration and cost of development of, and subsequently obtaining regulatory approval for, our product candidates.

Our future success depends on our successful development of AAV gene therapy and other biological therapy product candidates, including our anti-tau antibody candidate. Each of the product candidates we are advancing, either alone or together with our strategic collaborators, is currently in preclinical development.

AAV gene therapies are a relatively new technology. We cannot accurately predict when or if any of our product candidates will prove effective or safe in humans or whether these product candidates will receive marketing approval. Additionally, there can be no assurance that we will not experience problems or delays in the preclinical testing or development of our product candidates and that such problems or delays will not cause unanticipated costs, or that any such problems or delays can be solved in a timely or profitable basis, if at all. We also may experience unanticipated problems or delays in expanding our manufacturing capacity or outsourcing manufacturing activities to contract manufacturers.

The clinical trial requirements of the FDA, the EMA and other regulatory authorities and the criteria these regulators use to determine the safety and efficacy of a product candidate vary substantially according to the type, complexity, novelty and intended use and market of the product candidate. The regulatory approval process for novel product candidates such as gene therapies can be more expensive and take longer than for other, better known or more extensively studied product candidates. Until August 2017, the FDA had never approved an AAV gene therapy product. Since that time, it has approved a limited number of gene therapy products. In Europe, a similarly limited number of AAV gene therapy products have been granted marketing authorization.

It is difficult to determine how long it will take or how much it will cost to obtain regulatory approvals for our product candidates in either the United States or the European Union or how long it will take to commercialize our product candidates. The few regulatory approvals of gene therapies to date may not be indicative of what the FDA, EMA, or other regulatory authorities may require for approval or whether different or additional preclinical studies or clinical trials may be required to support regulatory approval in a particular jurisdiction. Delay or failure to obtain, or unexpected costs in obtaining, the regulatory approval necessary to bring a potential product candidate to market could decrease our ability to generate sufficient product revenue, and our business, financial condition, results of operations and prospects may be harmed.

Regulatory requirements governing biological and gene therapy products have changed frequently and may continue to change in the future. Such requirements may lengthen the regulatory review process, require us to modify current studies or perform additional studies or increase our development costs, which in turn may force us to delay, limit, or terminate certain of our programs.

The Center for Biologics Evaluation and Research, or CBER, of the FDA regulates biological products, for human use. The Office of Tissues and Advanced Therapies, or OTAT, formerly known as the Office of Cellular, Tissue and Gene Therapies, within CBER reviews gene therapy and related products and has established the Cellular, Tissue and Gene Therapies Advisory Committee to advise CBER in its review.

U.S. regulations require each clinical trial site's institutional review board, or IRB, to review proposed clinical trials to assess the safety of the trial. If the protocol for such a trial was amended, it would need to be re-reviewed by the respective institutional IRBs of each institution. Any delay in or failure to obtain institutional IRB approval for any protocol or protocol amendment could delay, interrupt, or limit the conduct of the clinical trial at one or more participating clinical trial sites.

Adverse or unforeseen developments in clinical trials of proprietary antibody and gene therapy products conducted by us or others may cause the FDA or other oversight bodies to change the requirements for approval of any of our product candidates. Similarly, EMA and local health authorities of individual countries within the European Union may issue new guidelines concerning the clinical development and marketing authorization for gene therapy medicinal products and require that we comply with these new guidelines. The EMA and agencies at both the federal and state level in the United States have expressed an interest in further regulating new biotechnologies, including gene therapy. In addition, gene therapy products are considered genetically-modified organism, or GMO, products and are regulated as such in each country. Designation of the type of GMO product and subsequent handling and disposal requirements can vary across countries and is variable throughout the European Union. Addressing each specific country requirement and obtaining approval to commence a clinical trial in these countries could result in delays in starting, conducting, or completing a clinical trial. Similar issues could be faced in other regions of the world including the Asia-Pacific region.

These regulatory review committees and advisory groups and the new guidelines they promulgate may lengthen the regulatory review process, require us to perform additional studies, increase our development costs, lead to changes in regulatory positions and interpretations, delay or prevent approval and commercialization of these product candidates or lead to significant post-approval limitations or restrictions. As we advance our product candidates, we will be required to consult with these regulatory and advisory groups and comply with applicable guidelines.

Any inability to receive timely, actionable feedback from regulatory authorities could also delay or otherwise hinder our development efforts. These and other regulatory delays may require us to incur additional clinical development costs, slow down our product candidate development and approval process and delay or potentially jeopardize our ability to commence product sales and generate revenue from our product candidates.

We plan to continue to seek and incorporate FDA guidance in our ongoing development plans for each of our potential clinical candidates. If we fail to consult or solicit guidance from regulators or are unable to obtain sufficiently frequent or detailed guidance from regulators, we may be required to delay or discontinue development of certain of our product candidates. These additional processes may result in a review and approval process that is longer than we otherwise would have expected. Delays as a result of increased or lengthier regulatory approval process and further restrictions on development of our product candidates can be costly and could negatively impact our or our collaborators' ability to complete clinical trials and commercialize our current and future product candidates in a timely manner, if at all.

Results from preclinical studies and early-stage clinical trials may not be indicative of efficacy in late-stage clinical trials.

All of our product candidates are in early stages of development, and the risk of failure is high. Clinical testing is expensive, is difficult to design and implement, can take many years to complete and is uncertain as to outcome. A failure of one or more clinical trials can occur at any stage of testing. Our product candidates may fail to show the

desired safety and efficacy in preclinical testing or clinical development despite demonstrating promising results in earlier preclinical studies or clinical trials. In addition, the outcome of preclinical testing and early clinical trials may not be predictive of the success of later stage clinical trials. For example, despite data we believed was promising from the earlier PD-1101 Phase 1b clinical trial and from the separate PD-1102 Phase 1 clinical trial evaluating the delivery of VY-AADC (NBIb-1817), we and our strategic collaborator Neurocrine did not receive favorable data, and were ultimately unable to complete, the RESTORE-1 Phase 2 clinical trial evaluating VY-AADC (NBIb-1817) for the treatment of Parkinson's disease. Similarly, interim results generated from clinical trials do not necessarily predict final results, and results from one completed clinical trial may not be replicated in a subsequent clinical trial with a similar study design. Some of our clinical trials were conducted with small patient populations and were not blinded or placebo-controlled, making it difficult to predict whether the favorable results that we observed in such trials will be sustained or repeated in larger and more advanced clinical trials. Moreover, preclinical and clinical data are often susceptible to varying interpretations and analyses, and many companies that have believed their product candidates performed satisfactorily in preclinical studies and clinical trials have nonetheless failed to obtain marketing approval of their products.

There is a high failure rate for product candidates proceeding through preclinical studies and clinical trials. A number of companies in the pharmaceutical and biotechnology industries have suffered significant setbacks in late-stage clinical trials even after achieving promising results in early-stage clinical trials. If a larger population of patients does not experience positive results, if these results are not reproducible, or if our products show diminishing activity over time, our products may not receive approval from the EMA or the FDA. Data obtained from preclinical and clinical activities are subject to varying interpretations, which may delay, limit or prevent regulatory approval. In addition, we may encounter regulatory delays or rejections as a result of many factors, including changes in regulatory policy during the period of product development. Failure to confirm favorable results from earlier trials by demonstrating the safety and effectiveness of our products in late-stage clinical trials with larger patient populations could harm our business and we may never succeed in commercialization or generating product revenue.

We may in the future conduct clinical trials for product candidates at sites outside the United States, and the FDA may not accept data from trials conducted in such locations.

To date, we have only conducted clinical trials in the United States. However, we may in the future choose to conduct one or more of our clinical trials or include sites in current or future clinical trials outside the United States. For example, we may include clinical trial sites outside the United States for our planned Phase 1 clinical trial to evaluate VY-TAU01.

Although the FDA may accept data from clinical trials conducted outside the United States, acceptance of these data is subject to conditions imposed by the FDA. In cases where data from foreign clinical trials are intended to serve as the sole basis for marketing approval in the United States, the FDA will generally not approve the application on the basis of foreign data alone unless (i) the data are applicable to the U.S. population and U.S. medical practice; (ii) the trials were performed by clinical investigators of recognized competence and pursuant to GCP regulations; and (iii) the data may be considered valid without the need for an on-site inspection by the FDA, or if the FDA considers such inspection to be necessary, the FDA is able to validate the data through an on-site inspection or other appropriate means.

In addition, even where the foreign trial data are not intended to serve as the sole basis for approval, the FDA will not accept the data as support for an application for marketing approval unless the trial satisfies certain conditions. For example, the clinical trial must be well-designed and conducted and performed by qualified investigators in accordance with ethical principles. The trial population must also adequately represent the U.S. population, and the data must be applicable to the U.S. population and U.S. medical practice in ways that the FDA deems clinically meaningful. In addition, while these clinical trials are subject to the applicable local laws, FDA acceptance of the data will depend on its determination that the trials also complied with all applicable U.S. laws and regulations. If the FDA does not accept the data from any trial we conduct outside the United States, it would likely result in the need for additional trials, which would be costly and time-consuming and would delay or permanently halt our development of the applicable product candidates. Even if the FDA accepted such data, it could require us to modify our planned clinical trials to receive clearance to initiate such trials in the United States or to continue such trials once initiated.

Other risks inherent in conducting international clinical trials or using international trial sites include:

- foreign regulatory requirements that could restrict or limit our ability to conduct our clinical trials;
- the administrative burden of complying with a variety of foreign laws, medical standards and regulatory requirements, including the regulation of pharmaceutical and biotechnology products and treatment;
- the failure of enrolled patients to adhere to clinical protocols or inadequate collection and assessment of clinical data as a result of differences in healthcare services or cultural customs;
- foreign exchange fluctuations;
- diminished or loss of protection of intellectual property in the relevant jurisdiction; and
- political, economic, environmental, and health risks relevant to specific foreign countries, including risks related to natural disasters or disease outbreaks.

We are early in our development efforts. All of our active product candidates are currently in preclinical development or are advancing into the clinic. We may encounter substantial delays or difficulties in commencement, enrollment or completion of our preclinical studies or clinical trials, or we may fail to demonstrate safety and efficacy to the satisfaction of applicable regulatory authorities, any of which could prevent us from commercializing our current and future product candidates on a timely basis, if at all.

We are early in our development efforts, and all of our active product candidates are currently in preclinical development. Before obtaining marketing approval from regulatory authorities for the sale of our current and future product candidates, we must conduct extensive clinical trials to demonstrate the safety and efficacy of the product candidates. To conduct clinical trials, we must first complete preclinical testing and studies to support IND applications or similar applications in other jurisdictions. We cannot be certain of the timely completion or successful outcome of our preclinical testing and studies.

Our ability to complete our preclinical testing and studies is contingent on, among other things, our ability to source animals and other supplies required for the conduct of such testing and studies. If we are unable to obtain such supplies, we may be unable to complete such preclinical testing and studies in a timely manner or at all. For example, some of our IND-enabling toxicology, capsid discovery, and other studies require certain non-human primates, or NHPs, that are customarily imported from outside the United States. Our inability to obtain access to a sufficient supply of these NHPs in a timely manner or at all may impair or delay our ability to complete preclinical studies to support capsid discovery efforts or IND applications or similar applications in other jurisdictions. We have previously encountered, and may encounter in the future, delays in obtaining a sufficient supply of such NHPs due to governmental or regulatory actions that result in importation restrictions in the United States or exportation restrictions in the country of origin. At times when the NHP supply in the United States has been constrained, we have conducted NHP studies at contract research facilities outside of the United States. When utilizing such facilities, we are required to observe export control regulations for the shipment of product candidates and their component materials and import control regulations for the shipment of samples to us for evaluation and storage. We may be required to incur delays or expenses in order to conduct our NHP studies in compliance with these regulations, and we may be subject to additional penalties, delays, or expenses if we fail to achieve compliance.

Additionally, we cannot predict if the FDA or similar regulatory authorities outside the United States will accept our planned clinical programs or if the outcome of our preclinical testing and studies will ultimately support the further development of our preclinical and clinical programs. In connection with our VY-HTT01 Program for the treatment of Huntington's disease, for example, we were unable to predict what the FDA would require and were unable to obtain a second pre-IND meeting with the FDA to discuss the product candidate's regulatory pathway with the FDA. As a result, in October 2020, the FDA notified us that the IND application for the planned Phase 1 and Phase 2 clinical trial to evaluate VY-HTT01 had been put on clinical hold.

In addition, the FDA's and other regulatory authorities' policies with respect to clinical trials may change and additional government regulations may be enacted. For example, in December 2022, with the passage of Food and Drug Omnibus Reform Act, known was FDORA, Congress required sponsors to develop and submit a diversity action plan for each Phase 3 clinical trial or any other "pivotal study" of a new drug or biological product. These plans are meant to encourage the enrollment of more diverse patient populations in late-stage clinical trials of FDA-regulated products. Specifically, action plans must include the sponsor's goals for enrollment, the underlying rationale for those goals, and an explanation of how the sponsor intends to meet them. In addition to these requirements, the legislation directs the FDA to issue new guidance on diversity action plans. Similarly, the regulatory landscape related to clinical trials in the European Union, or EU, recently evolved. The EU Clinical Trials Regulation, or CTR, which was adopted in April 2014 and repeals the EU Clinical Trials Directive, became applicable on January 31, 2022. While the Clinical Trials Directive required a separate clinical trial application, or CTA, to be submitted in each member state, to both the competent national health authority and an independent ethics committee, the CTR introduces a centralized process and only requires the submission of a single application to all member states concerned. The CTR allows sponsors to make a single submission to both the competent authority and an ethics committee in each member state, leading to a single decision per member state. The assessment procedure of the CTA has been harmonized as well, including a joint assessment by all member states concerned, and a separate assessment by each member state with respect to specific requirements related to its own territory, including ethics rules. Each member state's decision is communicated to the sponsor via the centralized EU portal. Once the CTA is approved, clinical study development may proceed. If we are slow or unable to adapt to changes in existing requirements or the adoption of new requirements or policies governing clinical trials, our development plans may be impacted.

We cannot guarantee that any clinical trials will be conducted as planned or completed on schedule, if at all. A clinical trial failure can occur at any stage of testing. Similarly, there may be delays or difficulties in our initiation of future clinical trials. Similarly, there may be delays or difficulties in our initiation of future clinical trials. Due to the additional regulatory uncertainties associated with gene therapy products, for example, we did not initiate the RESTORE-1 Phase 2 clinical trial for VY-AADC (NBIb-1817) as a treatment for Parkinson's disease until we met with OTAT to discuss our proposed trial design and overall development plan.

We also have very limited historical experience with clinical trials. Identifying and qualifying patients to participate in clinical trials of our product candidates is critical to our success. We may not be able to identify, recruit and enroll a sufficient number of patients, or those with required or desired characteristics, to complete our clinical trials in a timely manner or at all pursuant to the requirements of the FDA, EMA, or other regulatory authorities. Patient enrollment and trial completion are affected by many factors including:

- perceived risks and benefits of proprietary antibody and AAV gene therapy approaches for the treatment of neurological and other diseases;
- formulation changes to our product candidates, which may require us to conduct additional clinical studies to bridge our modified product candidates to earlier versions;
- size of the patient population and process for identifying patients;
- design of the trial protocol;
- eligibility and exclusion criteria;
- patients with preexisting antibodies to the gene therapy vector that preclude their participation in the trial;
- perceived risks and benefits of the product candidate under study;
- availability of competing therapies and clinical trials;
- severity of the disease under investigation;

- availability of genetic testing for potential patients;
- proximity and availability of clinical trial sites for prospective patients;
- lack of adequate compensation of patients;
- ability to obtain and maintain patient consent;
- risk that enrolled patients will drop out before completion of the trial;
- our ability to locate appropriately trained physicians to conduct such clinical trials, particularly for clinical trials requiring lengthy and highly complex surgical protocols, the performance of which may only be possible at major academic medical centers or specialized surgical centers;
- willingness of patients to participate in a placebo-controlled trial;
- patient referral practices of physicians; and
- ability to monitor patients adequately during and after treatment.

Further, we plan to seek marketing approvals in the United States, the European Union and other jurisdictions, which may require that we conduct clinical trials in foreign countries. Our ability to successfully initiate, enroll and complete a clinical trial in any foreign country is subject to numerous risks unique to conducting business in foreign countries, including:

- difficulty in establishing or managing relationships with clinical research organizations, or CROs, and physicians;
- different standards for the conduct of clinical trials;
- absence in some countries of established groups with sufficient regulatory expertise for review of AAV gene therapy protocols;
- our inability to locate qualified local partners or collaborators for such clinical trials; and
- the potential burden of complying with a variety of foreign laws, medical standards and regulatory requirements, including the regulation of pharmaceutical and biotechnology products and treatment.

If we have difficulty enrolling a sufficient number of patients to conduct our clinical trials as planned, we may need to delay, limit or terminate ongoing or planned clinical trials in some or all localities, any of which would harm our business, financial condition, results of operations and prospects.

Other events that may prevent successful or timely completion of clinical development include:

- delays in reaching a consensus with regulatory authorities or collaborators on trial design, implementation, management, or other aspects of the clinical trial;
- delays in reaching agreement on acceptable terms with prospective CROs and clinical trial sites;
- delays in opening clinical trial sites or obtaining required IRB or independent ethics committee approval at each clinical trial site;

- as a result of a serious adverse event, or SAE, or after an inspection of our clinical trial operations or trial sites or the decision by us or our collaborators, or the requirement of regulators or IRBs to suspend or terminate clinical research for various reasons, including noncompliance with regulatory requirements or a finding that the participants are being exposed to unacceptable health risks;
- failure by us, our collaboration partners, any CROs we engage, or any other third parties to adhere to clinical trial protocols or regulatory requirements;
- failure by us, our collaboration partners, any CROs we engage, or any other third parties to perform in accordance with the FDA's good clinical practices, or GCPs, or applicable regulatory guidelines in the European Union;
- failure by physicians to adhere to delivery protocols leading to variable results;
- delays in the testing, validation, manufacturing and delivery of our product candidates to the clinical sites, including delays by third parties with whom we have contracted to perform certain of those functions;
- insufficient or inadequate supply or quality of our product candidates or other materials necessary to conduct clinical trials of our product candidates;
- delays in having patients complete participation in a trial or return for post-treatment follow-up;
- clinical trial sites or patients dropping out of a trial at a rate higher than we anticipate;
- selection of clinical endpoints that require prolonged periods of clinical observation or analysis of the resulting data;
- receipt of negative or inconclusive clinical trial results;
- occurrence of SAEs associated with the product candidate that are viewed to outweigh its potential benefits;
- occurrence of SAEs in trials of the same class of agents conducted by other sponsors;
- changes in regulatory requirements and guidance that require amending or submitting new clinical protocols; or
- the cost of clinical trials of our product candidates may be greater than we anticipate.

Any inability to successfully initiate or complete preclinical studies and clinical trials could result in additional costs and potential delays to us or impair our ability to generate revenues from product sales, regulatory and commercialization milestones and royalties. We do not know whether any of our preclinical studies or clinical trials will begin as planned, will need to be restructured, or will be completed on schedule, or at all. For example, our decision to refocus our Huntington's disease program means we must conduct new preclinical studies, prepare a new IND, submit it to the FDA, and resolve any potential FDA objections before enrolling our first patient in a new clinical trial. In addition, if we make manufacturing or formulation changes to our product candidates, such as our previous transition to an HEK 293-based production system from a baculovirus/Sf9 AAV production system or as a result of unanticipated clinical trial results, we may need to conduct additional studies to bridge our modified product candidates to earlier versions. Clinical trial delays also could shorten any periods during which we may have the exclusive right to commercialize our product candidates or allow our competitors to bring products to market before we do, which could impair our ability to successfully commercialize our product candidates and may harm our business, financial condition, results of operations and prospects.

Additionally, if the results of our clinical trials are inconclusive or if there are safety concerns or SAEs associated with our product candidates, we may:

- be delayed in obtaining marketing approval for our product candidates, if we are able to do so at all;
- obtain approval for indications or patient populations that are not as broad as intended or desired;
- obtain approval with labeling that includes significant use or distribution restrictions or safety warnings;
- be subject to changes in the way the product is administered;
- be required to perform additional clinical trials to support approval or be subject to additional post-marketing testing requirements;
- have regulatory authorities withdraw, or suspend, their approval of the product or impose restrictions on its distribution in the form of a Risk Evaluation and Mitigation Strategy, or REMS;
- be subject to the addition of labeling statements, such as warnings or contraindications;
- be sued or otherwise become party to dispute proceedings; or
- experience damage to our reputation.

Our product candidates may cause undesirable side effects or have other properties that could delay or prevent their regulatory approval, limit their commercial potential or result in significant negative consequences following any potential marketing approval.

Our proprietary antibodies and gene therapy product candidates may cause an immunologic reaction, or an immune response against the relevant product candidate. Other potential side effects associated with our gene therapy product candidates could include insertional oncogenesis, which is the process whereby the insertion of a functional gene near a gene that is important in cell growth or division results in uncontrolled cell division, which could potentially enhance the risk of malignant transformation. In past clinical trials that were conducted by others using non-AAV gene therapy vectors, several significant side effects were caused by gene therapy treatments, including reported cases of leukemia and death. If our vectors demonstrate a similar adverse effect, or other adverse effects, we may be required to halt or delay further clinical development of our product candidates or withdraw the product from the market post-approval. For example, in a recently published review of patients with hepatocellular carcinomas, it was shown that a small subset contained an integrated genome sequence of wild-type AAV2 and it was suggested that AAV2 may be associated with insertional oncogenesis.

In addition to side effects caused by the product candidate, the administration process also could cause side effects. If in the future we are unable to demonstrate that such side effects were caused by the administration process or related procedures or are unable to modify the trial protocol adequately to address such side effects, the FDA, the European Commission, the EMA or other regulatory authorities could order us to cease further development of, or deny approval of, our product candidates for any or all targeted indications. For example, product candidates designed to “knock down” or reduce the expression of a gene or the production of its encoded protein, could have effects on other parts of the body, or “off target” effects, that could result in unforeseen toxicity. Even if we are able to demonstrate that any future SAEs are not product-related, and regulatory authorities do not order us to cease further development of our product candidates, such occurrences could affect patient recruitment or the ability of enrolled patients to complete the trial. Moreover, if we elect, or are required, to delay, suspend or terminate any clinical trial of any of our product candidates, the commercial prospects of such product candidates may be harmed and our ability to generate product revenues from any of these product candidates may be delayed or eliminated. Any of these occurrences may harm our ability to develop other product candidates and may harm our business, financial condition and prospects significantly.

Additionally, if any of our product candidates receives marketing approval, the FDA could require us to adopt a REMS to ensure that the benefits outweigh its risks. We believe that the likelihood of the FDA requiring a REMS may be higher for treatments with more invasive routes of administration such as direct delivery through brain surgery. Such REMS may include, among other things, a medication guide outlining the risks of the product for distribution to patients and a communication plan to health care practitioners or the limitation of the use of the product to specifically trained neurosurgeons and/or certain centers. Furthermore, adverse events which were initially considered unrelated to the study treatment of the clinical trial may later be found to be caused by the study treatment. If we or others later identify undesirable side effects caused by our product candidate, several potentially significant negative consequences could result, including:

- regulatory authorities may suspend or withdraw approvals of such product candidate;
- regulatory authorities may require additional warnings on the label;
- we may be required to change the way a product candidate is administered or conduct additional clinical trials;
- we could be sued and held liable for harm caused to patients; and
- our reputation may suffer.

Any of these events could prevent us from achieving or maintaining market acceptance of our product candidates and could significantly harm our business, prospects, financial condition and results of operations.

We may be unable to obtain orphan drug designation or exclusivity for any of our product candidates for which we seek such designation. If our competitors are able to obtain orphan drug exclusivity for products that constitute the "same drug" and treat the same indications as our product candidates, we may not be able to have competing products approved by the applicable regulatory authority for a significant period of time. For products for which we may obtain orphan drug designation or exclusivity, we may be unable to prevent the approval or marketing authorization of other similar products based upon regulatory decisions regarding product "sameness".

Regulatory authorities in some jurisdictions, including the United States and the European Union, may designate drugs for relatively small patient populations as orphan drugs. Under the Orphan Drug Act of 1983, or the Orphan Drug Act, the FDA may designate a product candidate as an orphan drug if it is intended to treat a rare disease or condition, which is generally defined as having a patient population of fewer than 200,000 individuals in the United States, or a patient population greater than 200,000 in the United States where there is no reasonable expectation that the cost of developing the drug or biological product will be recovered from sales in the United States. In the European Union, EMA's Committee for Orphan Medicinal Products grants orphan drug designation to promote the development of products that are intended for the diagnosis, prevention or treatment of a life-threatening or chronically debilitating condition affecting not more than five in 10,000 persons in the European Union. Additionally, orphan designation is granted for products intended for the diagnosis, prevention or treatment of a life-threatening, seriously debilitating or serious and chronic condition and when, without incentives, it is unlikely that sales of the drug in the European Union would be sufficient to justify the necessary investment in developing the drug or biologic product.

Generally, if a product candidate with an orphan drug designation receives the first marketing approval for the indication for which it has such designation, the product is entitled to a period of marketing exclusivity, which precludes the applicable regulatory authority from approving another marketing application for a product that constitutes the same drug treating the same indication for that marketing exclusivity period, except in limited circumstances. If another sponsor receives such approval before we do (regardless of our orphan drug designation), we may be precluded from receiving marketing approval for our product for the applicable exclusivity period. The applicable period is seven years in the United States and 10 years in the European Union. The exclusivity period in the United States can be extended by six months if the new drug application or BLA sponsor submits pediatric data that adequately respond to a written request from the FDA for such data. The exclusivity period in the European Union can be reduced to nine years if a

product no longer meets the criteria for orphan drug designation or if the product is sufficiently profitable so that market exclusivity is no longer justified. Orphan drug exclusivity may be revoked if any regulatory agency determines that the request for designation was materially defective or if the manufacturer is unable to assure sufficient quantity of the product to meet the needs of patients with the rare disease or condition.

We believe that certain of our current programs may qualify for orphan drug designation. Even if we obtain orphan drug exclusivity for a product candidate, that exclusivity may not effectively protect the product candidate from competition because different drugs or biological products can be approved for the same condition. In the United States, even after an orphan drug is approved, the FDA may subsequently approve another drug or biological product for the same condition if the FDA concludes that the other drug or biological product is not the "same drug" or biological product or even if it is, the FDA determines that it is clinically superior in that it is shown to be safer or more effective or makes a major contribution to patient care. In September 2021, the FDA issued final guidance describing its current thinking on when a gene therapy product is the "same" as another product for purposes of orphan exclusivity. Under the guidance, if either the transgene or vector differs between two gene therapy products in a manner that does not reflect "minor" differences, the two products would be considered different drugs for orphan drug exclusivity purposes. The FDA will determine whether two vectors from the same viral class are the same on a case-by-case basis and may consider additional key features in assessing the sameness.

In the European Union, marketing authorization may be granted to a similar medicinal product for the same orphan indication if:

- the second applicant can establish in its application that its medicinal product, although similar to the orphan medicinal product already authorized, is safer, more effective or otherwise clinically superior;
- the holder of the marketing authorization for the original orphan medicinal product consents to a second orphan medicinal product application; or
- the holder of the marketing authorization for the original orphan medicinal product cannot supply sufficient quantities of orphan medicinal product.

On August 3, 2017, the Congress passed the FDA Reauthorization Act of 2017, or FDARA. FDARA, among other things, codified the FDA's pre-existing regulatory interpretation to require that a drug sponsor demonstrate the clinical superiority of an orphan drug that is otherwise the same as a previously approved drug for the same rare disease in order to receive orphan drug exclusivity. The new legislation reverses prior precedent holding that the Orphan Drug Act unambiguously requires that the FDA recognize the orphan exclusivity period regardless of a showing of clinical superiority.

The FDA and Congress may further reevaluate the Orphan Drug Act and its regulations and policies, particularly in light of a decision from the U.S. Court of Appeals for the Eleventh Circuit in September 2021 finding that, for the purpose of determining the scope of exclusivity, the term "same disease or condition" means the designated "rare disease or condition" and could not be interpreted by the FDA to mean the "indication or use." Thus, the Court of Appeals concluded, orphan drug exclusivity applies to the entire designated disease or condition rather than the "indication or use." On January 23, 2023, the FDA announced that, in matters beyond the scope of that Court's order, the FDA will continue to apply its existing regulations tying orphan-drug exclusivity to the uses or indications for which the orphan drug was approved. We do not know if, when, or how the FDA may change the orphan drug regulations and policies in the future, and it is uncertain how any changes might affect our business. Depending on what changes the FDA may make to its orphan drug regulations and policies, our business could be adversely impacted.

A potential breakthrough therapy designation by the FDA for our product candidates may not lead to a faster development or regulatory review or approval process, and it does not increase the likelihood that our product candidates will receive marketing approval.

We have previously sought and may in the future seek a breakthrough therapy designation for some of our product candidates. A breakthrough therapy is defined as a drug or biological product that is intended, alone or in combination with one or more other drugs, to treat a serious or life-threatening disease or condition, and preliminary clinical evidence indicates that the drug or biological product may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development. For drugs or biological products that have been designated as breakthrough therapies, interaction and communication between the FDA and the sponsor of the trial can help to identify the most efficient path for clinical development while minimizing the number of patients placed in ineffective control regimens. Drugs designated as breakthrough therapies by the FDA may also be eligible for accelerated approval.

Designation as a breakthrough therapy is within the discretion of the FDA. Accordingly, even if we believe one of our product candidates meets the criteria for designation as a breakthrough therapy, the FDA may disagree and instead determine not to make such designation. In any event, the receipt of a breakthrough therapy designation for a product candidate may not result in a faster development process, review or approval compared to drugs considered for approval under conventional FDA procedures and does not assure ultimate approval by the FDA. In addition, even if one or more of our product candidates qualify as breakthrough therapies, the FDA may later decide that the drugs or biological products no longer meet the conditions for qualification.

A potential regenerative medicine advanced therapy designation by the FDA for our product candidates may not lead to a faster development or regulatory review or approval process, and it does not increase the likelihood that our product candidates will receive marketing approval.

We have sought and may in the future seek a regenerative medicine advanced therapy, or RMAT, designation for some of our product candidates. Under the 21st Century Cures Act, or the Cures Act, to be eligible to receive RMAT designation from the FDA, a product candidate must be (a) considered a “regenerative medicine therapy” as defined in the Cures Act; (b) intended to treat, modify, reverse, or cure one or more serious or life-threatening diseases or conditions; and (c) indicated, in preliminary clinical evidence, to have the potential to address unmet medical needs for such diseases or conditions. Gene therapies, including genetically modified cells, that lead to a durable modification of cells or tissues may meet the definition in the Cures Act of a regenerative medicine therapy.

The RMAT program is intended to facilitate efficient development and expedite review of such therapies. A new drug application or a BLA for a product candidate that has received an RMAT designation may be eligible for priority review or accelerated approval through (1) surrogate or intermediate endpoints reasonably likely to predict long-term clinical benefit or (2) reliance upon data obtained from a meaningful number of sites. Benefits of such designation also include early interactions with FDA to discuss any potential surrogate or intermediate endpoint to be used to support accelerated approval. A product candidate that has received an RMAT designation that is granted accelerated approval and is subject to post-approval requirements may fulfill such requirements through the submission of clinical evidence, clinical studies, patient registries, or other sources of real world evidence, such as electronic health records; the collection of larger confirmatory data sets; or post-approval monitoring of all patients treated with such therapy prior to its approval.

RMAT designation is within the discretion of the FDA. Accordingly, even if we believe one of our other product candidates meets the criteria for RMAT designation, the FDA may disagree and instead determine not to make such designation. In any event, the receipt of RMAT designation for a product candidate may not result in a faster development process, review or approval compared to drugs considered for approval under conventional FDA procedures and does not assure ultimate approval by the FDA. In addition, the FDA may later decide that a product candidate that received RMAT designation no longer meets the conditions for designation. Alternatively, we or our collaborative partners may decide not to proceed with the clinical development of a product candidate that has previously received RMAT designation or decide to pursue such product candidate for an indication for which it has not received RMAT designation.

Fast Track designation by the FDA may not actually lead to a faster development or regulatory review or approval process and does not assure FDA approval of our product candidate.

If a drug is intended for the treatment of a serious or life-threatening condition and the drug demonstrates the potential to address unmet medical need for this condition, the drug sponsor may apply for FDA Fast Track designation. We have sought and may in the future seek such a designation for our product candidates. A Fast Track designation does not ensure that the product candidate will receive marketing approval or that approval will be granted within any particular timeframe. Thus, Fast Track products may not experience a faster development process, review or approval compared to conventional FDA procedures. In addition, the FDA may withdraw Fast Track designation if it believes that the designation is no longer supported by data from a product candidate's clinical development program. Fast Track designation alone does not guarantee qualification for the FDA's priority review procedures.

Priority review designation by the FDA may not lead to a faster regulatory review or approval process and, in any event, does not assure FDA approval of our product candidate.

If the FDA determines that a product candidate offers major advances in treatment or provides a treatment where no adequate therapy exists, the FDA may designate the product candidate for priority review. A priority review designation means that the FDA's goal to review an application is six months, rather than the standard review period of ten months. We may request priority review for our product candidates. The FDA has broad discretion with respect to whether or not to grant priority review status to a product candidate, so even if we believe a particular product candidate is eligible for such designation or status, the FDA may decide not to grant it. Moreover, a priority review designation does not necessarily mean a faster regulatory review process or necessarily confer any advantage with respect to approval compared to conventional FDA procedures. Receiving priority review from the FDA does not guarantee approval within the six-month review cycle or thereafter.

Even if we complete the necessary preclinical studies and clinical trials, the marketing approval process is expensive, time-consuming and uncertain and may prevent us from obtaining approvals for the commercialization of some or all of our product candidates. If we or any current or future collaborators are not able to obtain, or if there are delays in obtaining, required regulatory approvals, we or they may not be able to commercialize our products, and our ability to generate revenue may be materially impaired.

Our product candidates and the activities associated with their development and commercialization, including their design, testing, manufacture, safety, efficacy, recordkeeping, labeling, storage, approval, advertising, promotion, sale and distribution, export and import, are subject to comprehensive regulation by the FDA and other regulatory agencies in the United States and by the EMA and comparable regulatory authorities in other countries. Failure to obtain marketing approval for a product candidate will prevent us from commercializing the product candidate. We have not received approval to market any of our product candidates from regulatory authorities in any jurisdiction. We have only limited experience in filing and supporting the applications necessary to gain marketing approvals and expect to rely on third-party CROs to assist us in this process.

Securing marketing approval requires the submission of extensive preclinical and clinical data and supporting information to the various regulatory authorities for each therapeutic indication to establish the product candidate's safety and efficacy. Securing regulatory approval also requires the submission of information about the product manufacturing process to, and inspection of manufacturing facilities by, the relevant regulatory authority. Our product candidates may not be effective, may be only moderately effective or may prove to have undesirable or unintended side effects, toxicities or other characteristics that may preclude our obtaining marketing approval or prevent or limit commercial use.

The FDA may also require that NDA submissions for our product candidates include pediatric data. Under the PREA, an NDA, BLA or supplement to an NDA or BLA for certain drugs and biological products must contain data to assess the safety and effectiveness of the drug or biological product in all relevant pediatric subpopulations and to support dosing and administration for each pediatric subpopulation for which the product is safe and effective, unless the sponsor receives a deferral or waiver from the FDA. Applicable legislation in the EU also requires sponsors to either conduct clinical trials in a pediatric population in accordance with a Pediatric Investigation Plan approved by the

Pediatric Committee of the European Medicines Agency, or EMA, or to obtain a waiver or deferral from the conduct of these studies by this Committee. For any of our product candidates for which we are seeking regulatory approval in the United States or the EU, we cannot guarantee that we will be able to obtain a waiver or alternatively complete any required studies and other requirements in a timely manner, or at all, which could result in associated reputational harm and subject us to enforcement action.

The process of obtaining marketing approvals, both in the United States and abroad, is expensive; may take many years if additional clinical trials are required, if approval is obtained at all and can vary substantially based upon a variety of factors, including the type, complexity and novelty of the product candidates involved. In the United States, for example, the application user fee to obtain FDA review of a marketing application is more than $4.0 million, and may be higher in the future. Changes in marketing approval policies during the development period, in or the enactment of additional statutes or regulations, or in regulatory review for each submitted product application, may cause delays in the approval or rejection of an application. The FDA and comparable authorities in other countries have substantial discretion in the approval process and may refuse to accept any application or may decide that our data are insufficient for approval and require additional preclinical, clinical or other studies. In addition, varying interpretations of the data obtained from preclinical and clinical testing could delay, limit or prevent marketing approval of a product candidate. Any marketing approval we, or any current or future collaborators, ultimately obtain may be limited or subject to restrictions or post-approval commitments that render the approved product not commercially viable.

Accordingly, if we or any current or future collaborators experience delays in obtaining approval or if we or they fail to obtain or retain approval of our product candidates and devices, the commercial prospects for our product candidates may be harmed and our ability to generate revenues could be materially impaired.

Even if we obtain regulatory approval for a product candidate, our products will remain subject to regulatory oversight.

Even if we obtain any regulatory approval for our product candidates, they will be subject to ongoing regulatory requirements for manufacturing, labeling, packaging, storing, advertising, promoting, sampling, record-keeping and submitting safety and other post-market information. Any regulatory approvals that we receive for our product candidates also may be subject to a REMS, limitations on the approved indicated uses for which the product may be marketed or to the conditions of approval or contain requirements for potentially costly post-marketing testing, including post-marketing studies or clinical trials, and surveillance to monitor the quality, safety and efficacy of the product. For example, the holder of an approved BLA is obligated to monitor and report adverse events and any failure of a product to meet the specifications in the BLA. FDA guidance advises that patients treated with some types of gene therapy undergo follow-up observations for potential adverse events for as long as 15 years. The holder of an approved BLA also must submit new or supplemental applications and obtain FDA approval for certain changes to the approved product, product labeling or manufacturing process. Advertising and promotional materials must comply with FDA rules and are subject to FDA review, in addition to other potentially applicable federal and state laws.

In addition, product manufacturers and their facilities are subject to payment of user fees and continual review and periodic inspections by the FDA and other regulatory authorities for compliance with cGMP requirements and adherence to commitments made in the BLA or foreign marketing application. If we, or a regulatory authority, discover previously unknown problems with a product, such as adverse events of unanticipated severity or frequency, or problems with the facility where the product is manufactured or such regulatory authority disagrees with the promotion, marketing or labeling of that product, the regulatory authority may impose restrictions relative to that product, the manufacturing facility or us, including requiring recall or withdrawal of the product from the market or suspension of manufacturing.

If we fail to comply with applicable regulatory requirements following approval of any of our product candidates, a regulatory authority may:

- issue a warning letter asserting that we are in violation of the law;
- seek an injunction or impose administrative, civil or criminal penalties or monetary fines;

- suspend or withdraw regulatory approval;
- suspend any ongoing clinical trials;
- refuse to approve a pending BLA or comparable foreign marketing application, or any supplements thereto, submitted by us or our collaboration partners;
- restrict the marketing or manufacturing of the product;
- seize or detain the product or otherwise require the withdrawal of the product from the market;
- refuse to permit the import or export of products; or
- refuse to allow us to enter into supply contracts, including government contracts.

Any government investigation of alleged violations of law could require us to expend significant time and resources in response and could generate negative publicity. The occurrence of any event or penalty described above may inhibit our ability to commercialize our product candidates and adversely affect our business, financial condition, results of operations and prospects.

Further, our ability to develop and market new drug products may be impacted by ongoing litigation challenging the FDA's approval of mifepristone. Specifically, on April 7, 2023, the U.S. District Court for the Northern District of Texas stayed the approval by the FDA of mifepristone, a drug product which was originally approved in 2000 and whose distribution is governed by various conditions adopted under a REMS. In reaching that decision, the District Court made a number of findings that may negatively impact the development, approval and distribution of drug products in the United States. Among other determinations, the District Court held that plaintiffs were likely to prevail in their claim that FDA had acted arbitrarily and capriciously in approving mifepristone without sufficiently considering evidence bearing on whether the drug was safe to use under the conditions identified in its labeling. Further, the District Court read the standing requirements governing litigation in federal court as permitting a plaintiff to bring a lawsuit against the FDA in connection with its decision to approve an NDA or establish requirements under a REMS based on a showing that the plaintiff or its members would be harmed to the extent that FDA's drug approval decision effectively compelled the plaintiffs to provide care for patients suffering adverse events caused by a given drug.

On April 12, 2023, the District Court decision was stayed, in part, by the U.S. Court of Appeals for the Fifth Circuit. Thereafter, on April 21, 2023, the U.S. Supreme Court entered a stay of the District Court's decision, in its entirety, pending disposition of the appeal of the District Court decision in the Court of Appeals for the Fifth Circuit and the disposition of any petition for a writ of certiorari to or the U.S. Supreme Court. The Court of Appeals for the Fifth Circuit held oral argument in the case on May 17, 2023 and, on August 16, 2023, issued its decision. The Court of Appeals declined to order the removal of mifepristone from the market, finding that a challenge to the FDA's initial approval in 2000 is barred by the statute of limitations. But the Court of Appeals did hold that plaintiffs were likely to prevail in their claim that changes allowing for expanded access of mifepristone that FDA authorized in 2016 and 2021 were arbitrary and capricious. On September 8, 2023, the Justice Department and a manufacturer of mifepristone filed petitions for a writ of certiorari, requesting that asked the United States Supreme Court to review the Court of Appeals decision. On December 13, 2023, the U.S. Supreme Court granted these petitions for writ of certiorari for the appeals court decision.

In addition, FDA policies, and those of equivalent foreign regulatory agencies, may change and additional government regulations may be enacted that could prevent, limit or delay regulatory approval of our product candidates. We cannot predict the likelihood, nature or extent of government regulation that may arise from future legislation or administrative action, either in the United States or abroad. If we are slow or unable to adapt to changes in existing requirements or the adoption of new requirements or policies, or if we are not able to maintain regulatory compliance, we may lose any marketing approval that we may have obtained and we may not achieve or sustain profitability, which would harm our business, financial condition, results of operations and prospects.

We face significant competition in an environment of rapid technological change and the possibility that our competitors may achieve regulatory approval before us or develop therapies that are more advanced or effective than ours, which may harm our business and financial condition and our ability to successfully market or commercialize our product candidates.

The biopharmaceutical industry is characterized by intense and dynamic competition to develop new technologies and proprietary therapies. Any product candidates that we successfully develop into products and commercialize may compete with existing therapies and new therapies that may become available in the future. While we believe that our gene therapy platform, vectorized antibody platform, product programs, product candidates and scientific expertise in the fields of proprietary antibodies, gene therapy, and neuroscience provide us with competitive advantages, we face potential competition from various sources, including larger and better-funded pharmaceutical, specialty pharmaceutical and biotechnology companies, as well as from academic institutions, governmental agencies and public and private research institutions.

We are aware of several companies focused on developing their proprietary antibodies or AAV gene therapies in various indications, as well as several companies addressing other methods for modifying genes and regulating gene expression. Any advances in antibody or gene therapy technology made by a competitor may be used to develop therapies that could compete against any of our product candidates.

We expect that our TRACER discovery platform and preclinical programs will compete with a variety of therapies in development, including:

- Our anti-tau antibody and tau silencing gene therapy programs for AD will potentially compete with tau antibodies being developed by Lundbeck Inc., Merck & Co. Inc. in collaboration with Teijin Limited, Roche Genentech Inc. in collaboration with AC Immune SA, Eisai Co., Ltd., Janssen Pharmaceuticals, Inc., UCB S.A., Bristol Myers Squibb Company in collaboration with Prothera Inc., along with several other companies, as well as an antisense oligonucleotide program being developed by Ionis in collaboration with Biogen;

- Our program for a monogenic form of ALS will potentially compete with Tofersen being developed by Biogen, in collaboration with Ionis, and gene therapies being developed by Novartis Gene Therapies, Inc. and uniQure, Inc.; and

- Our TRACER discovery platform will potentially compete with a variety of companies developing AAV capsids, including: 4D Molecular Therapeutics, Inc., Affinia Therapeutics Inc., Apertura Gene Therapy, LLC, Capsida Biotherapeutics, Inc., Capsigen, Inc., Dyno Therapeutics, Inc., Kate Therapeutics, Inc., and Shape Therapeutics Inc.

Many of our potential competitors, alone or with their strategic partners, have substantially greater financial, technical and other resources, such as larger research and development, clinical, marketing and manufacturing organizations. Mergers and acquisitions in the biotechnology and pharmaceutical industries, including recent transactions involving a number of gene therapy companies, may result in even more resources being concentrated among a smaller number of competitors. Smaller and other early-stage companies may also prove to be significant competitors, particularly through collaborative agreements with large and established companies. Our commercial opportunity could be reduced or eliminated if competitors develop and commercialize products that are safer, more effective, have fewer or less severe side effects, are more convenient or are less expensive than any products that we may develop. Competitors also may obtain FDA or other regulatory approval for their products more rapidly or earlier than us or may obtain orphan drug or other marketing exclusivity, which could result in our competitors establishing a strong market position before we are able to enter the market or reducing the number of available subjects for enrollment in our clinical trials to support regulatory submissions and approvals of our product. Additionally, technologies developed or acquired by our competitors may render our potential product candidates uneconomical or obsolete, and we may not be successful in marketing our product candidates against competitors. These third parties also compete with us in recruiting and retaining qualified scientific and management personnel, establishing clinical trial sites, and registering patients for clinical trials.

In addition, as a result of the expiration or successful challenge of our patent rights, we could face more litigation with respect to the validity and scope of patents relating to our competitors' products. The availability of our competitors' products could limit the demand, and the price we are able to charge, for any products that we may develop and commercialize. If we are not able to compete effectively against potential competitors, our business will not grow, and our financial condition and operations will be harmed.

Even if we obtain and maintain approval for our product candidates from the FDA, we may never obtain approval for our product candidates outside of the United States, which would limit our market opportunities and adversely affect our business.

Approval of a product candidate in the United States by the FDA does not ensure approval of such product candidate by regulatory authorities in other countries or jurisdictions, and approval by one foreign regulatory authority does not ensure approval by regulatory authorities in other foreign countries or by the FDA. Sales of our product candidates outside of the United States will be subject to foreign regulatory requirements governing clinical trials and marketing approval. Even if the FDA grants marketing approval for a product candidate, comparable regulatory authorities of foreign countries also must approve the manufacturing and marketing of the product candidates in those countries. Approval procedures vary among jurisdictions and can involve requirements and administrative review periods different from, and more onerous than, those in the United States, including additional preclinical studies or clinical trials or manufacturing control requirements. In many countries outside the United States, a product candidate must be separately approved for reimbursement before it can be approved for sale in that country. In some cases, the price that we intend to charge for our products, if approved, is also subject to approval. We intend to submit a marketing authorization application to EMA for approval of our product candidates in the European Union but obtaining such approval from the European Commission following the opinion of EMA is a lengthy and expensive process. Even if a product candidate is approved, the FDA or the European Commission may limit the indications for which the product may be marketed, require extensive warnings on the product labeling or require expensive and time-consuming additional clinical trials or reporting as conditions of approval. Regulatory authorities in countries outside of the United States and the European Union also have requirements for approval of product candidates with which we must comply prior to marketing in those countries. Obtaining foreign regulatory approvals and compliance with foreign regulatory requirements could result in significant delays, difficulties and costs for us and could delay or prevent the introduction of our product candidates in certain countries.

Further, clinical trials conducted in one country may not be accepted by regulatory authorities in other countries. Regulatory approval for any of our product candidates may be withdrawn. If we fail to comply with the regulatory requirements, our target market will be reduced and our ability to realize the full market potential of our product candidates will be harmed and our business, financial condition, results of operations and prospects will be harmed.

Further, we could face heightened risks with respect to obtaining marketing authorization in the UK as a result of the withdrawal of the UK from the EU, commonly referred to as Brexit. The UK is no longer part of the European Single Market and EU Customs Union. As of January 1, 2021, the Medicines and Healthcare products Regulatory Agency, or the MHRA, became responsible for supervising medicines and medical devices in Great Britain, comprised of England, Scotland and Wales under domestic law, whereas under the terms of the Northern Ireland Protocol, Northern Ireland is currently subject to EU rules. The UK and EU have however agreed to the Windsor Framework which fundamentally changes the existing system under the Northern Ireland Protocol, including with respect to the regulation of medicinal products in the UK Once implemented, the changes introduced by the Windsor Framework will see the MHRA be responsible for approving all medicinal products destined for the entire UK market (i.e., both Great Britain and Northern Ireland), and the EMA will no longer have any role in approving medicinal products destined for Northern Ireland.

In addition, foreign regulatory authorities may change their approval policies and new regulations may be enacted. For instance, the EU pharmaceutical legislation is currently undergoing a complete review process, in the context of the Pharmaceutical Strategy for Europe initiative, launched by the European Commission in November 2020. The European Commission's proposal for revision of several legislative instruments related to medicinal products (potentially reducing the duration of regulatory data protection, revising the eligibility for expedited pathways, etc.) was

published on April 26, 2023. The proposed revisions remain to be agreed and adopted by the European Parliament and European Council and the proposals may therefore be substantially revised before adoption, which is not anticipated before early 2026. The revisions may however have a significant impact on the pharmaceutical industry and our business in the long term.

We expect that we will be subject to additional risks in commercializing any of our product candidates that receive marketing approval outside the United States, including tariffs, trade barriers and regulatory requirements; economic weakness, including inflation, increasing interest rates, or political instability in particular foreign economies and markets; compliance with tax, employment, immigration and labor laws for employees living or traveling abroad; foreign currency fluctuations, which could result in increased operating expenses and reduced revenue, and other obligations incident to doing business in another country; and workforce uncertainty in countries where labor unrest is more common than in the United States.

If approved, our product candidates that are licensed and regulated as biologics may face competition from biosimilars approved through an abbreviated regulatory pathway.

The Biologics Price Competition and Innovation Act of 2009, or BPCIA, was enacted as part of the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Affordability Reconciliation Act, or collectively, the ACA, to establish an abbreviated pathway for the approval of biosimilar and interchangeable biological products. The regulatory pathway establishes legal authority for the FDA to review and approve biosimilar biologics, including the possible designation of a biosimilar as “interchangeable” based on its similarity to an approved biologic. Under the BPCIA, a reference biological product is granted 12 years of data exclusivity from the time of first licensure of the product, and the FDA will not accept an application for a biosimilar or interchangeable product based on the reference biological product until four years after the date of first licensure of the reference product In addition, the licensure of a biosimilar product may not be made effective by the FDA until 12 years from the date on which the reference product was first licensed. During this 12-year period of exclusivity, another company may still develop and receive approval of a competing biologic, so long as its BLA does not rely on the reference product or sponsor’s data, or the company does not submit the application as a biosimilar application.

We believe that any of the product candidates we develop as a biological product under a BLA should qualify for the 12-year period of exclusivity. However, there is a risk that this exclusivity could be shortened due to congressional action or otherwise, or that the FDA will not consider the subject product candidates to be reference products for competing products, potentially creating the opportunity for biosimilar competition sooner than anticipated. Moreover, the extent to which a biosimilar, once approved, will be substituted for any one of the reference products in a way that is similar to traditional generic substitution for non-biological products will depend on a number of marketplace and regulatory factors that are still developing. Nonetheless, the approval of a biosimilar to our product candidates would have a material adverse impact on our business due to increased competition and pricing pressure.

Risks Related to Third Parties

To date, all of our revenue has been derived from our ongoing collaborations and licensing agreements with Neurocrine, Novartis, Alexion, and Sangamo Therapeutics, Inc., or Sangamo, and from our prior collaborations with Sanofi Genzyme, AbbVie Biotechnology Ltd and AbbVie Ireland Unlimited Company, or AbbVie. If any ongoing or future collaboration, option and license, or license agreements were to be terminated, our business financial condition, results of operations and prospects could be harmed.

To date, all of our revenue has been derived from our ongoing collaborations and licensing agreements with Neurocrine, Novartis, Alexion and Sangamo and from our prior collaborations with Sanofi Genzyme Corporation, AbbVie Biotechnology Ltd and AbbVie Ireland Unlimited Company. If any ongoing or future collaboration, option and license, or license agreements were to be terminated, our business financial condition, results of operations and prospects could be harmed. For example, certain of our prior collaborations were terminated. As a result of the terminations of our collaborations with Sanofi Genzyme and AbbVie, we ceased to be eligible to receive option and milestone payments pursuant to the collaborations or to receive royalties in connection with any potential products developed under the collaborations.

On February 2, 2021, Neurocrine notified us that it had elected to terminate the 2019 Neurocrine Collaboration Agreement solely with regards to the VY-AADC Program. This termination became effective August 2, 2021, which we refer to as the Neurocrine VY-AADC Program Termination Effective Date. The 2019 Neurocrine Collaboration Agreement remains in full force and effect for each other program thereunder. Upon the termination of the VY-AADC Program, the license granted by us to Neurocrine regarding the VY-AADC Program expired, and we regained worldwide intellectual property rights to the VY-AADC Program in accordance with the collaboration agreement, and the restrictions on us to develop, manufacture or commercialize a gene therapy product directed to the targets specified in the VY-AADC Program terminated. If Neurocrine were to terminate the remainder of the 2019 Neurocrine Collaboration Agreement, we would become responsible for all research and development expenses relating to the remaining Neurocrine Programs and would not receive any future milestone payments or royalty payments under the 2019 Neurocrine Collaboration Agreement with respect to such programs.

In October 2021, we entered into an option and license agreement with Pfizer, or the Pfizer Agreement, pursuant to which we granted Pfizer options to receive an exclusive license, or the Pfizer License Options, to certain novel capsids we have generated using our TRACER Capsid discovery platform, or TRACER Capsids, to develop and commercialize certain AAV gene therapy candidates comprised of a capsid and specified Pfizer transgenes, or Pfizer Transgenes. Effective as of September 30, 2022, Pfizer exercised a Pfizer License Option with respect to a capsid for the specified Pfizer Transgene for potential treatment of a rare neurological disease. In connection with the exercise of the Pfizer License Option for a rare neurological disease, we granted Pfizer an exclusive, worldwide license, with the right to sublicense, under certain of our intellectual property, the rights to develop and commercialize rare neurological disease products utilizing the capsid candidate and incorporating the corresponding Pfizer Transgene, or the Pfizer Licensed CNS Products. Pfizer did not exercise its option to license a capsid for the potential treatment of a cardiovascular disease. As result, Pfizer's right to exercise a Pfizer License Option for a cardiovascular disease has terminated in accordance with the terms of the Pfizer Agreement and all rights to capsids for that cardiovascular disease have reverted to us. On July 28, 2023, Alexion, AstraZeneca Rare Disease, or Alexion, entered into a definitive purchase and license agreement for preclinical gene therapy assets and enabling technologies from Pfizer. Effective upon the closing of the transaction on September 20, 2023, Alexion acquired all of Pfizer's rights under the Pfizer Agreement and became the successor-in-interest to Pfizer thereunder. We refer to the Pfizer Agreement following the acquisition, as the Alexion Agreement. The acquisition does not impact the material terms of the option and license agreement.

In March 2022, we entered into an option and license agreement with Novartis, or the 2022 Novartis Option and License Agreement, pursuant to which we granted Novartis options to receive an exclusive license to TRACER Capsids to develop and commercialize certain AAV gene therapy candidates comprised of a TRACER Capsid and specified genetic payloads for specific genetic targets. Under the terms of the 2022 Novartis Option and License Agreement, we received an upfront payment of $54.0 million. Effective as of March 1, 2023, Novartis exercised its options to license TRACER Capsids for use in gene therapy programs against two undisclosed neurologic disease targets. With Novartis' option exercise on two targets, we received a $25.0 million option exercise payment in April of 2023, and we are eligible to receive associated potential development, regulatory, and commercial milestone payments, as well as mid- to high-single-digit tiered royalties based on net sales of Novartis products incorporating the licensed capsids. In addition, during the research term, Novartis retains the right to expand the agreement to include options to license capsids for up to two additional rare CNS targets, subject to their availability, for a fee of $18.0 million per target. Under such an expansion, we would be eligible to receive a $12.5 million license option exercise fee for each target exercised, as well as future potential milestone payments per target and mid- to high-single-digit tiered royalties on products incorporating the licensed capsids. Novartis elected not to license a capsid for one CNS target under the 2022 Novartis Option and License Agreement prior to the expiration of the applicable option period. As a result we are no longer eligible to receive development, regulatory, and commercial milestone payments or royalties in connection with this target, and all capsid rights with respect to that target have returned to us.

Our current collaborators or any future collaborator might not be successful in obtaining approvals for the product candidates arising from our collaboration or commercializing or manufacturing the resulting products. Further, such collaborator's objectives in connection with the collaboration may not be consistent with our best interests. With respect to the rights granted to a collaborator by us, the collaborator could take actions that may be adverse to us, or it could halt, slow, or deprioritize its development and commercialization efforts under the collaboration. In any such instances, our business, financial condition, results of operations and prospects could be materially harmed.

We may seek to enter into collaborations, and out-licensing transactions in the future with other third parties. If we are unable to enter into such collaborations or out-licensing transactions, or if these collaborations or out-licensing transactions are not successful, our business could be adversely affected.

We may seek to enter into additional collaborations in the future, including sales, marketing, distribution, development, option, licensing, and/or broader collaboration agreements. For example, on January 8, 2023, we entered into a second collaboration agreement, or the 2023 Neurocrine Collaboration Agreement, with Neurocrine for the research, development, manufacture and commercialization of gene therapy products directed to the gene that encodes GBA1, for the treatment of Parkinson's disease and other diseases associated with GBA1, or the GBA1 Program, and three new programs focused on the research, development, manufacture and commercialization of gene therapies designed to address central nervous system diseases or conditions associated with rare genetic targets, or the 2023 Discovery Programs, and, collectively with the GBA1 Program, the 2023 Neurocrine Programs. On December 28, 2023, we entered into the 2023 Novartis Collaboration Agreement to (a) provide rights to Novartis with respect to certain of our TRACER Capsids for use in the research, development, and commercialization by Novartis of AAV gene therapy products and product candidates, comprising such TRACER Capsids and payloads intended for the treatment of spinal muscular atrophy, or the Novartis SMA Program, and (b) collaborate to develop AAV gene therapy products and product candidates intended for the treatment of Huntington's disease under the Novartis HD Program, in each case, leveraging our TRACER Capsids and other intellectual property controlled by us.

Our likely collaborators, optionees, and licensees include large and mid-size pharmaceutical companies, regional and national pharmaceutical companies, biotechnology companies, and medical device manufacturers. However, we may not be able to enter into additional collaborations or option and license transactions on favorable terms or at all. Our ability to generate revenues from our collaborations and option and license transactions will depend on our and our collaborators', optionees', and licensees' abilities to successfully perform the functions assigned to each of us in these arrangements. In addition, our collaborators, optionees, and licensees might have the ability to abandon research or development projects and terminate applicable agreements. Moreover, an unsuccessful outcome in any clinical trial for which our collaborator, optionee, or licensee is responsible could be harmful to the public perception and prospects of our proprietary antibody program and gene therapy and vectorized antibody platforms.

Our relationship with any current or future collaborators, optionees, or licensees may pose several risks, including the following:

- collaborators, optionees, and licensees have significant discretion in determining the amount and timing of the efforts and resources that they will apply to these collaborations and option and license transactions;
- collaborators, optionees, or licensees may not perform their obligations as expected or desired;
- the preclinical studies and clinical trials conducted as part of these collaborations or by our licensees may not be successful;
- collaborators, optionees, or licensees may not pursue development and commercialization of any product candidates that achieve regulatory approval or may elect not to continue or renew development or commercialization programs based on preclinical study or clinical trial results, changes in the collaborators', optionees', or licensees' strategic focus or available funding or external factors, such as an acquisition, which divert resources or create competing priorities;
- collaborators, optionees, or licensees may delay preclinical studies and clinical trials, provide insufficient funding for preclinical studies and clinical trials, stop a preclinical study or clinical trial or abandon a product candidate, repeat or conduct new preclinical studies or clinical trials or require a new formulation of a product candidate for preclinical studies or clinical trials;

- we may not have access to, or may be restricted from disclosing, certain information regarding product candidates being developed or commercialized under a collaboration or by a licensee and, consequently, may have limited ability to inform our stockholders about the status of such product candidates;

- collaborators, optionees, or licensees could independently develop, or develop with third parties, products that compete directly or indirectly with our product candidates if the collaborators, optionees, or licensees believe that competitive products are more likely to be successfully developed or can be commercialized under terms that are more economically attractive than ours;

- product candidates developed in collaboration with us or by a licensee may be viewed by our collaborators or licensees as competitive with their own product candidates or products, which may cause collaborators or licensees to cease to devote resources to the commercialization of our product candidates;

- a collaborator or licensee with marketing and distribution rights to one or more of our product candidates that achieve regulatory approval may not commit sufficient resources to the marketing and distribution of any such product candidate;

- disagreements with collaborators, optionees, or licensees, including disagreements over proprietary rights, contract interpretation or the preferred course of development of any product candidates, may cause delays or termination of the research, development or commercialization of such product candidates, may lead to additional responsibilities or expenses for us with respect to such product candidates (in the case of collaborations) or may result in litigation or arbitration, any of which would be time-consuming and expensive;

- in collaboration, licensing, and option arrangements where we have licensed intellectual property rights to collaborators, licensees, and optionees who have the right to control prosecution of the licensed intellectual property rights, disputes may arise with respect to the prosecution strategy for the relevant intellectual property rights, which may impair our ability to pursue our preferred prosecution strategy or achieve the desired protection from any relevant patents;

- collaborators, optionees, or licensees may not properly maintain or defend our intellectual property rights or may use our proprietary information in such a way as to invite litigation that could jeopardize or invalidate our intellectual property or proprietary information or expose us to potential litigation;

- disputes may arise with respect to the ownership or inventorship of intellectual property developed pursuant to our collaborations or option and license transactions;

- collaborators, optionees, or licensees may infringe the intellectual property rights of third parties, which may expose us to litigation and potential liability;

- the terms of our collaboration or license agreement may restrict us from entering into certain relationships with other third parties, thereby limiting our options; and

- collaborations may be terminated for the convenience of the collaborator and, if terminated, we could be required to raise additional capital to pursue further development or commercialization of the applicable product candidates.

Collaboration and license agreements may not lead to the development or commercialization of product candidates in the most efficient manner, or at all. If our collaborations or option and license transactions do not result in the successful development and commercialization of products, or if one of our collaborators, optionees, or licensees terminates its agreement with us, we may not receive any future research funding or milestone or royalty payments under

the collaboration or option and license transactions. If we do not receive the funding we expect under these agreements, our development of our product candidates could be delayed, and we may need additional resources to develop our product candidates. In the event we are unable to achieve milestones necessary to demonstrate progress on our programs relevant to our ongoing collaborations with Neurocrine or Novartis, Neurocrine or Novartis may be unwilling to fund these programs at the desired levels or at all, which could require us to fund these programs to a greater extent than we have expected, to decline to pursue certain program objectives or to discontinue one or more of the programs. Additionally, subject to its contractual obligations to us, if a collaborator, optionee, or licensee of ours were to be involved in a business combination, it might deemphasize or terminate the development or commercialization of any product candidate optioned or licensed to it by us. If one of our collaborators, optionees, or licensees terminates its agreement with us, we may find it more difficult to attract new collaborators, optionees, or licensees, and the perception of us in the business and financial communities could be adversely affected. All of the risks relating to product development, regulatory approval and commercialization described in this periodic report also apply to the activities of our collaborators, optionees, and licensees.

We will face significant competition in seeking appropriate collaborators, optionees, and licensees, and the negotiation process is time-consuming and complex. Our ability to reach a definitive collaboration or license agreement with any future collaborators, optionees, and licensees will depend, among other things, upon our assessment of the collaborator's, optionee's, or licensee's resources and expertise, the terms and conditions of the proposed collaboration or option and license transactions and the proposed collaborator's, optionee's, or licensee's evaluation of several factors. Those factors may include the design or results of clinical trials, the likelihood of approval by the FDA or similar regulatory authorities outside the United States, the potential market for the subject product candidate, the costs and complexities of manufacturing and delivering such product candidate to patients, the potential of competing products, the existence of uncertainty with respect to our ownership of technology, which can exist if there is a challenge to such ownership without regard to the merits of the challenge, and industry and market conditions generally. The collaborator, optionee, or licensee may also consider alternative product candidates or technologies for similar indications that may be available to collaborate on and whether such a collaboration or option and license transaction could be more attractive than the one with us for our product candidate. We may also be restricted under future license agreements from entering into agreements on certain terms with potential collaborators, optionees, or licensees. In addition, there have been a significant number of recent business combinations among large pharmaceutical companies that have resulted in a reduced number of potential future collaborators, optionees, and licensees.

If we are unable to reach agreements with suitable collaborators on a timely basis, on acceptable terms, or at all, we may have to curtail the development of a product candidate, reduce or delay its development program or one or more of our other development programs, delay its potential commercialization or reduce the scope of any sales or marketing activities, or increase our expenditures and undertake development or commercialization activities at our own expense. If we elect to fund and undertake development or commercialization activities on our own, we may need to obtain additional expertise and additional capital, which may not be available to us on acceptable terms or at all. If we fail to enter into collaborations or option and license transactions and do not have sufficient funds or expertise to undertake the necessary development and commercialization activities, we may not be able to further develop our product candidates or bring them to market or continue to develop our proprietary antibody program or gene therapy and vectorized antibody platforms and programs. If we license rights to product candidates, we may not be able to realize the benefit of such transactions if we are unable to successfully integrate them with our existing operations and company culture.

We and our collaborators have relied, and we and our collaborators expect to continue to rely, on third parties to conduct, supervise and monitor our preclinical studies and clinical trials, and if these third parties perform in an unsatisfactory manner, our business could be harmed.

We and our collaborators expect to rely on CROs, clinical trial sites, and other vendors to ensure our preclinical studies and clinical trials are conducted properly and on time. We and our collaborators may also engage third parties such as clinical data management organizations, medical institutions and clinical investigators to conduct or assist in our clinical trials or other preclinical and clinical research and development work. While we and our collaborators will have agreements governing their activities, we and our collaborators will have limited influence over their actual performance. We and our collaborators will control only certain aspects of our third-party service providers' activities. Nevertheless, we and our collaborators will be responsible for ensuring that each of our preclinical studies and clinical trials is

conducted in accordance with the applicable protocol, legal, quality, regulatory and scientific standards. Our reliance on these third parties does not relieve us of our regulatory responsibilities. For example, the PD-1101 Phase 1b clinical trial of VY-AADC (NBIb-1817) and the separate PD-1102 Phase 1 clinical trial exploring the delivery of VY-AADC (NBIb-1817) using a posterior trajectory were conducted at several locations. Additionally, we had expected to initiate the planned VYTAL Phase 1 and 2 clinical trial for VY-HTT01 at multiple sites in the United States before our decision to refocus the Huntington's disease program. If any locations terminate a particular clinical trial, we or our collaborators would be required to find other parties or locations to conduct such clinical trial. We and our collaborators may be unable to find a new party to conduct new trials of our product candidates or obtain clinical supply of our product candidates or AAV vectors for such trials. If we or our collaborators elect to internalize some or all activities related to the conduct of our preclinical studies or clinical trials that are currently performed by our third-party service providers, or if we or our collaborators are required to do so due to a service provider's termination of our relationship, then we or our collaborators may be required to source additional technology and personnel in order to perform the relevant activities. We and our collaborators may be unsuccessful in our efforts to internalize some or all relevant activities, either on the desired timeline or at all.

We, our collaborators, and our third-party service providers are required to comply with the FDA's good laboratory practices, or GLPs, and GCPs for conducting, recording and reporting the results of IND-enabling preclinical studies and clinical studies to assure that the data and reported results are credible and accurate and that the rights, integrity and confidentiality of clinical trial participants are protected. We and our collaborators are also required to register ongoing clinical trials and post the results of completed clinical trials on a government-sponsored database, ClinicalTrials.gov, within specified timeframes. The FDA enforces these GLPs and GCPs through periodic inspections of trial sponsors, principal investigators, clinical trial sites, and laboratories at which the FDA may determine that our preclinical studies and clinical trials did not comply with GLPs or GCPs. If we, our collaborators, or our third-party service providers fail to comply with applicable GLPs or GCPs, the preclinical or clinical data generated in our future preclinical studies or clinical trials may be deemed unreliable and the FDA may require us to perform additional preclinical studies or clinical trials before approving the relevant INDs or marketing applications. In addition, our future clinical trials will require a sufficient number of patients to evaluate the safety and effectiveness of our product candidates. Accordingly, if we, our collaborators, or our third-party service providers fail to comply with these regulations or fail to recruit a sufficient number of patients, we may be required to repeat such preclinical studies or clinical trials, which would delay the regulatory approval process. Failure to comply can also result in fines, adverse publicity, and civil and criminal sanctions.

Our third-party service providers are not our employees, and we and our collaborators are therefore unable to directly monitor whether or not they devote sufficient time, attention, expertise and resources to our clinical and nonclinical programs. These third-party service providers may also have relationships with other commercial entities, including our competitors, for whom they may also be conducting clinical trials or other drug development activities that could harm our competitive position. If our third-party service providers do not successfully carry out their contractual duties or obligations, fail to meet expected deadlines, or if the quality or accuracy of the preclinical or clinical data they obtain is compromised due to the failure to adhere to our clinical protocols or regulatory requirements, or for any other reasons, our preclinical studies or clinical trials may be extended, delayed or terminated, and we may not be able to obtain regulatory approval for, or successfully commercialize our product candidates. As a result, our financial results and the commercial prospects for our product candidates could be harmed, our costs could increase, and our ability to generate revenues could be delayed.

Risks Related to Manufacturing

Our gene therapies are novel, complex and difficult to manufacture. We could experience manufacturing problems that result in delays in the development or commercialization of our product candidates or otherwise harm our business.

The manufacture of gene therapy products is technically complex and necessitates substantial expertise and capital investment. Production difficulties caused by unforeseen events may delay the availability of material for our clinical studies. To meet the requirements of our current and planned future trials we have developed a flexible manufacturing platform that is based on proprietary technology and provides a scalable process for preclinical and

clinical AAV production. We are using a HEK 293 based transient transfection manufacturing process to support our preclinical research activities. We also have expertise with the baculovirus/Sf9 AAV production system, a technology for producing AAV gene therapy vectors at scale in insect-derived cells, which we have used for our clinical development activities in the past and may use in the future for clinical development activities. As the field advances, we will continue to evaluate additional novel manufacturing technologies that may be suitable for future clinical and commercial manufacturing.

We presently contract with third parties for the manufacturing of our program materials for our proprietary antibody and gene therapy product candidates. We have also built an onsite, state-of-the-art process research and development facility to enable the manufacturing of preclinical AAV gene therapy vectors for large animal studies including IND enabling GLP toxicology materials. We are currently assessing our manufacturing capabilities, and we do not currently have our own clinical or commercial scale manufacturing. The use of contract manufacturing and reliance on collaboration partners is relatively cost-efficient and eliminates the need for our direct investment in manufacturing facilities and additional staff early in development. Although we rely on contract manufacturers, we have personnel with manufacturing and quality experience to oversee our contract manufacturers.

To date, our third-party manufacturers have met our manufacturing requirements for our program materials. We expect third-party manufacturers to be capable of providing sufficient quantities of our program materials to meet anticipated clinical trial scale demands. To meet our projected needs for clinical and commercial manufacturing, third parties with whom we currently work might need to increase their scale of production or we may need to secure additional suppliers as part of our external manufacturing network. We believe that there are alternate sources of supply for our program materials that can satisfy our clinical and commercial requirements, although we cannot be certain that identifying and establishing relationships and technology transfers with such sources, if necessary, would not result in significant delay or material additional costs. However, if a third-party manufacturer decided to not enter into a new contract with us for program materials or if they did not have the capacity to meet our needs for program materials, we may be required to contract with additional suppliers on terms which may be less favorable to us or would result in additional material costs.

To date, our third-party manufacturers have met our quality standards for our program materials. The manufacturers of pharmaceutical products must comply with strictly enforced cGMP requirements, state and federal regulations, as well as foreign requirements when applicable. Any failure by us or our contract manufacturing organizations to adhere to or document compliance to such regulatory requirements could lead to a delay or interruption in the availability of our program materials for clinical study. If we or our manufacturers were to fail to comply with the FDA, EMA, or other regulatory authority, it could result in sanctions being imposed on us, including clinical holds, fines, injunctions, civil penalties, delays, suspension or withdrawal of approvals, license revocation, seizures or recalls of product candidates or products, operating restrictions and criminal prosecutions, any of which could significantly and adversely affect supplies of our product candidates. Our potential future dependence upon others for the manufacture of our product candidates may also adversely affect our future profit margins and our ability to commercialize any product candidates that receive regulatory approval on a timely and competitive basis.

Biological products are inherently difficult to manufacture. Our program materials are manufactured using technically complex processes requiring specialized equipment and facilities, highly specific raw materials, cells, and reagents, and other production constraints. Several of these raw materials, cells, and reagents are provided by a limited number of suppliers. Even though we aim to have backup supplies and suppliers of raw materials, cells, and reagents whenever possible, we cannot be certain they will be sufficient if our primary sources are unavailable. A shortage of a critical raw material, cell line, or reagent, or a technical issue during manufacturing may lead to delays in clinical development or commercialization plans. Any changes in the manufacturing of components of the raw materials we use could result in unanticipated or unfavorable effects on our manufacturing processes, including delays.

Delays in obtaining regulatory approval of our or our collaborators' manufacturing processes and facilities or disruptions in such manufacturing processes may delay or disrupt our commercialization efforts. Until recently, no cGMP gene therapy manufacturing facility in the United States had received approval from the FDA for the manufacture of an approved gene therapy product.

Before we can begin to commercially manufacture a product candidate in our own facility, or the facility of a collaborator, we must obtain regulatory approval from the FDA for our manufacturing process and our collaborator's facility. A manufacturing authorization must also be obtained from the appropriate European Union regulatory authorities. Until recently, no cGMP gene therapy manufacturing facility in the United States had received approval from the FDA for the manufacture of an approved gene therapy product and, therefore, the timeframe required for us to obtain such approval is uncertain. In addition, we must pass a pre-approval inspection of our or our collaborator's manufacturing facility by the FDA and other relevant regulatory authorities before any of our product candidates can obtain marketing approval. In order to obtain approval, we will need to ensure that all of our processes, methods and equipment are compliant with cGMP, and perform extensive audits of vendors, contract laboratories and suppliers. If any of our vendors, contract laboratories or suppliers is found to be out of compliance with cGMP, we may experience delays or disruptions in manufacturing while we work with these third parties to remedy the violation or while we work to identify suitable replacement vendors. The cGMP requirements govern quality control of the manufacturing process and documentation policies and procedures. In complying with cGMP, we will be obligated to expend time, money and effort in production, record keeping and quality control to assure that the product meets applicable specifications and other requirements. If we fail to comply with these requirements, we would be subject to possible regulatory action and may not be permitted to sell any products that we may develop.

Failure to comply with ongoing regulatory requirements could cause us to suspend production or put in place costly or time-consuming remedial measures.

The regulatory authorities may, at any time, following approval of a product for sale, audit the manufacturing facilities for such product or institute biennial inspections. If any such inspection or audit identifies a failure to comply with applicable regulations, or if a violation of product specifications or applicable regulations occurs independent of such an inspection or audit, the relevant regulatory authority may require remedial measures that may be costly or time-consuming to implement and that may include the temporary or permanent suspension of a clinical trial or commercial sales or the temporary or permanent closure of a manufacturing facility. Any such remedial measures imposed upon our third-party manufacturers, our collaborators, or us could harm our business, financial condition, results of operations and prospects.

If our third-party manufacturers, our collaborators, or we fail to comply with applicable cGMP regulations, FDA and foreign regulatory authorities can impose regulatory sanctions including, among other things, refusal to approve a pending application for a new product candidate or suspension or revocation of a pre-existing approval. Such an occurrence may cause our business, financial condition, results of operations and prospects to be harmed.

Additionally, if supply from any third-party manufacturers is delayed or interrupted, there could be a significant disruption in the supply of our clinical or commercial material. We have agreements in place with our contract manufacturers pursuant to which we are collaborating on cGMP manufacturing processes and analytical methods for the manufacture of our proprietary antibody and AAV product candidates. Therefore, if we are unable to enter into an agreement with our contract manufacturers to manufacture clinical or commercial material for our product programs, or if our agreement with our contract manufacturers were terminated, we would have to find suitable alternative manufacturers. This could delay our or our collaborators' ability to conduct clinical trials or commercialize our current and future product candidates. The regulatory authorities also may require additional trials if a new manufacturer is relied upon for commercial production. Switching manufacturers may involve substantial costs and could result in a delay in our desired clinical and commercial timelines.

Any contamination in the manufacturing process for our products or product candidates, shortages of raw materials, cells or reagents, or failure of any of our key suppliers to deliver necessary components could result in delays in our clinical development or marketing schedules.

Given the nature of biologics manufacturing, there is a risk of contamination. Any contamination could adversely affect our ability to produce product candidates on schedule and could, therefore, harm our results of operations and cause reputational damage.

Some of the raw materials required in our manufacturing process are derived from biologic sources. Such raw materials are difficult to procure and may be subject to contamination or recall. A material shortage, contamination, recall or restriction on the use of biologically derived substances in the manufacture of our product candidates could adversely impact or disrupt the commercial manufacturing or the production of clinical material, which could adversely affect our development timelines and our business, financial condition, results of operations and prospects.

Failure to obtain access to or to protect intellectual property related to the manufacturing of our products or product candidates may result in changes, delays and/or inability to manufacture such products or product candidates.

The intellectual property related to the manufacture of biological products is complex. If we are unable to maintain control of manufacturing technology such as our trade secrets, or we are unable to protect ongoing improvements comprehensively and in a sufficient number of jurisdictions, it would impact our ability to produce products for commercial sale or product candidates for preclinical testing or clinical trials and our development timelines and operations timelines could be adversely affected.

We presently manufacture our AAV product candidates using a mammalian cell system. We are aware of third parties which also use this system in the manufacture of their products and who hold intellectual property on their AAV manufacturing systems. If we determine that access to certain third-party intellectual property is necessary for the manufacturing of our products and product candidates and are unable to license or otherwise access this intellectual property, it would impact our ability to produce products for commercial sale or product candidates for preclinical testing or clinical trials and our development timelines and operations timelines could be adversely affected.

Risks Related to Our Business Operations

We may not be successful in our efforts to identify or discover additional product candidates and may fail to capitalize on programs or product candidates that may be a greater commercial opportunity, or for which there is a greater likelihood of success.

The success of our business depends upon our ability to identify, develop and commercialize product candidates generated through our proprietary antibody program and our gene therapy and vectorized antibody platforms and programs. Research programs to identify new product candidates require substantial technical, financial and human resources. Our product candidates are in preclinical development. Our current portfolio of product candidates is subject to change as we continue to conduct preclinical testing and to develop product candidates and prioritize or abandon product candidates based on such results and other factors. For example, in August 2022, we announced a re-prioritization of our portfolio based on a review evaluating our programs based on, among other things, our assessment of their potential for competitive differentiation, the efficiency of such product candidate's path to human proof of biology or proof of mechanism (reflecting the availability of validated biomarkers), unmet medical need, commercial opportunity, and alignment with our overall strategy, as well as supportive preclinical data. We may also fail to identify other product candidates for clinical development for several reasons. For example, our research may be unsuccessful in identifying potential product candidates or our potential product candidates may be shown to have harmful side effects, may be commercially impracticable to manufacture or may have other characteristics that may make the products unmarketable or unlikely to receive marketing approval.

Additionally, because we have limited resources, we may forego or delay pursuit of opportunities with certain programs or product candidates or for indications that later prove to have greater commercial potential. Similar to our prior investments with regard to our VY-AADC (NBIb-1817) and VY-HTT01 programs, our spending on current and

future research and development programs may not yield any commercially viable products. If we do not accurately evaluate the commercial potential for a particular product candidate, we may relinquish valuable rights to that product candidate through strategic collaboration, option and license, or other arrangements in cases in which it would have been more advantageous for us to retain sole development and commercialization rights to such product candidate. Alternatively, we may allocate internal resources to a product candidate in a therapeutic area in which it would have been more advantageous to enter into a partnering arrangement. Several of our current preclinical programs have previously been part of collaborations with third parties. While we have invested significant resources in these programs, we may decide in the future to cease development activities on one or more of them.

If any of these events occur, we may be forced to abandon our development efforts with respect to a particular product candidate or fail to develop a potentially successful product candidate, which could harm our business, financial condition, results of operations and prospects.

Our future success depends on our ability to retain key members of our management and research and development teams, and to attract, retain and motivate qualified personnel.

We are highly dependent on the management, technical, and scientific expertise of principal members of our management, scientific, and clinical teams. While we have entered into employment agreements or offer letters with each of our executive officers, any of them could leave our employment at any time, as all of our employees are “at will” employees. We currently do not have “key person” insurance on any of our employees. The loss of the services of one or more of our current employees might impede the achievement of our research, development and commercialization objectives.

Recruiting and retaining qualified employees, consultants and advisors for our business, including scientific and technical personnel, is critical to our success. There currently is a shortage of skilled individuals with substantial gene therapy experience, which is likely to continue. As a result, competition for skilled personnel, including in gene therapy research and vector manufacturing, is intense and the turnover rate can be high. We may not be able to attract and retain personnel on acceptable terms, if at all, given the competition among numerous pharmaceutical and biotechnology companies and academic institutions for individuals with similar skill sets. Our consultants and advisors may be employed by employers other than us and may have commitments under consulting or advisory contracts with other entities that may limit their availability to us. In addition, failure to succeed in preclinical or clinical trials or applications for marketing approval, the termination of relationships with collaborators, and the reduction of our workforce in connection with the development of a new portfolio and platform strategy may make it more challenging to recruit and retain qualified personnel. The inability to recruit, or loss of services of, certain executives, key employees, consultants or advisors, may impede the progress of our research, development and commercialization objectives and could harm our business, financial condition, results of operations and prospects.

If we are unable to manage expected growth in the scale and complexity of our operations, our performance may suffer.

If we are successful in executing our business strategy, we will need to expand our managerial, operational, financial and other systems and resources to manage our operations, continue our research and development activities and, in the longer term, build a commercial infrastructure to support commercialization of any of our product candidates that are approved for sale. We can provide no assurances that we will have sufficient resources in the future to manage all of our planned programs. Future growth would impose significant added responsibilities on members of management, may lead to significant added costs, and may divert our management and business development resources. It is likely that our management, finance, development personnel, systems and facilities currently in place may not be adequate to support this future growth. Our need to effectively manage our operations, growth and product candidates requires that we continue to develop more robust business processes and improve our systems and procedures in each of these areas and to attract and retain sufficient numbers of talented employees. We may be unable to successfully implement these tasks on a larger scale and, accordingly, may not achieve our research, development and growth goals.

Our employees, principal investigators, consultants and commercial partners may engage in misconduct or other improper activities, including non-compliance with regulatory standards and requirements and insider trading.

We are exposed to the risk of fraud or other misconduct by our employees, principal investigators, consultants, collaborators, and commercial partners. Misconduct by these parties could include intentional failures to comply with FDA regulations or the regulations applicable in the European Union and other jurisdictions, provide accurate information to the FDA, the European Commission and other regulatory authorities, comply with healthcare fraud and abuse laws and regulations in the United States and abroad, report financial information or data accurately or disclose unauthorized activities to us. In particular, sales, marketing and business arrangements in the healthcare industry are subject to extensive laws and regulations intended to prevent fraud, misconduct, kickbacks, self-dealing and other abusive practices. These laws and regulations restrict or prohibit a wide range of pricing, discounting, marketing and promotion, sales commission, customer incentive programs and other business arrangements. Such misconduct also could involve the improper use of information obtained in the course of clinical trials or interactions with the FDA or other regulatory authorities, which could result in regulatory sanctions and cause serious harm to our reputation. We have adopted a code of conduct applicable to all of our employees, but it is not always possible to identify and deter employee misconduct, and the precautions we take to detect and prevent this activity may not be effective in controlling unknown or unmanaged risks or losses or in protecting us from government investigations or other actions or lawsuits stemming from a failure to comply with these laws or regulations. If any such actions are instituted against us, and we are not successful in defending ourselves or asserting our rights, those actions could have a significant impact on our business, financial condition, results of operations and prospects, including the imposition of significant fines or other sanctions.

Current and future legislation may increase the difficulty and cost for us and any collaborators to obtain marketing approval of and commercialize our product candidates and affect the prices we, or they, may obtain.

In the United States and foreign jurisdictions, there have been a number of legislative and regulatory changes and proposed changes regarding the healthcare system that could prevent or delay marketing approval of our product candidates, restrict or regulate post-approval activities and affect our ability, or the ability of any collaborators, to profitably sell any products for which we obtain marketing approval. We expect that current laws, as well as other healthcare reform measures that may be adopted in the future, may result in more rigorous coverage criteria and in additional downward pressure on the price that we, or any collaborators, may receive for any approved products. If reimbursement of our products is unavailable or limited in scope, our business could be materially harmed.

In March 2010, President Obama signed the ACA into law. In addition, other legislative changes have been proposed and adopted since the ACA was enacted. In August 2011, the Budget Control Act of 2011, among other things, created measures for spending reductions by Congress. A Joint Select Committee on Deficit Reduction, tasked with recommending a targeted deficit reduction of at least $1.2 trillion for the years 2013 through 2021, was unable to reach required goals, thereby triggering the legislation's automatic reduction to several government programs. These changes included aggregate reductions to Medicare payments to providers of up to 2% per fiscal year, which went into effect in April 2013 and will remain in effect through 2030 under the Coronavirus Aid, Relief, and Economic Security Act, or the CARES Act. The American Taxpayer Relief Act of 2012, among other things, reduced Medicare payments to several providers and increased the statute of limitations period for the government to recover overpayments to providers from three to five years. These laws may result in additional reductions in Medicare and other healthcare funding and otherwise affect the prices we may obtain for any of our product candidates for which we may obtain regulatory approval or the frequency with which any such product candidate is prescribed or used.

Since enactment of the ACA, there have been, and continue to be, numerous legal challenges and Congressional actions to repeal and replace provisions of the law. For example, with enactment of the Tax Cuts and Jobs Act of 2017, or the TCJA, which was signed by President Trump on December 22, 2017, Congress repealed the "individual mandate." The repeal of this provision, which requires most Americans to carry a minimal level of health insurance, became effective in 2019. Further, on December 14, 2018, a U.S. District Court judge in the Northern District of Texas ruled that the individual mandate portion of the ACA is an essential and inseverable feature of the ACA, and therefore because the mandate was repealed as part of the TCJA, the remaining provisions of the ACA are invalid as well. On December 18, 2019, the Court of Appeals for the Fifth Circuit affirmed the lower court's ruling that the individual mandate portion of

the ACA is unconstitutional and it remanded the case to the district court for reconsideration of the severability question and additional analysis of the provisions of the ACA. Thereafter, the U.S. Supreme Court agreed to hear this case. Oral argument in the case took place on November 10, 2020. On June 17, 2021, the U.S. Supreme Court dismissed this case after finding that plaintiffs do not have standing to challenge the constitutionality of the ACA. Litigation and legislation over the ACA are likely to continue, with unpredictable and uncertain results.

The Trump Administration also took executive actions to undermine or delay implementation of the ACA, including directing federal agencies with authorities and responsibilities under the ACA to waive, defer, grant exemptions from, or delay the implementation of any provision of the ACA that would impose a fiscal or regulatory burden on states, individuals, healthcare providers, health insurers, or manufacturers of pharmaceuticals or medical devices. On January 28, 2021, however, President Biden issued a new Executive Order which directs federal agencies to reconsider rules and other policies that limit Americans' access to health care, and consider actions that will protect and strengthen that access. Under this Executive Order, federal agencies are directed to re-examine: policies that undermine protections for people with pre-existing conditions, including complications related to COVID-19; demonstrations and waivers under Medicaid and the ACA that may reduce coverage or undermine the programs, including work requirements; policies that undermine the Health Insurance Marketplace or other markets for health insurance; policies that make it more difficult to enroll in Medicaid and the ACA; and policies that reduce affordability of coverage or financial assistance, including for dependents.

The prices of prescription pharmaceuticals in the United States and foreign jurisdictions are subject to considerable legislative and executive actions and could impact the prices we obtain for our drug products, if and when approved.

The prices of prescription pharmaceuticals have also been the subject of considerable discussion in the United States and other jurisdictions. To date, there have been several recent U.S. congressional inquiries, as well as proposed and enacted state and federal legislation designed to, among other things, bring more transparency to pharmaceutical pricing, review the relationship between pricing and manufacturer patient programs, reduce the costs of pharmaceuticals under Medicare and Medicaid, and reform government program reimbursement methodologies for products. In 2020, former President Trump issued several executive orders intended to lower the costs of prescription products and certain provisions in these orders have been incorporated into regulations. These regulations include an interim final rule implementing a most favored nation model for prices that would tie Medicare Part B payments for certain physician-administered pharmaceuticals to the lowest price paid in other economically advanced countries, effective January 1, 2021. That rule, however, has been subject to a nationwide preliminary injunction and, on December 29, 2021, the Centers for Medicare & Medicaid Services, or CMS, issued a final rule to rescind it. With issuance of this rule, CMS stated that it will explore all options to incorporate value into payments for Medicare Part B pharmaceuticals and improve beneficiaries' access to evidence-based care.

In addition, in October 2020, the Department of Health and Human Services, or the HHS and the FDA published a final rule allowing states and other entities to develop a Section 804 Importation Program, or SIP, to import certain prescription drugs from Canada into the United States. That regulation was challenged in a lawsuit by the Pharmaceutical Research and Manufacturers of America, or PhRMA, but the case was dismissed by a federal district court in February 2023 after the court found that PhRMA did not have standing to sue HHS. Nine states (Vermont, Colorado, Florida, Maine, New Mexico, New Hampshire, North Dakota, Texas, and Wisconsin) have passed laws allowing for the importation of drugs from Canada. Certain of these states have submitted Section 804 Importation Program proposals and are awaiting FDA approval. On January 5, 2023, the FDA approved Florida's plan for Canadian drug importation.

Further, in November 2020, the HHS finalized a regulation removing safe harbor protection for price reductions from pharmaceutical manufacturers to plan sponsors under Part D, either directly or through pharmacy benefit managers, unless the price reduction is required by law. The rule also creates a new safe harbor for price reductions reflected at the point-of-sale, as well as a safe harbor for certain fixed fee arrangements between pharmacy benefit managers and manufacturers. Pursuant to court order, the removal and addition of the aforementioned safe harbors were delayed and recent legislation imposed a moratorium on implementation of the rule until January 1, 2026. The Inflation Reduction Act of 2022, or IRA, further delayed implementation of this rule to January 1, 2032.

In July 2021, President Biden signed Executive Order 14063, which focuses on, among other things, the price of pharmaceuticals. The order directed the HHS to create a plan within 45 days to combat "excessive pricing of prescription pharmaceuticals and enhance domestic pharmaceutical supply chains, to reduce the prices paid by the federal government for such pharmaceuticals, and to address the recurrent problem of price gouging." In September 2021, the HHS released its plan to reduce pharmaceutical prices. The key features of that plan are to: (a) make pharmaceutical prices more affordable and equitable for all consumers and throughout the health care system by supporting pharmaceutical price negotiations with manufacturers; (b) improve and promote competition throughout the prescription pharmaceutical industry by supporting market changes that strengthen supply chains, promote biosimilars and generic drugs, and increase transparency; and (c) foster scientific innovation to promote better healthcare and improve health by supporting public and private research and making sure that market incentives promote discovery of valuable and accessible new treatments.

More recently, on August 16, 2022, the IRA was signed into law by President Biden. The new legislation has implications for Medicare Part D, which is a program available to individuals who are entitled to Medicare Part A or enrolled in Medicare Part B to give them the option of paying a monthly premium for outpatient prescription drug coverage. Among other things, the IRA requires manufacturers of certain drugs to engage in price negotiations with Medicare (beginning in 2026), with prices that can be negotiated subject to a cap; imposes rebates under Medicare Part B and Medicare Part D to penalize price increases that outpace inflation (first due in 2023); and replaces the Part D coverage gap discount program with a new discounting program (beginning in 2025). The IRA permits the Secretary of the HHS to implement many of these provisions through guidance, as opposed to regulation, for the initial years.

Specifically, with respect to price negotiations, Congress authorized Medicare to negotiate lower prices for certain costly single-source drug and biologic products that do not have competing generics or biosimilars and are reimbursed under Medicare Part B and Part D. CMS may negotiate prices for ten high-cost drugs paid for by Medicare Part D starting in 2026, followed by 15 Part D drugs in 2027, 15 Part B or Part D drugs in 2028, and 20 Part B or Part D drugs in 2029 and beyond. This provision applies to drug products that have been approved for at least 9 years and biologics that have been licensed for 13 years, but it does not apply to drugs and biologics that have been approved for a single rare disease or condition. Nonetheless, since CMS may establish a maximum price for these products in price negotiations, we would be fully at risk of government action if our products became the subject of Medicare price negotiations. Moreover, given the risk that could be the case, these provisions of the IRA may also further heighten the risk that we would not be able to achieve the expected return on any drug products or full value of our patents protecting our products if prices are set after such products have been on the market for nine years.

Further, the legislation subjects drug manufacturers to civil monetary penalties and a potential excise tax for failing to comply with the legislation by offering a price that is not equal to or less than the negotiated "maximum fair price" under the law or for taking price increases that exceed inflation. The legislation also requires manufacturers to pay rebates for drugs in Medicare Part D whose price increases exceed inflation. The new law also caps Medicare out-of-pocket drug costs at an estimated $4,000 a year in 2024 and, thereafter beginning in 2025, at $2,000 a year. In addition, the IRA potentially raises legal risks with respect to individuals participating in a Medicare Part D prescription drug plan who may experience a gap in coverage if they required coverage above their initial annual coverage limit before they reached the higher threshold, or "catastrophic period" of the plan. Individuals requiring services exceeding the initial annual coverage limit and below the catastrophic period must pay 100% of the cost of their prescriptions until they reach the catastrophic period. Among other things, the IRA contains many provisions aimed at reducing this financial burden on individuals by reducing the co-insurance and co-payment costs, expanding eligibility for lower income subsidy plans, and price caps on annual out-of-pocket expenses, each of which could have potential pricing and reporting implications.

On June 6, 2023, Merck & Co. filed a lawsuit against the HHS and CMS asserting that, among other things, the IRA's Drug Price Negotiation Program for Medicare constitutes an uncompensated taking in violation of the Fifth Amendment of the Constitution. Subsequently, a number of other parties, including the U.S. Chamber of Commerce, Bristol Myers Squibb Company, the PhRMA, Astellas, Novo Nordisk, Janssen Pharmaceuticals, Novartis, AstraZeneca and Boehringer Ingelheim, also filed lawsuits in various courts with similar constitutional claims against the HHS and CMS. We expect that litigation involving these and other provisions of the IRA will continue, with unpredictable and uncertain results.

Accordingly, while it is currently unclear how the IRA will be effectuated, we cannot predict with certainty what impact any federal or state health reforms will have on us, but such changes could impose new or more stringent regulatory requirements on our activities or result in reduced reimbursement for our products, any of which could adversely affect our business, results of operations and financial condition.

At the state level, individual states are increasingly aggressive in passing legislation and implementing regulations designed to control pharmaceutical and biological product pricing, including price or patient reimbursement constraints, discounts, restrictions on certain product access and marketing cost disclosure and transparency measures, and, in some cases, designed to encourage importation from other countries and bulk purchasing. In addition, health care organizations and individual hospitals are increasingly using bidding procedures to determine what pharmaceutical products and which suppliers will be included in their prescription drug and other health care programs. These measures could reduce the ultimate demand for our products, once approved, or put downward pressure on our product pricing. We expect that additional state and federal healthcare reform measures will be adopted in the future, any of which could limit the amounts that federal and state governments will pay for healthcare products and services, which could result in reduced demand for our product candidates or additional pricing pressures.

In other countries, particularly the countries of the European Union, the pricing of prescription pharmaceuticals is subject to governmental control and access. In these countries, pricing negotiations with governmental authorities can take considerable time after the receipt of marketing approval for a product. To obtain reimbursement or pricing approval in some countries, we, or our collaborators, may be required to conduct a clinical trial that compares the cost-effectiveness of our products or product candidates to other available therapies. If reimbursement of our products or product candidates is unavailable or limited in scope or amount, or if pricing is set at unsatisfactory levels, our business could be materially harmed.

These measures, as well as others adopted in the future, may result in additional downward pressure on the price that we receive for any approved product we or our collaborators might bring to market. Accordingly, such reforms, if enacted, could have an adverse effect on anticipated revenue from that we, or our collaborators, may successfully develop and for which we, or they, may obtain marketing approval and may affect our overall financial condition and ability to develop or commercialize product candidates.

Our relationships with healthcare providers, physicians and third-party payors will be subject, directly or indirectly, to applicable anti-kickback, fraud and abuse and other healthcare laws and regulations, which, in the event of a violation, could expose us to criminal sanctions, civil penalties, contractual damages, reputational harm and diminished profits and future earnings.

Healthcare providers, physicians and third-party payors will play a primary role in the recommendation and prescription and use of our products and any product candidates for which we obtain marketing approval. Our future arrangements with healthcare providers, physicians and third-party payors may expose us to broadly applicable fraud and abuse and other healthcare laws and regulations that may constrain the business or financial arrangements and relationships through which we market, sell and distribute any products for which we obtain marketing approval. Restrictions under applicable federal and state healthcare laws and regulations include the following:

- the federal Anti-Kickback Statute prohibits, among other things, persons from knowingly and willfully soliciting, offering, receiving or providing remuneration, directly or indirectly, in cash or in kind, to induce or reward, or in return for, either the referral of an individual for, or the purchase, order or recommendation or arranging of, any good or service, for which payment may be made under a federal healthcare program such as Medicare and Medicaid;

- the federal False Claims Act imposes criminal and civil penalties, including through civil whistleblower or qui tam actions, against individuals or entities for, among other things, knowingly presenting, or causing to be presented, false or fraudulent claims for payment by a federal healthcare program or making a false statement or record material to payment of a false claim or avoiding, decreasing or concealing an obligation to pay money to the federal government, with potential liability including mandatory treble damages and significant per-claim penalties;

- the federal Health Insurance Portability and Accountability Act of 1996, or HIPAA, imposes criminal and civil liability for executing a scheme to defraud any healthcare benefit program or making false statements relating to healthcare matters;

- HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act, and its implementing regulations, also imposes obligations, including mandatory contractual terms, with respect to safeguarding the privacy, security and transmission of individually identifiable health information;

- the federal Physician Payments Sunshine Act requires applicable manufacturers of covered products to report payments and other transfers of value to physicians, other healthcare providers, and teaching hospitals; and

- analogous state and foreign laws and regulations, such as state anti-kickback, false claims, and transparency laws, may apply to sales or marketing arrangements and claims involving healthcare items or services reimbursed by non-governmental third-party payors, including private insurers. Some state laws require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government and may require product manufacturers to report information related to payments and other transfers of value to physicians and other healthcare providers or marketing expenditures. State and foreign laws also govern the privacy and security of health information in some circumstances, many of which differ from each other in significant ways and often are not preempted by HIPAA, thus complicating compliance efforts.

If our operations or the operations of our present and future collaborators are found to be in violation of any of the laws described above or any government regulations that apply to us or them, we or they may be subject to penalties, including civil and criminal penalties, damages, fines, and the curtailment or restructuring of our operations. Any penalties, damages, fines, curtailment or restructuring of our operations could adversely affect our or their financial results. We are developing and implementing a corporate compliance program designed to ensure that we will market and sell any future products that we successfully develop from our product candidates in compliance with all applicable laws and regulations, but we cannot guarantee that this program will protect us from governmental investigations or other actions or lawsuits stemming from a failure to be in compliance with such laws or regulations. If any such actions are instituted against us and we are not successful in defending ourselves or asserting our rights, those actions could have a significant impact on our business, including the imposition of significant fines or other sanctions.

Efforts to ensure that our business with third parties will comply with applicable healthcare laws and regulations will involve substantial costs. For example, we are engaged in an ongoing effort to improve our healthcare compliance program and establish a more robust compliance infrastructure. We may fail to establish appropriate compliance measures, and even with a stronger program in place, it is possible that governmental authorities will conclude that our business practices may not comply with current or future statutes, regulations or case law involving applicable fraud and abuse or other healthcare laws and regulations. If our operations are found to be in violation of any of these laws or any other governmental regulations that may apply to us, we may be subject to significant civil, criminal and administrative penalties, damages, fines, imprisonment, exclusion of products from government funded healthcare programs, such as Medicare and Medicaid, and the curtailment or restructuring of our operations. If any of the physicians or other healthcare providers or entities with whom we expect to do business is found to be not in compliance with applicable laws, they may be subject to criminal, civil or administrative sanctions, including exclusions from government funded healthcare programs.

The provision of benefits or advantages to physicians to induce or encourage the prescription, recommendation, endorsement, purchase, supply, order or use of medicinal products is also prohibited in other jurisdictions. The provision of benefits or advantages to physicians is governed by anti bribery laws of European Union Member States and the UK Bribery Act 2010.

Payments made to physicians in certain European Union Member States must be publicly disclosed and often must be the subject of prior notification and approval by the physician's employer, his or her competent professional

organization and/or the regulatory authorities of the individual European Union Member States. Failure to comply with these requirements could result in reputational risk, public reprimands, administrative penalties, fines or imprisonment.

We are subject to stringent privacy laws, information security laws, regulations, policies and contractual obligations related to data privacy and security and changes in such laws, regulations, policies, and contractual obligations or our failure to comply with such requirements could subject us to significant fines and penalties, which may have a material adverse effect on our business, financial condition or results of operations.

We are subject to data privacy and protection laws and regulations that apply to the collection, transmission, storage and use of personally-identifying information, which among other things, impose certain requirements relating to the privacy, security and transmission of personal information, including comprehensive regulatory systems in the United States, European Union, and United Kingdom. The legislative and regulatory landscape for privacy and data protection continues to evolve in jurisdictions worldwide, and there has been an increasing focus on privacy and data protection issues with the potential to affect our business. Failure to comply with any of these laws and regulations could result in enforcement action against us, including fines, imprisonment of company officials and public censure, claims for damages by affected individuals, costly changes to our business practices, damage to our reputation and loss of goodwill, any of which could have a material adverse effect on our business, financial condition, results of operations or prospects.

There are numerous U.S. federal and state laws and regulations related to the privacy and security of personal information. In particular, regulations promulgated pursuant to HIPAA establish privacy and security standards that limit the use and disclosure of individually identifiable health information, or protected health information that we may obtain directly or indirectly from health care providers, health plans or other health care industry stakeholders and require the implementation of administrative, physical and technological safeguards to protect the privacy of protected health information and ensure the confidentiality, integrity and availability of electronic protected health information. Determining whether we handle protected health information and whether it has been handled in compliance with applicable privacy standards and our contractual obligations can be complex and may be subject to changing interpretation. These obligations may be applicable to some or all of our business activities now or in the future.

In 2018, California passed into law the California Consumer Privacy Act, or CCPA, which took effect on January 1, 2020 and imposed many requirements on certain businesses that process the personal information of California residents. Many of the CCPA's requirements are similar to those found in the European Union's General Data Protection Regulation, or GDPR, including requiring businesses to provide notice to data subjects regarding the information collected about them and how such information is used and shared, and providing data subjects the right to request access to such personal information and, in certain cases, request the erasure of such personal information. The CCPA also affords California residents the right to opt-out of "sales" of their personal information. The CCPA prescribes significant penalties for companies that violate its requirements. On November 3, 2020, California voters passed a ballot initiative for the California Privacy Rights Act, or CPRA, which went into effect on January 1, 2023 and significantly expanded the CCPA to incorporate additional GDPR-like provisions including requiring that the use, retention, and sharing of personal information of California residents be reasonably necessary and proportionate to the purposes of collection or processing, granting additional protections for sensitive personal information, and requiring greater disclosures related to notice to residents regarding retention of information. The CPRA also created a new enforcement agency – the California Privacy Protection Agency – whose sole responsibility is to enforce the CPRA, which will further increase compliance risk. The CPRA may apply to some of our business activities. In addition, other states, including Connecticut, Colorado, Florida, Indiana, Iowa, Montana, New Jersey, Tennessee, Texas, Utah, and Virginia, have recently passed state privacy laws; the laws in Connecticut, Colorado, Utah, and Virginia became effective in 2023, the laws in Florida, Montana, and Texas are scheduled to go into effect later in 2024, the laws in Iowa, New Jersey, and Tennessee are scheduled to go into effect in 2025, and the law in Indiana is scheduled to go into effect in 2026. Congress, at the federal level, and other states are expected to consider similar laws in the future. These laws may impact our business activities, including our identification of research subjects, relationships with business partners and ultimately the marketing and distribution of our products.

The collection, use, disclosure, transfer, or other processing of personal data regarding individuals in the European Union, including personal health data, is subject to the GDPR, which became effective on May 25, 2018. The

GDPR is wide-ranging in scope and imposes numerous requirements on companies that process personal data, including requirements relating to processing health and other sensitive data, obtaining consent of the individuals to whom the personal data relates, providing information to individuals regarding data processing activities, implementing safeguards to protect the security and confidentiality of personal data, providing notification of data breaches, and taking certain measures when engaging third-party processors. The GDPR also imposes strict rules on the transfer of personal data to countries outside the European Union, including the United States, and permits data protection authorities to impose large penalties for violations of the GDPR, including potential fines of up to €20 million or 4% of annual global revenues, whichever is greater. The GDPR also confers a private right of action on data subjects and consumer associations to lodge complaints with supervisory authorities, seek judicial remedies, and obtain compensation for damages resulting from violations of the GDPR. Compliance with the GDPR has been and will continue to require a rigorous and time-intensive process that has increased and will continue to increase our cost of doing business or require us to change our business practices. Despite those efforts, there is a risk that we or our collaborators may be subject to fines and penalties, litigation, and reputational harm in connection with any activities occurring in the European Union, which could adversely affect our business, prospects, financial condition and results of operations.

GDPR restrictions on transfers of personal data from the European Union to the United States are unsettled and may impact our business operations. The GDPR generally prohibits transfers of personal data of European Union data subjects outside of the European Union, unless a lawful data transfer solution has been implemented or a specific exception applies. In July 2020, the European Court of Justice invalidated the Privacy Shield program, a voluntary self-certification privacy protection mechanism that facilitated transfers of personal data from the European Union to the United States. The court upheld the validity of an alternative contractual mechanism for such data transfers but required companies to take additional steps, such as evaluating supplementary measures that may need to be taken to protect the transferred personal data. In October 2022, President Biden signed an executive order to implement the European Union -U.S. Data Privacy Framework, which would replace the Privacy Shield. In December 2022, the European Commission began the European Union's process for adopting the European Union-U.S. Data Privacy Framework, but it is unclear if and when the framework will be finalized and whether it will be challenged in court. Continued uncertainty relating to European Union -U.S. data transfers may adversely impact our business operations in the European Union.

Beyond GDPR, there are privacy and data security laws in a growing number of countries around the world. Following the exit of the United Kingdom, or UK, from the European Union, the United Kingdom's Data Protection Act of 2018 applies to the processing of personal data that takes place in the UK and includes parallel obligations to those set forth by GDPR. Privacy and data security laws in several other countries loosely follow GDPR as a model but often contain different or conflicting provisions. These laws will impact our ability to conduct our business activities, including both our clinical trials and any eventual commercialization and distribution of commercial products, through increased compliance costs, costs associated with contracting and potential enforcement actions. Any failure to comply with data protection and privacy laws could result in government-imposed fines or orders requiring that we change our practices, claims for damages or other liabilities, regulatory investigations and enforcement action, litigation and significant costs for remediation, any of which could adversely affect our business. Even if we are not determined to have violated these laws, government investigations into these issues typically require the expenditure of significant resources and generate negative publicity, which could harm our business, financial condition, results of operations or prospects.

Product liability lawsuits against us could cause us to incur substantial liabilities and could limit commercialization of any product candidates that we may develop.

We face an inherent risk of product liability exposure related to the testing of our product candidates in clinical trials and may face an even greater risk if we commercialize any products that we may develop. If we cannot successfully defend ourselves against claims that our product candidates caused injuries, we could incur substantial liabilities. Regardless of merit or eventual outcome, liability claims may result in:

- decreased demand for any product candidates that we may develop;
- loss of revenue;

- substantial monetary awards to trial participants or patients;
- significant time and costs to defend the related litigation;
- withdrawal of clinical trial participants;
- the inability to commercialize any product candidates that we may develop; and
- injury to our reputation and significant negative media attention.

Although we maintain clinical trial liability insurance in the amount of $10.0 million per occurrence and $10.0 million in the aggregate, this insurance may not be adequate to cover all liabilities that we may incur. We anticipate that we will need to increase our insurance coverage each time we commence a clinical trial. In addition, if we successfully commercialize any product candidate, we will need to obtain product liability insurance. Insurance coverage is increasingly expensive. We may not be able to maintain insurance coverage at a reasonable cost or in an amount adequate to satisfy any liability that may arise.

If we, our collaborators, or any third-party manufacturers engaged by us or our collaborators fail to comply with environmental, health and safety laws and regulations, we could become subject to fines or penalties or incur costs that could harm our business.

We, our collaborators, and any third-party manufacturers we engage are subject to numerous environmental, health and safety laws and regulations, including those governing laboratory procedures and the generation, handling, use, storage, treatment, manufacture, transportation and disposal of, and exposure to, hazardous materials and wastes, as well as laws and regulations relating to occupational health and safety. Our operations involve the use of hazardous and flammable materials, including chemicals and biologic and radioactive materials. Our operations also produce hazardous waste products. We generally contract with third parties for the disposal of these materials and wastes. We cannot eliminate the risk of contamination or injury from these materials. In the event of contamination or injury resulting from our use of hazardous materials or from any other work-related injuries, we could be held liable for any resulting damages, and any liability could exceed our resources. We also could incur significant costs associated with civil or criminal fines and penalties.

Although we maintain general liability insurance and workers' compensation insurance for certain costs and expenses we may incur due to injuries to our employees resulting from the use of hazardous materials or other work-related injuries, this insurance may not provide adequate coverage against potential liabilities. We do not maintain insurance for environmental liability or toxic tort claims that may be asserted against us in connection with our storage or disposal of biologic, hazardous or radioactive materials.

In addition, we may incur substantial costs in order to comply with current or future environmental, health and safety laws and regulations, which have tended to become more stringent over time. These current or future laws and regulations may impair our research, development or production efforts. Failure to comply with these laws and regulations also may result in substantial fines, penalties or other sanctions or liabilities, which could harm our business, financial condition, results of operations and prospects.

Further, with respect to the operations of any current or future collaborators or third-party contract manufacturers, it is possible that if they fail to operate in compliance with applicable environmental, health and safety laws and regulations or properly dispose of wastes associated with our products, we could be held liable for any resulting damages, suffer reputational harm or experience a disruption in the manufacture and supply of our product candidates or products.

Risks Related to the Commercialization of Our Product Candidates

The affected populations for our product candidates may be smaller than we or third parties currently project, which may affect the addressable markets for our product candidates.

Our projections of the number of people who have the diseases we are seeking to treat, as well as the subset of people with these diseases who have the potential to benefit from treatment with our product candidates, are estimates based on our knowledge and understanding of these diseases. The total addressable market opportunity for our product candidates will ultimately depend upon a number of factors including the diagnosis and treatment criteria included in the final label, if approved for sale in specified indications, acceptance by the medical community, patient access and product pricing and reimbursement. Prevalence estimates are frequently based on information and assumptions that are not exact and may not be appropriate, and the methodology is forward-looking and speculative. The process we have used in developing an estimated prevalence range for the indications we are targeting has involved collating limited data from multiple sources. While we believe these sources are reliable, we have not independently verified the data. Accordingly, the prevalence estimates included in our periodic reports and other reports filed with or furnished to the Securities and Exchange Commission, or SEC, should be viewed with caution. Further, the data and statistical information used in such reports, including estimates derived from them, may differ from information and estimates made by our competitors or from current or future studies conducted by independent sources.

The use of such data involves risks and uncertainties, and such data is subject to change based on various factors. Our estimates may prove to be incorrect and new studies may change the estimated incidence or prevalence of the diseases we seek to address. The number of patients with the diseases we are targeting in the United States, the European Union and elsewhere may turn out to be lower than expected or may not be otherwise amenable to treatment with our products, or new patients may become increasingly difficult to identify or access, all of which would harm our results of operations and our business. Additionally, because some patients with the diseases we are targeting in the United States, the European Union, and elsewhere may have increased susceptibility to COVID-19, the recent COVID-19 pandemic could limit the number of patients willing to participate in clinical trials related to our products or amenable to treatment with our products, which would harm our results of operations and our business.

If we are unable to establish sales, medical affairs and marketing capabilities or enter into agreements with third parties to market and sell our product candidates, we may be unable to generate any product revenue.

To successfully commercialize any products that may result from our clinical development programs, we will need to further develop these capabilities, either on our own or with others. The establishment and development of our own commercial team or the establishment of a contract sales force to market any products we may develop will be expensive and time-consuming and could delay any product launch. Moreover, we cannot be certain that we will be able to successfully develop this capability.

Under the 2019 Neurocrine Collaboration Agreement, Neurocrine agreed to fund the clinical development through the readout of the RESTORE-1 Phase 2 clinical trial for VY-AADC (NBIb-1817). If Neurocrine had not terminated the 2019 Neurocrine Collaboration Agreement with respect to VY-AADC (NBIb-1817), after the data readout of the RESTORE-1 Phase 2 clinical trial, we would have had the option to either: (1) co-commercialize VY-AADC (NBIb-1817) with Neurocrine in the United States under a 50/50 cost- and profit-sharing arrangement and receive milestones and royalties based on ex-U.S. sales, or (2) retain the right to receive milestone payments and royalties based on global sales pursuant to the full global commercial rights granted to Neurocrine. Under the terms of the 2019 Neurocrine Collaboration Agreement for the FA Program, Neurocrine has agreed to fund the development through the Phase 1 clinical trial of VY-FXN01. After the achievement of milestones or metrics specified in the applicable development plan, as determined by the JSC, we have the option to either: (1) co-commercialize VY-FXN01 with Neurocrine in the United States under a 60/40 cost and profit-sharing arrangement, 60% to Neurocrine and 40% to us, or (2) retain the right to receive milestone payments and royalties based on global sales pursuant to the full global commercial rights granted to Neurocrine.

Under the 2023 Neurocrine Collaboration Agreement, Neurocrine agreed to fund the non-clinical development activities for the GBA1 Program. Upon our receipt of topline data from the first Phase 1 clinical trial for a product

candidate being developed pursuant to the GBA1 Program, we will have the option to either: (1) co-commercialize collaboration products in the GBA1 Program with Neurocrine in the United States under a 50/50 cost- and profit-sharing arrangement and receive milestones and royalties based on ex-U.S. sales, or (2) retain the right to receive milestone payments and royalties based on global sales pursuant to the full global commercial rights granted to Neurocrine. In the event we exercise our 2023 Co-Co Option, the parties have also agreed that Neurocrine is entitled to receive (in addition to its 50% share of profits) 50% of our share of profits until our obligation to repay 50% of all development costs incurred by Neurocrine in connection with the GBA1 Program prior to such exercise have been paid off out of such 50% of our share of profits. The 2023 Co-Co Trigger Event is the date on which we receive topline data from the first Phase 1 clinical trial for a product candidate being developed pursuant to the GBA1 Program.

Under the 2023 Novartis Collaboration Agreement, Novartis is solely responsible for, and has sole decision-making authority with respect to, at its own expense, the exploitation of a product or product candidate under the Novartis SMA Program, or the Novartis SMA Program Product. With respect to the Novartis HD Program, the parties have agreed to conduct research and pre-clinical development of Novartis HD Program Products pursuant to a research plan, with Novartis reimbursing us for our activities thereunder in accordance with the agreed-to budget. From and after the first IND application filing for the Novartis HD Program, the parties have agreed that Novartis will assume sole responsibility for the development and commercialization of Novartis HD Program Products, including all further preclinical and clinical development and any commercialization of the Novartis HD Program products and product candidates. With respect to each of the Novartis SMA Program Products and Novartis HD Program Products, Novartis is obligated to use commercially reasonable efforts to develop and obtain regulatory approval for at least one of each such product in the United States and in certain other international markets specified in the 2023 Novartis Collaboration Agreement.

In the future, we may seek to enter into collaborations regarding other of our product candidates with other entities to utilize their established marketing and distribution capabilities, but we may be unable to enter into such agreements on favorable terms, if at all. If any current or future collaborators do not commit sufficient resources to commercialize our products, or we are unable to develop the necessary capabilities on our own, we will be unable to generate sufficient product revenue to sustain our business. We compete with many companies that currently have extensive, experienced and well-funded medical affairs, marketing and sales operations to recruit, hire, train and retain marketing and sales personnel. We also face competition in our search for third parties to assist us with the sales and marketing efforts of our product candidates. We might face unforeseen costs and expenses associated with creating an independent sales and marketing organization. Our sales personnel might also face difficulties obtaining access to physicians or being able to persuade adequate numbers of physicians to use or prescribe our products or selling our products if we lack complementary products, which could disadvantage us compared to companies with more extensive product lines. Without an internal team or the support of a third party to perform marketing and sales functions, we may be unable to compete successfully against these more established companies.

Our efforts to educate the medical community and third-party payors on the benefits of our product candidates may require significant resources and may never be successful. Such efforts may require more resources than are typically required due to the complexity and uniqueness of certain of our potential products. If any of our product candidates is approved but fails to achieve market acceptance among physicians, patients, or third-party payors, we will not be able to generate significant revenues from such product, which could harm our business, financial condition, results of operations and prospects.

The insurance coverage and reimbursement status of newly-approved products is uncertain. Failure to obtain or maintain adequate coverage and reimbursement for our product candidates, if approved, could limit our ability to market those products and decrease our ability to generate product revenue.

We expect the cost of a single administration of gene therapy products, such as those we are developing, to be substantial, when and if they receive regulatory approval. We expect that coverage and reimbursement by government and private payors will be essential for most patients to be able to afford these treatments. Accordingly, sales of our product candidates will depend substantially, both domestically and abroad, on the extent to which the costs of our product candidates will be paid by health maintenance, managed care, pharmacy benefit and similar healthcare management organizations, or will be reimbursed by government authorities, private health coverage insurers and other

third-party payors. Coverage and reimbursement by a third-party payor may depend upon several factors, including the third-party payor's determination that use of a product is:

- a covered benefit under its health plan;
- safe, effective and medically necessary;
- appropriate for the specific patient and the indication;
- convenient and easy-to-administer compared to alternative treatments;
- cost-effective compared to alternative treatments; and
- neither experimental nor investigational.

No uniform policy requirement for coverage and reimbursement for biopharmaceutical products exists among third-party payors. Therefore, coverage and reimbursement for such products can differ significantly from payor to payor. As a result, obtaining coverage and reimbursement for a product from third-party payors is a time-consuming and costly process that could require us to provide to each different payor supporting scientific, clinical and cost-effectiveness data, and to receive the support of medical associations and technology assessment committees. We may not be able to provide data sufficient to gain acceptance with respect to coverage and reimbursement. If coverage and reimbursement are not available, or are available only at limited levels, we may not be able to successfully commercialize our product candidates. Even if coverage is provided, the approved reimbursement amount may not be adequate to realize a sufficient return on our investment including our research, development, manufacture, sales, and distribution expenses. Interim reimbursement levels for new drugs, if applicable, may also not be sufficient to cover our costs and may not be made permanent. Reimbursement rates may vary according to the use of the drug and the clinical setting in which it is used, may be based on reimbursement levels already set for lower cost drugs and may be incorporated into existing payments for other services. Assuming we obtain coverage for a given product by a third-party payor, the resulting reimbursement payment rates may not be adequate or may require co-payments that patients find unacceptably high. Patients who are prescribed medications for the treatment of their conditions, and their prescribing physicians, generally rely on third-party payors to reimburse all or part of the costs associated with their prescription drugs. Patients are unlikely to use our products unless coverage is provided and reimbursement is adequate to cover all or a significant portion of the cost of our products. Therefore, coverage and adequate reimbursement are critical to new product acceptance. Additionally, there may be significant delays in obtaining coverage and reimbursement for newly approved drugs and biologics, and coverage may be more limited than the purposes for which the drug is approved by the FDA or comparable foreign regulatory authorities.

There is significant uncertainty related to third-party coverage and reimbursement of newly approved products. In the United States, third-party payors, including government payors such as the Medicare and Medicaid programs, play an important role in determining the extent to which new drugs and biologics will be covered and reimbursed. The Medicare and Medicaid programs increasingly are used as models for how private payors and government payors develop their coverage and reimbursement policies. A primary trend in the U.S. healthcare industry and elsewhere is cost containment. Government authorities and third-party payors have attempted to control costs by limiting coverage and the amount of reimbursement for particular medications. Increasingly, third-party payors are requiring that drug companies provide them with predetermined discounts from list prices and are challenging the prices charged for medical products.

The CMS is responsible for determining whether a product should be approved for coverage and reimbursement under the Medicare program. It is difficult to predict what CMS will decide with respect to coverage and reimbursement for novel products such as ours, as there is no body of established practices and precedents for these types of products. Currently, no gene therapy product has been approved for coverage and reimbursement by the CMS. Moreover, reimbursement agencies in the European Union may be more conservative than CMS. For example, several cancer drugs have been approved for reimbursement in the United States and have not been approved for reimbursement in certain European Union Member States. It is difficult to predict what third-party payors will decide with respect to the coverage

and reimbursement for our product candidates, especially given that the cost of our product candidates is likely to be very high and pricing of such products is highly uncertain.

Outside the United States, international operations generally are subject to extensive government price controls and other market regulations, and increasing emphasis on cost-containment initiatives in the European Union, Canada and other countries may put pricing pressure on us. In many countries, the prices of medical products are subject to varying price control mechanisms as part of national health systems. In general, the prices of medicines under such systems are substantially lower than in the United States. Other countries allow companies to fix their own prices for medical products, but monitor and control company profits. Additional foreign price controls or other changes in pricing regulation could restrict the amount that we are able to charge for our product candidates. Accordingly, in markets outside the United States, the reimbursement for our products may be reduced compared with the United States and may be insufficient to generate commercially reasonable product revenues.

Moreover, increasing efforts by government and third-party payors in the United States and abroad to cap or reduce healthcare costs may cause such organizations to limit both coverage and the level of reimbursement for new products approved and, as a result, they may not cover or provide adequate payment for our product candidates. Payors increasingly are considering new metrics as the basis for reimbursement rates, such as average sales price, or ASP, average manufacturer price, or AMP, and Actual Acquisition Cost. The existing data for reimbursement based on some of these metrics is relatively limited, although certain states have begun to survey acquisition cost data for the purpose of setting Medicaid reimbursement rates, and CMS has begun making pharmacy National Average Drug Acquisition Cost and National Average Retail Price data publicly available on at least a monthly basis. The regulations that govern marketing approvals, pricing, coverage and reimbursement for new drug and device products vary widely from country to country. Current and future legislation may significantly change the approval requirements in ways that could involve additional costs and cause delays in obtaining approvals. Some countries require approval of the sale price of a drug before it can be marketed. In many countries, the pricing review period begins after marketing or product licensing approval is granted. In some foreign markets, prescription pharmaceutical pricing remains subject to continuing governmental control even after initial approval is granted. As a result, we might obtain marketing approval for a product in a particular country, but then be subject to price regulations that delay our commercial launch of the product, possibly for lengthy time periods, and negatively impact the revenues we are able to generate from the sale of the product in that country. To obtain reimbursement or pricing approval in some countries, we may be required to conduct a clinical trial that compares the cost-effectiveness of our product candidate to other available therapies. Adverse pricing limitations may hinder our ability to recoup our investment in one or more product candidates, even if our product candidates obtain marketing approval.

Therefore, it is difficult to project the impact of these evolving reimbursement metrics on the willingness of payors to cover candidate products that we or our partners are able to commercialize. We expect to experience pricing pressures in connection with the sale of any of our product candidates due to the trend toward managed healthcare, the increasing influence of health maintenance organizations and additional legislative changes. The downward pressure on healthcare costs in general, particularly prescription drugs and surgical procedures and other treatments, has become intense. As a result, increasingly high barriers are being erected to the entry of new products such as ours.

The commercial success of any of our product candidates will depend upon its degree of market acceptance by physicians, patients, third-party payors and others in the medical community.

Ethical, social and legal concerns about gene therapy could result in additional regulations restricting or prohibiting our gene therapy products. Even with the requisite approvals from the FDA in the United States, EMA in the European Union and other regulatory authorities internationally, the commercial success of our product candidates will depend, in part, on the support and acceptance of medical associations and technology assessment committees, physicians, patients and health care payors of proprietary antibody and gene therapy products in general, and our product candidates in particular, as medically necessary, cost-effective and safe. Any product that we commercialize may not gain acceptance by physicians, patients, health care payors and others in the medical community. If these products do not achieve an adequate level of acceptance, we may not generate significant product revenue and may not become

profitable. The degree of market acceptance of proprietary antibody and gene therapy products and, in particular, our product candidates, if approved for commercial sale, will depend on several factors, including:

- the efficacy and safety of such product candidates as demonstrated in clinical trials;
- the potential and perceived advantages of product candidates over alternative treatments;
- the cost of treatment relative to alternative treatments;
- the clinical indications for which the product candidate is approved by the FDA or the European Commission, or other regulatory authorities;
- patient awareness of, and willingness to seek, genotyping;
- the willingness of physicians to prescribe new therapies;
- the willingness of physicians to undergo specialized training with respect to administration of our product candidates;
- the willingness of the target patient population to try new therapies;
- the prevalence and severity of any side effects;
- product labeling or product insert requirements of the FDA, EMA or other regulatory authorities, including any limitations or warnings contained in a product's approved labeling or restrictions on the use of our products together with other medications;
- relative convenience and ease of administration;
- the strength of marketing and distribution support;
- the timing of market introduction of competitive products;
- publicity concerning our products or competing products and treatments; and
- sufficient third-party payor coverage and reimbursement.

Even if a potential product displays a favorable efficacy and safety profile in preclinical studies and clinical trials, market acceptance of the product will not be fully known until after it is launched.

Our gene therapy and vectorized antibody approaches utilize vectors derived from viruses that are selectively engineered, which may be perceived as unsafe or may result in unforeseen adverse events. Negative public opinion and increased regulatory scrutiny of gene therapy may damage public perception of the safety of our gene therapy product candidates and adversely affect our ability to conduct our business or obtain regulatory approvals for our gene therapy product candidates.

Gene and vectorized antibody therapies remain novel technologies, with few gene therapy products approved to date in the United States and the European Union. Public perception may be influenced by claims that gene therapy is unsafe, and gene therapy may not gain the acceptance of the public or the medical community. Medical events such as the recent COVID-19 pandemic that emphasize harmful effects of certain viruses could also indirectly foster negative public perception of virus-based therapies. In particular, our success will depend upon physicians who specialize in the treatment of genetic diseases targeted by our product candidates, prescribing treatments that involve the use of our

product candidates in lieu of, or in addition to, existing treatments with which they are familiar and for which greater clinical data may be available. More restrictive government regulations or negative public opinion would have an adverse effect on our business, financial condition, results of operations and prospects and may delay or impair the development and commercialization of our product candidates or demand for any products we may develop.

For example, earlier gene therapy trials led to several well-publicized adverse events, including cases of leukemia and death seen in other trials using non-AAV gene therapy vectors. Adverse events and SAEs in our clinical trials such as the MRI abnormalities detected in some patients dosed in the RESTORE-1 Phase 2 clinical trial, or other clinical trials involving gene therapy products or our competitors' products, even if not ultimately attributable to the relevant product candidates, and the resulting publicity, could result in increased government regulation, unfavorable public perception, potential regulatory delays in the testing or approval of our product candidates, stricter labeling requirements for those product candidates that are approved and a decrease in demand for any such product candidates.

If we obtain approval to commercialize our product candidates outside of the United States, in particular in the United Kingdom or European Union, a variety of risks associated with international operations could harm our business.

We expect that we will be subject to additional risks in commercializing our product candidates outside the United States, including:

- different regulatory requirements for approval of drugs and biologics in foreign countries;
- reduced or loss of protection under our intellectual property rights;
- unexpected changes in tariffs, trade barriers and regulatory requirements;
- economic weakness, including inflation, or political instability in particular foreign economies and markets;
- compliance with tax, employment, immigration and labor laws for employees living or traveling abroad;
- foreign currency fluctuations, which could result in increased operating expenses and reduced revenues, and other obligations incident to doing business in another country;
- workforce uncertainty in countries where labor unrest is more common than in the United States;
- shortages resulting from any events affecting raw material supply or manufacturing capabilities abroad;
- business interruptions resulting from geopolitical actions, including war and terrorism, from natural disasters including earthquakes, typhoons, floods and fires, or from economic, social, or political instability; and
- greater difficulty with enforcing our contracts in jurisdictions outside of the United States.

We must dedicate additional resources to comply with numerous laws and regulations in each jurisdiction in which we plan to operate. The creation, implementation and maintenance of international business practices compliance programs is costly and such programs are difficult to enforce, particularly where reliance on third parties is required. The Foreign Corrupt Practices Act, or FCPA, prohibits any U.S. individual or business from paying, offering, authorizing payment or offering anything of value, directly or indirectly, to any foreign official, political party or candidate for the purpose of influencing any act or decision of the foreign entity in order to assist the individual or business in obtaining or retaining business. The FCPA also obligates companies whose securities are listed in the United States to comply with certain accounting provisions requiring the company to maintain books and records that accurately and fairly reflect all transactions of the corporation, including international subsidiaries, and to devise and maintain an adequate system of

internal accounting controls for international operations. The anti-bribery provisions of the FCPA are enforced primarily by the Department of Justice. The SEC is involved with enforcement of the books and records provisions of the FCPA.

Compliance with the FCPA is expensive and difficult, particularly in countries in which corruption is a recognized problem. In many foreign countries, it is common for others to engage in business practices that are prohibited by U.S. laws and regulations applicable to us, including the FCPA. In addition, the FCPA presents particular challenges in the pharmaceutical industry because, in many countries, hospitals are operated by the government, and doctors and other hospital employees are considered foreign officials. Certain payments to hospitals in connection with clinical trials and other work have been deemed to be improper payments to government officials and have led to FCPA enforcement actions.

Various laws, regulations and executive orders also restrict the use and dissemination outside of the United States, or the sharing with certain non-U.S. nationals, of information classified for national security purposes, as well as certain products and technical data relating to those products. If we expand our presence outside of the United States, we will be required to dedicate additional resources to comply with these laws, and these laws may preclude us from developing, manufacturing, or selling certain products and product candidates outside of the United States, which could limit our growth potential and increase our development costs.

The failure to comply with laws governing international business practices may result in substantial civil and criminal penalties and suspension or debarment from government contracting. The SEC also may suspend or bar issuers from trading securities on U.S. exchanges for violations of the FCPA's accounting provisions. Although we expect to implement policies and procedures designed to comply with these laws and policies, there can be no assurance that our employees, contractors and agents will comply with these laws and policies. If we are unable to successfully manage the challenges of international expansion and operations, our business and operating results could be harmed.

Risks Related to Our Intellectual Property

Our rights to develop and commercialize our product candidates are subject to, in part, the terms and conditions of licenses granted to us by others.

We are reliant upon licenses to certain patent rights and proprietary technology from third parties that are important or necessary to the development of our technology and products, including technology related to our manufacturing process and our product candidates. These and other licenses may not provide exclusive rights to use such intellectual property and technology in all relevant fields of use and in all territories in which we may wish to develop or commercialize our technology and products in the future. As a result, we may not be able to prevent competitors from developing and commercializing competitive products in territories included in all of our licenses. These licenses may also require us to grant back certain rights to licensors and/or to pay certain amounts relating to the use of the licensed intellectual property. For example, the Touchlight License Agreement obligates us to make future milestone and royalty payments if we, or our collaboration partners or TRACER Capsid licensees, use a capsid created using certain DNA preparation processes licensed under the Touchlight License Agreement.

In some circumstances, particularly in-licenses with academic institutions, we may not have the right to control the preparation, filing and prosecution of patent applications, or to maintain, enforce or defend the patents, covering technology that we license from third parties. Therefore, we cannot be certain that these patents and applications will be prosecuted, maintained and enforced in a manner consistent with the best interests of our business. If our licensors fail to maintain such patents, or lose rights to those patents or patent applications, the rights we have licensed may be reduced or eliminated and our right to develop and commercialize any of our products that are the subject of such licensed rights could be adversely affected. In certain circumstances, we have or may license technology from third parties on a non-exclusive basis. In such instances, other licensees may have the right to enforce our licensed patents in their respective fields, without our oversight or control. Those other licensees may choose to enforce our licensed patents in a way that harms our interest, for example, by advocating for claim interpretations or agreeing on invalidity positions that conflict with our positions or our interest. In addition to the foregoing, the risks associated with patent rights that we license from third parties will also apply to patent rights we own or may own in the future.

Further, in many of our license agreements we are responsible for bringing any actions against any third party for infringing on the patents we have licensed. Certain of our license agreements also require us to meet development thresholds to maintain the license, including establishing a set timeline for developing and commercializing products and minimum yearly diligence obligations in developing and commercializing the product. Certain of our license agreements contain "no challenge" clauses which preclude and prevent us from taking any action to limit or narrow the intellectual property of a licensor. In some cases, these limitations extend to any intellectual property of our licensor and not just that which is licensed to us. Such constraints may limit our ability to develop or commercialize products or to expand such efforts beyond the scope of any license. Disputes may arise regarding intellectual property subject to a licensing agreement, including:

- the scope of rights granted under the license agreement and other interpretation-related issues;
- the extent to which our technology and processes infringe on intellectual property of the licensor that is not subject to the licensing agreement;
- the sublicensing of patent and other rights under our collaborative development relationships;
- our diligence obligations under the license agreement and what activities satisfy those diligence obligations;
- the inventorship or ownership of inventions and know-how resulting from the creation or use of intellectual property by our licensors and us and our partners; and
- the priority of invention of patented technology.

If disputes over intellectual property that we have licensed prevent or impair our ability to maintain our current licensing arrangements on acceptable terms, we may be unable to successfully develop and commercialize the affected product candidates.

If we fail to comply with our obligations under these license agreements, or we are subject to a bankruptcy, the licensor may have the right to terminate the license, in which event we would not be able to develop, manufacture, or market products covered by the license or may face other penalties under the agreements. Termination of any of our agreements involving intellectual property or reduction or elimination of our rights under these agreements may result in our having to negotiate new or reinstated agreements with less favorable terms or cause us to lose our rights under these agreements, including our rights to important intellectual property or technology. Termination may also result in unfavorable terms associated with such termination or may result in obligations on our part to license or grant back intellectual property rights to prior licensors.

Furthermore, the research resulting in certain of our licensed patent rights and technology was funded by the U.S. government. As a result, the government may have certain rights, or march-in rights, to such patent rights and technology. When new technologies are developed with U.S. government funding, the U.S. government generally obtains certain rights in any resulting patents, including a non-exclusive, royalty-free license authorizing the U.S. government, or a third party on its behalf, to use the invention for non-commercial purposes. These rights may permit the government to disclose our confidential information to third parties and to exercise march-in rights to use or allow third parties to use our licensed technology. The U.S. government can exercise its march-in rights if it determines that action is necessary because we fail to achieve practical application of the government-funded technology, because action is necessary to alleviate health or safety needs, to meet requirements of federal regulations or to give preference to U.S. industry. In addition, our rights in such inventions may be subject to certain requirements to manufacture products embodying such inventions in the United States. Any exercise by the government, or a third party on its behalf, of such rights could harm our competitive position, business, financial condition, results of operations and prospects.

If we are unable to obtain and maintain patent protection for our products and technology, or if the scope of the patent protection obtained is not sufficiently broad, our competitors could develop and commercialize products and technology similar or identical to ours, and our ability to successfully commercialize our products and technology may be adversely affected.

Our success depends, in large part, on our and our licensors' ability to obtain and maintain patent protection in the United States and other countries with respect to our product candidates and manufacturing technology. We and our licensors have sought, and we intend to seek in the future, to protect our proprietary position by filing patent applications in the United States and abroad related to many of our technologies and product candidates that are important to our business.

The patent prosecution process is expensive, time-consuming and complex, and we may not have and may not in the future be able to file, prosecute, maintain, enforce, defend or license all necessary or desirable patent applications in some or all relevant jurisdictions at a reasonable cost or in a timely manner. For example, in some cases, the work of certain academic researchers and biotechnology and biopharmaceutical companies in the gene therapy field has entered the public domain, which may compromise our ability to obtain patent protection for certain inventions related to or building upon such prior work. Consequently, we may not be able to obtain any such patents to prevent others from using our technology for, and developing and marketing competing products to treat, these indications. It is also possible that we will fail to identify patentable aspects of our research and development output before it is too late to obtain patent protection. In some cases, we may be able to obtain patent protection, but such protections may expire before we commercialize the product protected by those rights, leaving us no meaningful protection for our products. In other cases, where our intellectual property is being managed by a third-party collaborator, licensee or partner, that third party may fail to act diligently in prosecuting, maintaining, defending or enforcing our patents. Such conduct may result in the failure to maintain or obtain protections, loss of rights, loss of patent term or, in cases where a third party has acted negligently or inequitably, patents being found unenforceable.

The patent position of biotechnology and pharmaceutical companies generally is highly uncertain, involves complex legal and factual questions and has, in recent years, been the subject of much litigation. As a result, the issuance, scope, validity, enforceability and commercial value of our and our licensors' patent rights are highly uncertain. Our pending and future patent applications may not result in patents being issued which protect our technology or product candidates or which effectively prevent others from commercializing competitive technologies and product candidates. In particular, during prosecution of any patent application, the issuance of any patents based on the application may depend upon our ability to generate additional preclinical or clinical data that support the patentability of our proposed claims. We may not be able to generate sufficient additional data on a timely basis, or at all. Changes in either the patent laws or interpretation of the patent laws in the United States and other countries may diminish the value, narrow the scope, or eliminate the enforceability of our and our licensors' patent protection.

We may not be aware of all third-party intellectual property rights potentially relating to our product candidates, particularly due to the competitive and rapidly-evolving gene therapy patent landscape. Publications of discoveries in the scientific literature often lag behind the actual discoveries, and patent applications in the United States and other jurisdictions are typically not published until 18 months after filing or, in some cases, only upon issuance or not at all. Therefore, we cannot be certain that we, or a licensor, were the first to make the inventions claimed in any owned or any licensed patents or pending patent applications, respectively, or which entity was the first to file for patent protection until such patent application publishes or issues as a patent. Databases for patents and publications, and methods for searching them, are inherently limited, so it is not practical to review and know the full scope of all issued and pending patent applications. As a result, the issuance, scope, validity, enforceability, and commercial value of our and our licensed patent rights are uncertain.

Even if the patent applications we license or may own in the future do issue as patents, they may not issue in a form that will provide us with any meaningful protection, prevent competitors or other third parties from competing with us or otherwise provide us with any competitive advantage. Our competitors or other third parties may be able to circumvent our patents by developing similar or alternative technologies or products in a non-infringing manner.

In spite of a legal presumption of validity, the issuance of a patent is not conclusive as to its inventorship, ownership, scope, validity, or enforceability which may be challenged in the courts and patent offices in the United States and abroad. Such challenges may result in loss of exclusivity or in patent claims being narrowed, invalidated or held unenforceable, which could limit our ability to stop others from using or commercializing similar or identical technology and products, or limit the duration of the patent protection of our technology and product candidates. Given the amount of time required for the development, testing and regulatory review of new product candidates, patents protecting such candidates might expire before or shortly after such candidates are commercialized. As a result, our intellectual property may not provide us with sufficient rights to exclude others from commercializing products similar or identical to ours.

Our intellectual property licenses with third parties may be subject to disagreements over contract interpretation, which could narrow the scope of our rights to the relevant intellectual property or technology, resulting in termination of our access to such intellectual property, or increase our financial or other obligations to our licensors.

The agreements under which we currently license intellectual property or technology from third parties are complex, and certain provisions in such agreements may be susceptible to multiple interpretations. The resolution of any contract interpretation disagreement that may arise could narrow what we believe to be the scope of our rights to the relevant intellectual property or technology, result in loss of access, or increase what we believe to be our financial or other obligations under the relevant agreement, any of which could harm our business, financial condition, results of operations and prospects.

We may not be successful in obtaining necessary rights to our product candidates through acquisitions and in-licenses.

We currently have rights to certain intellectual property, through licenses from third parties, to develop our product candidates. Because our programs may require the use of proprietary rights held by third parties, the growth of our business likely will depend, in part, on our ability to acquire, in-license or use these proprietary rights. We may be unable to acquire or in-license any compositions, methods of use, processes or other intellectual property rights from third parties that we identify as necessary for our product candidates. The licensing or acquisition of third-party intellectual property rights is a competitive area, and several more established companies may pursue strategies to license or acquire third-party intellectual property rights that we may consider attractive. These established companies may have a competitive advantage over us due to their size, capital resources and greater clinical or technical development and commercialization capabilities. In addition, companies that perceive us to be a competitor may be unwilling to assign or license rights to us. We also may be unable to license or acquire third-party intellectual property rights on terms that would allow us to make an appropriate return on our investment.

We currently co-own certain intellectual property rights with one or more third parties. We may not be able to obtain a license to the third parties' interest such that we have exclusive access and control of such co-owned assets. In this case, and depending on the jurisdiction of the patent filing, we may not be able to license, enforce, or exploit the co-owned rights without the consent from, or an accounting to, the other co-owners.

We sometimes collaborate with non-profit and academic institutions to accelerate our preclinical research or development under written agreements with these institutions. Typically, these institutions provide us with an option to negotiate a license to any of the institution's rights in technology resulting from the collaboration. Regardless of such option, we may be unable to negotiate a license within the specified timeframe or under terms that are acceptable to us. If we are unable to do so, the institution may offer the intellectual property rights to other parties, potentially blocking our ability to develop our program. We may also decide not to exercise an option to such institutional rights.

If we decide not to obtain, or are unable to successfully obtain rights to required third-party intellectual property rights or maintain the existing intellectual property rights we have, we may be required to expend significant time and resources to redesign our product candidates or the methods for manufacturing them or to develop or license replacement technology, all of which may not be feasible on a technical or commercial basis. If we are unable to do so, we may be unable to develop or commercialize the affected product candidates, which could harm our business significantly.

Obtaining and maintaining our patent protection depends on compliance with various procedural, document submission, fee payment and other requirements imposed by government patent agencies, and our patent protection could be reduced or eliminated for non-compliance with these requirements.

Periodic maintenance fees, renewal fees, annuity fees and various other government fees on patents and/or applications will be due to be paid to the United States Patent and Trademark Office, or USPTO, and various government patent agencies outside of the United States over the lifetime of our licensed patents and/or applications and any patent rights we may own in the future. We rely on our outside counsel or our licensors to pay these fees due to patent agencies. The USPTO and various non-U.S. government patent agencies require compliance with several procedural, documentary, fee payment and other similar provisions during the patent application process. We employ reputable law firms and other professionals to help us comply and we are also dependent on our licensors to take the necessary action to comply with these requirements with respect to our licensed intellectual property. In many cases, an inadvertent lapse can be cured by payment of a late fee or by other means in accordance with the applicable rules. There are situations, however, in which non-compliance can result in abandonment or lapse of the patent or patent application, resulting in partial or complete loss of patent rights in the relevant jurisdiction, and may compromise the strength of other intellectual property in our portfolio. In such an event, potential competitors might be able to enter the market and this circumstance could harm our business.

On February 1, 2019 the government of Venezuela, in response to certain U.S. sanctions, began to require that foreign entities pay all official fees, including patent fees (either for pending matters or new petitions), in PETRO, a “cryptocurrency” created by the Nicolás Maduro administration in February 2018 as a way to collect U.S. dollars while avoiding American financial sanctions issued under an Executive Order of President Trump on March 19, 2018. The Executive Order banned transactions involving “any digital currency, digital coin, or digital token, that was issued by, for, or on behalf of the Government of Venezuela on or after January 9, 2018.” The prohibition is applicable to any U.S. entity unless exempted by license. We do not hold such a license and therefore may not be able to secure patents in Venezuela.

We may not be able to protect our intellectual property rights throughout the world.

Filing, prosecuting and defending patents on product candidates in all countries throughout the world would be prohibitively expensive. Our intellectual property rights may vary from country to country and foreign protections could be less extensive than those in the United States. In addition, the laws of some foreign countries do not protect intellectual property rights to the same extent as federal and state laws in the United States. Consequently, we may not be able to prevent third parties from practicing our inventions in all countries outside the United States, or from selling or importing products made using our inventions in and into the United States or other jurisdictions. Competitors may use our technologies in jurisdictions where we have not obtained patent protection to develop their own products and, further, may export otherwise infringing products to territories where we have patent protection, but enforcement is not as strong as that in the United States. These products may compete with our products and our patents or other intellectual property rights may not be effective or sufficient to prevent them from competing.

Many companies have encountered significant problems in protecting and defending intellectual property rights in foreign jurisdictions. The legal systems of certain countries, particularly certain developing countries, do not favor the enforcement of patents, trade secrets and other intellectual property protection, particularly those relating to biotechnology products or methods of treatment, which could make it difficult for us to stop the infringement of our patents or marketing of competing products in violation of our proprietary rights generally. For example, an April 2023 report from the Office of the United States Trade Representative identified a number of countries, including India and China, where challenges to the procurement and enforcement of patent rights have been reported. Several countries, including India and China, have been listed in the report every year since 1989. With Brexit, there is uncertainty associated with obtaining, defending, and enforcing intellectual property rights in the United Kingdom. International treaties and regulations promulgated as a result of this transition could impede or eliminate our ability to obtain or maintain meaningful intellectual property rights in the United Kingdom. Proceedings to enforce our patent rights in foreign jurisdictions could result in substantial costs and divert our efforts and attention from other aspects of our business, could put our patents at risk of being invalidated or interpreted narrowly and our patent applications at risk of not issuing and could provoke third parties to assert claims against us. We may not prevail in any lawsuits that we

initiate, and the damages or other remedies awarded, if any, may not be commercially meaningful. Accordingly, our efforts to enforce our intellectual property rights around the world may be inadequate to obtain a significant commercial advantage from the intellectual property that we develop or license.

Issued patents covering our technology or product candidates could be found invalid or unenforceable if challenged in court. We may not be able to protect our trade secrets in court.

If one of our licensees or licensors or we initiate legal proceedings against a third party to enforce a patent covering our technology or one of our product candidates, the defendant could counterclaim that the patent covering such technology or product candidate is invalid or unenforceable. In patent litigation in the United States, defendant counterclaims alleging invalidity or unenforceability are commonplace. Grounds for a validity challenge could be an alleged failure to meet any of several statutory requirements, including subject matter eligibility, lack of novelty, obviousness, lack of written description, failure to enable third parties to practice the relevant invention, or double patenting. Grounds for an unenforceability assertion could be an allegation that an individual connected with prosecution of the patent, including an inventor, an employee of the company, a collaborator or advisor, withheld information material to patentability from the USPTO, or made a misleading statement, during prosecution. Third parties also may raise similar claims before administrative bodies in the United States or abroad, even outside the context of litigation. Such mechanisms include pre-issuance submissions, *ex parte* re-examination, post-grant review, *inter partes* review and equivalent proceedings in foreign jurisdictions. Some of these mechanisms may even be exploited anonymously by third parties. Such proceedings could result in the revocation or cancellation of or amendment to our patents in such a way that they no longer cover our technology or product candidates. The outcome following legal assertions of invalidity and unenforceability is unpredictable. With respect to the validity question, for example, we cannot be certain that there is no invalidating prior art, of which the patent examiner and we or our licensees or licensors were unaware during prosecution. If a defendant were to prevail on a legal assertion of invalidity or unenforceability, we could lose part or, all of the patent protection on one or more of our product candidates or our supporting technology. Such a loss of patent protection could harm our business.

In addition to the protection afforded by patents, we rely on trade secret protection, nondisclosure, and confidentiality agreements to protect proprietary know-how that is not patentable or that we elect not to patent, processes for which patents are difficult to enforce and any other elements of our product candidate discovery and development processes that involve proprietary know-how, information or technology that is not covered by patents. However, trade secrets can be difficult to protect. Some courts inside and outside the United States are less willing or unwilling to protect trade secrets. We seek to protect our proprietary technology and processes, in part, by entering into confidentiality agreements with our employees, consultants, scientific advisors, collaborators, contractors, and other third parties. We cannot guarantee that we have entered into such agreements with each party that may have or have had access to our trade secrets or proprietary technology and processes. We also seek to preserve the integrity and confidentiality of our data and trade secrets by maintaining physical security of our premises and physical and electronic security of our information technology systems. While we have confidence in these individuals, organizations and systems, agreements or security measures may be breached, and we may not have adequate remedies for any breach. In addition, our trade secrets may otherwise become known or be independently discovered by competitors.

Third parties may initiate legal proceedings alleging that we are infringing their intellectual property rights, the outcome of which would be uncertain and could harm our business.

Our commercial success depends upon our ability and the ability of our collaborators to develop, manufacture, market and sell our product candidates and use our proprietary technologies without infringing the proprietary rights and intellectual property of third parties. The biotechnology and pharmaceutical industries are characterized by extensive and complex litigation regarding patents and other intellectual property rights. We may become party to, or threatened with, infringement litigation claims regarding our products and technology, including claims from competitors or from non-practicing entities that have no relevant product revenue and against whom our own patent portfolio may have no deterrent effect. Moreover, we may become party to, or be threatened with, adversarial proceedings or litigation regarding intellectual property rights with respect to our product candidates and technology, including *ex parte* re-examination, post-grant review and *inter partes* review before the USPTO or foreign patent offices. Third parties may

assert infringement claims against us based on existing patents or patents that may be granted in the future, regardless of the merit of the claim.

In November 2022, we and Touchlight entered into the Touchlight License Agreement to allow for our historical use of a certain DNA preparation process, or the Subject DNA Preparation Process, and to authorize the prospective exploitation of TRACER Capsids that we have previously created using the Subject DNA Preparation Process. As previously referenced in the Risk Factor section of our prior periodic reports, Touchlight had made us aware in early 2022 that it believed that some of its intellectual property rights could potentially be asserted against us, although we disagreed with this assessment. In connection with entering into the Touchlight License Agreement, Touchlight also agreed to release any potential claims against us regarding the alleged historical use of certain of Touchlight's intellectual property rights and exploitation of TRACER Capsids created with the alleged use of such intellectual property rights.

Potential parties may emerge and choose to engage in litigation with us to enforce or to otherwise assert their patent rights against us. Even if we believe such claims are without merit, a court of competent jurisdiction could hold that these third-party patents are valid, enforceable and infringed, which could adversely affect our ability to commercialize our product candidates or any other of our product candidates or technologies covered by the asserted third-party patents. In order to successfully challenge the validity of any such asserted third-party U.S. patent in federal court, we would need to overcome a presumption of validity. As this burden is a high one requiring us to present clear and convincing evidence as to the invalidity of any such U.S. patent claim, there is no assurance that a court of competent jurisdiction would invalidate the claims of any such U.S. patent. Similar challenges exist in other jurisdictions. If we are found to infringe a third-party's valid and enforceable intellectual property rights, we could be required to obtain a license from such third-party to continue developing, manufacturing and marketing our product candidates and technology. However, we may not be able to obtain any required license on commercially reasonable terms or at all. Even if we were able to obtain a license, it could be non-exclusive, thereby giving our competitors and other third parties access to the same technologies licensed to us, and it could require us to make substantial licensing and royalty payments. We could be forced, including by court order, to cease developing, manufacturing and commercializing the infringing technology or product candidates. In addition, we could be found liable for monetary damages, including treble damages and attorneys' fees, if we are found to have willfully infringed a patent or other intellectual property right. A finding of infringement could prevent us from manufacturing and commercializing our product candidates or force us to cease some of our business operations, which could harm our business. In addition, we may be forced to redesign our product candidates, seek new regulatory approvals, and indemnify third parties pursuant to contractual agreements. Claims that we have misappropriated the confidential information or trade secrets of third parties could have a similar negative impact on our business, reputation, financial condition, results of operations and prospects.

Intellectual property litigation could cause us to spend substantial resources and distract our personnel from their normal responsibilities.

Competitors may infringe our intellectual property rights or the intellectual property rights of our licensees or licensors, or we may be required to defend against claims of infringement. To counter infringement or unauthorized use claims or to defend against claims of infringement can be expensive and time-consuming. Even if resolved in our favor, litigation or other legal proceedings relating to intellectual property claims may cause us to incur significant expenses and could distract our technical and management personnel from their normal responsibilities. In addition, there could be public announcements of the results of hearings, motions or other interim proceedings or developments. If securities analysts or investors perceive these results to be negative, it could have a substantial adverse effect on the price of our common stock. Such litigation or proceedings could substantially increase our operating losses and reduce the resources available for development activities or any future sales, marketing or distribution activities. We may not have sufficient financial or other resources to conduct such litigation or proceedings adequately. Some of our competitors may be able to sustain the costs of such litigation or proceedings more effectively than we can because of their greater financial resources and more mature and developed intellectual property portfolios. Uncertainties resulting from the initiation and continuation of patent litigation or other proceedings could adversely affect our ability to compete in the marketplace.

We may be subject to claims asserting that our employees, consultants or advisors have wrongfully used or disclosed alleged trade secrets or other proprietary confidential information or know-how of their current or former employers or claims asserting ownership of what we regard as our own intellectual property.

Many of our directors, employees, consultants, and advisors are currently, or were previously, employed at universities or other biotechnology or pharmaceutical companies, including our competitors or potential competitors. Although we try to ensure that these individuals do not use the proprietary information or know-how of others in their work for us, we may be subject to claims that these individuals or we have used or disclosed intellectual property, including trade secrets or other proprietary information, of any such individual's current or former employer. Litigation may be necessary to defend against these claims. If we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights or personnel. Even if we are successful in defending against such claims, litigation could result in substantial costs and be a distraction to management.

In addition, while it is our policy to require our employees, consultants, advisors and contractors who may be involved in the conception or development of intellectual property to execute agreements assigning such intellectual property to us, we may be unsuccessful in executing such an agreement with each party who, in fact, conceives or develops intellectual property that we regard as our own. The assignment of intellectual property rights may not be self-executing or the assignment agreements may be breached, and we may be forced to bring claims against third parties, or defend claims that they may bring against us, to determine the ownership of what we regard as our intellectual property.

If we fail in prosecuting or defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights or personnel. Even if we are successful in prosecuting or defending against such claims, litigation could result in substantial costs and be a distraction to management.

Changes in U.S. patent law could diminish the value of patents in general, thereby impairing our ability to protect our products.

Patent reform legislation could increase the uncertainties and costs surrounding the prosecution of patent applications and the enforcement or defense of issued patents. On September 16, 2011, the Leahy-Smith America Invents Act, or the Leahy-Smith Act, was signed into law. The Leahy-Smith Act includes several significant changes to U.S. patent law. These include provisions that affect the way patent applications are prosecuted and also may affect patent litigation. These also include provisions that switched the United States from a "first-to-invent" system to a "first-inventor-to-file" system, allow third-party submission of prior art to the USPTO during patent prosecution and set forth additional procedures to attack the validity of a patent by the USPTO administered post grant proceedings. Under a first-inventor-to-file system, assuming the other requirements for patentability are met, the first inventor to file a patent application generally will be entitled to the patent on an invention regardless of whether another inventor had made the invention earlier. The USPTO has promulgated regulations and procedures to govern administration of the Leahy-Smith Act, and many of the substantive changes to patent law associated with the Leahy-Smith Act, and in particular, the first-inventor-to-file provisions, became effective on March 16, 2013. The Leahy-Smith Act has resulted in increased pressure to invest in filing applications earlier, and consequently has increased the uncertainties and costs surrounding the prosecution of our patent applications, and may increase the enforcement or defense of our issued patents, all of which could harm our business, financial condition, results of operations and prospects.

The administrative tribunal created by the Leahy-Smith Act, known as the Patent Trial and Appeals Board, or PTAB, may have an impact on the operation of our business in the future. For example, the initial results of patent challenge proceedings before the PTAB since its inception in 2013 have resulted in the invalidation of many U.S. patent claims. The availability of the PTAB as a lower-cost, faster and potentially more potent tribunal for challenging patents could therefore increase the likelihood that our own licensed patents will be challenged, thereby increasing the uncertainties and costs of maintaining and enforcing them. Moreover, if such challenges occur, we may not have the right to control the defense. In certain situations, we may be required to rely on our licensor to consider our suggestions and to defend such challenges, with the possibility that it may not do so in a way that best protects our interests.

We also may be subject to a third-party pre-issuance submission of prior art to the USPTO or become involved in other contested proceedings such as opposition, derivation, reexamination, *inter partes* review, or post-grant review proceedings challenging our patent rights or the patent rights of others. An adverse determination in any such submission, proceeding or litigation could reduce the scope of, or invalidate, our patent rights, allow third parties to commercialize our technology or products and compete directly with us, without payment to us, or result in our inability to manufacture or commercialize products without infringing third-party patent rights. In addition, if the breadth or strength of protection provided by our patents and patent applications is threatened, it could dissuade companies from collaborating with us to license, develop or commercialize current or future products.

The patent positions of companies engaged in the development and commercialization of biologics and pharmaceuticals are particularly uncertain as the courts address issues such as patenting genes or gene products. Recent guidance provided under *Berkheimer v HP, Inc*. (April 19, 2018) and *Vanda Pharmaceuticals, Inc. v West-Ward Pharmaceuticals* (June 7, 2018) instruct USPTO examiners on the ramifications of the court rulings as applied to method of treatment claims, natural products and principles including all naturally occurring nucleic acids. Patents for certain of our product candidates contain claims related to specific DNA sequences that are naturally occurring and, therefore, could be the subject of future challenges made by third parties. In addition, the recent USPTO guidance could make it impossible for us to pursue similar patent claims in patent applications we may prosecute in the future.

We cannot assure you that our efforts to seek patent protection for our technology and products will not be negatively impacted by the court decisions referenced above, rulings in other cases or changes in guidance or procedures issued by the USPTO. We cannot fully predict what impact decisions from the U.S. Supreme Court's decisions in *Mayo Collaborative Services v. Prometheus Laboratories* and *Molecular Pathology v. Myriad Genetics, Inc.* or other applicable court decisions may have on the ability of life science companies to obtain or enforce patents relating to their products and technologies in the future. These decisions, the guidance issued by the USPTO and rulings in other cases or changes in USPTO guidance or procedures could have an adverse effect on our existing patent portfolio and our ability to protect and enforce our intellectual property in the future.

Moreover, although the U.S. Supreme Court has held that isolated segments of naturally occurring DNA are not patent-eligible subject matter, certain third parties could allege that activities that we may undertake infringe other gene-related patent claims, and we may deem it necessary to defend ourselves against these claims by asserting non-infringement and/or invalidity positions, or paying to obtain a license to these claims. In any of the foregoing or in other situations involving third-party intellectual property rights, if we are unsuccessful in defending against claims of patent infringement, we could be forced to pay damages or be subjected to an injunction that would prevent us from utilizing the patented subject matter. Such outcomes could harm our business, financial condition, results of operations or prospects.

Outside the United States, other courts have also begun to address the patenting of genetic material. In August 2015, the Australian High Court ruled that isolated genes cannot be patented in Australia. The decision did not address methods of using genetic material. Any ruling of a similar scope in other countries could affect the scope of our intellectual property rights. The ambiguities and changing law in all countries as to patenting genetic material may directly affect our ability to secure and/or maintain patent protection for our gene therapy products.

If we do not obtain patent term extension and regulatory exclusivity for our product candidates, our business may be harmed.

Patents have a limited lifespan. In the United States, if all maintenance fees are timely paid, the natural expiration of a patent is generally 20 years from its earliest U.S. non-provisional filing date. Various extensions may be available, but the life of a patent, and the protection it affords, is limited. Even if patents covering our product candidates are obtained, once the patent life has expired, we may be open to competition from competitive products, including generics or biosimilars.

Depending upon the timing, duration and specifics of any FDA marketing approval of our product candidates, one or more of our U.S. patents, which may cover non-gene therapy compounds, may be eligible for limited patent term extension under the Drug Price Competition and Patent Term Restoration Act of 1984, or the Hatch-Waxman Act. The

Hatch-Waxman Act permits a patent extension term of up to five years as compensation for patent term lost during the FDA regulatory review process. A patent term extension cannot extend the remaining term of a patent beyond a total of 14 years from the date of product approval, only one patent may be extended per FDA-approved product, and only those claims covering the approved drug, an approved method for using it, or a method for manufacturing it may be extended. Further, certain of our licenses currently or in the future may not provide us with the right to control decisions the licensor or its other licensees on Orange Book listings or patent term extension decisions under the Hatch-Waxman Act. Thus, if one of our important licensed patents is eligible for a patent term extension under the Hatch-Waxman Act, and it covers a product of another licensee in addition to our own product candidate, we may not be able to obtain that extension if the other licensee seeks and obtains that extension first. However, we may not be granted an extension because of, for example, failing to exercise due diligence during the testing phase or regulatory review process, failing to apply within applicable deadlines, failing to apply prior to expiration of relevant patents or otherwise failing to satisfy applicable requirements.

The BPCIA provides up to 12 years of market exclusivity for a reference biological product. We may not be able to obtain such exclusivity for our products. Moreover, the applicable time-period or the scope of patent protection afforded during any such extension could be less than we request. If we are unable to obtain patent term extension or the scope of term of any such extension is less than we request, the period during which we will have the right to exclusively market our product may be shortened and our competitors may obtain approval of competing products following our patent expiration, and our revenue could be materially reduced.

Intellectual property rights do not necessarily address all potential threats.

The degree of future protection afforded by our intellectual property rights is uncertain because intellectual property rights have limitations, and such rights may not adequately protect our business or permit us to maintain our competitive advantage. For example:

- others may be able to make antibody or gene therapy products that are similar to our product candidates but that are not covered by the claims of the patents that we own, license or may access in the future;
- we, or our license partners or current or future collaborators, might not have been the first to make the inventions covered by the issued patent or pending patent application that we license or may own in the future;
- we, or our license partners or current or future collaborators, might not have been the first to file patent applications covering certain of our or their inventions;
- others may independently develop similar or alternative technologies or duplicate any of our technologies without infringing our owned or licensed intellectual property rights;
- it is possible that our pending patent applications or those that we may own in the future will not lead to issued patents;
- issued patents that we hold rights to may be held invalid or unenforceable, including as a result of legal challenges by our competitors;
- our competitors might conduct research and development activities in countries where we do not have patent rights and then use the information learned from such activities to develop competitive products for sale in our major commercial markets;
- we may not develop additional proprietary technologies that are patentable;
- the patents of others may have an adverse effect on our business; and

- we may choose not to file a patent for certain inventions, trade secrets or know-how, and a third party may subsequently file a patent covering such intellectual property.

Should any of these events occur, they could significantly harm our business, financial condition, results of operations and prospects.

We may not be able to maintain sufficient control over our proprietary know-how or trade secrets when employees, consultants, advisors or persons with access to our proprietary information terminate their relationship with us.

Despite our efforts to protect our proprietary know-how and trade secrets, our competitors may discover this information, or obtain the benefit of this information, through a breach of confidentiality and/or non-competition obligations by persons who were formerly associated with us but who have established relationships as employees, contractors, consultants or advisors with other companies, including our competitors. The recent departures of certain executives, key employees, consultants or advisors, and the restructuring of our organization, may make it more difficult to enforce our rights in protecting this information. Further, if discovered in a timely manner, our efforts to enforce rights to protect against these types of breaches may not be possible under law, or may not be successful if commenced.

It is also possible that, as we grow and establish ourselves in multiple geographic areas, alignment and/or compliance with company polices may not be consistently maintained. In any such cases, the risk of loss of control or proper management of our proprietary information could jeopardize our intellectual property.

Our reliance on third parties requires us to share our trade secrets, confidential information and know-how, which increases the possibility that a competitor will discover them or that our trade secrets, confidential information and/or know-how will be misappropriated or disclosed.

Because we currently rely on certain third parties to manufacture all or part of our product candidates and to perform quality testing, and because we collaborate with various organizations and academic institutions for the advancement of our proprietary antibody program and gene therapy and vectorized antibody platforms and programs, we must, at times, share our proprietary technology and confidential information, including trade secrets, with them. We seek to protect our proprietary technology, in part, by entering into confidentiality agreements and, if applicable, material transfer agreements, collaborative research agreements, consulting agreements or other similar agreements with our collaborators, advisors, employees and consultants prior to beginning research or disclosing proprietary information. These agreements typically limit the rights of the third parties to use or disclose our confidential information. Despite the contractual provisions employed when working with third parties, the need to share trade secrets and other confidential information and know-how increases the risk that such trade secrets and confidential information and know-how become known by our competitors, are inadvertently incorporated into the technology of others or are disclosed or used in violation of these agreements. Given that our proprietary position is based, in part, on our know-how and trade secrets, a competitor's discovery of our proprietary technology and confidential information or other unauthorized use or disclosure would impair our competitive position and may harm our business, financial condition, results of operations and prospects.

Despite our efforts to protect our trade secrets and know-how, our competitors may discover our trade secrets or know-how, either through breach of these agreements, independent development or publication of information including our trade secrets or know-how by third parties. A competitor's discovery of our trade secrets and/or know-how would impair our competitive position and have an adverse impact on our business, financial condition, results of operations and prospects.

Risks Related to Ownership of Our Common Stock

Sales of a substantial number of shares of our common stock in the public market could cause our stock price to fall.

Persons who were our stockholders prior to our initial public offering continue to hold a substantial number of shares of our common stock. If such persons sell, or indicate an intention to sell, substantial amounts of our common stock in the public market, the trading price of our common stock could decline.

In January 2024, we completed a private placement of 2,145,002 shares of our common stock to Novartis and an underwritten public offering of 7,777,778 shares of our common stock and pre-funded warrants to purchase up to 3,333,333 shares of our common stock. In addition, shares of common stock that are either subject to outstanding options or restricted stock units, or RSUs, or reserved for future issuance under our stock incentive plans will become eligible for sale in the public market to the extent permitted by the provisions of various vesting schedules and Rule 144 and Rule 701 under the Securities Act of 1933, as amended. We have also filed registration statements on Form S-8 permitting shares of common stock issued on exercise of options or the settlement of RSUs to be freely sold in the public market. If these additional shares of common stock are sold, or if it is perceived that they will be sold, in the public market, the trading price of our common stock could decline. We also have an effective registration statement on Form S-3 for the sale of up to $300.0 million in aggregate of an indeterminate number of shares of common stock and preferred stock, an indeterminate principal amount of debt securities, and an indeterminate number of depositary shares, subscription rights, warrants, purchase contract and units, of which we have reserved $75.0 million for the offering, issuance, and sale of common stock through at-the-market offerings or negotiated transactions under a sales agreement we entered into with Cowen and Company, LLC, on November 8, 2022.

Certain holders of our common stock have rights, subject to specified conditions, to require us to file registration statements covering their shares or to include their shares in registration statements that we may file for ourselves or other stockholders. Any sales of securities by these stockholders could have a material adverse effect on the trading price of our common stock.

The price of our common stock may be volatile and fluctuate substantially, which could result in substantial losses for purchasers of our common stock.

The price of our common stock is likely to be volatile and may fluctuate substantially. From January 1, 2023 through December 31, 2023, the sales price of our common stock ranged from a high of $14.34 to a low of $5.87 on the Nasdaq Global Select Market. As a result of this volatility, our stockholders may not be able to sell their common stock at or above the price at which they purchased it. The market price for our common stock may be influenced by many factors, including:

- our success in commercializing any product candidates for which we obtain marketing approval;
- regulatory action and results of clinical trials of our product candidates or those of our competitors;
- the success of competitive products or technologies;
- the results of clinical trials of our product candidates;
- the results of clinical trials of product candidates of our competitors;
- the commencement, termination, and success of our collaborations, including the ability or willingness of our collaboration partners to fulfill their obligations to us;
- regulatory or legal developments in the United States and other countries;
- developments or disputes concerning patent applications, issued patents or other proprietary rights;
- the recruitment or departure of key personnel;
- the level of expenses related to any of our product candidates or clinical development programs;
- the results of our efforts to discover, develop, acquire or in-license additional product candidates or technologies, the cost of commercializing such product candidates, and the cost of development of any such product candidates or technologies;

- actual or anticipated changes in estimates as to financial results, development timelines or recommendations by securities analysts;
- variations in our financial results or those of companies that are perceived to be similar to us;
- the ability to secure third-party reimbursement for our product candidates;
- changes in the structure of healthcare payment systems;
- market conditions in the pharmaceutical and biotechnology sectors;
- general economic, industry and market conditions, including interest rates and inflation; and
- other factors described in this "Risk Factors" section and elsewhere in this Annual Report on Form 10-K.

If our operating results fall below the expectations of investors or securities analysts for a given period, the price of our common stock could decline substantially. Furthermore, any fluctuations in our operating results from period to period may, in turn, cause the price of our stock to fluctuate substantially. We believe that such comparisons of our financial results are not necessarily meaningful and should not be relied upon as an indication of our future performance.

In the past, following periods of volatility in the market price of a company's securities, securities class-action litigation often has been instituted against that company. We also may face securities class-action litigation if we cannot obtain regulatory approvals for or if we otherwise fail to commercialize our product candidates. We and certain of our current and former officers and directors were previously named as defendants in a purported class action lawsuit. This proceeding and other similar litigation, if instituted against us, could cause us to incur substantial costs to defend such claims and divert management's attention and resources, which could seriously harm our business, financial condition, results of operations and prospects.

We have broad discretion in how we apply our available funds, and we may not use these funds effectively, which could affect our results of operations and cause our stock price to decline.

Our management will have broad discretion in the application of our existing cash, cash equivalents and marketable securities and could spend these funds in ways that do not improve our results of operations or enhance the value of our common stock. The failure by our management to apply our available funds effectively could result in financial losses that could cause the price of our common stock to decline and delay the development of our product candidates and preclinical programs. Pending their use, we may invest our available funds in a manner that does not produce income or that loses value.

We are a "smaller reporting company" and the reduced disclosure requirements applicable to such companies may make our common stock less attractive to investors.

We are a "smaller reporting company," as defined in Rule 12b-2 under the Securities Exchange Act of 1934, as amended. We would cease to qualify as a smaller reporting company if we have (a) a non-affiliate public float in excess of $250 million and annual revenues in excess of $100 million during our last fiscal year, or (b) a non-affiliate public float in excess of $700 million, in each case determined on an annual basis as of the last business day of our second quarter. As a smaller reporting company, we are permitted and intend to rely on exemptions from certain disclosure requirements that are applicable to other public companies that are not smaller reporting companies. These exemptions include:

- being permitted to provide only two years of audited consolidated financial statements in this Annual Report on Form 10-K, with correspondingly reduced "Management's Discussion and Analysis of Financial Condition and Results of Operations" disclosure;
- reduced disclosure obligations regarding executive compensation; and

- not being required to furnish a stock performance graph in our annual report.

We expect to continue to take advantage of some or all of the available exemptions until we cease to be a smaller reporting company. We may cease to qualify as a smaller reporting company as early as June 30, 2024, which would require us to comply with disclosure requirements that are applicable to other public companies that are not smaller reporting companies following the filing of our Annual Report on Form 10-K for the year ending December 31, 2024, and any portions of our definitive proxy statement relating to our 2025 Annual Meeting of Stockholders incorporated by reference therein. We cannot predict whether investors will find our common stock less attractive if we rely on these exemptions. If some investors find our common stock less attractive as a result, there may be a less active trading market for our common stock and our stock price may be more volatile.

We have been, and could in the future be, subject to legal actions and proceedings related to the decline in our stock price, which could distract our management and could result in substantial costs or large judgments against us.

The market prices of securities of companies in the biotechnology and pharmaceutical industry, including the market price of our common stock, have been extremely volatile and have experienced fluctuations that have often been unrelated or disproportionate to the operating performance of these companies. In January 2021, a putative class action lawsuit was filed against us and certain of our current and former officers and directors. In July 2021, the lead plaintiff voluntarily dismissed the action without prejudice against all defendants and as to all claims, and this action is no longer pending. Nonetheless, due to the volatility in, or the unfulfilled expectations of stockholders for, our stock price, we may be the target of similar litigation in the future.

In connection with such legal proceedings, we could incur substantial costs and such costs and any related settlements or judgments may not be covered by insurance. We could also suffer an adverse impact on our reputation and a diversion of management's attention and resources, which could cause serious harm to our business, operating results and financial condition.

Provisions in our amended and restated certificate of incorporation and bylaws and Delaware law could make an acquisition of us, which may be beneficial to our stockholders, more difficult and may prevent attempts by our stockholders to replace or remove our current management.

Provisions in our amended and restated certificate of incorporation and bylaws may discourage, delay or prevent a merger, acquisition or other change in control of us that stockholders may consider favorable, including transactions in which our stockholders might otherwise receive a premium for their shares. These provisions also could limit the price that investors might be willing to pay in the future for shares of our common stock, thereby depressing the market price of our common stock. In addition, because our board of directors is responsible for appointing the members of our management team, these provisions may frustrate or prevent any attempts by our stockholders to replace or remove our current management by making it more difficult for stockholders to replace members of our board of directors. Among other things, these provisions:

- establish a classified board of directors such that only one of three classes of members of the board is elected each year;
- allow the authorized number of our directors to be changed only by resolution of our board of directors;
- limit the manner in which stockholders can remove directors from the board;
- establish advance notice requirements for stockholder proposals that can be acted on at stockholder meetings and nominations to our board of directors;
- require that stockholder actions must be effected at a duly called stockholder meeting and prohibit actions by our stockholders by written consent;
- limit who may call stockholder meetings;

- authorize our board of directors to issue preferred stock without stockholder approval, which could be used to institute a stockholder rights plan, or so-called “poison pill,” that would work to dilute the stock ownership of a potential hostile acquirer, effectively preventing acquisitions that have not been approved by our board of directors; and

- require the approval of the holders of at least 75% of the votes that all our stockholders would be entitled to cast to amend or repeal certain provisions of our amended and restated certificate of incorporation or bylaws.

Moreover, because we are incorporated in Delaware, we are governed by the provisions of Section 203 of the Delaware General Corporation Law, which prohibits a person who owns in excess of 15% of our outstanding voting stock from merging or combining with us for a period of three years after the date of the transaction in which the person acquired in excess of 15% of our outstanding voting stock, unless the merger or combination is approved in a prescribed manner.

Our amended and restated certificate of incorporation designates the Court of Chancery of the State of Delaware as the sole and exclusive forum for certain types of actions and proceedings that may be initiated by our stockholders, which could limit our stockholders’ ability to obtain a favorable judicial forum for disputes with us or our directors, officers or employees.

Our amended and restated certificate of incorporation provides that, unless we consent in writing to an alternative forum, the Court of Chancery of the State of Delaware will be the sole and exclusive forum for (a) any derivative action or proceeding brought on our behalf, (b) any action asserting a claim of breach of a fiduciary duty owed by any of our directors, officers and employees to us or our stockholders, (c) any action asserting a claim arising pursuant to any provision of the Delaware General Corporation Law, our certificate of incorporation or our bylaws or (d) any action asserting a claim that is governed by the internal affairs doctrine, in each case subject to the Court of Chancery having personal jurisdiction over the indispensable parties named as defendants therein. Any person purchasing or otherwise acquiring any interest in any shares of our capital stock shall be deemed to have notice of and to have consented to this provision of our amended and restated certificate of incorporation. This choice of forum provision is inapplicable to actions arising under the Securities Exchange Act of 1934, as amended, and we likewise do not intend to apply this choice of forum provision to actions arising under the Securities Act of 1933, as amended.

This choice of forum provision may limit a stockholder’s ability to bring a claim that is not arising under the Securities Exchange Act of 1934, as amended, or the Securities Act of 1933, as amended, in a judicial forum that he, she or it finds favorable for disputes with us or our directors, officers or employees, which may discourage such lawsuits against us and our directors, officers and employees even though an action, if successful, might benefit our stockholders. Stockholders who do bring a claim in the Court of Chancery could face additional litigation costs in pursuing any such claim, particularly if they do not reside in or near the State of Delaware. The Court of Chancery may also reach different judgments or results than would other courts, including courts where a stockholder considering an action may be located or would otherwise choose to bring the action, and such judgments or results may be more favorable to us than to our stockholders. Alternatively, if a court were to find this provision of our amended and restated certificate of incorporation inapplicable to, or unenforceable in respect of, one or more of the specified types of actions or proceedings, we may incur additional costs and business interruption that could have a material adverse effect on our business, financial condition or results of operations.

Because we do not anticipate paying any cash dividends on our capital stock in the foreseeable future, capital appreciation, if any, will be our stockholders’ sole source of gain.

We have never declared or paid cash dividends on our capital stock. We currently intend to retain all of our future earnings, if any, to finance the growth and development of our business. In addition, the terms of any future debt agreements may preclude us from paying dividends. As a result, capital appreciation, if any, of our common stock will be the sole source of gain for our stockholders for the foreseeable future.

General Risk Factors

We might not be able to utilize a significant portion of our net operating loss carryforwards.

As of December 31, 2023, we had both federal and state net operating loss, or NOL, carryforwards of $55.3 million and $33.2 million, respectively. The state NOLs will expire beginning in 2041 while the federal NOLs do not expire. These state NOL carryforwards could expire unused and be unavailable to offset our future income tax liabilities. As described above under the heading “Changes in tax laws or in their implementation or interpretation may adversely affect our business and financial condition,” the TCJA, as amended by the CARES Act, includes changes to U.S. federal tax rates and the rules governing NOL carryforwards that may significantly impact our ability to utilize our NOLs to offset taxable income in the future. Nor is it clear how various states will respond to the TCJA, the Families First Coronavirus Response Act or the CARES Act. In addition, state NOLs generated in one state cannot be used to offset income generated in another state. Furthermore, the use of NOL carryforwards may become subject to an annual limitation under Section 382 of the Internal Revenue Code, or the Code, and similar state provisions in the event of certain cumulative changes in the ownership interest of significant shareholders in excess of 50 percent over a three-year period. This could limit the amount of NOL carryforwards that can be utilized annually to offset future taxable income or tax liabilities. The amount of the annual limitation is determined based on the value of a company immediately prior to the ownership change. Our company has completed several transactions since its inception which resulted in an ownership change under Section 382 of the Code. In addition, future changes in our stock ownership, some of which are outside of our control, could result in ownership changes in the future. For these reasons, even if we attain profitability, we may be unable to use a material portion of our NOLs and other tax attributes.

Our internal computer systems, or those of our collaborators or other contractors or consultants, may fail or suffer security breaches, which could result in a material disruption of our product development programs.

Our internal computer systems and those of our current and any future collaborators and other contractors or consultants are vulnerable to damage from cyber-attacks, computer viruses, unauthorized access, ransom requests, sabotage, natural disasters, terrorism, war and telecommunication and electrical failures. While we have not experienced any such material system failure, accident or security breach to date, if such an event were to occur and cause interruptions in our operations or the operations of those third parties with which we contract, it could result in a material disruption of our development programs and our business operations, whether due to a loss of our trade secrets or other proprietary information or other similar disruptions, and could require a substantial expenditure of resources to remedy. For example, the loss of clinical trial data from completed or ongoing clinical trials could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data. We could also be subject to risks caused by misappropriation, misuse, leakage, falsification or intentional or accidental release or loss of information maintained in our information systems and networks, including personal information of our employees. Outside parties may attempt to penetrate our systems or those of the third parties with which we contract or to fraudulently induce our employees or employees of such third parties to disclose sensitive information to gain access to our data or to use such access to request cash compensation in the form of a ransom for the return of such data.

The number and complexity of these threats continue to increase over time. Although we develop and maintain systems and controls designed to prevent these events from occurring, and we have a process to identify and mitigate threats, the development and maintenance of these systems, controls and processes is costly and requires ongoing monitoring and updating as technologies change and efforts to overcome security measures become more sophisticated. Despite our efforts, the possibility of these events occurring cannot be eliminated entirely. Although we maintain cyber risk insurance for certain costs we may incur due to a cyber-related event, this insurance may not provide adequate coverage against potential liabilities. To the extent that any disruption or security breach were to result in a loss of, or damage to, our data or applications, or inappropriate disclosure of confidential or proprietary information, or a loss of cash in response to ransom threats, we could incur liability, our competitive and financial position and the market perception of the effectiveness of our security measures could be harmed, our credibility could be damaged, and the further development and commercialization of our product candidates could be delayed.

ITEM 1B. UNRESOLVED STAFF COMMENTS

Not applicable.

ITEM 1C. CYBERSECURITY

We have certain processes for assessing, identifying and managing cybersecurity risks, which are built into our overall risk management program/information technology function. These processes are designed to help protect our information assets and operations from internal and external cyber threats, protect employee, vendor, and collaborator information from unauthorized access or attack, and secure our networks and systems. They include physical, procedural and technical safeguards, response plans, regular tests on our systems, incident simulations, and routine review of our policies and procedures to identify risks and improve our cybersecurity risk management practices. We engage certain external parties, including consultants with expertise in information security, cybersecurity incident response, and governance, to enhance our cybersecurity oversight. We consider the internal risk oversight programs of third-party information technology service providers before engaging them in order to help protect us from any related vulnerabilities.

Our Board of Directors does not believe that there are currently any known risks from cybersecurity threats that are reasonably likely to materially affect us or our business strategy, results of operations or financial condition. Our Audit Committee provides direct oversight over cybersecurity risk, and provides updates to the Board of Directors regarding such oversight. The Audit Committee receives updates from management twice a year regarding cybersecurity matters and is notified between such updates regarding significant new cybersecurity threats or incidents.

Our Vice President of Information Technology leads operational oversight of company-wide cybersecurity strategy, policy, standards, and processes, and works across the relevant departments to assess and help prepare us to address cybersecurity risks. Additionally, our Vice President of Information Technology participates in management's semi-annual updates to the Audit Committee. In the event of a cybersecurity incident, our Vice President of Information Technology works with our information technology department and appropriate representatives of other affected departments to assess and respond to the incident, and to determine whether internal and external reporting of the incident is merited under the circumstances. Our Vice President of Information Technology has more than 15 years of experience managing cybersecurity, including service as the Chief Information Security Officer for a multi-national company in the specialty materials industry. He has experience with compliance programs for cybersecurity-related regulations and standards such as the European Union's General Data Protection Regulation, The Payment Card Industry Data Security Standard (PCI-DSS), and the U.S. Food and Drug Administration's regulations on electronic records and electronic signatures. He has also participated in Gartner cybersecurity programs and conferences and received training in the identification and prevention of cyber threats such as spam, phishing, spear-phishing, malware, and social engineering.

In an effort to deter and detect cyber threats, we annually provide all of our personnel, including full-time and part-time employees and temporary staff, with a data protection, cybersecurity and incident response and prevention training and compliance program, which covers timely and relevant topics, including social engineering, phishing, password protection, confidential data protection, asset use and mobile security, and the importance of reporting all incidents immediately. We also use technology-based tools to mitigate cybersecurity risks and to bolster our employee-based cybersecurity programs. We have implemented a proactive cybersecurity monitoring program that is designed to achieve threat detection and swift response, which includes the use of best-in-class tools to mitigate vulnerabilities and protect our information assets and operations. We also utilize a stringent endpoint security practice, geo-fencing and geo-blocking on firewalls, and automatic prioritization of emails to enable our information technology department to efficiently prioritize response to the most dangerous threats.

ITEM 2. PROPERTIES

Our corporate headquarters are located in Lexington, Massachusetts. Other operations, including laboratory space, are located in Cambridge, Massachusetts. We lease our office and laboratory space, which consist of approximately 26,148 square feet located in Cambridge, Massachusetts and 93,449 square feet located in Lexington, Massachusetts.

ITEM 3. LEGAL PROCEEDINGS

In the ordinary course of business, we are from time to time involved in lawsuits, claims, investigations, proceedings, and threats of litigation relating to intellectual property, commercial arrangements and other matters. While the outcome of any such matters cannot be predicted with certainty, as of December 31, 2023, we were not party to any material pending proceedings. No material governmental proceedings are pending or, to our knowledge, contemplated against us. We are not a party to any material proceedings in which any director, member of senior management or affiliate of ours is either a party adverse to us or our subsidiaries or has a material interest adverse to us or our subsidiaries.

ITEM 4. MINE SAFETY DISCLOSURES

Not applicable.

PART II

ITEM 5. MARKET FOR REGISTRANT'S COMMON EQUITY, RELATED STOCKHOLDER MATTERS AND ISSUER PURCHASES OF EQUITY SECURITIES.

Our common stock has been traded on the Nasdaq Global Select Market under the symbol "VYGR" since November 11, 2015. Prior to this time, there was no public market for our common stock.

Stockholders

As of February 21, 2024, there were approximately 12 holders of record of our common stock. The actual number of stockholders is greater than this number of record holders, and includes stockholders who are beneficial owners, but whose shares are held in street name by brokers and other nominees. This number of holders of record also does not include stockholders whose shares may be held in trust by other entities.

Dividends

We have not paid any cash dividends on our common stock since inception and do not anticipate paying cash dividends in the foreseeable future.

Recent Sales of Unregistered Securities

Set forth below is information regarding shares of our common stock issued and stock options granted by us for the twelve months ended December 31, 2023 that were not registered under the Securities Act of 1933, as amended, or the Securities Act and that have not otherwise been described in a Quarterly Report on Form 10-Q or a Current Report on Form 8-K.

On October 1, 2023, we granted to six employees restricted stock unit awards settleable for an aggregate of 211,500 shares of our common stock. These restricted stock units were made outside of our 2015 Stock Option and Incentive Plan as an inducement material to such individual's acceptance of an offer of employment with us in accordance with Nasdaq Listing Rule 5635(c)(4). We intend to file a registration statement on a Form S-8 to register the shares of common stock underlying these inducement awards prior to the time at which the awards become exercisable or settleable, as applicable.

ITEM 6. RESERVED

ITEM 7. MANAGEMENT'S DISCUSSION AND ANALYSIS OF FINANCIAL CONDITION AND RESULTS OF OPERATIONS

You should read the following discussion and analysis of our financial condition and results of operations together with our consolidated financial statements and related notes appearing elsewhere in this Annual Report on Form 10-K. In addition to historical information, this discussion and analysis contains forward-looking statements that involve risks, uncertainties and assumptions. Our actual results may differ materially from those anticipated in these forward-looking statements as a result of certain factors. We discuss factors that we believe could cause or contribute to these differences below and elsewhere in this report, including those set forth under Item 1A. "Risk Factors" and under "Forward-Looking Statements" in this Annual Report on Form 10-K.

We are a biotechnology company whose mission is to leverage the power of human genetics to modify the course of and ultimately cure neurological diseases. Our pipeline includes programs for Alzheimer's disease, or AD; amyotrophic lateral sclerosis, or ALS; Parkinson's disease, and multiple other diseases of the central nervous system. Many of our programs are derived from our TRACER™ (Tropism Redirection of AAV by Cell Type-Specific Expression of RNA) adeno-associated virus, or AAV, capsid discovery platform, which we have used to generate novel capsids, or TRACER Capsids, and identify associated receptors to potentially enable high brain penetration with genetic medicines following intravenous dosing. Some of our programs are wholly-owned, and some are advancing with licensees and collaborators including Alexion, AstraZeneca Rare Disease, or Alexion; Novartis Pharma AG, or Novartis; Neurocrine Biosciences, Inc., or Neurocrine; and Sangamo Therapeutics, Inc., or Sangamo.

We focus on leveraging our expertise in capsid discovery and neuropharmacology to address the delivery hurdles that have constrained the genetic medicine and neurology disciplines, with the goal of either halting or slowing disease progression or reducing symptom severity, and therefore providing clinically meaningful impact to patients. We are advancing our own proprietary pipeline of drug candidates for neurological diseases, with a focus on AD. Our wholly-owned prioritized pipeline programs include an anti-tau antibody for AD; a superoxide dismutase 1, or SOD1, gene therapy for ALS; and a tau silencing gene therapy for AD. We identified a lead development candidate for our anti-tau antibody program in the first quarter of 2023 and expect to submit an investigational new drug, or IND, application to the U.S. Food and Drug Administration, or the FDA, for this program in the first half of 2024. We believe this trial could result in the potential to generate proof-of-concept data for slowing the spread of pathological tau via tau positron emission tomography imaging in 2026. We identified a lead development candidate for the SOD1 silencing gene therapy program in the fourth quarter of 2023, and we expect to submit the IND application for this program in mid-2025. We promoted our tau silencing gene therapy program to a prioritized program in the first quarter of 2024, based on preclinical data demonstrating robust reductions in tau messenger RNA, or mRNA, in a murine model, and we anticipate submission of an IND in 2026. Our proprietary pipeline also includes an early research initiative to develop a gene therapy for the treatment of AD. This program seeks to combine a vectorized anti-amyloid antibody with a TRACER Capsid.

We are also working with our collaboration partners on multiple programs. In January 2019 and January 2023, we entered into collaboration and license agreements with Neurocrine. Under our agreements with Neurocrine, we are actively advancing two later preclinical stage programs: a glucocerebrosidase 1, or GBA1, gene therapy program for Parkinson's disease and other GBA1-mediated diseases, and a frataxin gene therapy program for Friedreich's ataxia, or the FA Program. Pursuant to such agreements, we are also working with Neurocrine on five early-stage programs for the research, development, manufacture and commercialization of gene therapies designed to address central nervous system diseases or conditions associated with rare genetic targets. We have also entered into agreements with licensees including Novartis, Alexion, and Sangamo, to license or to provide options to receive exclusive licenses to certain TRACER Capsids. As described further below, in December 2023, we entered into a license and collaboration agreement with Novartis to provide Novartis certain rights regarding the development of potential gene therapy product candidates for the treatment of spinal muscular atrophy and to collaborate with Novartis to develop gene therapy product candidates for the treatment of Huntington's disease. The joint steering committee with Neurocrine has selected a development candidate for the FA Program, during the first quarter of 2024, and expects to advance into first-in-human

clinical trials in 2025. We anticipate that our collaborative partners and licensees will submit at least one additional IND application for a partnered program and initiate clinical development for the associated program by the end of 2025.

Despite reporting $132.3 million in net income for the year ended December 31, 2023, we have a history of incurring significant losses. We reported a net loss of $46.4 million for the year ended December 31, 2022. We reported a net loss of $71.2 million for the year ended December 31, 2021. As of December 31, 2023, we had an accumulated deficit of $261.2 million. We expect to continue to incur significant expenses and operating losses for the foreseeable future. We anticipate that our expenses will increase substantially in connection with our ongoing activities, as we:

- conduct preclinical development activities and initiate investigational new drug, or IND, application-enabling studies and clinical trials in connection with our anti-tau antibody program and our SOD1 ALS gene therapy program;
- continue investing in our proprietary antibody program, gene therapy and vectorized antibody platforms and programs, and other research and development initiatives;
- increase our investment in and support for TRACER, our proprietary discovery platform to facilitate the selection of AAV capsids and expand our investment to discover TRACER Capsids with broad tropism in central nervous system, or CNS, and other tissues with cell-specific transduction properties for particular therapeutic applications;
- increase our investment in the discovery and development of modalities for receptor-mediated non-viral delivery of therapeutic payloads to the CNS;
- conduct joint research and development under our strategic collaborations for the research, development, and commercialization of certain of our pipeline programs, including our FA Program pursuant to our collaboration and license agreement with Neurocrine entered into in January 2019, or the 2019 Neurocrine Collaboration Agreement, and our GBA1 Program pursuant to our collaboration and license agreement with Neurocrine entered into on January 8, 2023, or the 2023 Neurocrine Collaboration Agreement; and our Huntington's disease program, or the Novartis HD Program, pursuant to our license and collaboration agreement with Novartis entered into on December 28, 2023, or the 2023 Novartis Collaboration Agreement;
- initiate additional preclinical studies and clinical trials for, and continue research and development of, our other programs;
- continue our process research and development activities, as well as establish our research-grade manufacturing capabilities;
- identify additional diseases for treatment with our AAV gene therapies and develop additional programs or product candidates;
- seek marketing and regulatory approvals for any of our product candidates that successfully complete clinical development;
- maintain, expand, protect and enforce our intellectual property portfolio;
- identify, acquire or in-license other product candidates and technologies;
- expand our operational, financial and management systems and personnel, including personnel to support our clinical development, manufacturing and commercialization efforts;
- continue our clinical trial insurance coverage as we expand our clinical trials and increase our product liability insurance once we engage in commercialization efforts; and

- continue to operate as a public company.

Financial Operations Overview

Revenue

To date, we have not generated any revenue from product sales and do not expect to generate any revenue from product sales for the foreseeable future. For the year ended December 31, 2023, we recognized $80.8 million of collaboration revenue from the 2023 Neurocrine Collaboration Agreement, $80.0 million of collaboration revenue from the 2023 Novartis Collaboration Agreement, $79.0 million of collaboration revenue from our option and license agreement with Novartis entered into on March 4, 2022, or the 2022 Novartis Agreement, $9.8 million of collaboration revenue from the 2019 Neurocrine Collaboration Agreement, and $0.4 million of other collaboration revenue. For additional information about our revenue recognition policy, see the section titled "—Summary of significant accounting policies and basis of presentation."

For the foreseeable future, we expect substantially all of our revenue will be generated from the 2019 Neurocrine Collaboration Agreement, the 2023 Neurocrine Collaboration Agreement, the 2023 Novartis Collaboration Agreement, the 2022 Novartis Option and License Agreement, and our option and license agreement with Alexion entered into on October 1, 2021, or the Alexion Agreement, and any other strategic collaborations and out-licensing arrangements we may enter into in the future. If our development efforts are successful, we may also generate revenue from product sales.

Expenses

Research and Development Expenses

Research and development expenses consist primarily of costs incurred for our research activities, including our program discovery efforts, and the development of our proprietary antibody program and gene therapy and vectorized antibody platforms and programs which include:

- employee-related expenses including salaries, benefits, and stock-based compensation expense;
- costs of funding research performed by third parties that conduct research and development, preclinical activities, manufacturing and production design on our behalf;
- the cost of purchasing laboratory supplies and non-capital equipment used in designing, developing and manufacturing preclinical study materials;
- consultant fees;
- facility costs including rent, depreciation and maintenance expenses;
- the cost of securing and protecting intellectual property rights associated with our research and development activities; and
- fees for maintaining licenses under our third-party licensing agreement.

Research and development costs are expensed as incurred. Costs for certain activities, such as manufacturing, preclinical studies, and clinical trials, are generally recognized based on an evaluation of the progress to completion of specific tasks using information and data provided to us by our vendors and collaborators.

Research and development activities are central to our business model. We are in the early stages of development of our product candidates. During the year ended December 31, 2023, our research and development expenses have increased as compared to the amounts recorded in the same period in the prior year. As our development programs progress and as we identify product candidates and initiate preclinical studies and clinical trials, including as we initiate our planned Phase 1 clinical trial to evaluate VY-TAU01 in 2024, we expect research and development costs

to continue to increase. However, at this time, we cannot reasonably estimate or know the nature, timing and estimated costs of the efforts that will be necessary to complete the development of our product candidates.

Because of the numerous risks and uncertainties associated with pharmaceutical product development, we are unable to accurately predict the timing or amount of increased expenses. Our expenses will increase if:

- we are required by the FDA or the European Medicines Agency or other regulatory agencies to redesign or modify trials or studies or to perform trials or studies in addition to those currently expected;
- there are any delays in the receipt of regulatory clearance to begin our planned clinical programs; or
- there are any delays in enrollment of patients in or completing our clinical trials or the development of our product candidates.

General and Administrative Expenses

General and administrative expenses consist primarily of salaries and other related costs, including stock-based compensation, for personnel in executive, finance, accounting, information technology, business development, legal and human resource functions. Other significant costs include corporate facility costs not otherwise included in research and development expenses, legal fees related to patent and corporate matters and fees for accounting and consulting services.

During the year ended December 31, 2023, our general and administrative expenses have increased as compared to the amount recorded in the same period in prior year. As our development programs progress and we identify product candidates and initiate preclinical studies and clinical trials, including as we initiate our planned Phase 1 clinical trial to evaluate VY-TAU01 in 2024, we continue to expect general and administrative expenses to increase to support these additional research and development activities.

Other Income, Net

Other income, net consists primarily of interest income on our marketable securities.

Critical Accounting Policies and Estimates

Our management's discussion and analysis of our consolidated financial condition and results of operations are based on our consolidated financial statements, which have been prepared in accordance with U.S. generally accepted accounting principles. The preparation of these consolidated financial statements requires us to make judgments and estimates that affect the reported amounts of assets, liabilities, revenues and expenses and the disclosure of contingent assets and liabilities in our consolidated financial statements. We base our estimates on historical experience, known trends and events, and various other factors that are believed to be reasonable under the circumstances. Actual results may differ from these estimates under different assumptions or conditions. On an ongoing basis, we evaluate our judgments and estimates in light of changes in circumstances, facts and experience. The effects of material revisions in estimates, if any, will be reflected in the financial statements prospectively from the date of change in estimates.

While our significant accounting policies are described in more detail in the notes to our consolidated financial statements appearing elsewhere in this Annual Report on Form 10-K, we believe that certain aspects of our accounting policy on revenue recognition require the most significant judgments and estimates in the preparation of our financial statements.

Revenue Recognition – ASC 606

We recognize revenue in accordance with Financial Accounting Standards Board, or FASB, Accounting Standards Codification, or ASC, Topic 606 *Revenue from Contracts with Customers,* or ASC 606.

We enter into license, option, and collaboration agreements which are within the scope of ASC 606, under which we license or provide options to license certain of our product candidates and, in certain cases, perform research and development. The terms of these arrangements typically include payment of one or more of the following: non-refundable, upfront fees; reimbursement of research and development costs; development, regulatory and commercial milestone payments; option exercise fees; and royalties on net sales of licensed products.

We estimate the transaction price based on the amount expected to be received for transferring the promised goods or services in the contract. The consideration may include fixed consideration and/or variable consideration. At the inception of each arrangement that includes variable consideration, we evaluate the amount of potential payment and the likelihood that the payments will be received. We utilize either the most likely amount method or expected amount method to estimate the amount expected to be received based on which method best predicts the amount expected to be received. The amount of variable consideration which is included in the transaction price may be constrained, and is included in the transaction price only to the extent that it is probable that a significant reversal in the amount of the cumulative revenue recognized will not occur in a future period.

Our contracts often include development and regulatory milestone payments which are assessed under the most likely amount method and constrained if it is probable that a significant revenue reversal would occur. Milestone payments that are not within our control or the licensee's control, such as regulatory approvals, are not considered probable of being achieved until those approvals are received. At the end of each reporting period, we re-evaluate the probability of achievement of such development milestones and any related constraint, and if necessary, adjust our estimate of the overall transaction price. Given the nature of the milestone payments in our contracts with customers, most of the variable consideration is subject to a constraint that does not involve significant judgement.

We allocate the transaction price based on the estimated stand-alone selling price of each of the performance obligations. We must develop assumptions that require judgment to determine the stand-alone selling price for each performance obligation identified in the contract. We utilize key assumptions to determine the stand-alone selling price for performance obligations, which may include other comparable transactions, pricing considered in negotiating the transaction and the estimated costs. Additionally, in determining the standalone selling price for material rights, we utilize comparable transactions, industry standards for product development and clinical trial success probabilities and estimates of option exercise likelihood. We do not believe that reasonable changes in the assumptions used to determine stand-alone selling price for our performance obligations would materially impact the amount of revenue we recognize.

The consideration allocated to each performance obligation is recognized as revenue when control is transferred for the related goods or services. For performance obligations which consist of licenses and other promises, we utilize judgment to assess the nature of the combined performance obligation to determine whether the combined performance obligation is satisfied over time or at a point in time and, if over time, the appropriate method of measuring progress. We evaluate the measure of progress each reporting period and, if necessary, adjust the measure of performance and related revenue recognition.

A significant portion of revenue recognized from the 2019 Neurocrine Collaboration Agreement and the 2023 Neurocrine Collaboration Agreement is related to performance obligations pursuant to which revenue is recognized using a proportional performance model. Revenue is recognized using input-based measurements, which involves the measurement of progress toward each performance obligation based on the actual costs incurred compared to total projected costs. We use judgement in estimating the expected remaining costs to complete the research and development services for each performance obligation based on discussions with the joint steering committee for the program and discussions with our collaboration partners. We evaluate the measure of progress each reporting period and, if necessary, adjust the measure and related revenue recognition. Changes in our estimates of the expected remaining costs to complete the research and development services for our performance obligations, such as the significant change that occurred in the fourth quarter of 2021 as a result of decisions made by the JSC for the 2019 Neurocrine Collaboration Agreement, can result in significant changes to the amount of revenue we recognize each period.

Results of Operations

Comparison of the years ended December 31, 2023 and 2022:

The following table summarizes our results of operations for the years ended December 31, 2023 and 2022, respectively, together with the changes in those items in dollars:

	Year ended December 31,		
	2023	2022	Change
		(in thousands)	
Collaboration revenue	$ 250,008	$ 40,907	$ 209,101
Operating expenses:			
Research and development	92,172	60,764	31,408
General and administrative	35,822	30,980	4,842
Total operating expenses	127,994	91,744	36,250
Other income, net:			
Interest income	11,721	1,792	9,929
Other income	3	2,653	(2,650)
Total other income, net	11,724	4,445	7,279
Income (loss) before income taxes	133,738	(46,392)	180,130
Income tax provision	1,408	16	1,392
Net income (loss)	$ 132,330	$ (46,408)	$ 178,738

Collaboration Revenue

Collaboration revenue was $250.0 million and $40.9 million for the years ended December 31, 2023 and 2022, respectively. The increase in collaboration revenue was the result of $79.0 million in revenue recognized during the year ended December 31, 2023, in connection with Novartis' decision to exercise two of its license options under the 2022 Novartis Option and License Agreement, or Novartis License Options, along with the expiration of a third Novartis License Option. In addition, during the year ended December 31, 2023, we recognized $80.0 million of revenue associated with the 2023 Novartis Collaboration Agreement, $80.8 million of revenue associated with the 2023 Neurocrine Collaboration Agreement, $9.8 million of revenue associated with the 2019 Neurocrine Collaboration Agreement, and $0.4 million of other collaboration revenue. During the year ended December 31, 2022, collaboration revenue was primarily related to Pfizer's decision, as Alexion's predecessor-in-interest under the Alexion Agreement, to exercise the first material right for a license option under the Alexion Agreement, or the Pfizer License Option, along with the expiration of the second material right associated with the Pfizer License Option, which resulted in revenue recognized of $40.0 million.

Research and Development Expense

Research and development expense increased by $31.4 million from $60.8 million for the year ended December 31, 2022 to $92.2 million for the year ended December 31, 2023. The following table summarizes our research and development expenses for the years ended December 31, 2023 and 2022:

	Year ended December 31,		
	2023	2022	Change
		(in thousands)	
Employee and consultant	$ 42,445	$ 29,209	$ 13,236
External research and development	34,185	15,679	18,506
Facilities and other	6,790	7,863	(1,073)
Professional fees	8,752	8,013	739
Total research and development expenses	$ 92,172	$ 60,764	$ 31,408

The increase in research and development expense for the year ended December 31, 2023 was primarily attributable to the following:

- approximately $13.2 million for increased employee and consultant-related costs associated with higher headcount in research and development functions as compared to the same period in the prior year due to the progress in our development programs; and

- approximately $18.5 million for external research and development costs related to increased program-related spending, particularly on manufacturing and IND-enabling studies for our anti-tau antibody program and SOD1 program, along with increased Neurocrine program support during the 2023 period, and the fee due to Touchlight pursuant to our license agreement;

- approximately $0.7 million for increased professional fees to support our pipeline programs; partially offset by

- approximately $1.1 million for decreased facility and other costs primarily related to the termination of the lease for office and laboratory space at 75 Sidney Street during the second quarter of 2022.

General and Administrative Expense

General and administrative expense increased by $4.8 million from $31.0 million for the year ended December 31, 2022 to $35.8 million for the year ended December 31, 2023. The increase in general and administrative expense was primarily attributable to increased compensation costs and stock-based compensation associated with higher headcount in general and administrative functions as compared to the same period in the prior year, as well as the recognition of $1.9 million of business development costs related to the 2023 Novartis Collaboration Agreement as general and administrative expenses during the year ended December 31, 2023.

Other Income, Net

Other income, net of approximately $11.7 million was recognized during the year ended December 31, 2023, as compared to $4.4 million during the year ended December 31, 2022. Other income, net during the year ended December 31, 2023 was primarily related to interest income due to increased interest rates on increased balances of marketable securities, while other income, net during the year ended December 31, 2022 was primarily related to an employee retention tax credit under the Coronavirus Aid, Relief, and Economic Security Act and interest income on marketable securities balances.

Liquidity and Capital Resources

Sources of Liquidity

We have funded our operations primarily through private placements of redeemable convertible preferred stock, public offerings and private placements of our common stock, strategic collaborations and option and license arrangements, including our 2019 Neurocrine Agreement, 2023 Neurocrine Collaboration Agreement, 2022 Novartis Agreement, 2023 Novartis Collaboration Agreement, Alexion Agreement, and with our prior collaboration agreements.

As of December 31, 2023, we had cash, cash equivalents, and marketable securities of $230.9 million. Based upon our current operating plan, we expect that our existing cash, cash equivalents, and marketable securities at December 31, 2023, together with the $80.0 million upfront payment received in January 2024 in connection with the 2023 Novartis Collaboration Agreement, the $20.0 million in proceeds from Novartis' stock purchase, the $93.5 million in net proceeds received from a public offering in January 2024 (refer to Note 15 of our consolidated financial statements included elsewhere in this Annual Report on Form 10-K), along with amounts expected to be received as reimbursement for development costs under our collaboration and license agreements with Neurocrine and Novartis, certain near term milestones, and interest income, to be sufficient to meet our planned operating expenses and capital expenditure requirements into 2027.

Cash Flows

The following table provides information regarding our cash flows for the years ended December 31, 2023, 2022, and 2021:

	Year ended December 31,		
	2023	2022	2021
	(in thousands)		
Net cash provided by (used in):			
Operating activities	$ 77,919	$ (12,509)	$ (53,525)
Investing activities	(141,643)	(7,339)	65,906
Financing activities	33,645	1,110	612
Net (decrease) increase in cash, cash equivalents, and restricted cash	$ (30,079)	$ (18,738)	$ 12,993

Cash Flows from Operating Activities

Net cash provided by operating activities was $77.9 million during the year ended December 31, 2023 as compared to $12.5 million of net cash used in operating activities for the year ended December 31, 2022. The increase was primarily due to our net income for the year ended December 31, 2023 of $132.3 million as compared to our net loss for the year ended December 31, 2022 of $46.4 million. This is offset by an increase in accounts receivable of $80.2 million during the year ended December 31, 2023 primarily attributable to the upfront payment due in connection with the 2023 Novartis Collaboration Agreement, as compared to no accounts receivable activity during the year ended December 31, 2022.

Net cash used in operating activities was $12.5 million during the year ended December 31, 2022. The cash used in operating activities for the year ended December 31, 2022 was primarily driven by operating expenses, net of stock-based compensation and depreciation, offset by an increase in deferred revenue partially driven by the upfront payment of $54.0 million from Novartis in connection with our entry into the 2022 Novartis Agreement during the year ended December 31, 2022.

Net cash used in operating activities was $53.5 million during the year ended December 31, 2021. The cash used in operating activities for the year ended December 31, 2021 was primarily driven by operating expenses, net of stock-based compensation and depreciation. We also received an upfront payment of $30.0 million from Pfizer as the predecessor-in-interest to Alexion, in connection with our entry into the Alexion Agreement.

Cash Flows from Investing Activities

Net cash used in investing activities was $141.6 million during the year ended December 31, 2023. The cash used in investing activities for the year ended December 31, 2023 was primarily due to $224.0 million for purchases of marketable securities and $3.3 million for purchases of property and equipment, offset by $85.6 million from proceeds from maturities and sales of marketable securities.

Net cash used in investing activities was $7.3 million during the year ended December 31, 2022. The cash used in investing activities for the year ended December 31, 2022 was primarily due to $54.8 million for purchases of marketable securities and $2.5 million for purchases of property and equipment, offset by $50.0 million from proceeds from maturities and sales of marketable securities.

Net cash provided by investing activities was $65.9 million during the year ended December 31, 2021. The cash provided by investing activities for the year ended December 31, 2021 was primarily due to $70.0 million from maturities of marketable securities and $12.6 million from proceeds of sales of marketable securities partially offset by $15.1 million for purchases of marketable securities and $1.6 million for purchases of property and equipment.

Cash Flows from Financing Activities

Net cash provided by financing activities was $33.6 million during the year ended December 31, 2023 primarily due to the $31.1 million in proceeds from the issuance of common stock in connection with the 2023 Neurocrine Collaboration Agreement along with proceeds from the exercise of stock options, and purchases by our employees of our common stock under our employee stock purchase plan.

Net cash provided by financing activities was $1.1 million during the year ended December 31, 2022 primarily due to the proceeds from the exercise of stock options, and purchases by our employees of our common stock under our employee stock purchase plan.

Net cash provided by financing activities was $0.6 million during the year ended December 31, 2021 primarily due to the proceeds from the exercise of stock options, and purchases by our employees of our common stock under our employee stock purchase plan.

Funding Requirements

Our expenses increased during the year ended December 31, 2023 as compared with the prior year as our development programs progressed and we increased headcount. We expect our expenses to continue to increase as we continue the research and development of, conduct clinical trials of, and seek marketing approval for our product candidates including as we initiate our planned Phase 1 clinical trial to evaluate VY-TAU01 in 2024, and as we continue to enter into or conduct activities in connection with our collaboration agreements. In addition, if we obtain marketing approval for any of our product candidates, we expect to incur significant expenses related to program sales, marketing, manufacturing and distribution to the extent that such sales, marketing and distribution are not the responsibility of potential collaborators. Furthermore, we expect to incur increasing costs associated with operating as a public company, executing financial statement controls, satisfying regulatory and quality standards, fulfilling healthcare compliance requirements, and maintaining product, clinical trial and directors' and officers' liability insurance coverage. We also anticipate the cost of goods and services and the levels of compensation paid to employee will increase due to inflationary conditions existing in the general economy. Accordingly, we will need to obtain substantial additional funding in connection with our continuing operations. If we are unable to raise capital or enter into business development transactions when needed or on acceptable terms, we could be forced to delay, reduce or eliminate our research and development programs or any future commercialization efforts.

As of December 31, 2023, we had cash, cash equivalents, and marketable securities of $230.9 million. Based upon our current operating plan, we expect that our existing cash, cash equivalents, and marketable securities at December 31, 2023, together with the $80.0 million upfront payment received in January 2024 in connection with the 2023 Novartis Collaboration Agreement, the $20.0 million in proceeds from Novartis' stock purchase, the $93.5 million in net proceeds received from a public offering executed in January 2024, along with amounts expected to be received as reimbursement for development costs under our collaboration and license agreements with Neurocrine and Novartis, certain near term milestones, and interest income, to be sufficient to meet our planned operating expenses and capital expenditure requirements into 2027. Our future capital requirements will depend on many factors, including:

- the scope, progress, results, and costs of product discovery, preclinical studies and clinical trials for our product candidates;
- the scope, progress, results, costs, prioritization, and number of our research and development programs;
- the progress and status of our strategic collaborations and option and license agreements and any similar arrangements we may enter into in the future, including any research and development costs for which we are responsible, future additional obligations that may be committed to within or outside these agreements, and our receipt of any future milestone payments and royalties from our collaboration partners or licensors;
- the extent to which we are obligated to reimburse preclinical development and clinical trial costs, or the achievement of milestones or occurrence of other developments that trigger milestone and royalty

payments, under any collaboration or license agreements to which we might become a party, such as the Touchlight License Agreement;

- the costs, timing and outcome of regulatory review of our product candidates;
- our ability to establish and maintain collaboration, distribution, or other marketing arrangements for our product candidates on favorable terms, if at all;
- the costs and timing of preparing, filing and prosecuting patent applications, maintaining and enforcing our intellectual property rights and defending intellectual property-related claims;
- the extent to which we acquire or in-license other product candidates and technologies, including any intellectual property associated with such candidates or technologies, acquire or invest in other businesses, or out-license our product candidates, capsids or other technologies;
- the costs of advancing our manufacturing capabilities and securing manufacturing arrangements for pre-commercial and commercial production;
- the level of product sales by us or our collaborators from any product candidates for which we obtain marketing approval in the future;
- the costs of operating as a public company and maintaining adequate product, clinical trial, and directors' and officers' liability insurance coverage; and
- the costs of establishing or contracting for sales, manufacturing, marketing, distribution, and other commercialization capabilities if we obtain regulatory approvals to market our product candidates.

Identifying potential product candidates and conducting preclinical studies and clinical trials is a time-consuming, expensive and uncertain process that takes years to complete. We may never generate the necessary data or results required to obtain marketing approval and achieve product sales. In addition, our product candidates, if approved, may not achieve commercial success. Our product revenues, if any, and any commercial milestone payments or royalty payments under our collaboration agreements, will be derived from sales of products that may not be commercially available for many years, if at all. Accordingly, we will need to continue to rely on additional financing and business development transactions to achieve our business objectives. Adequate additional financing may not be available to us on acceptable terms, or at all.

Until such time, if ever, as we can generate product revenues sufficient to achieve consistent profitability, we expect to finance our cash needs through a combination of equity offerings, debt financings, collaborations, strategic alliances, and option and license arrangements. We do not have any committed external source of funds other than the amounts we are entitled to receive from our collaboration partners and licensors for reimbursement of certain research and development expenses, potential option exercises, the achievement of specified regulatory and commercial milestones, and royalty payments under our collaboration, and option and license agreements, as applicable. To the extent that we raise additional capital through the sale of equity or equity-linked securities, including convertible debt, our stockholders' ownership interests will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect our existing stockholders' rights as holders of our common stock. Debt financing and preferred equity financing, if available, may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, obtaining additional capital, acquiring or divesting businesses, making capital expenditures or declaring dividends.

If we raise additional funds through collaborations, strategic alliances, or option and license arrangements with third parties, we may have to relinquish valuable rights to our technologies, future revenue streams, research programs or product candidates or to grant licenses on terms that may not be favorable to us. If we are unable to raise additional funds through equity or debt financings when needed, we may be required to delay, limit, reduce or terminate our product

development or future commercialization efforts or grant rights to develop and market product candidates that we would otherwise prefer to develop and market ourselves.

Contractual Obligations

We enter into agreements in the normal course of business with clinical research organizations, contract manufacturing organizations, and institutions to license intellectual property. These contracts generally are cancelable at any time by us, upon 30 to 90 days prior written notice.

Our agreements to license intellectual property include potential milestone payments that are dependent upon the development of products using the intellectual property licensed under the agreements and contingent upon the achievement of clinical trial or regulatory approval milestones. We may also be required to pay annual maintenance fees or minimum amounts payable ranging from low-four digits to low five-digits depending upon the terms of the applicable agreement. In certain instances, we are also obligated to pay our licensors royalties based on sales of products, if approved, using the intellectual property licensed under the applicable agreement.

We also have non-cancelable operating lease commitments arising from our leases of office and laboratory space at our facilities in Cambridge and Lexington, Massachusetts. For more information, refer to Note 7 to our consolidated financial statements included elsewhere in this Annual Report on Form 10-K.

Off-Balance Sheet Arrangements

We did not have, during the periods presented, and we do not currently have, any off-balance sheet arrangements, as defined under applicable rules of the Securities and Exchange Commission, or the SEC.

Smaller Reporting Company Status

As of June 30, 2023, we have requalified as a “smaller reporting company,” as defined in Rule 12b-2 under the Securities Exchange Act of 1934, as amended. We would cease to qualify as a smaller reporting company if we have (a) a non-affiliate public float in excess of $250 million and annual revenues in excess of $100 million during our last fiscal year, or (b) a non-affiliate public float in excess of $700 million, in each case determined on an annual basis as of the last business day of our second quarter. As a smaller reporting company, we are permitted and intend to rely on exemptions from certain disclosure requirements that are applicable to other public companies that are not smaller reporting companies. These exemptions include:

- being permitted to provide only two years of audited consolidated financial statements in this Annual Report on Form 10-K, with correspondingly reduced Management's Discussion and Analysis of Financial Condition and Results of Operations disclosure;
- reduced disclosure obligations regarding executive compensation; and
- not being required to furnish a stock performance graph in our annual report.

We expect to continue to take advantage of some or all of the available exemptions until we cease to be a smaller reporting company. We may cease to qualify as a smaller reporting company as early as June 30, 2024, which would require us to comply with disclosure requirements that are applicable to other public companies that are not smaller reporting companies following the filing of our Annual Report on Form 10-K for the year ending December 31, 2024, and any portions of our definitive proxy statement relating to our 2025 Annual Meeting of Stockholders incorporated by reference therein.

ITEM 7A. QUANTITATIVE AND QUALITATIVE DISCLOSURES ABOUT MARKET RIS K

We are exposed to market risk related to changes in interest rates. We have policies requiring us to invest in high-quality issuers, limit our exposure to any individual issuer, and ensure adequate liquidity. Our primary exposure to

market risk is interest rate sensitivity, which is affected by changes in the general level of U.S. interest rates, particularly because our investments, including cash equivalents, are in the form of money market funds and marketable securities and are invested in U.S. Treasury notes. Due to the short-term duration of our investment portfolio and the low risk profile of our investments, we believe an immediate 100 basis point change in interest rates would not have a material effect on the fair market value of our portfolio.

We are not currently exposed to market risk related to changes in foreign currency exchange rates; however, we may contract with vendors that are located in Asia and Europe in the future and may be subject to fluctuations in foreign currency rates at that time.

Inflation generally affects us by increasing our costs of labor, goods, and services. We do not believe that inflation had, or that an immediate 100 basis point change in inflation would have had, a material effect on our business, financial condition, or results of operations during the year ended December 31, 2023.

ITEM 8. FINANCIAL STATEMENTS AND SUPPLEMENTARY DAT A

The financial statements required to be filed pursuant to this Item 8 are appended to this report. An index of those financial statements is found in Item 15.

ITEM 9. CHANGES IN AND DISAGREEMENTS WITH ACCOUNTANT S ON ACCOUNTING AND FINANCIAL DISCLOSURE

None.

ITEM 9A. CONTROLS AND PROCEDURES

Management's Evaluation of Disclosure Controls and Procedures

We maintain "disclosure controls and procedures," as defined in Rules 13a-15(c) or 15d-15(e) under the Exchange Act of 1934, or Exchange Act, to mean controls and other procedures of a company that are designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is recorded, processed, summarized, and reported within the time periods specified in the Securities and Exchange Commission's rules and forms. Our disclosure controls and procedures include, without limitation, controls and other procedures designed to ensure that information required to be disclosed by us in the reports we file or submit under the Exchange Act is accumulated and communicated to our management, including our principal executive officer and principal financial officer, as appropriate, to allow timely decisions regarding required disclosure.

Our management, with the participation of our principal executive officer and principal financial officer, evaluated the effectiveness of our disclosure controls and procedures as of December 31, 2023. Our management recognizes that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving their objectives, and management necessarily applies its judgment in evaluating the cost-benefit relationship of possible controls and procedures. Our principal executive officer and principal financial officer have concluded based upon the evaluation described above that, as of December 31, 2023, our disclosure controls and procedures were effective at the reasonable assurance level.

We continue to review and document our disclosure controls and procedures and may from time to time make changes aimed at enhancing their effectiveness and to ensure that our systems evolve with our business.

Management's Annual Report on Internal Control over Financial Reporting

Our management is responsible for establishing and maintaining adequate internal control over financial reporting. Internal control over financial reporting is defined in Rules 13a-15(f) and 15d-15(f) under the Exchange Act as a process designed by, or under the supervision of, a company's principal executive officer and principal financial officer, or persons performing similar functions, and effected by a company's board of directors, management, and other personnel, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial

statements for external purposes in accordance with generally accepted accounting principles and includes those policies and procedures that:

- pertain to the maintenance of records that in reasonable detail accurately and fairly reflect the transactions and dispositions of a company's assets;
- provide reasonable assurance that transactions are recorded as necessary to permit preparation of financial statements in accordance with generally accepted accounting principles, and that a company's receipts and expenditures are being made only in accordance with authorizations of our management and directors; and
- provide reasonable assurance regarding prevention or timely detection of unauthorized acquisition, use or disposition of our assets that could have a material effect on the financial statements.

Because of its inherent limitations, internal control over financial reporting may not prevent or detect misstatements. Projections of any evaluation of effectiveness to future periods are subject to the risk that controls may become inadequate because of changes in conditions, or that the degree of compliance with the policies or procedures may deteriorate.

Under the supervision of and with the participation of our principal executive officer and principal financial and accounting officer, our management assessed the effectiveness of our internal control over financial reporting as of December 31, 2023 based on the criteria set forth by the Committee of Sponsoring Organizations of the Treadway Commission (COSO) in Internal Control—Integrated Framework (2013 framework). Based on this assessment, management concluded that our internal control over financial reporting was effective as of December 31, 2023.

Changes in Internal Control over Financial Reporting

No change in our internal control over financial reporting (as defined in Rules 13a-15(f) and 15d-15(f) under the Exchange Act) occurred during our fiscal quarter ended December 31, 2023 that has materially affected, or is reasonably likely to materially affect, our internal control over financial reporting.

ITEM 9B. OTHER INFORMATION

Director and Officer Trading Arrangements

None of our directors or officers adopted or terminated a Rule 10 b5- 1 trading arrangement or a non-Rule 10 b5-1 trading arrangement (as defined in Item 408(c) of Regulation S-K) during the quarterly period ended December 31, 2023.

ITEM 9C. DISCLOSURE REGARDING FOREIGN JURISDICTIONS THAT PREVENT INSPECTIONS

Not applicable.

PART III

ITEM 10. DIRECTORS, EXECUTIVE OFFICERS AND CORPORATE GOVERNANCE

Incorporated by reference from the information in our Proxy Statement for our 2024 Annual Meeting of Stockholders, which we expect to file with the SEC within 120 days of the end of the fiscal year to which this Annual Report on Form 10-K relates.

ITEM 11. EXECUTIVE COMPENSATIO N

Incorporated by reference from the information in our Proxy Statement for our 2024 Annual Meeting of Stockholders, which we expect to file with the SEC within 120 days of the end of the fiscal year to which this Annual Report on Form 10-K relates.

ITEM 12. SECURITY OWNERSHIP OF CERTAIN BENEFICIAL OWNER S AND MANAGEMENT AND RELATED STOCKHOLDER MATTERS

Incorporated by reference from the information in our Proxy Statement for our 2024 Annual Meeting of Stockholders, which we expect to file with the SEC within 120 days of the end of the fiscal year to which this Annual Report on Form 10-K relates.

ITEM 13. CERTAIN RELATIONSHIPS AND RELATED TRANSACTION S, AND DIRECTOR INDEPENDENCE

Incorporated by reference from the information in our Proxy Statement for our 2024 Annual Meeting of Stockholders, which we expect to file with the SEC within 120 days of the end of the fiscal year to which this Annual Report on Form 10-K relates.

ITEM 14. PRINCIPAL ACCOUNTANT FEES AND SERVICE S

Incorporated by reference from the information in our Proxy Statement for our 2024 Annual Meeting of Stockholders, which we expect to file with the SEC within 120 days of the end of the fiscal year to which this Annual Report on Form 10-K relates.

PART IV

ITEM 15. EXHIBITS AND FINANCIAL STATEMENT SCHEDULE S

(a)(1) Financial Statements.

	Pages
Report of independent registered public accounting firm PCAOB ID 42	F-1
Consolidated Balance Sheets	F-3
Consolidated Statements of Operations and Comprehensive Income (Loss)	F-4
Consolidated Statements of Stockholders' Equity	F-5
Consolidated Statements of Cash Flows	F-6
Notes to consolidated financial statements	F-7

(a)(2) Financial Statement Schedules.

All schedules have been omitted because they are not required or because the required information is given in the Consolidated Financial Statements or Notes thereto set forth under Item 8 above.

(a)(3) Exhibits.

See the Exhibit Index immediately preceding the signature page of this Annual Report on Form 10-K. The exhibits listed in the Exhibit Index below are filed or incorporated by reference as part of this Annual Report on Form 10-K.

ITEM 16. FORM 10-K SUMMARY

This Annual Report on Form 10-K does not include a summary.

Report of Independent Registered Public Accounting Firm

To the Stockholders and the Board of Directors of Voyager Therapeutics, Inc.

Opinion on the Financial Statements

We have audited the accompanying consolidated balance sheets of Voyager Therapeutics, Inc. (the Company) as of December 31, 2023 and 2022, the related consolidated statements of operations and comprehensive income (loss), stockholders' equity, and cash flows for each of the three years in the period ended December 31, 2023, and the related notes (collectively referred to as the "consolidated financial statements"). In our opinion, the consolidated financial statements present fairly, in all material respects, the financial position of the Company at December 31, 2023 and 2022, and the results of its operations and its cash flows for each of the three years in the period ended December 31, 2023, in conformity with U.S. generally accepted accounting principles.

Basis for Opinion

These financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on the Company's financial statements based on our audits. We are a public accounting firm registered with the Public Company Accounting Oversight Board (United States) (PCAOB) and are required to be independent with respect to the Company in accordance with the U.S. federal securities laws and the applicable rules and regulations of the Securities and Exchange Commission and the PCAOB.

We conducted our audits in accordance with the standards of the PCAOB. Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the financial statements are free of material misstatement, whether due to error or fraud. The Company is not required to have, nor were we engaged to perform, an audit of its internal control over financial reporting. As part of our audits we are required to obtain an understanding of internal control over financial reporting but not for the purpose of expressing an opinion on the effectiveness of the Company's internal control over financial reporting. Accordingly, we express no such opinion.

Our audits included performing procedures to assess the risks of material misstatement of the financial statements, whether due to error or fraud, and performing procedures that respond to those risks. Such procedures included examining, on a test basis, evidence regarding the amounts and disclosures in the financial statements. Our audits also included evaluating the accounting principles used and significant estimates made by management, as well as evaluating the overall presentation of the financial statements. We believe that our audits provide a reasonable basis for our opinion.

Critical Audit Matter

The critical audit matter communicated below is a matter arising from the current period audit of the financial statements that was communicated or required to be communicated to the audit committee and that: (1) relates to accounts or disclosures that are material to the financial statements and (2) involved our especially challenging, subjective or complex judgments. The communication of the critical audit matter does not alter in any way our opinion on the consolidated financial statements, taken as a whole, and we are not, by communicating the critical audit matter below, providing a separate opinion on the critical audit matter or on the accounts or disclosures to which it relates.

Revenue recognition under the proportional performance model

Description of the Matter	As discussed in Note 9 to the consolidated financial statements, the Company recorded collaboration revenue of $11.0 million for the year ended December 31, 2023, and had total deferred revenue of $75.2 million, as of December 31, 2023 pursuant to certain of its license and collaboration agreements under the proportional performance method. The Company recognizes arrangement consideration allocated to certain performance obligations that are delivered over time using the proportional performance method. Revenue is recognized using input-based measurements, which involves the measurement of progress toward each performance obligation based on the actual costs incurred compared to total projected costs.

	Auditing collaboration revenue recognized was especially challenging and judgmental because the proportional performance calculation involves subjective management assumptions about estimates of the expected remaining costs to complete the research and development services for each performance obligation. Changes in expected remaining costs to complete can have a material effect on the amount of collaboration revenue recognized.
How We Addressed the Matter in Our Audit	Our audit procedures included, among others, the inspection of the Company's contracts and testing of the completeness and accuracy of the underlying data used to determine the expected remaining costs to complete the research and development services for each performance obligation that is accounted for using proportional performance. We performed inquiries of research and development personnel and inspected the minutes of joint steering committee meetings to validate management's estimates and to assess the reasonableness of the proportional performance calculation. We also performed a retrospective review to assess the Company's historical estimates of the remaining costs to complete the research and development services and a sensitivity analysis to evaluate the materiality of reasonable changes in management's assumptions.

/s/ *Ernst & Young LLP*

We have served as the Company's auditor since 2015.

Boston, Massachusetts
February 28, 2024

Voyager Therapeutics, Inc.
Consolidated Balance Sheets
(amounts in thousands, except share and per share data)

	December 31, 2023	December 31, 2022
Assets		
Current assets:		
Cash and cash equivalents	$ 68,802	$ 98,959
Marketable securities	162,073	19,889
Accounts receivable	80,150	—
Related party collaboration receivable	3,341	257
Prepaid expenses and other current assets	5,318	5,394
Total current assets	319,684	124,499
Property and equipment, net	16,494	17,857
Deposits and other non-current assets	1,593	1,515
Operating lease, right-of-use assets	13,510	15,485
Total assets	$ 351,281	$ 159,356
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable	$ 1,604	$ 2,566
Accrued expenses	16,823	7,816
Other current liabilities	3,200	2,832
Deferred revenue, current	42,881	59,377
Total current liabilities	64,508	72,591
Deferred revenue, non-current	32,359	6,450
Other non-current liabilities	18,094	21,295
Total liabilities	114,961	100,336
Commitments, contingencies, and other liabilities (see note 8)		
Stockholders' equity:		
Preferred stock, $ 0.001 par value: 5,000,000 shares authorized; no shares issued and outstanding at December 31, 2023 and 2022	—	—
Common stock, $ 0.001 par value: 120,000,000 shares authorized; 44,038,333 and 38,613,891 shares issued and outstanding at December 31, 2023 and 2022, respectively	44	38
Additional paid-in capital	497,506	452,713
Accumulated other comprehensive loss	(48)	(219)
Accumulated deficit	(261,182)	(393,512)
Total stockholders' equity	236,320	59,020
Total liabilities and stockholders' equity	$ 351,281	$ 159,356

The accompanying notes are an integral part of these consolidated financial statements.

Voyager Therapeutics, Inc.
Consolidated Statements of Operations and Comprehensive Income (Loss)
(amounts in thousands, except share and per share data)

	Year ended December 31,		
	2023	**2022**	**2021**
Collaboration revenue	$ 250,008	$ 40,907	$ 37,415
Operating expenses:			
Research and development	92,172	60,764	73,787
General and administrative	35,822	30,980	37,246
Total operating expenses	127,994	91,744	111,033
Operating income (loss)	122,014	(50,837)	(73,618)
Other income, net:			
Interest income (expense)	11,721	1,792	(390)
Other income	3	2,653	2,811
Total other income, net	11,724	4,445	2,421
Income (loss) before income taxes	133,738	(46,392)	(71,197)
Income tax provision	1,408	16	—
Net income (loss)	$ 132,330	$ (46,408)	$ (71,197)
Other comprehensive income (loss)			
Net unrealized gain (loss) on available-for-sale-securities	171	(81)	(4)
Total other comprehensive income (loss)	171	(81)	(4)
Comprehensive income (loss)	$ 132,501	$ (46,489)	$ (71,201)
Net income (loss) per share, basic	$ 3.08	$ (1.21)	$ (1.89)
Net income (loss) per share, diluted	$ 2.97	$ (1.21)	$ (1.89)
Weighted-average common shares outstanding, basic	43,020,747	38,356,810	37,668,947
Weighted-average common shares outstanding, diluted	44,569,334	38,356,810	37,668,947

The accompanying notes are an integral part of these consolidated financial statements.

Voyager Therapeutics, Inc.
Consolidated Statements of Stockholders' Equity
(amounts in thousands, except share data)

	Common Stock		Additional Paid-In	Accumulated Other Comprehensive	Accumulated	Stockholders'
	Shares	Amount	Capital	Income (Loss)	Deficit	Equity
Balance at December 31, 2020	37,368,027	$ 37	$ 430,324	$ (134)	$ (275,907)	$ 154,320
Exercises of vested stock options	3,811	1	27	—	—	28
Vesting of restricted stock units	346,551	—	—	—	—	—
Issuance of common stock under ESPP	200,006	—	918	—	—	918
Stock-based compensation expense	—	—	10,990	—	—	10,990
Unrealized loss on available-for-sale securities	—	—	—	(4)	—	(4)
Net loss	—	—	—	—	(71,197)	(71,197)
Balance at December 31, 2021	37,918,395	$ 38	$ 442,259	$ (138)	$ (347,104)	$ 95,055
Exercises of vested stock options	89,012	—	629	—	—	629
Vesting of restricted stock units	456,219	—	—	—	—	—
Issuance of common stock under ESPP	150,265	—	672	—	—	672
Stock-based compensation expense	—	—	9,153	—	—	9,153
Unrealized loss on available-for-sale securities	—	—	—	(81)	—	(81)
Net loss	—	—	—	—	(46,408)	(46,408)
Balance at December 31, 2022	38,613,891	$ 38	$ 452,713	$ (219)	$ (393,512)	$ 59,020
Exercises of vested stock options	385,655	1	1,851	—	—	1,852
Vesting of restricted stock units	531,560	—	—	—	—	—
Issuance of common stock in connection with the 2023 Neurocrine Collaboration Agreement	4,395,588	5	31,116	—	—	31,121
Issuance of common stock under ESPP	111,639	—	959	—	—	959
Stock-based compensation expense	—	—	10,867	—	—	10,867
Unrealized gain on available-for-sale securities	—	—	—	171	—	171
Net income	—	—	—	—	132,330	132,330
Balance at December 31, 2023	44,038,333	$ 44	$ 497,506	$ (48)	$ (261,182)	$ 236,320

The accompanying notes are an integral part of these consolidated financial statements

Voyager Therapeutics, Inc.
Consolidated Statements of Cash Flows
(amounts in thousands)

	Year ended December 31,		
	2023	**2022**	**2021**
Cash flow from operating activities			
Net income (loss)	$ 132,330	$ (46,408)	$ (71,197)
Adjustments to reconcile net income (loss) to net cash provided by (used in) operating activities:			
Stock-based compensation expense	11,153	9,344	11,324
Depreciation	4,441	6,191	5,165
Amortization of premiums and discounts on marketable securities	(3,626)	(16)	349
Gain on lease termination	—	(2,468)	—
Change in fair value of common stock and warrants to purchase equity securities	—	—	(2,460)
Loss on disposal of fixed assets	178	377	—
Changes in operating assets and liabilities:			
Accounts receivable	(80,150)	—	—
Related party collaboration receivable	(3,084)	475	7,280
Prepaid expenses and other assets	76	(1,967)	1,883
Operating lease, right-of-use assets	1,975	3,462	2,606
Other non-current assets	—	(152)	69
Accounts payable	(962)	1,992	(60)
Accrued expenses	9,007	(3,148)	(3,335)
Operating lease liabilities	(2,833)	(3,922)	(3,428)
Deferred revenue	9,414	23,731	(1,721)
Net cash provided by (used in) operating activities	77,919	(12,509)	(53,525)
Cash flow from investing activities			
Purchases of property and equipment	(3,256)	(2,491)	(1,609)
Purchases of marketable securities	(223,968)	(54,848)	(15,117)
Proceeds from sales and maturities of marketable securities	85,581	50,000	82,632
Net cash (used in) provided by investing activities	(141,643)	(7,339)	65,906
Cash flow from financing activities			
Proceeds from the exercise of stock options	1,852	629	28
Proceeds from the issuance of common stock in connection with the 2023 Neurocrine Collaboration Agreement	31,121	—	—
Proceeds from the purchase of common stock under ESPP	672	481	584
Net cash provided by financing activities	33,645	1,110	612
Net (decrease) increase in cash and cash equivalents	(30,079)	(18,738)	12,993
Cash, cash equivalents, and restricted cash beginning of period	100,474	119,212	106,219
Cash, cash equivalents, and restricted cash end of period	$ 70,395	$ 100,474	$ 119,212
Supplemental disclosure of cash and non-cash activities			
Operating lease right-of-use assets obtained in exchange for operating lease liabilities	$ —	$ —	$ 664
Capital expenditures incurred but not yet paid	$ —	$ 14	$ 80

The accompanying notes are an integral part of these consolidated financial statements.

VOYAGER THERAPEUTICS INC.
NOTES TO CONSOLIDATED FINANCIAL STATEMENTS

1. Nature of business

Voyager Therapeutics, Inc. (the "Company") is a biotechnology company whose mission is to leverage the power of human genetics to modify the course of and ultimately cure neurological diseases. The Company's pipeline includes programs for Alzheimer's disease ("AD"); amyotrophic lateral sclerosis ("ALS"); Parkinson's disease, and multiple other diseases of the central nervous system, ("CNS"). Many of the Company's programs are derived from its TRACER™ adeno-associated virus ("AAV") capsid discovery platform, which the Company has used to generate novel capsids ("TRACER Capsids") and identify associated receptors to potentially enable high brain penetration with genetic medicines following intravenous dosing. Some of the Company's programs are wholly-owned, and some are advancing with licensees and collaborators including Alexion, AstraZeneca Rare Disease ("Alexion"); Novartis Pharma AG, ("Novartis"); Neurocrine Biosciences, Inc. ("Neurocrine"); and Sangamo Therapeutics, Inc. ("Sangamo").

The Company focuses on leveraging its expertise in capsid discovery and neuropharmacology to address the delivery hurdles that have constrained the genetic medicine and neurology disciplines, with the goal of either halting or slowing disease progression or reducing symptom severity, and therefore providing clinically meaningful impact to patients. The Company is advancing its own proprietary pipeline of drug candidates for neurological diseases, with a focus on AD. The Company's wholly-owned prioritized pipeline programs include an anti-tau antibody for AD; a superoxide dismutase 1 ("SOD1") silencing gene therapy for ALS; and a tau silencing gene therapy for AD. The Company identified a lead development candidate for its anti-tau antibody program in the first quarter of 2023, initiated good laboratory practices ("GLP") toxicology studies in the third quarter of 2023, and expects to submit an investigational new drug ("IND"), application to the U.S. Food and Drug Administration ("FDA") for this program in the first half of 2024. The Company believes this trial could result in the potential to generate proof-of-concept data for slowing the spread of pathological tau via tau positron emission tomography imaging in 2026. The Company identified a lead development candidate for its SOD1 silencing gene therapy program in the fourth quarter of 2023, and the Company expects to submit the IND application for its SOD1 silencing gene therapy program in mid-2025. The Company promoted its tau silencing gene therapy program to a prioritized program in the first quarter of 2024, based on preclinical data demonstrating robust reductions in tau messenger RNA ("mRNA") in a murine model, and it anticipates submission of an IND in 2026. The Company's proprietary pipeline also includes an early research initiative to develop a gene therapy for the treatment of AD. This program seeks to combine a vectorized anti-amyloid antibody with a TRACER Capsid.

The Company is also working with its collaboration partners on multiple programs. In January 2019 and January 2023, the Company entered into collaboration and license agreements with Neurocrine. Under the agreements with Neurocrine, the Company is actively advancing two later preclinical stage programs: a glucocerebrosidase 1 ("GBA1") gene therapy program for Parkinson's disease and other GBA1-mediated diseases, and a frataxin gene therapy program for Friedreich's ataxia (the "FA Program"). Pursuant to such agreements, the Company is also working with Neurocrine on five early-stage programs for the research, development, manufacture and commercialization of gene therapies designed to address CNS diseases or conditions associated with rare genetic targets. The Company has also entered into agreements with licensees including Novartis, Alexion, and Sangamo, to license or to provide options to receive exclusive licenses to certain TRACER Capsids. As described further below, in December 2023, the Company entered into a license and collaboration agreement with Novartis to provide Novartis certain rights regarding the development of potential gene therapy product candidates for the treatment of spinal muscular atrophy and to collaborate with Novartis to develop gene therapy product candidates for the treatment of Huntington's disease. The joint steering committee with Neurocrine selected a development candidate for the FA Program, during the first quarter of 2024, and expects to advance into first-in-human clinical trials in 2025. The Company also anticipates that its collaborative partners and licensees will submit at least one additional IND application for a partnered program and initiate clinical development for the associated program by the end of 2025.

The Company has a history of incurring annual net operating losses prior to the operating income in 2023. As of December 31, 2023, the Company had an accumulated deficit of $ 261.2 million. The Company has not generated any

product revenue and has financed its operations primarily through public offerings and private placements of its equity securities and funding from fees, milestone payments, and cost reimbursements associated with its prior and current collaborations and license agreements.

As of December 31, 2023, the Company had cash, cash equivalents, and marketable securities of $ 230.9 million. Based upon its current operating plan, the Company expects that its existing cash, cash equivalents, and marketable securities at December 31, 2023, together with the upfront $ 80.0 million payment received in January 2024 in connection with the Collaboration and License Agreement by and between the Company and Novartis dated as of December 28, 2023 (the "2023 Novartis Collaboration Agreement"), $ 20.0 million in proceeds from Novartis' equity purchase, and $ 93.5 million in net proceeds received from a public offering closed in January 2024, to be sufficient to meet the Company's planned operating expenses and capital expenditure requirements for at least twelve months from the issuance of these consolidated financial statements.

There can be no assurance that the Company will be able to obtain additional debt or equity financing on terms acceptable to the Company or generate product revenue or revenue from collaboration partners, on a timely basis or at all. The failure of the Company to obtain sufficient funds on acceptable terms when needed could have a material adverse effect on the Company's business, results of operations, and financial condition.

2. Summary of significant accounting policies and basis of presentation

Basis of presentation

The accompanying consolidated financial statements have been prepared in conformity with accounting principles generally accepted in the United States of America ("GAAP") and pursuant to the rules and regulations of the U.S. Securities and Exchange Commission ("SEC") for reporting on Form 10-K. The Company's consolidated financial statements include the accounts of Voyager Therapeutics, Inc. and its wholly-owned subsidiary, Voyager Securities Corporation. All intercompany balances and transactions have been eliminated.

Use of Estimates

The preparation of consolidated financial statements in conformity with GAAP requires management to make estimates and assumptions that affect the amounts reported in the consolidated financial statements and accompanying notes. On an ongoing basis, the Company's management evaluates its estimates, which include, but are not limited to, estimates related to revenue recognition, research and development accrued expenses, stock-based compensation expense, and income taxes. The Company bases its estimates on historical experience and other market specific or other relevant assumptions that it believes to be reasonable under the circumstances. Actual results may differ from those estimates or assumptions.

Fair Value of Financial Instruments

ASC Topic 820, *Fair Value Measurement* ("ASC 820"), establishes a fair value hierarchy for instruments measured at fair value that distinguishes between assumptions based on market data (observable inputs) and the Company's own assumptions (unobservable inputs). Observable inputs are inputs that market participants would use in pricing the asset or liability based on market data obtained from sources independent of the Company. Unobservable inputs are inputs that reflect the Company's assumptions about the inputs that market participants would use in pricing the asset or liability, and are developed based on the best information available in the circumstances.

ASC 820 identifies fair value as the exchange price, or exit price, representing the amount that would be received to sell an asset or paid to transfer a liability in an orderly transaction between market participants. As a basis for considering market participant assumptions in fair value measurements, ASC 820 establishes a three-tier fair value hierarchy that distinguishes between the following:

- *Level 1*—Quoted market prices in active markets for identical assets or liabilities.

- *Level 2*—Inputs other than Level 1 inputs that are either directly or indirectly observable, such as quoted market prices, interest rates, and yield curves.
- *Level 3*—Unobservable inputs developed using estimates of assumptions developed by the Company, which reflect those that a market participant would use.

To the extent that the valuation is based on models or inputs that are less observable or unobservable in the market, the determination of fair value requires more judgment. Accordingly, the degree of judgment exercised by the Company in determining fair value is greatest for instruments categorized in Level 3. A financial instrument's level within the fair value hierarchy is based on the lowest level of any input that is significant to the fair value measurement.

The carrying amounts reflected in the balance sheets for cash and cash equivalents, prepaid expenses and other current assets, accounts payable and accrued expenses approximate their fair values, due to their short-term nature.

Cash and Cash Equivalents

The Company considers all highly liquid investments purchased with original maturities of 90 days or less at acquisition to be cash equivalents. Cash and cash equivalents include cash held in banks and amounts held in money market funds.

Marketable Securities

The Company classifies marketable securities with a remaining maturity of greater than three months when purchased as available-for-sale. Marketable securities with a remaining maturity date greater than one year are classified as non-current where the Company has the intent and ability to hold these securities for at least the next 12 months.

All available-for-sale debt securities are carried at fair value with the unrealized gains and losses included in other comprehensive income (loss) as a component of stockholders' equity until realized. Any premium or discount arising at purchase is amortized and/or accreted to interest income and/or expense. Realized gains and losses are determined using the specific identification method and are included in other income. If any adjustment to fair value reflects a decline in value of the investment, the Company uses a forward-looking approach based on expected losses to estimate credit losses on certain types of financial instruments, including trade receivables and available-for-sale debt securities. No other than temporary losses or credit losses have been recognized.

Cash, cash equivalents, and marketable securities as of December 31, 2023 and 2022 consist of the following:

	Amortized Cost	Unrealized Gains	Unrealized Losses	Fair Value
		(in thousands)		
As of December 31, 2023				
Money market funds included in cash and cash equivalents	$ 65,589	—	—	$ 65,589
Marketable securities:				
U.S. Treasury notes	102,966	81	(3)	103,044
U.S. Government agency securities	31,068	10	(3)	31,075
Corporate bonds	23,975	2	(7)	23,970
Commercial paper	3,985	—	—	3,985
Total money market funds and marketable securities	$ 227,583	$ 93	$ (13)	$ 227,663
As of December 31, 2022				
Money market funds included in cash and cash equivalents	$ 91,724	$ —	$ —	$ 91,724
Marketable securities:				
U.S. Treasury notes	19,980	—	(91)	19,889
Total money market funds and marketable securities	$ 111,704	$ —	$ (91)	$ 111,613

All of the Company's marketable securities at December 31, 2023 and 2022 have a contractual maturity of one year or less.

The Company reviews investments whenever the fair value of an investment is less than the amortized cost and evidence indicates that an investment's carrying amount is not recoverable within a reasonable period of time. In connection with these investments, the Company evaluates whether the decline in fair value has resulted from credit losses or other factors, considering the extent to which fair value is less than amortized cost, any changes to the rating of the security by a rating agency, and adverse conditions specifically related to the security, among other factors. If this assessment indicates that a credit loss exists, the present value of cash flows expected to be collected from the security is compared to the amortized cost basis of the security. If the present value of cash flows expected to be collected is less than the amortized cost basis, a credit loss exists and an allowance for credit losses is recorded for the credit loss on the condensed consolidated balance sheet, limited by the amount that the fair value is less than the amortized cost basis. Any impairment that is not related to credit is recognized in other comprehensive income (loss). Changes in the allowance for credit losses are recorded as a provision for (or reversal of) credit loss expense in general and administrative expenses within the consolidated statement of operations and comprehensive income (loss). Losses are charged against the allowance when the Company believes the uncollectability of an available-for-sale security is confirmed or when either of the criteria regarding intent or requirement to sell is met.

The Company held $ 44.2 million and $ 19.9 million in marketable securities that were in an unrealized loss position as of December 31, 2023 and December 31, 2022, respectively. The unrealized losses at December 31, 2023 and December 31, 2022 were attributable to changes in interest rates and the unrealized losses do not represent credit losses. The Company does not intend to sell these securities and it is not more likely than not that it will be required to sell them before recovery of their amortized cost basis.

Restricted Cash

As of December 31, 2023 and 2022, the Company maintained restricted cash totaling approximately $ 1.6 million and $ 1.5 million, respectively, held in the form of money market accounts as collateral for the Company's facility lease obligations. The balance is included within deposits and other non-current assets in the accompanying consolidated balance sheets. The following table provides a reconciliation of cash, cash equivalents, and restricted cash within the consolidated balance sheets that sum to the total of the same such amounts shown in the statements of cash flows:

	As of December 31,		
	2023	2022	2021
		(in thousands)	
Cash and cash equivalents	$ 68,802	$ 98,959	$ 117,433
Restricted cash included in deposits and other non-current assets	1,593	1,515	1,779
Total cash, cash equivalents, and restricted cash	$ 70,395	$ 100,474	$ 119,212

Property and Equipment

Property and equipment consists of laboratory equipment, furniture and office equipment, and leasehold improvements and is stated at cost, less accumulated depreciation. Maintenance and repairs that do not improve or extend the lives of the respective assets are expensed to operations as incurred; while costs of major additions and betterments are capitalized. Depreciation is calculated over the estimated useful lives of the assets using the straight-line method.

Impairment of Long-Lived Assets

The Company evaluates long-lived assets for potential impairment when events or changes in circumstances indicate the carrying value of the assets may not be recoverable. Recoverability is measured by comparing the book values of the assets to the expected future net undiscounted cash flows that the assets are expected to generate. If such assets are considered to be impaired, the impairment to be recognized is measured by the amount by which the book

values of the assets exceed their fair value. The Company has not recognized any impairment losses from inception through December 31, 2023.

Revenue Recognition

The Company enters into license, option, and collaboration agreements which are within the scope of ASC 606, *Revenue from Contracts with Customers* ("ASC 606"), under which the Company licenses or provides options to license certain of the Company's product candidates and, in certain cases, performs research and development services. The terms of these arrangements typically include payment of one or more of the following: non-refundable, upfront fees; reimbursement of research and development costs; option exercise fees; development, regulatory, and commercial milestone payments; and royalties on net sales of licensed products.

The promised goods or services in the Company's arrangements typically consist of license rights to the Company's intellectual property and research and development services. The Company provides options to additional items in the contracts, which are accounted for as separate contracts when the customer elects to exercise such options, unless the option provides a material right to the customer. The Company evaluates the customer options for material rights, or options to acquire additional goods or services for free or at a discount. If the customer options are determined to represent a material right, the material right is recognized as a separate performance obligation at the outset of the arrangement. Performance obligations are promised goods or services in a contract to transfer a distinct good or service to the customer and are considered distinct when (a) the customer can benefit from the good or service on its own or together with other readily available resources and (b) the promised good or service is separately identifiable from other promises in the contract. In assessing whether promised goods or services are distinct, the Company considers factors such as the stage of development of the underlying intellectual property, the capabilities of the customer to develop the intellectual property on its own or whether the required expertise is readily available and whether the goods or services are integral or dependent to other goods or services in the contract.

The Company estimates the transaction price based on the amount expected to be received for transferring the promised goods or services in the contract. The consideration may include fixed consideration or variable consideration. At the inception of each arrangement that includes variable consideration, the Company evaluates the amount of potential payments and the likelihood that the payments will be received. The Company utilizes either the most likely amount method or expected amount method to estimate the amount expected to be received based on which method best predicts the amount expected to be received. The amount of variable consideration which is included in the transaction price may be constrained, and is included in the transaction price only to the extent that it is probable that a significant reversal in the amount of the cumulative revenue recognized will not occur in a future period.

The Company's contracts often include development and regulatory milestone payments which are assessed under the most likely amount method and constrained if it is probable that a significant revenue reversal would occur. Milestone payments that are not within the Company's control or the licensee's control, such as regulatory approvals, are not considered probable of being achieved until those approvals are received. At the end of each reporting period, the Company re-evaluates the probability of achievement of such development milestones and any related constraint, and if necessary, adjusts its estimate of the overall transaction price. Any such adjustments are recorded on a cumulative catch-up basis, which would affect collaboration revenue in the period of adjustment. To date, the Company has not recognized any consideration related to the achievement of development, regulatory, or commercial milestone revenue resulting from any of the Company's collaboration or license arrangements.

For arrangements that include sales-based royalties, including milestone payments based on the level of sales, in which the license is deemed to be the predominant item to which the royalties relate, the Company recognizes revenue at the later of (a) when the related sales occur, or (b) when the performance obligation to which some or all of the royalty has been allocated has been satisfied (or partially satisfied). To date, the Company has not recognized any consideration related to sales-based royalty revenue resulting from any of the Company's collaboration or license arrangements.

The Company allocates the transaction price based on the estimated stand-alone selling price of each of the performance obligations. The Company must develop assumptions that require judgment to determine the stand-alone selling price for each performance obligation identified in the contract. The Company utilizes key assumptions to

determine the stand-alone selling price for service obligations, which may include other comparable transactions, pricing considered in negotiating the transaction and the estimated costs. Additionally, in determining the standalone selling price for material rights, the Company utilizes comparable transactions, clinical trial success probabilities, and estimates of option exercise likelihood. Variable consideration is allocated specifically to one or more performance obligations in a contract when the terms of the variable consideration relate to the satisfaction of the performance obligation and the resulting amounts allocated are consistent with the amounts the Company would expect to receive for the satisfaction of each performance obligation.

The consideration allocated to each performance obligation is recognized as revenue when control is transferred for the related goods or services. For performance obligations which consist of licenses and other promises, the Company utilizes judgment to assess the nature of the combined performance obligation to determine whether the combined performance obligation is satisfied over time or at a point in time and, if over time, the appropriate method of measuring progress. The Company evaluates the measure of progress each reporting period and, if necessary, adjusts the measure of performance and related revenue recognition.

Upfront payments and fees are recorded as contract liabilities within deferred revenue on the consolidated balance sheets until the Company performs its obligations under these arrangements. Amounts are recorded as accounts receivable when the Company's right to consideration is unconditional. A portion of revenue recognized from the 2019 and 2023 Neurocrine Collaboration Agreements and the 2023 Novartis Collaboration Agreement is related to performance obligations pursuant to which revenue is recognized using a proportional performance model. Revenue is recognized using input-based measurements, which involves the measurement of progress toward each performance obligation based on the actual costs incurred compared to total projected costs. The Company estimates the expected remaining costs to complete the research and development services for each performance obligation. The Company evaluates the measure of progress each reporting period and, if necessary, adjusts the measure and related revenue recognition.

Research and Development

Research and development costs are charged to expense as incurred in performing research and development activities. The costs include employee compensation costs, external research, consultant costs, sponsored research, license fees, process development and facilities costs. Facilities costs primarily include the allocation of rent, utilities and depreciation.

Leases

The Company determines if an arrangement is or contains a lease at inception under Accounting Standards Codification (ASC) 842 *Leases*. For leases with a term of 12 months or less, the Company does not recognize a right-of-use asset or lease liability. The Company's operating leases are recognized on its consolidated balance sheet as operating lease, right-of-use asset, other current liabilities, and other non-current liabilities. The Company does not have any finance leases.

Right-of-use assets represent the Company's right to use an underlying asset for the lease term and lease liabilities represent the Company's obligation to make lease payments arising from the lease. Operating lease right-of-use assets and liabilities are recognized at the lease commencement date based on the present value of lease payments over the lease term. As the Company's leases typically do not provide an implicit rate, the Company uses an estimate of its incremental borrowing rate based on the information available at the lease commencement date in determining the present value of lease payments. Operating lease right-of-use assets also include the effect of any lease prepaid or deferred lease payments and are reduced by lease incentives. The lease terms may include options to extend or terminate the lease when it is reasonably certain that the Company will exercise that option. Lease expense is recognized on a straight-line basis over the lease term.

The Company has lease agreements with lease and non-lease components, which are generally accounted for separately. Non-lease components as it pertains to the Company's leased premises generally refer to common area maintenance charges related to the premises.

Research Contract Costs and Accruals

The Company has entered into various research and development contracts with research institutions and other companies. These agreements are generally cancelable. The Company records accruals for estimated ongoing research costs. When evaluating the adequacy of the accrued liabilities, the Company analyzes progress of the studies, including the phase or completion of events, invoices received and contracted costs. Significant judgments and estimates may be made in determining the accrued balances at the end of any reporting period. Actual results could differ from the Company's estimates. The Company's historical accrual estimates have not been materially different from the actual costs.

Patent Costs

The Company expenses patent application and related legal costs as incurred and classifies such costs as general and administrative expenses in the accompanying statements of operations.

Stock-Based Compensation Expense

The Company accounts for its stock-based compensation awards in accordance with ASC Topic 718 *Compensation—Stock Compensation* ("ASC 718"). ASC 718 requires all stock-based payments to employees, directors, and other service providers, referred to as non-employees, including grants of restricted stock units and stock options, to be recognized as expense in the consolidated statements of operations and comprehensive income (loss) based on their grant date fair values. The Company estimates the fair value of options granted using the Black-Scholes option pricing model. The Company uses the fair value of its common stock to determine the fair value of restricted stock awards and restricted stock units.

The Black-Scholes option pricing model requires inputs based on certain subjective assumptions, including (a) the expected stock price volatility, (b) the expected term of the award, (c) the risk-free interest rate and (d) expected dividends. The Company bases the estimate of expected volatility on the historical volatility of its common stock. The historical volatility is calculated based on a period of time commensurate with the expected term assumption. The Company uses the simplified method as prescribed by the SEC Staff Accounting Bulletin No. 107, *Share-Based Payment*, to calculate the expected term for stock options granted to employees as it does not have sufficient historical exercise data to provide a reasonable basis upon which to estimate the expected term. For stock options granted to non-employees, the Company utilizes the contractual term of the arrangement as the basis for the expected term assumption. The risk-free interest rate is based on a treasury instrument whose term is consistent with the expected term of the stock options. The expected dividend yield is assumed to be zero as the Company has never paid dividends and has no current plans to pay any dividends on its common stock.

The Company expenses the fair value of its stock-based compensation awards on a straight-line basis over the associated service period, which is generally the period in which the related services are received, adjusted for actual forfeitures of unvested awards as they occur.

The Company records the expense for stock-based compensation awards subject to performance conditions over the remaining service period when management determines that achievement of the performance condition is probable. Management evaluates when the achievement of a performance condition is probable based on the expected satisfaction of the performance conditions as of the reporting date.

Income Taxes

Income taxes are recorded in accordance with ASC Topic 740, *Income Taxes* ("ASC 740"), which provides for deferred taxes using an asset and liability approach. Under this method, deferred tax assets and liabilities are determined based on the difference between the financial reporting and the tax reporting basis of assets and liabilities and are measured using the enacted tax rates and laws that are expected to be in effect when the differences are expected to

reverse. The Company provides a valuation allowance against net deferred tax assets unless, based upon the weight of available evidence, it is more likely than not that the deferred tax assets will be realized.

The Company accounts for uncertain tax positions in accordance with the provisions of ASC 740. When uncertain tax positions exist, the Company recognizes the tax benefit of tax positions to the extent that the benefit will more likely than not be realized. The determination as to whether the tax benefit will more likely than not be realized is based upon the technical merits of the tax position as well as consideration of the available facts and circumstances. As of December 31, 2023, the Company does not have any significant uncertain tax positions.

Comprehensive Income (Loss)

Comprehensive income (loss) is comprised of net income (loss) and other comprehensive income or loss. Other comprehensive income or loss consists of unrealized gains or losses on marketable securities.

Net Income (Loss) Per Share

Basic net income (loss) per share is calculated by dividing the net income (loss) by the weighted-average number of shares of common stock outstanding during the period, without consideration for potentially dilutive securities. Diluted net income (loss) per share is computed by dividing the net income (loss) by the weighted-average number of shares of common stock and potentially dilutive securities outstanding for the period determined using the treasury-stock and if-converted methods.

For purposes of the diluted net income (loss) per share, unvested restricted common stock and outstanding stock options are considered to be potentially dilutive securities. Unvested restricted common stock and outstanding stock options were excluded from the calculation of diluted net loss per share in the years ended December 31, 2023 and 2022, because their effect would be anti-dilutive and therefore, basic and diluted net loss per share were the same for the years ended December 31, 2023 and 2022.

The following table sets forth the outstanding potentially dilutive securities that have been excluded in the calculation of diluted net income (loss) per share because to do so would be anti-dilutive:

	As of December 31,		
	2023	2022	2021
Unvested restricted common stock awards	22,500	45,000	137,255
Unvested restricted common stock units	1,370,897	1,112,563	806,379
Outstanding stock options	7,425,444	6,199,571	5,013,193
Total	8,818,841	7,357,134	5,956,827

Basic net income (loss) and diluted weighted-average shares outstanding are as follows for the years ended December 31, 2023, 2022, and 2021:

	Year Ended December 31,		
	2023	2022	2021
	(in thousands, except share data)		
Numerator:			
Net income (loss)	$ 132,330	$ (46,408)	$ (71,197)
Denominator for basic net income (loss) per share:			
Weighted average shares outstanding-basic	43,020,747	38,356,810	37,668,947
Denominator for diluted net income (loss) per share:			
Weighted average shares outstanding	43,020,747	38,356,810	37,668,947
Common stock options and restricted stock units	1,548,587	—	—
Weighted average shares outstanding-diluted	44,569,334	38,356,810	37,668,947

Concentrations of Credit Risk and Significant Suppliers

The Company has no financial instruments with off-balance sheet risk such as foreign exchange contracts, option contracts or other foreign currency hedging arrangements. Financial instruments that potentially subject the Company to a concentration of credit risk are cash and cash equivalents. The Company's cash is held in accounts at financial institutions that may exceed federally insured limits. The Company has not experienced any credit losses in such accounts and does not believe it is exposed to any significant credit risk on these funds.

The Company is dependent on third-party manufacturers to supply certain products for research and development activities in its programs. In particular, the Company relies on a sole manufacturer to supply it with specific vectors related to the Company's research and development programs.

Segment Information

Operating segments are defined as components of an enterprise about which separate discrete information is available for evaluation by the chief operating decision maker ("CODM") in deciding how to allocate resources and assess performance. The Company and the Company's CODM, the Company's Chief Executive Officer, view the Company's operations and manages its business as a single operating segment, which is the business of developing and commercializing genetic medicine.

Recent Accounting Pronouncements

In August 2020, the Financial Accounting Standards Board ("FASB") issued ASU No. 2020-06, *Debt – Debt with Conversion and Other Options (Subtopic 470-20) and Derivatives and Hedging—Contracts in Entity's Own Equity (Subtopic 815-40)*. This standard amends the guidance on convertible instruments and the derivatives scope exception for contracts in an entity's own equity and amends the related earnings per share ("EPS") guidance. The ASU will be effective for smaller reporting companies for fiscal years beginning after December 15, 2023 and interim periods within those fiscal years. Early adoption is permitted in fiscal years beginning after December 15, 2020, including interim periods within those fiscal years. The Company is assessing the impact of ASU 2020-06 on the consolidated financial statements and does not expect it to have a material impact.

In November 2023 the FASB issued ASU No. 2023-07, *Segment Reporting (Topic 280): Improvements to Reportable Segment Disclosure*. This standard requires annual and interim disclosure of significant segment expenses that are regularly provided to the CODM. The amendments in this update also expand the interim segment disclosure requirements. The disclosures required under ASU 2023-07 are also required for public entities with a single reportable segment. This standard is effective for fiscal years beginning after December 15, 2023, and interim periods within fiscal years beginning after December 15, 2024. Early adoption is permitted and the amendments in this update are required to be applied on a retrospective basis. The Company is evaluating the impact of ASU 2023-07 on its consolidated financial statements and does not expect it to have a material impact.

In December 2023, the FASB issued ASU 2023-09, "*Income Taxes (Topic 740) - Improvements to Income Tax Disclosures."* ASU 2023-09 enhances the transparency and decision usefulness of income tax disclosures by requiring consistent categories and greater disaggregation of information in the rate reconciliation and income taxes paid disaggregated by jurisdiction. The amendments in ASU 2023-09 are effective for fiscal years, and interim periods within those fiscal years, beginning after December 15, 2024, and is applicable to the Company in fiscal 2025. However, retrospective application is permitted. Early adoption is also permitted. Besides a change in income tax disclosures, the Company does not expect the adoption of ASU 2023-09 to have a material impact on its consolidated financial statements.

3. Fair value measurements

Assets and liabilities measured at fair value on a recurring basis as of December 31, 2023 and 2022 are as follows:

Assets	Total	Quoted Prices in Active Markets for Identical Assets (Level 1)	Significant Other Observable Inputs (Level 2)	Significant Unobservable Inputs (Level 3)
		(in thousands)		
December 31, 2023				
Money market funds included in cash and cash equivalents	$ 65,589	$ 65,589	$ —	$ —
Marketable securities:				
U.S. Treasury notes	103,044	103,044	—	—
U.S. Government agency securities	31,075	31,075	—	—
Corporate bonds	23,970	—	23,970	—
Commercial paper	3,985	—	3,985	—
Total money market funds and marketable securities	$ 227,663	$ 199,708	$ 27,955	$ —
December 31, 2022				
Money market funds included in cash and cash equivalents	$ 91,724	$ 91,724	$ —	$ —
Marketable securities:				
U.S. Treasury notes	19,889	19,889	—	—
Total money market funds and marketable securities	$ 111,613	$ 111,613	$ —	$ —

The Company measures the fair value of money market funds, U.S. Treasury notes and U.S. Government agency securities based on quoted prices in active markets for identical securities. The Company measures the fair value of the Level 2 securities, corporate bonds and commercial paper, based on recent trades of securities in inactive markets or based on quoted market prices of similar instruments and other significant inputs derived from or corroborated by observable market data.

4. Prepaid expenses and other current assets

Prepaid expenses and other current assets consist of the following:

	As of December 31,	
	2023	2022
	(in thousands)	
Other current assets	$ 2,628	$ 4,233
Prepaid insurance	607	696
Prepaid research and development contracts	1,119	83
Accrued interest receivable	964	382
Total	$ 5,318	$ 5,394

5. Property and equipment, net

Property and equipment, net consists of the following:

	As of December 31,	
	2023	2022
	(in thousands)	
Laboratory equipment	$ 20,536	$ 19,675
Leasehold improvements	7,106	12,554
Furniture and office equipment	2,625	2,333
Other	1,265	502
Total property and equipment	31,532	35,064
Less: accumulated depreciation	(15,038)	(17,207)
Property and equipment, net	$ 16,494	$ 17,857

The Company recorded $ 4.4 million, $ 6.2 million, and $ 5.2 million in depreciation expense during the years ended December 31, 2023, 2022, and 2021, respectively.

6. Accrued expenses

Accrued expenses consist of the following:

	As of December 31,	
	2023	2022
	(in thousands)	
Employee compensation costs	$ 6,614	$ 4,559
Research and development costs	5,225	1,895
Accrued goods and services	4,229	636
Professional services	755	726
Total	$ 16,823	$ 7,816

7. Lease obligation

Operating Leases

As of December 31, 2023, the Company has a lease for laboratory and office space at 75 Hayden Avenue in Lexington, Massachusetts through January 31, 2031 and a lease for additional office and laboratory space at 64 Sidney Street in Cambridge, Massachusetts through November 30, 2026.

In September 2021, the Company entered into an agreement with BioNTech US, Inc. (“BioNTech US”) to sublease part of the office and laboratory space leased by the Company at 75 Sidney Street in Cambridge, Massachusetts (the “Sublease Agreement”) at that time. The sublease term was for approximately 3.3 years. The sublease did not relieve the Company of its original obligation under the lease, and therefore the Company did not adjust the operating lease right-of-use asset as a result of the sublease and accounted for the sublease as a separate lease.

On June 22, 2022 the Company entered into a Lease Termination Agreement (the “Lease Termination Agreement”) and terminated the lease for office and laboratory space at 75 Sidney Street (the “75 Sidney Street Lease”), effective immediately. In connection with the Lease Termination Agreement, the Company also entered into a Sublease Termination Agreement (the “Sublease Termination Agreement”) and terminated the Sublease Agreement with BioNTech US. The Company did not incur any termination penalties in connection with the Lease Termination Agreement or Sublease Termination Agreement. The Company derecognized the related right-of-use asset of approximately $ 14.5 million and the operating lease liabilities of $ 17.0 million, accordingly, resulting in a gain of $ 2.5 million in the year ended December 31, 2022.

The Company's lease agreements require the Company to maintain a cash deposit or irrevocable letter of credit in the aggregate amount of $ 1.5 million payable to its landlords as security for the performance of its obligations under the leases.

On August 11, 2023, the Company entered into a first amendment (the "First Amendment") to its existing lease for laboratory and office space at 75 Hayden Avenue in Lexington, Massachusetts, pursuant to which the Company agreed to lease approximately 61,307 square feet of additional office and laboratory space. The term of the First Amendment commences on the date on which the landlord makes the space available for use by the Company, and expires on January 31, 2031, unless sooner terminated or extended. As of December 31, 2023, the Company did not have control of the space and therefore, the lease had not yet commenced. The commencement date for the First Amendment occurred on February 1, 2024. The expected contractual obligation under the First Amendment to the Company's existing lease is approximately $ 37.8 million, to be paid over the 7 year term of the lease.

Total lease cost for operating leases of approximately $ 3.6 million, $ 4.6 million, and $ 6.8 million was incurred during the years ended December 31, 2023, 2022, and 2021, respectively. As of December 31, 2023, the weighted average remaining lease term was 5 years and the weighted average incremental borrowing rate used to determine the operating lease liability was 7.4 %.

The following table summarizes the operating sublease income generated under the Sublease Agreement for the years ended December 31, 2023, 2022, and 2021:

	Years ended December 31,		
	2023	2022	2021
		(in thousands)	
Operating sublease income	$ —	$ 1,380	$ 838

8. Commitments, contingencies, and other liabilities

As of December 31, 2023 and 2022, other current and non-current liabilities consisted of the following:

	As of December 31,	
	2023	2022
	(in thousands)	
Other current liabilities		
Lease liabilities	3,200	2,832
Total other current liabilities	$ 3,200	$ 2,832
Other non-current liabilities		
Lease liabilities	$ 17,093	$ 20,294
Other	1,001	1,001
Total other non-current liabilities	$ 18,094	$ 21,295

Strategic Restructuring

On August 6, 2021, the board of directors of the Company approved a strategic restructuring plan to eliminate a portion of its workforce as part of an initiative to reduce expenses and enhance operations. The strategic restructuring plan was approved in connection with its portfolio reevaluation efforts and its strategic shift to invest additional resources in the Company's TRACER Capsid development efforts.

During the year ended December 31, 2021, the Company incurred restructuring costs of approximately $ 2.6 million, which consists of severance-related costs. These costs are reported within the Company's research and development expenses and general and administrative expenses. All costs have been paid as of December 31, 2023.

Litigation

The Company was not a party to any material legal matters or claims and did no t have contingency reserves established for any litigation liabilities as of December 31, 2023 or 2022.

9. Significant Agreements

2023 Novartis License and Collaboration Agreement

On December 28, 2023 (the "2023 Novartis Collaboration Agreement Effective Date"), the Company entered into a License and Collaboration Agreement (the "2023 Novartis Collaboration Agreement") with Novartis to (a) provide rights to Novartis with respect to certain TRACER Capsids for use in the research, development, and commercialization by Novartis of AAV gene therapy products and product candidates, comprising such TRACER Capsids and payloads intended for the treatment of spinal muscular atrophy (the "Novartis SMA Program") and (b) collaborate to develop AAV gene therapy products and product candidates intended for the treatment of Huntington's disease (the "Novartis HD Program"), in each case, leveraging TRACER Capsids and other intellectual property controlled by the Company.

Novartis SMA Program and Novartis HD Program Licenses

Under the terms of the 2023 Novartis Collaboration Agreement, the Company granted Novartis and its affiliates:

- a non-exclusive, non-transferable, non-sublicensable (except in limited circumstances for contractors), worldwide, royalty-free right and license under any patents or know-how controlled by the Company and related to the TRACER Capsids to evaluate the same for use in the development of a product or product candidate under the Novartis SMA Program (a "Novartis SMA Program Product") comprising such a TRACER Capsid and a payload selected by Novartis during the period beginning on the 2023 Novartis Collaboration Agreement Effective Date and ending on the third anniversary of the 2023 Novartis Collaboration Agreement Effective Date;

- an exclusive (even as to the Company), sublicensable, non-transferable, worldwide, royalty-bearing right and license under any patents or know-how controlled by the Company and relating to the selected TRACER Capsids to exploit the same as incorporated into a Novartis SMA Program Product for all human and veterinary diagnostic, prophylactic and therapeutic uses during the Term (as defined below); and

- an exclusive (even as to the Company), non-transferable, sublicensable, worldwide, royalty-bearing right and license under any patents and know-how controlled by the Company and relating to the development of a product or product candidate under the Novartis HD Program (a "Novartis HD Program Product") to exploit the same for all human and veterinary diagnostic, prophylactic and therapeutic uses during the Term.

Governance

The Company and Novartis have agreed to manage the Novartis HD Program through a joint steering committee until dissolved after the first IND application filing for a Novartis HD Program Product. The Company and Novartis have further agreed that day-to-day activities of both the Novartis SMA Program and the Novartis HD Program shall be managed through designees from each of the Company and Novartis, acting as alliance managers.

Development, Regulatory Approval, Commercialization and Diligence

Under the 2023 Novartis Collaboration Agreement, Novartis is solely responsible for, and has sole decision-making authority with respect to, at its own expense, the exploitation of a Novartis SMA Program Product.

With respect to the Novartis HD Program, the parties have agreed to conduct research and pre-clinical development of Novartis HD Program Products pursuant to a research plan, with Novartis reimbursing the Company for

its activities thereunder in accordance with the agreed-to budget. From and after the first IND application filing for the Novartis HD Program, the parties have agreed that Novartis will assume sole responsibility for the development and commercialization of Novartis HD Program Products, including all further preclinical and clinical development and any commercialization of the Novartis HD Program products and product candidates.

With respect to each of the Novartis SMA Program Products and Novartis HD Program Products, Novartis is obligated to use commercially reasonable efforts to develop and obtain regulatory approval for at least one of each such product in the United States and in certain other international markets specified in the 2023 Novartis Collaboration Agreement.

Termination

Unless earlier terminated, with respect to any licensed product(s) under the Collaboration Agreement, on a country-by-country basis, the 2023 Novartis Collaboration Agreement expires upon the expiration of the last-to-expire royalty term with respect to such licensed product in such country in the territory. Subject to a cure period, either party may terminate the 2023 Novartis Collaboration Agreement, in whole or in part, subject to specified conditions, in the event of the other party's uncured material breach. Novartis may also terminate the 2023 Novartis Collaboration Agreement, in whole or in part, subject to specified conditions, for the Company's insolvency, for the occurrence of a violation of global trade control laws, or for the Company's non-compliance with certain anti-bribery or anti-corruption covenants. Novartis may terminate the 2023 Novartis Collaboration Agreement, in whole or in part, for any or no reason upon ninety days ' written notice to the Company. In the event that Novartis has the right to terminate the 2023 Novartis Collaboration Agreement as a result of an uncured material breach by the Company that materially impairs the ability of Novartis to exploit one or more licensed products, Novartis may, in lieu of such termination, elect for the 2023 Novartis Collaboration Agreement to remain in full force and effect, and all milestone payments and royalties that would have otherwise been payable by Novartis under such licenses had the 2023 Novartis Collaboration Agreement not been breached would be substantially reduced.

Financial Terms

Under the 2023 Novartis Collaboration Agreement, Novartis agreed to pay the Company an initial upfront payment of $ 80.0 million. The Company is eligible to receive specified development, regulatory, and commercialization milestone payments of up to an aggregate of $ 200.0 million for the Novartis SMA Program and up to an aggregate of $ 225.0 million for the Novartis HD Program, in each case for the first corresponding product to achieve the corresponding milestone. The Company is also eligible to receive (a) specified sales milestone payments of up to an aggregate of $ 400.0 million for the Novartis SMA Program and up to an aggregate of $ 375.0 million for the Novartis HD Program and (b) tiered, escalating royalties in the high single-digit to low double-digit percentages of annual net sales of the Novartis SMA Program Products and the Novartis HD Program Products. The royalties are subject to potential customary reductions, including patent claim expiration, payments for certain third-party licenses, and biosimilar market penetration, subject to specified limits.

Stock Purchase Agreement

Under the stock purchase agreement with Novartis entered into on December 28, 2023 (the "2023 Novartis Stock Purchase Agreement"), Novartis agreed to purchase 2,145,002 shares of common stock of the Company (the "Novartis Shares") for an aggregate purchase price of approximately $ 20.0 million.

Accounting Analysis

The Company determined the 2023 Novartis Collaboration Agreement represents a contract with a customer under ASC 606. In addition, the 2023 Novartis Collaboration Agreement did not modify the scope or price of the 2022 Novartis Option and License Agreement, as discussed below. The Company therefore determined that the 2023 Novartis Collaboration Agreement should be accounted for separately. The 2023 Novartis Collaboration Agreement includes the following performance obligations: (i) the development and commercialization license for the Novartis SMA Program, (ii) the development and commercialization license for the Novartis HD Program; and (iii) the research and development

services for the Novartis HD Program (“Novartis HD Research Services”). The development and commercialization licenses for the Novartis HD Program and Novartis SMA Program are each distinct, as Novartis can benefit from such licenses on their own or from other resources commonly available in the industry given the stage of development of the product candidates subject to the licenses. Similarly, the research and development services for the Novartis HD Program provide a distinct benefit to Novartis within the context of the contract, separate from the licenses.

The transaction consideration allocated to the performance obligations within the 2023 Novartis Collaboration Agreement includes fixed consideration of $ 80.0 million, and variable consideration, which is comprised of an estimated $ 24.2 million of cost reimbursements for Novartis HD Research Services, up to $ 425.0 of potential development milestone payments, up to $ 775.0 million of potential sales milestone payment, and sales-based royalties. The consideration related to the Novartis HD Research Services, becomes due and payable on a quarterly basis as the services are being performed.

The Company estimates variable consideration using the most likely amount approach. At the outset of the contract, the Company has determined this consideration should be constrained. The sales milestone payments and royalties will be recognized in the period the underlying sales occur, as this consideration is related to the two development and commercialization licenses, the predominant performance obligations in the contract.

The Company allocated the fixed transaction price to the separate performance obligations based on the relative standalone selling price of each performance obligation. The standalone selling prices for development and commercialization licenses for the Novartis SMA Program and Novartis HD Program were estimated using an adjusted-market approach. The Company allocated the variable consideration related to the Novartis HD Research Services as the consideration becomes payable as the Company delivers the Novartis HD Research Services and allocating the entirety of this consideration to the Novartis HD Research Services reflects the amount the Company expects to be entitled to for performing the services. The development milestone payments, the sales milestone payments and the royalties are allocated to the respective development and commercialization licenses for the Novartis SMA Program and Novartis HD Program as the variable consideration relates directly to those performance obligations.

The Company recognized the $ 80.0 million fixed transaction price allocated to the development and commercialization licenses for the Novartis SMA Program and Novartis HD Program, as collaboration revenue upon delivery of the development and commercialization licenses to Novartis in December 2023. The issuance of the Novartis Shares to Novartis pursuant to the 2023 Novartis Stock Purchase Agreement in January 2024 resulted in a premium of $ 0.7 million. The premium will be allocated to the development and commercialization licenses for the Novartis HD Program and Novartis SMA Program and is expected to be recognized as collaboration revenue during the first quarter of 2024, upon the issuance of the Novartis Shares under the 2023 Novartis Stock Purchase Agreement. The Novartis HD Research Services commenced in the first quarter of 2024. The $ 80.0 million fixed transaction price was recorded in accounts receivable as of December 31, 2023. The Company had an unconditional right to the payment, and it was collected in January of 2024.

The Company incurred approximately $ 1.9 million of business development costs related to the 2023 Novartis Collaboration Agreement which were payable only upon the execution of the agreement, and therefore are considered incremental costs of obtaining a contract with a customer. Given the substantial value associated with the development and commercialization licenses for the Novartis SMA Program and Novartis HD Program that were delivered in December 2023, the Company recognized the $ 1.9 million of costs in general and administrative expenses during the year ended December 31, 2023.

2022 Novartis Option and License Agreement

Summary of Agreement

On March 4, 2022 (the “Novartis Effective Date”), the Company entered into an option and license agreement with Novartis (the “2022 Novartis Agreement”). Pursuant to the 2022 Novartis Agreement, the Company granted Novartis options (the “Novartis License Options”) to license TRACER Capsids (“Novartis Licensed Capsids”) for

exclusive use with certain targets to develop and commercialize adeno-associated virus gene therapy candidates comprised of Novartis Licensed Capsids and payloads directed to such targets (the “Novartis Payloads”).

During the period commencing on the Novartis Effective Date and ending on the first anniversary thereof or, in the event Novartis exercises a Novartis License Option, the third anniversary thereof, on a target-by-target basis (the “Novartis Research Term”), the Company granted Novartis a non-exclusive research license to evaluate the Company’s TRACER Capsids for potential use, in combination with Novartis Payloads, in programs targeting three specified genes (the “Initial Novartis Targets”). Upon the payment of additional fees, Novartis may also assess the Company’s TRACER Capsids for use with up to two other targets (the “Additional Novartis Targets”), subject to certain conditions including that such target is not part of, or reasonably competitive with, the Company’s current development programs (the Initial Novartis Targets and the Additional Novartis Targets collectively, the “Novartis Targets”). During the Novartis Research Term, as applicable, the Company may, at its sole discretion and expense, conduct further research activities to identify additional TRACER Capsids. If the Company elects to do so, the Company has agreed to disclose performance characteristics of such new TRACER Capsids to Novartis on a rolling basis.

During the applicable Novartis Research Term, Novartis may exercise up to three Novartis License Options—or up to five Novartis License Options if Novartis is evaluating the Additional Novartis Targets—in the aggregate, provided that Novartis may only exercise one Novartis License Option for each Novartis Target. Upon the exercise of any Novartis License Option, the Company has agreed to grant Novartis a target-exclusive, worldwide license, with the right to sublicense, under certain of the Company’s intellectual property, the rights to develop and commercialize the applicable Novartis Licensed Capsid as incorporated into products containing the corresponding Novartis Payload (the “Novartis Licensed Products”). Upon the exercise of a Novartis License Option, the Company has agreed to provide certain additional know-how to enable Novartis to exploit the Novartis Licensed Capsid and the corresponding Novartis Payload for use in a Novartis Licensed Product. Novartis may, during the applicable Novartis Research Term but following the exercise of a Novartis License Option, conduct additional evaluation of the Company’s capsid candidates and has the right to substitute any other TRACER Capsid for a Novartis Licensed Capsid.

Effective March 1, 2023, Novartis exercised its Novartis License Options to license TRACER Capsids for use in gene therapy programs against two undisclosed Initial Novartis Targets.

Subject to the Company’s disclosure obligations described above, the Company and Novartis have agreed to conduct their respective research and evaluation activities independently, with communications being managed by two alliance managers comprised of a designee from each of the Company and Novartis.

Under the 2022 Novartis Agreement, Novartis is solely responsible for, and has sole decision-making authority with respect to, development and commercialization of the Novartis Licensed Products. In the event Novartis exercises a Novartis License Option, Novartis is required to use commercially reasonable efforts to develop and obtain regulatory approval for at least one Novartis Licensed Product for each Novartis Target for which it has exercised a Novartis License Option in (a) the United States and (b) at least three of the following countries: the United Kingdom, France, Germany, Italy, Spain and Japan (each of which, a “Novartis Major Market Country”), subject to certain limitations. Novartis is also required to use commercially reasonable efforts to commercialize each Novartis Licensed Product in the United States and at least three Novartis Major Market Countries where Novartis or its designated affiliates or sublicensees has received regulatory approval for such Novartis Licensed Product, subject to certain limitations.

During the Novartis Research Term, the Company has agreed to provide plasmids to Novartis for the production of TRACER Capsids for evaluation upon request. The Company has also granted Novartis a non-exclusive license, effective upon an exercise of a Novartis License Option and in addition to its options for target-exclusive licenses under certain of the Company’s intellectual property described above, on a Novartis Licensed Capsid-by-Novartis Licensed Capsid basis, under certain of the Company’s know-how to exploit the applicable Novartis Licensed Capsid as incorporated into Novartis Licensed Products containing the corresponding Novartis Payload.

Under the terms of the 2022 Novartis Agreement, Novartis paid the Company an upfront payment of $ 54.0 million. Effective as of March 1, 2023, Novartis exercised its Novartis License Options to license TRACER Capsids for use in gene therapy programs against two undisclosed Initial Novartis Targets.

Under the terms of the 2022 Novartis Agreement, each party owns the entire right, title, and interest in and to all patents or know-how controlled by such party and existing as of or before the Novartis Effective Date, or invented, developed, created, generated or acquired solely by or on behalf of such party after the Novartis Effective Date. Subject to certain specified exceptions, any patents and know-how that are invented or otherwise developed jointly by or on behalf of the parties during the term of the 2022 Novartis Agreement and in the course of the parties' activities under the 2022 Novartis Agreement will follow inventorship under U.S. patent law.

Subject to certain limitations and exceptions, the Company has agreed (a) during the Novartis Research Term, not to conduct any internal program or program on behalf of a third party that is directed to the development or commercialization of any Company's capsids, or grant any third party or affiliate any right or license under the Company's rights in such capsids, to exploit any therapeutic product containing a capsid in combination with a payload designed to have therapeutic effect on any of the Novartis Targets; and (b) after Novartis's exercise of any Novartis License Option, not to grant any third party or affiliate any right or license under the Company's patents to exploit any Novartis Licensed Capsid for the applicable Novartis Target.

Unless earlier terminated, the 2022 Novartis Agreement expires on the expiration of the last-to-expire royalty term with respect to all Novartis Licensed Products in all countries. Subject to a cure period, either party may terminate the 2022 Novartis Agreement, in whole or in part, subject to specified conditions, in the event of the other party's uncured material breach. Novartis may also terminate the 2022 Novartis Agreement, in whole or in part, subject to specified conditions, for the Company's insolvency, the occurrence of a violation of global trade control laws, or for the Company's non-compliance with certain anti-bribery or anti-corruption covenants. Novartis may terminate the 2022 Novartis Agreement, in whole or in part, for any or no reason upon ninety days ' written notice to the Company.

Upon certain terminations for cause by Novartis, the licenses granted by the Company to Novartis under the 2022 Novartis Agreement shall become irrevocable and perpetual, and all milestone payments and royalties that would have otherwise been payable by Novartis under such licenses had the Novartis Agreement remained in effect would be substantially reduced.

Accounting Analysis

At inception, the Company determined the 2022 Novartis Agreement was a contract with a customer under ASC 606. The Company assessed the promised goods and services and determined that the 2022 Novartis Agreement contains three performance obligations consisting of three material rights, one for each of the Novartis License Options. The Company concluded that each Novartis License Option provides a material right as consideration for each option is less than the amount that the Company would otherwise have expected to receive outside the context of the contract. The promises at inception do not include the underlying goods or services that would be delivered upon exercise of the option, but rather represent the value to the customer of having the right to exercise the Novartis License Option at the specified exercise fee. Upon the exercise of a Novartis License Option, until March 4, 2025, while the Company is not obligated to conduct additional research activities upon any option exercise to identify additional proprietary capsids that may be useful for AAV gene therapies for the treatment of central nervous system or cardiovascular diseases, it has agreed to continue to disclose to Novartis, on a rolling basis, the performance characteristics identified for all such capsid candidates, if and when available. Novartis may conduct additional evaluation of such capsid candidates and has the right to substitute any other capsid candidate for the Novartis Licensed Capsid it previously elected to license when it exercised the Novartis License Option. The Company determined that this promise to provide Novartis the ability to evaluate and potentially substitute other capsid candidates for the Novartis Licensed Capsid it previously elected to license when it exercised the Novartis License Option, if and when available, is an additional performance obligation in the arrangement (the "Novartis Substitution Right Performance Obligation"). The Company concluded the options for Additional Novartis Targets are not material rights as the price reflects the standalone selling price of the options. The Company will therefore account for the options for Additional Novartis Targets separately, if and when exercised.

The Company received a nonrefundable, upfront payment of $ 54.0 million as consideration under the 2022 Novartis Agreement, which represents the transaction price at inception. Additional consideration to be paid to the Company upon exercise of the Novartis License Options or upon reaching certain milestones are excluded from the

transaction price as they relate to option fees and milestones that could only be achieved subsequent to an option exercise.

The Company allocated the transaction price to the three material rights based on their relative standalone selling prices. The estimated standalone selling price for each material right was based on an adjusted market assessment approach. The Company concluded that the market would be willing to pay an equal amount for each Novartis License Option on a standalone basis. The Company reached this conclusion after considering (i) the downstream economics including option fees, milestones and royalties related to each Novartis License Option being identical and (ii) comparable market data. The Company determined the standalone selling price for the Novartis Substitution Right Performance Obligation was insignificant to the allocation of the transaction price using the relative standalone selling price model and did not allocate any transaction price to the Novartis Substitution Right Performance Obligation, accordingly. This determination was supported by qualitative and quantitative assessments of the standalone selling price that considered the cost of identifying other potential capsid candidates and the likelihood of license substitution. As such, based on the relative standalone selling price for each of the three material rights, the allocation of the transaction price to the separate performance obligations is $ 18.0 million for each material right.

The amount allocated to each material right was recorded as deferred revenue.

During the year ended December 31, 2023, the Company recognized $ 79.0 million in collaboration revenue related to the Novartis Agreement. Of this $ 79.0 million, $ 54.0 million is attributable to the exercise of the two material rights for Novartis License Options and the expiration of the third material right and was previously deferred as of December 31, 2022. The remaining $ 25.0 million represents the option exercise fee. This amount was received by the Company during the second quarter of 2023.

2023 Neurocrine Collaboration Agreement

Summary of Agreement

On January 8, 2023, the Company entered into a collaboration and license agreement with Neurocrine (the “2023 Neurocrine Collaboration Agreement”) for the research, development, manufacture and commercialization of gene therapy products directed to the GBA1 Program, and three early research programs focused on the research, development, manufacture and commercialization of gene therapies designed to address central nervous system (“CNS”) diseases or conditions associated with rare genetic targets (the “2023 Discovery Programs” and, collectively with the GBA1 Program, the “2023 Neurocrine Programs”). The 2023 Neurocrine Collaboration Agreement became effective on February 21, 2023 (the “Neurocrine Effective Date”).

Collaboration and License

Under the 2023 Neurocrine Collaboration Agreement, the Company and Neurocrine have agreed to collaborate on the conduct of the 2023 Neurocrine Programs. Under the terms of the 2023 Neurocrine Collaboration Agreement, subject to the rights retained by the Company thereunder, the Company granted to Neurocrine, as of the Neurocrine Effective Date, an exclusive, royalty-bearing, sublicensable, worldwide license, under certain of the Company’s intellectual property rights, to research, develop, manufacture and commercialize gene therapy products (the “2023 Collaboration Products”), arising under the 2023 Neurocrine Programs.

Pursuant to mutually-agreed development plans, during the period beginning on the Neurocrine Effective Date and ending on the third anniversary of the Neurocrine Effective Date, which period may be extended upon mutual written agreement of the Company and Neurocrine (the “2023 Discovery Period”), and as overseen by the Joint Steering Committee (“JSC”) for the ongoing collaboration with Neurocrine, the Company is responsible for identifying capsids meeting target criteria, producing development candidates, and conducting other pre-clinical activities regarding the 2023 Collaboration Products. Neurocrine has agreed to be responsible for all costs the Company incurs in conducting pre-clinical development activities for each 2023 Neurocrine Program, in accordance with JSC agreed upon workplans and budgets. If the Company breaches its responsibilities during this time or, in certain circumstances, upon a change of

control, Neurocrine has the right, but not the obligation, to assume the conduct of the Company's activities under such 2023 Neurocrine Program.

The Company has been granted the option ("2023 Co-Co Option") to co-develop and co-commercialize 2023 Collaboration Products in the GBA1 Program in the United States upon the occurrence of the Company receiving topline data from the first Phase 1 clinical trial for a product candidate being developed pursuant to the GBA1 Program. Should the Company elect to exercise its 2023 Co-Co Option, the Company and Neurocrine agree to enter into a cost and profit-sharing arrangement (a "2023 Co-Co Agreement"), whereby the Company and Neurocrine agree to jointly develop and commercialize 2023 Collaboration Products in the GBA1 Program ("2023 Co-Co Products") in the United States and share equally in the GBA1 Program's costs, profits and losses in the United States, with each party entitled to or responsible for 50 % of profits and losses with respect to each 2023 Co-Co Product in the United States, subject to specified exceptions. The parties have agreed that the 2023 Co-Co Agreement will provide the Company the right to terminate the 2023 Co-Co Agreement for any reason upon prior written notice to Neurocrine and provide Neurocrine the right to terminate or amend the 2023 Co-Co Agreement upon a change of control under certain circumstances. In the event the Company exercises its 2023 Co-Co Option, the parties have also agreed that Neurocrine is entitled to receive (in addition to its 50 % share of profits) 50 % of the Company's share of profits until the Company's obligation to repay 50 % of all development costs incurred by Neurocrine in connection with the GBA1 Program prior to such exercise have been paid off out of such 50 % of the Company's share of profits.

Candidate Selection

Either party may notify the JSC of any gene therapy product candidate that includes a Company capsid and a payload that is being developed under a 2023 Neurocrine Program (a "Collaboration Candidate"), that it desires to nominate as a development candidate. In such event, the JSC shall determine whether such nominated Collaboration Candidate meets certain development criteria. There will be a maximum of four potential development candidates for which development is being performed under any 2023 Neurocrine Program at any given time during the 2023 Discovery Period. If a Collaboration Candidate fails to meet criteria established by the JSC and is removed from consideration to become a development candidate or is named a development candidate, then a new Collaboration Candidate may be nominated to be a potential development candidate to replace the Collaboration Candidate that has failed or succeeded such that not more than four potential development candidates per program are under consideration at any one time during the 2023 Discovery Period.

Manufacturing

The parties have agreed that the applicable development plans shall specify the allocation between the Company and Neurocrine of responsibilities for the manufacturing of Collaboration Candidates associated with the applicable 2023 Neurocrine Program during the 2023 Discovery Period. In accordance with the 2023 Collaboration Agreement, the parties have also agreed that, if the Company conducts any portion of the manufacturing of a Collaboration Candidate, the applicable development plan shall include an obligation for the Company to assist with the technology transfer of such manufacturing responsibilities to Neurocrine or a third-party contract manufacturing organization, as reasonably requested by Neurocrine, on terms to be mutually-agreed by the Company and Neurocrine. Following the end of the 2023 Discovery Period, Neurocrine shall be responsible for the manufacturing of all Collaboration Candidates and products.

Financial Terms

Under the terms of the 2023 Neurocrine Collaboration Agreement, Neurocrine paid the Company an upfront payment of approximately $ 136.0 million and approximately $ 39.0 million for the purchase of 4,395,588 shares of common stock of the Company at a price of $ 8.88 per share in February 2023. The 2023 Collaboration Agreement provides for aggregate development milestone payments from Neurocrine to the Company for 2023 Collaboration Products under (a) the GBA1 Program of up to $ 985.0 million; and (b) each of the three 2023 Discovery Programs of up to $ 175.0 million for each 2023 Discovery Program. The Company may be entitled to receive aggregate commercial milestone payments for up to two 2023 Collaboration Products under the GBA1 Program of up to $ 950.0 million per

2023 Collaboration Product and for one 2023 Collaboration Product under each 2023 Discovery Program of up to $ 275.0 million per 2023 Discovery Program.

Neurocrine has also agreed to pay the Company tiered royalties, based on future net sales of the 2023 Collaboration Products. Such royalty percentages, for net sales in and outside the United States, range from (a) for the GBA1 Program, the low double-digits to twenty and the high single-digits to mid-teens, respectively, and (b) for each 2023 Discovery Program, high single-digits to mid-teens and mid-single digits to low double-digits, respectively. On a country-by-country and 2023 Neurocrine Program-by-2023 Neurocrine Program basis, the parties have agreed royalty payments would commence on the first commercial sale of a 2023 Collaboration Product in such country and terminate upon the latest of (a) the expiration, invalidation or the abandonment of the last patent covering the composition of the 2023 Collaboration Product or its approved method of use in such country, (b) ten years from the first commercial sale of the 2023 Collaboration Product in such country and (c) the expiration of regulatory exclusivity in such country (the “2023 Royalty Term”). Royalty payments may be reduced by up to 50 % in specified circumstances, including expiration of patent rights related to a 2023 Collaboration Product, approval of biosimilar products in each country, or required payment of licensing fees to third parties related to the development and commercialization of any 2023 Collaboration Product. Additionally, the licenses granted to Neurocrine shall automatically convert to a fully-paid, perpetual, irrevocable royalty-free license on a country-by-country and 2023 Collaboration Product-by-2023 Collaboration Product basis upon the expiration of the 2023 Royalty Term applicable to the 2023 Collaboration Product in such country.

Termination

Unless earlier terminated, the 2023 Neurocrine Collaboration Agreement expires on the later of (a) the expiration of the last to expire 2023 Royalty Term with respect to all 2023 Collaboration Products worldwide or (b) the expiration or termination of any 2023 Co-Co Agreement. Neurocrine may terminate the 2023 Neurocrine Collaboration Agreement in its entirety or on a 2023 Neurocrine Program-by-2023 Neurocrine Program and/or country-by-country basis by providing at least (a) 180 -day advance notice if such notice is provided prior to the first commercial sale of any 2023 Collaboration Product to which the termination applies or (b) one-year advance notice if such notice is provided after the first commercial sale of any product to which the termination applies. Neurocrine may terminate the 2023 Neurocrine Collaboration Agreement with respect to a given 2023 Collaboration Product by providing written notice of termination to the Company within thirty days after complete readout of any clinical trial if the results of such clinical trial fail to meet the pre-specified primary endpoint(s) set forth in the applicable protocol or if there is a safety finding during the clinical trial relating to such 2023 Collaboration Product that either (a) is substantially irreversible or not monitorable in patients or (b) results in Neurocrine’s decision to designate such 2023 Collaboration Product as a terminated product under the 2023 Collaboration Agreement.

The Company may terminate the 2023 Neurocrine Collaboration Agreement with respect to a particular patent right of the Company’s, if Neurocrine challenges the validity or enforceability of such patent right. Subject to a cure period, either party may terminate the 2023 Neurocrine Collaboration Agreement in the event of a material breach in whole or in part, subject to specified conditions.

2023 Neurocrine Stock Purchase Agreement

In connection with the execution of the 2023 Neurocrine Collaboration Agreement, Neurocrine and the Company also entered into a stock purchase agreement on January 8, 2023 (“the “2023 Neurocrine Stock Purchase Agreement”), for the sale and issuance of 4,395,588 shares of common stock to Neurocrine at a price of $ 8.88 per share, for an aggregate purchase price of approximately $ 39.0 million. In accordance with the terms and conditions of the 2023 Neurocrine Stock Purchase Agreement, the Company issued and sold these shares to Neurocrine on February 23, 2023.

Accounting Analysis

At inception, the Company determined the 2023 Neurocrine Collaboration Agreement was a contract with a customer under ASC 606 and that modification accounting was not required given that the 2023 Neurocrine Collaboration Agreement did not modify the scope or price of the 2019 Neurocrine Collaboration Agreement. The Company therefore determined that the 2023 Neurocrine Agreement should be accounted for separately. The 2023

Neurocrine Collaboration Agreement includes the following performance obligations: (i) the development and commercialization license for the GBA1 Program, (ii) the research and development services for the GBA1 Program, and (iii) the research and development services for each of the 2023 Discovery Programs combined with a development and commercialization license for each program. The license for the GBA1 Program is distinct as Neurocrine can benefit from such license on its own or from other resources commonly available in the industry given the stage of development of the product candidates subject to the license. Similarly, the research and development services for the GBA1 Program provide a distinct benefit to Neurocrine within the context of the contract, separate from the license. The research and development services for the 2023 Discovery Programs are not distinct as Neurocrine cannot benefit from such licenses on its own or from other resources commonly available in the industry, without the corresponding research services due to the unique and specialized expertise of the Company that is not readily available in the marketplace. The GBA1 license, GBA1 research and development services and the combined licenses and research and development services for the 2023 Discovery Programs are distinct from one another as Neurocrine can benefit from each program separately.

The Company identified $ 143.9 million of fixed transaction price consisting of the $ 136.0 million upfront fee, and a premium of $ 7.9 million related to the $ 39.0 million equity investment of 4,395,588 shares when measured at fair value on the date of issuance. The Company is also entitled to reimbursement of costs incurred by the Company during the 2023 Discovery Period associated with each of the GBA1 Program and 2023 Discovery Programs.

These amounts are determinable based on development plans, and the Company has a contractual right to the payment of costs incurred under the agreed upon program development plans.

The Company utilizes the most likely amount approach to estimate the cost reimbursement and has concluded this consideration should be constrained. As of December 31, 2023, the estimate of the expected reimbursement was $ 11.3 million of costs incurred based on expectations as of such date. The sales milestone payments and royalties will be recognized in the period the underlying sales occur, as this consideration is related to the two development and commercialization licenses, the predominant performance obligations in the contract.

The Company has allocated the fixed transaction price to the separate performance obligations based on the relative standalone selling price of each performance obligation. The estimated standalone selling prices for performance obligations were developed using the estimated selling price of the license for the GBA1 Program and each of the three 2023 Discovery Programs, using primarily adjusted market assessment approaches that considered discounted, probability-weighted cash flow analyses and entity-specific and market factors. The Company did not allocate any of the fixed transaction price to the GBA1 research and development services performance obligation as the consideration for such services reflects a market rate.

The Company concluded that the variable consideration related to the cost reimbursement of each program will be allocated to each respective program as the cost reimbursement relates specifically to the respective program services being performed under the 2023 Neurocrine Collaboration Agreement. The reimbursement of research services is at a market rate and the allocation of the fixed consideration to each of the three 2023 Discovery Program performance obligations depicts the estimated amounts in which it would expect to receive for these obligations, absent the variable consideration related to the research reimbursement. Based on the initial development plans, the total variable consideration allocated to each program related to the expected cost reimbursement was as follows as of December 31, 2023:

Performance Obligation	Amount
	(in thousands)
Variable Consideration	
GBA1 Program	$ 5,920
2023 Discovery Program 1	3,779
2023 Discovery Program 2	780
2023 Discovery Program 3	780
Total	$ 11,259

Based on the relative standalone selling price allocation, the allocation of the fixed transaction price to the separate performance obligations was as follows:

Performance Obligation	Amount
	(in thousands)
Fixed Consideration	
GBA1 Program	$ 69,459
2023 Discovery Program 1	24,807
2023 Discovery Program 2	24,807
2023 Discovery Program 3	24,807
Total	$ 143,880

The Company recognized the fixed transaction price allocated to the development and commercialization license for the GBA1 Program as collaboration revenue in the first quarter of 2023, upon delivery of the development and commercialization license for the GBA1 Program to Neurocrine. The Company is recognizing the consideration allocated to each of the three 2023 Discovery Program performance obligations on a proportional performance basis over the period of service using input-based measurements such as costs incurred to date, to estimate proportion performed, and remeasures its progress towards completion at the end of each reporting period. Proportional performance is determined based on the workplan cost and timeline estimates.

During the year ended December 31, 2023, the Company recognized $ 69.5 million of revenue associated with the 2023 Neurocrine Collaboration Agreement related to the delivery of the development and commercialization license for the GBA1 Program. During the year ended December 31, 2023, the Company recognized $ 5.8 million of collaboration revenue associated with research and development services performed during the period and the corresponding cost reimbursement receivable for the GBA1 Program. During the year ended December 31, 2023, the Company recognized a total of $ 5.5 million of revenue associated with the fixed transaction price allocated to the three 2023 Discovery Programs, and for research and development services performed during the period. As of December 31, 2023, there was $ 69.1 million of deferred revenue related to the 2023 Neurocrine Collaboration Agreement, of which $ 38.4 million was classified as current and $ 30.7 million was classified as non-current in the accompanying balance sheets based on the period the services are expected to be delivered. Additionally, as of December 31, 2023, there was $ 1.8 million of related party collaboration receivable related to reimbursable costs expected to be received from Neurocrine for research and development services performed.

The Company incurred approximately $ 0.4 million of costs to obtain the 2023 Neurocrine Collaboration Agreement which were payable only upon the close of the transaction and therefore considered incremental costs of obtaining a contract with a customer and capitalized. The costs are recorded in prepaid expenses and are being amortized to operating expenses consistent with the manner in which the consideration allocated to the performance obligations is recognized.

2019 Neurocrine Collaboration Agreement

Summary of Agreement

Effective March 2019, the Company entered into a collaboration agreement with Neurocrine (the “2019 Neurocrine Collaboration Agreement”) for the research, development and commercialization of certain of its AAV gene therapy products. Under the 2019 Neurocrine Collaboration Agreement, the Company agreed to collaborate on the conduct of four collaboration programs (the “2019 Neurocrine Programs”) which include: (a) VY-AADC (NBIb-1817) for Parkinson’s disease (the “VY-AADC Program”), (b) the FA Program (collectively, with the VY-AADC Program, the “Legacy Programs”), and (c) two programs to be determined by the Company and Neurocrine at a later date (the “2019 Discovery Programs”).

In June 2019, in conjunction with the termination of the collaboration agreement with Sanofi Genzyme (the “Sanofi Genzyme Collaboration Agreement”), the Company gained ex-U.S. rights to the FA Program. The Company’s ex-U.S. rights to the FA Program were subsequently transferred to Neurocrine under the terms of the 2019 Neurocrine

Collaboration Agreement. To facilitate the transfer of the ex-U.S. rights to the FA Program to Neurocrine, the Company and Neurocrine executed an amendment to the 2019 Neurocrine Collaboration Agreement (the “June 2019 Modification”), and Neurocrine paid $ 5.0 million to the Company. There were no other changes in pricing or scope of the obligations required to be performed under the 2019 Neurocrine Collaboration Agreement.

In February 2021, Neurocrine notified the Company that it had elected to terminate the 2019 Neurocrine Collaboration Agreement solely with regards to the VY-AADC Program, effective August 2, 2021 (the “Neurocrine VY-AADC Program Termination Effective Date”). The 2019 Neurocrine Collaboration Agreement remains in full force and effect for each other program thereunder. As a result of the termination, Neurocrine is no longer obligated to reimburse the Company for research and development activities related to the VY-AADC Program.

Under the terms of the 2019 Neurocrine Collaboration Agreement, the Company originally agreed to collaborate with Neurocrine on, and to grant, exclusive, royalty-bearing, non-transferable, sublicensable licenses to certain of its intellectual property rights, for all human and veterinary diagnostic, prophylactic, and therapeutic uses, for the research, development, and commercialization of gene therapy products (the “ 2019 Collaboration Products”) under (a) the VY-AADC Program on a worldwide basis; (b) the FA Program in the United States and, all countries in the world in which the 2019 Neurocrine Collaboration Agreement remains in effect with respect to the FA Program; and (c) each 2019 Discovery Program on a worldwide basis. As a result of the termination of the 2019 Neurocrine Collaboration Agreement with regards to the VY-AADC Program, in accordance with the terms of the 2019 Neurocrine Collaboration Agreement, the licenses granted by the Company to Neurocrine regarding the VY-AADC Program have expired, and the Company has regained worldwide intellectual property rights regarding the VY-AADC Program, in each case as of the VY-AADC Termination Effective Date.

Pursuant to development plans agreed by the parties, which are overseen by a JSC, the Company has operational responsibility, subject to certain exceptions, for the conduct of each 2019 Neurocrine Program prior to the occurrence of a specified event for such 2019 Neurocrine Program (a “2019 Transition Event”), as described below, and is required to use commercially reasonable efforts to develop the corresponding 2019 Collaboration Products. Neurocrine has agreed to be responsible for all costs incurred by the Company in conducting these activities for each 2019 Neurocrine Program, in accordance with an agreed budget for each 2019 Neurocrine Program. If the Company breaches its development responsibilities or in certain circumstances upon a change in control, Neurocrine has the right but not the obligation to assume the activities under such 2019 Neurocrine Program.

Upon the occurrence of a 2019 Transition Event for each 2019 Neurocrine Program, Neurocrine has agreed to assume responsibility for development, manufacturing and commercialization activities for such 2019 Neurocrine Program from the Company and to pay milestones and royalties on future net sales as described further below. As a result of Neurocrine’s termination of the 2019 Neurocrine Collaboration Agreement with respect to the VY-AADC Program, the 2019 Transition Event with respect to the VY-AADC Program is no longer applicable. The 2019 Transition Events for the remaining programs are (a) with respect to the FA Program, the Company’s receipt of topline data for the initial Phase 1 clinical trial for an FA Program product candidate; and (b) with respect to each 2019 Discovery Program, the preparation by the Company and the approval by Neurocrine of an IND application to be filed with the FDA by Neurocrine for the first development candidate in such 2019 Discovery Program. For the FA Program, the Company was granted the option (the “2019 FA Co-Co Option”) to co-develop and co-commercialize the FA Program upon the occurrence of a specified event (a “2019 FA Co-Co Trigger Event”). The Company agreed, upon its exercise of the FA Co-Co Option, to enter into a cost- and profit-sharing arrangement with Neurocrine (the “2019 FA Co-Co Agreement”), and (a) jointly develop and commercialize the 2019 Collaboration Products for the FA Program (“FA Collaboration Products”), (b) share in its costs, profits and losses, and (c) forfeit certain milestones and royalties on net sales in the United States during the effective period of the 2019 FA Co-Co Agreement. The 2019 FA Co-Co Trigger Event for the FA Program is the achievement of milestones or metrics specified in the applicable development plan , as determined by the JSC.

Under the 2019 Neurocrine Collaboration Agreement, subject to exceptions specified therein, the Company and Neurocrine agreed that profits and losses under the Company’s 2019 FA Co-Co Option would be allocated 60 % to Neurocrine and 40 % to the Company for any FA Collaboration Product. The parties agreed that 2019 FA Co-Co

Agreement would provide the Company the right to terminate for any reason upon prior written notice to Neurocrine and Neurocrine the right to terminate in certain circumstances upon change of control.

The Company's research and development activities under the 2019 Neurocrine Collaboration Agreement are conducted pursuant to plans agreed to by the parties, on a program-by-program basis, and overseen by the JSC, as detailed in the 2019 Neurocrine Collaboration Agreement.

Under the 2019 Neurocrine Collaboration Agreement, the parties committed to agree on a list of up to eight target genes (the "Targets") from which Neurocrine had the right to nominate Targets for the two 2019 Discovery Programs. The Company and Neurocrine completed the nomination process, and the JSC has approved the two Targets for development under the 2019 Discovery Programs. The two Targets are currently under development.

The 2019 Neurocrine Collaboration Agreement provides for an upfront non-refundable payment of $ 115.0 million, as well as for aggregate development and regulatory milestone payments from Neurocrine to the Company for 2019 Collaboration Products under (a) the VY-AADC Program of up to $ 170.0 million, which the Company is no longer eligible to receive in light of the partial termination of the 2019 Neurocrine Collaboration Agreement; (b) the FA Program of up to $ 195.0 million, and (c) each of the two 2019 Discovery Programs of up to $ 130.0 million per 2019 Discovery Program. The Company may be entitled to receive aggregate commercial milestone payments for each 2019 Collaboration Product of up to $ 275.0 million, subject to an aggregate cap on commercial milestone payments across all 2019 Neurocrine Programs of $ 1.1 billion. Furthermore, in connection with the 2019 Neurocrine Collaboration Agreement, Neurocrine purchased 4,179,728 shares of the Company's common stock at a price of $ 11.9625 per share, for an aggregate purchase price of $ 50.0 million.

Neurocrine also agreed to pay the Company royalties, based on future net sales of the 2019 Collaboration Products. Such royalty percentages, for net sales in and outside the United States, as applicable, range (a) for the VY-AADC Program, from the mid-teens to low thirties and the low-teens to low twenties, respectively, which the Company is no longer eligible to receive in light of the partial termination of the 2019 Neurocrine Collaboration Agreement; (b) for the FA Program, from the low-teens to high-teens and high-single digits to mid-teens, respectively; and (c) for each 2019 Discovery Program, from the high-single digits to mid-teens and mid-single digits to low-teens, respectively. On a country-by-country and program-by-program basis, royalty payments would commence on the first commercial sale of a 2019 Collaboration Product and terminate on the later of (a) the expiration of the last patent covering the 2019 Collaboration Product or its method of use in such country, (b) ten years from the first commercial sale of the 2019 Collaboration Product in such country and (c) the expiration of regulatory exclusivity in such country (the "2019 Royalty Term"). Royalty payments may be reduced by up to 50 % in specified circumstances, including expiration of patents rights related to a 2019 Collaboration Product, approval of biosimilar products in a given country or required payment of licensing fees to third parties related to the development and commercialization of any 2019 Collaboration Product. As a result of Neurocrine's termination of the 2019 Neurocrine Collaboration Agreement with respect to the VY-AADC Program, the Company is no longer entitled to receive royalties related to the VY-AADC Program. Additionally, the licenses granted to Neurocrine shall automatically convert to fully paid-up, non-royalty bearing, perpetual, irrevocable, exclusive licenses on a country-by-country and product-by-product basis upon the expiration of the 2019 Royalty Term applicable to such 2019 Collaboration Product in such country.

Under the terms of the 2019 Neurocrine Collaboration Agreement and subject to specified exceptions therein, each party owns the entire right, title and interest in and to all intellectual property rights made solely by its employees or agents in the course of the collaboration. The parties jointly own all rights, title and interest in and to all intellectual property rights made or invented jointly by employees or agents of both parties.

During the term of the 2019 Neurocrine Collaboration Agreement, neither party nor any of its respective affiliates is permitted to directly or indirectly exploit any AAV-based gene therapy products directed to a Target to which a 2019 Collaboration Product is directed, subject to specified exceptions, including the parties' conduct of basic research activities.

Unless earlier terminated, the 2019 Neurocrine Collaboration Agreement expires on the later of (a) the expiration of the last to expire 2019 Royalty Term with respect to a 2019 Collaboration Product in all countries in the

relevant territory or (b) the expiration or termination of any 2019 FA Co-Co Agreement. Neurocrine may terminate the 2019 Neurocrine Collaboration Agreement in its entirety or on a program-by-program or country-by-country basis by providing at least (x) 180 -day advance notice if such notice is provided prior to the first commercial sale of the 2019 Collaboration Product to which the termination applies or (y) one-year advance notice if such notice is provided after the first commercial sale of the 2019 Collaboration Product to which the termination applies. The Company may terminate the 2019 Neurocrine Collaboration Agreement, subject to specified conditions, if Neurocrine challenges the validity or enforceability of certain of the Company's intellectual property rights. Subject to a cure period, either party may terminate the 2019 Neurocrine Collaboration Agreement in the event of a material breach by the other party in whole or in part, subject to specified conditions.

Upon termination in certain cases, Neurocrine has agreed to grant to the Company licenses to certain Neurocrine intellectual property, subject to a negotiation between the parties to establish royalty rates for use of such intellectual property. In the event of a breach by the Company with respect to a 2019 Neurocrine Program, if such termination were to occur after a 2019 Transition Event, then (a) with respect to the FA Program, if a 2019 FA Co-Co Agreement is in effect, Neurocrine can terminate the 2019 FA Co-Co Agreement for such program and the Company would no longer have co-development and co-commercialization rights with respect to the FA Collaboration Products and (b) subject to any license agreements, Neurocrine would no longer have any obligations with respect to any 2019 Collaboration Products resulting from such program.

Termination of VY-AADC Program

As described above, as of the Neurocrine VY-AADC Program Termination Effective Date, the license granted by the Company to Neurocrine thereunder regarding the VY-AADC Program expired, the Company regained worldwide intellectual property rights regarding the VY-AADC Program, and the restrictions on the Company to develop, manufacture or commercialize a gene therapy product directed to the target of the VY-AADC Program terminated, in each case in accordance with the terms of the 2019 Neurocrine Collaboration Agreement. As of the Neurocrine VY-AADC Program Termination Effective Date, Neurocrine no longer is obligated to reimburse the Company for research and development activities related to the VY-AADC Program, and the Company is no longer entitled to receive future milestone or royalty payments related to the VY-AADC Program. The Company is supporting Neurocrine, the study sponsor and IND holder, should there be any ongoing or future matters regarding the program.

Accounting Analysis

At inception, the Company determined the 2019 Neurocrine Collaboration Agreement was a contract with a customer under ASC 606, and included the following performance obligations: (a) research and development services for each Legacy Program combined with a development and commercialization license for each such program and (b) research and development services for each 2019 Discovery Program combined with a development and commercialization license for each program. The research services and license on a program-by-program basis are not distinct as Neurocrine cannot benefit from such license on its own or from other resources commonly available in the industry, without the corresponding research services due to the unique and specialized expertise of the Company that is not readily available in the marketplace.

The Company identified $ 92.4 million of fixed transaction price consisting of the $ 115.0 million upfront fee and $ 5.0 million payment from the June 2019 Modification, offset by a discount of $ 27.6 million related to the $ 50.0 million equity investment of 4,179,728 shares when measured at fair value on the date of issuance. The Company is also entitled to reimbursement of costs incurred by the Company prior to the 2019 Transition Events associated with each 2019 Neurocrine Program. These amounts are determinable based on program plans and budgets, and the Company has a contractual right to the payment of cost incurred under the agreed upon program plans. The Company utilized the most likely amount approach and estimated the expected cost reimbursement to be $ 431.1 million at inception. The Company concluded that these amounts do not require a constraint and are included in the transaction price at inception. The Company considers this estimate at each reporting date and updates the estimate based on information available. During the fourth quarter of 2021, the Company revised the estimate of the expected reimbursement to approximately $ 80.0 million based on expectations as a result from decisions made at the JSC meeting held in the fourth quarter of 2021, which resulted in significantly less research and development services to be provided by the Company under the 2019

Neurocrine Collaboration Agreement. During the fourth quarter of 2022, the Company further revised the estimate of the expected reimbursement to approximately $ 81.7 million, based on expectations resulting from decisions made at the JSC meeting held in the fourth quarter of 2022. During the fourth quarter of 2023, the Company further revised the estimate of the expected reimbursement to approximately $ 83.3 million, based on expectations resulting from decisions made at the JSC meeting held in the fourth quarter of 2023. Additional consideration to be paid to the Company upon reaching certain milestones are excluded from the transaction price at inception due to the uncertainty of achieving the development and regulatory milestones.

The Company allocated the fixed transaction price to the separate performance obligations based on the relative standalone selling price of each performance obligation or in the case of certain variable consideration to one or more performance obligations. The estimated standalone selling prices for performance obligations, which include a license and research services, were developed using the estimated selling price of the license, using comparable and market data, and an estimate of the overall effort to perform the research services along with a reasonable profit for research services.

The total variable consideration allocated to each program related to the expected cost reimbursement as of December 31, 2023 was as follows:

Performance Obligation	Amount
	(in thousands)
Variable Consideration	
VY-AADC Program	$ 53,863
FA Program	20,309
2019 Discovery Program 1	4,286
2019 Discovery Program 2	4,793
Total	$ 83,251

Based on the relative standalone selling price allocation, the allocation of the transaction price, exclusive of the variable consideration allocated to the individual performance obligations, to the separate performance obligations was as follows:

Performance Obligation	Amount
	(in thousands)
Fixed Consideration	
VY-AADC Program	$ 49,045
FA Program	20,647
2019 Discovery Program 1	14,443
2019 Discovery Program 2	8,247
Total	$ 92,382

The Company recognizes the transaction price associated with each performance obligation on a proportional performance basis over the period of service using input-based measurements such as costs incurred to date, to estimate proportion performed, and remeasures its progress towards completion at the end of each reporting period.

The Company determined the partial termination of the 2019 Neurocrine Collaboration Agreement with respect to the VY-AADC Program represented a modification of the arrangement under ASC 606 and that the remaining fixed transaction price at the Neurocrine VY-AADC Program Termination Effective Date of $ 42.2 million should be re-allocated to the FA Program and 2019 Discovery Program 1 and 2 based on their standalone selling prices. Accordingly, the Company recorded a cumulative adjustment to revenue of approximately $ 0.9 million on the partially satisfied remaining performance obligations, as the remaining services to be performed under each of the performance obligations are not distinct from the services prior to the modification. The Company determined that reasonable changes to the Company's estimates of standalone selling prices for the FA Program, 2019 Discovery Program 1 and 2019 Discovery Program 2 performance obligations did not have a material impact on the re-allocation or the amount of revenue recorded pursuant to the cumulative catch-up adjustment.

During the year ended December 31, 2023 and 2022, the Company recognized $ 9.8 million and $ 0.9 million of revenue, respectively, associated with its collaboration with Neurocrine related to fixed transaction price allocated to the three active programs, and research and development services performed during the period and the corresponding cost reimbursement receivable. As of December 31, 2023, there was $ 6.1 million of deferred revenue related to the 2019 Neurocrine Collaboration Agreement, which is classified as either current or non-current in the accompanying consolidated balance sheet based on the period the services are expected to be delivered. Additionally, as of December 31, 2023, there was $ 1.6 million of collaboration receivable related to reimbursable costs expected to be received from Neurocrine for research and development services performed.

Costs incurred relating to the Company's collaboration programs under the 2019 Neurocrine Collaboration Agreement consist of internal and external research and development costs, which primarily include: salaries and benefits, laboratory supplies, preclinical research studies, clinical studies, consulting services, and commercial development. These costs are included in research and development expenses in the Company's consolidated statements of operations.

The Company incurred approximately $ 0.8 million of costs to obtain the 2019 Neurocrine Collaboration Agreement which were payable only upon the close of the deal and therefore considered incremental costs of obtaining a contract with a customer and capitalized. The costs are recorded in prepaid expenses and other non-current assets and are being amortized over the period in which the research services will be provided.

The following table presents changes in the balances of the Company's related party collaboration receivable and contract liabilities for both the 2023 Neurocrine Collaboration Agreement and the 2019 Neurocrine Collaboration Agreement during the year ended December 31, 2023:

	Balance at December 31, 2022	Additions	Deductions	Balance at December 31, 2023
		(in thousands)		
Related party collaboration receivable	$ 257	$ 10,081	$ (6,997)	$ 3,341
Contract liabilities:				
Deferred revenue	$ 11,827	$ 74,420	$ (11,007)	$ 75,240

The change in the related party collaboration receivable balance for the year ended December 31, 2023 is primarily driven by amounts owed to the Company for research and development services provided, offset by amounts collected from Neurocrine during the period, for both the 2023 Neurocrine Collaboration Agreement and the 2019 Neurocrine Collaboration Agreement. Deferred revenue activity for the year ended December 31, 2023 includes the recording of $ 74.4 million of deferred revenue during the first quarter of 2023 related to the fixed transaction price allocated to each of the three 2023 Discovery Program performance obligations under the 2023 Neurocrine Collaboration Agreement, offset by $ 11.0 million of collaboration revenue recognized on the proportional performance model during the year ended December 31, 2023 for both the 2023 Neurocrine Collaboration Agreement and the 2019 Neurocrine Collaboration Agreement.

Alexion Option and License Agreement (Formerly Pfizer Option and License Agreement)

Summary of Agreement

On October 1, 2021, the Company entered into an option and license agreement (the "Pfizer Agreement") with Pfizer, Inc. ("Pfizer") pursuant to which the Company granted Pfizer options to receive an exclusive license (the "Pfizer License Options") to certain TRACER Capsids to develop and commercialize certain AAV gene therapy candidates comprised of a capsid and specified Pfizer transgenes (the "Pfizer Transgenes"). Under the terms of the Pfizer Agreement, during an initial research term that ended as of October 1, 2022 (the "Pfizer Research Term"), Pfizer had the right to evaluate the potential use of the capsids in combination with up to two Pfizer Transgenes to help treat respective CNS and cardiovascular diseases.

During the Pfizer Research Term, the Company agreed to provide Pfizer with certain quantities of materials encoding specified existing capsids for Pfizer's evaluation. Further, during the Pfizer Research Term, the Company

agreed to disclose to Pfizer, on a rolling basis, the performance characteristics identified during the Pfizer Research Term for all such capsid candidates. Pfizer had the right, in its sole discretion, to select any capsid candidate for evaluation to determine its interest in exercising a Pfizer License Option with respect to such capsid candidate. Pfizer had the right to exercise up to two Pfizer License Options, provided that it could exercise only one Pfizer License Option for each Pfizer Transgene.

Effective as of September 30, 2022, Pfizer exercised its Pfizer License Option with respect to a capsid for the specified Pfizer Transgene for potential treatment of a rare neurological disease. Pfizer did not exercise its option to license a capsid for the potential treatment of a cardiovascular disease. As result, Pfizer's right to exercise a Pfizer License Option for a cardiovascular disease has terminated in accordance with the terms of the Pfizer Agreement and all rights to capsids for that cardiovascular disease have reverted to the Company. Pfizer's exercise of a Pfizer License Option extended the Pfizer Research Term to October 1, 2024, during which period the Company may, at its sole discretion and expense, conduct additional research activities to identify additional proprietary capsids that may be useful for AAV gene therapies for the treatment of the rare neurological disease associated with the exercise of the applicable Pfizer License Option.

Pursuant to the exercise of the Pfizer License Option, the Company granted Pfizer an exclusive, worldwide license, with the right to sublicense, under certain of the Company's intellectual property, the rights to develop and commercialize rare neurological disease products utilizing the capsid candidate and incorporating the corresponding Pfizer Transgene (the "Pfizer Licensed CNS Products").

On July 28, 2023, Alexion entered into a definitive purchase and license agreement for preclinical gene therapy assets and enabling technologies from Pfizer. Effective upon the closing of the transaction on September 20, 2023, Alexion acquired all of Pfizer's rights under the Pfizer Agreement (now the "Alexion Agreement") and became the successor-in-interest to Pfizer thereunder. The acquisition does not impact the material terms of the option and license agreement. Until October 1, 2024, while the Company is not obligated to conduct additional research activities to identify additional proprietary capsids that may be useful for AAV gene therapies for the treatment of rare neurological diseases, it has agreed to continue to disclose to Alexion, on a rolling basis, the performance characteristics identified for all such capsid candidates, if and when available. Alexion may, during the Pfizer Research Term (now the "Alexion Research Term"), conduct additional evaluations of such capsid candidates and has the right to substitute any other capsid candidate for the capsid Pfizer elected to license when it exercised the Pfizer License Option.

Under the Alexion Agreement, Alexion is solely responsible for, and has sole decision-making authority with respect to, development and commercialization of the Pfizer Licensed CNS Products (now the "Alexion Licensed Products"). Alexion is required to use commercially reasonable efforts to develop and obtain regulatory approval for at least one Alexion Licensed CNS Product for which Pfizer exercised its Pfizer License Option in (a) the United States and (b) at least one of the following countries: the United Kingdom, France, Germany, Italy, Spain and Japan (each of which is referred to as an "Alexion Major Market Country"), subject to certain limitations. Alexion is also required to use commercially reasonable efforts to commercialize each Alexion Licensed CNS Product in the United States and at least one Alexion Major Market Country where Alexion or its designated affiliates or sublicensees has received regulatory approval for such Alexion Licensed CNS Product, subject to certain limitations.

Under the terms of the Alexion Agreement, Pfizer paid the Company an upfront payment of $ 30.0 million in October 2021. Following the exercise of the Pfizer License Option, Pfizer paid the Company a fee of $ 10.0 million. The Company is also eligible to receive specified development, regulatory, and commercialization milestone payments of up to an aggregate of $ 115.0 million for the first corresponding Alexion Licensed CNS Product to achieve the corresponding milestone. On an Alexion Licensed CNS Product-by-Alexion Licensed CNS Product basis, the Company is also eligible to receive (a) specified sales milestone payments of up to an aggregate of $ 175.0 million per Alexion Licensed CNS Product and (b) tiered, escalating royalties in the mid- to high-single-digit percentages of annual net sales of each Alexion Licensed CNS Product. The royalties are subject to potential reductions in customary circumstances including patent claim expiration, payments for certain third-party licenses, and biosimilar market penetration, subject to specified limits.

Under the terms of the Alexion Agreement, each of the Company and Alexion owns the entire right, title, and interest in and to all patents or know-how controlled by such party and existing as of or before the effective date of the

Alexion Agreement, or invented, developed, created, generated or acquired solely by or on behalf of such party after such effective date.

Subject to certain specified exceptions, any patents and know-how that are invented or otherwise developed jointly by or on behalf of the parties during the term of the Alexion Agreement and in the course of the Company's and Alexion's activities under the Alexion Agreement will follow inventorship under U.S. patent law. Subject to certain limitations and exceptions, the Company agreed (a) during the Alexion Research Term, not to conduct any internal program or program on behalf of a third party that is directed to development or commercialization of any capsid candidates, or grant any third party or affiliate any right or license under the Company's rights in such capsid candidates to exploit any therapeutic product, in combination with any Pfizer Transgene (now an "Alexion Transgene") in any indication for therapeutic, diagnostic and prophylactic human and veterinary use; and (b) not to grant any third party or affiliate any right or license under the Company's patents to exploit any licensed capsid in combination with any Alexion Transgene.

Unless earlier terminated, the Alexion Agreement expires on the expiration of the last-to-expire royalty term with respect to all Alexion Licensed CNS Products in all countries. Subject to a cure period, either party may terminate the Alexion Agreement, in whole or in part, subject to specified conditions, in the event of the other party's uncured material breach. Alexion may also terminate the Alexion Agreement, in whole or in part, subject to specified conditions, for the Company's insolvency, the occurrence of a violation of global trade control laws, or for the Company's noncompliance with certain anti-bribery or anti-corruption covenants. Alexion may also terminate the Alexion Agreement, in whole or in part, for any or no reason upon ninety days ' written notice to the Company.

Upon certain terminations for cause by Alexion, the license that the Company has granted to Alexion under the Alexion Agreement shall become irrevocable and perpetual, and all milestone payments and royalties that would have otherwise been payable by Alexion under such license had the Alexion Agreement remained in effect would be substantially reduced.

Accounting Analysis

At inception, the Company determined the Alexion Agreement was a contract with a customer under ASC 606. The Company assessed the promised goods and services under the Alexion Agreement, in accordance with ASC 606, and determined that the Alexion Agreement contains two performance obligations consisting of two material rights, one for each of the Pfizer License Options. The Company concluded that each Pfizer License Option provided a material right as consideration for each option is less than the amount that the Company would otherwise have expected to receive outside the context of the contract. The promises at inception do not include the underlying goods or services that would be delivered upon exercise of the option, but rather represent the value to the customer of having the right to exercise the Pfizer License Option at the specified exercise fee. Upon the exercise of a Pfizer License Option, until October 1, 2024, while the Company is not obligated to conduct additional research activities upon option exercise to identify additional proprietary capsids that may be useful for AAV gene therapies for the treatment of central nervous system or cardiovascular diseases, it has agreed to continue to disclose to Alexion, on a rolling basis, the performance characteristics identified for all such capsid candidates, if and when available. Alexion may, conduct additional evaluations of such capsid candidates and has the right to substitute any other capsid candidate for the capsid Pfizer elected to license when it exercised the Pfizer License Option. The Company determined that this promise to provide Alexion the ability to evaluate and potentially substitute other capsid candidates for the capsid Pfizer elected to license when it exercised the Pfizer License Option, if and when available, is an additional performance obligation in the arrangement (the "Alexion Substitution Right Performance Obligation").

The Company received a nonrefundable, upfront payment of $ 30.0 million as consideration under the Alexion Agreement, which represented the transaction price at inception. Additional consideration to be paid to the Company upon exercise of the Pfizer License Option or upon reaching certain milestones are excluded from the transaction price as they relate to option fees and milestones that could only be achieved subsequent to an option exercise.

The Company allocated the transaction price to the Pfizer License Options based on their relative standalone selling prices. The estimated standalone selling price for each material right was based on an adjusted market assessment approach. The Company concluded that the market would be willing to pay an equal amount for each Pfizer License

Option on a standalone basis. The Company reached this conclusion after considering (a) the downstream economics including option fees, milestones and royalties related to each Pfizer License Option being identical and (b) comparable market data. The Company determined the standalone selling price for the Alexion Substitution Right Performance Obligation was insignificant to the allocation of the transaction price using the relative standalone selling price model and, accordingly, did not allocate any transaction price to the Alexion Substitution Right Performance Obligation. This determination was supported by qualitative and quantitative assessments of the standalone selling price that considered the cost of identifying other potential capsid candidates and the likelihood of license substitution. As such, based on the relative standalone selling price for each of the two material rights, the allocation of the transaction price to the separate performance obligations was $ 15.0 million for each material right. The amount allocated to each material right was initially recorded as deferred revenue.

During the year ended December 31, 2022, the Company recognized $ 40.0 million in collaboration revenue related to the Alexion Agreement. No revenue was recognized under the Alexion Agreement for the year ended December 31, 2023.

License Agreement with Sangamo

On June 28, 2023, the Company entered into a definitive license agreement for a potential treatment of prion disease with Sangamo. Using their proprietary epigenetic regulation platform, Sangamo has developed zinc finger transcriptional regulators which they believe can specifically and potently block expression of the prion protein, the pathogenic driver of prion disease. The Company is eligible to earn certain license fees, royalties on potential commercial sales of any products using Voyager's capsid, and, in the event the prion program is out licensed by Sangamo, a portion of all licensing revenues received with respect to this program. During the year ended December 31, 2023 the Company recognized $ 0.4 million of collaboration revenue related to the license agreement with Sangamo.

License Agreement with Touchlight IP Limited

On November 3, 2022, the Company and Touchlight IP Limited ("Touchlight") entered into a license agreement (the "Touchlight License Agreement") to authorize historical use by the Company of a certain DNA preparation process ("Subject DNA Preparation Process"), and to authorize the prospective exploitation of TRACER Capsids created with the use of the Subject DNA Preparation Process.

The terms of the Touchlight License Agreement included a one-time, non-refundable technology access fee of $ 5.0 million, which was paid during the fourth quarter of 2022. The Company recorded the $ 5.0 million to research and development expense in the year ended December 31, 2022, accordingly.

The terms of the Touchlight License Agreement also include future milestone payments and low single-digit royalties payable to Touchlight if the Company or its program collaborators or licensees choose to utilize in a therapeutic product TRACER Capsids that were created with the historical use of the Subject DNA Preparation Process. Additionally, the Company is obligated to pay low single-digit royalties to Touchlight on future payments the Company receives in connection with licensing of TRACER Capsids that were created with the historical use of the Subject DNA Preparation Process, excluding the licensing of or collaboration on any Company therapeutic programs.

During the year ended December 31, 2023, the Company recorded $ 0.5 million in research and development expense associated with amounts due to Touchlight in conjunction with the 2023 Novartis Collaboration Agreement.

10. Common stock

As of December 31, 2023 and 2022, the Company had authorized 120,000,000 shares of common stock, at $ 0.001 par value per share.

General

The voting, dividend and liquidation rights of the holders of the common stock are subject to and qualified by the rights, powers and preferences of the holders of preferred stock. The common stock has the following characteristics:

Liquidation

The holders of shares of common stock are entitled to share ratably in the Company's remaining assets available for distribution to its stockholders in the event of any voluntary or involuntary liquidation, dissolution or winding up of the Company or upon occurrence of a deemed liquidation event.

Shares Reserved For Future Issuance

	As of December 31,	
	2023	2022
Shares reserved for vesting of restricted stock awards under the Founder Agreements	22,500	45,000
Shares reserved for exercise of outstanding stock options	7,425,444	6,199,571
Shares reserved for vesting of outstanding restricted stock units	1,370,897	1,112,563
Shares reserved for issuances under the 2015 Stock Option Plan	3,572,195	3,536,932
Shares reserved for issuances under the 2015 Employee Stock Purchase Plan	2,158,966	1,884,309
	14,550,002	12,778,375

11. Stock-based compensation

2014 Stock Option and Grant Plan

In January 2014, the Company adopted the 2014 Stock Option and Grant Plan (the "2014 Plan"), under which it could grant incentive stock options, non-qualified stock options, restricted stock awards, unrestricted stock awards, or restricted stock units to purchase up to 823,529 shares of common stock to employees, officers, directors and consultants of the Company.

The terms of stock option agreements, including vesting requirements, were determined by the board of directors and were subject to the provisions of the 2014 Plan. Restricted stock awards granted by the Company generally vest based on each grantee's continued service with the Company during a specified period following grant. Stock options granted to employees generally vest over four years , with 25 % vesting on the one year anniversary and 75 % vesting ratably, on a monthly basis, over the remaining three years . Stock options granted to non-employee consultants generally vest monthly over a period of one to four years .

Founder Awards

In January 2014, the Company issued 1,188,233 shares of restricted stock to its founders (each, a "Founder") at an original issuance price of $ 0.0425 per share. Of the total restricted shares awarded to the Founders, 835,292 shares were slated to vest over one to four years , based on each Founder's continued service to the Company in varying capacity as a Scientific Advisory Board member, consultant, director, officer or employee, as set forth in each grantee's individual restricted stock purchase agreement.

The remainder of the restricted stock awards were slated to vest upon the achievement of certain performance objectives as well as continued service to the Company, as set forth in the agreements. Stock-based compensation

expense associated with these performance-based awards is recognized when the achievement of the performance condition is considered probable, using management's best estimates. The Company has modified certain of the awards, including repurchasing a total of 131,470 shares underlying the awards through December 31, 2023, and modifying the vesting provisions such that the modified awards vest over time rather than based on performance. The stock-based compensation expense recorded related to these awards during the years ended December 31, 2023, 2022, and 2021 were immaterial to the Company's consolidated financial statements.

2015 Stock Option Plan

In October 2015, the Company's board of directors and stockholders approved the 2015 Stock Option and Incentive Plan ("2015 Stock Option Plan"), which became effective upon the completion of the Company's initial public offering ("IPO"). The 2015 Stock Option Plan provides the Company with the flexibility to use various equity-based incentive and other awards as compensation tools to motivate its workforce. These tools include stock options, stock appreciation rights, restricted stock, restricted stock units, unrestricted stock, performance share awards and cash-based awards. The 2015 Stock Option Plan replaced the 2014 Plan. Any options or awards outstanding under the 2014 Plan remained outstanding and effective. The number of shares initially reserved for issuance under the 2015 Stock Option Plan is the sum of (a) 1,311,812 shares of common stock and (b) the number of shares under the 2014 Plan that are not needed to fulfill the Company's obligations for awards issued under the 2014 Plan as a result of forfeiture, expiration, cancellation, termination or net issuances of awards thereunder. The number of shares of common stock that may be issued under the 2015 Stock Option Plan is also subject to increase on the first day of each fiscal year by up to 4 % of the Company's issued and outstanding shares of common stock on the immediately preceding December 31.

Effective January 1, 2016 and every anniversary thereafter an additional 4 % of outstanding common stock was added to the Company's 2015 Stock Option Plan pursuant to its "evergreen" provision, for future issuance. This has accumulated to a total of 12,531,505 shares through January 1, 2024. During the year ended December 31, 2023, the Company granted options to purchase 1,753,800 shares of common stock to employees and directors under the 2015 Stock Option Plan. As of December 31, 2023, there were 3,572,195 shares available for future issuance under the 2015 Stock Option Plan.

2015 Employee Stock Purchase Plan

In October 2015, the Company's board of directors and stockholders approved the 2015 Employee Stock Purchase Plan (the "2015 ESPP"). Under the 2015 ESPP, all full-time employees of the Company are eligible to purchase common stock of the Company twice per year, at the end of each six-month payment period. During each payment period, eligible employees who so elect, may authorize payroll deductions in an amount of 1 % to 10 % (whole percentages only) of the employee's base pay for each payroll period. At the end of each payment period, the accumulated deductions are used to purchase shares of common stock from the Company at a discount. A total of 262,362 shares of common stock were initially authorized for issuance under this plan.

Effective January 1, 2016 and every anniversary thereafter an additional 1 % of outstanding common stock was added to the 2015 ESPP, pursuant to its evergreen provision, for future issuance. This has accumulated to a total of 2,692,838 shares through January 1, 2023. The Company issued 111,639 and 150,265 shares of common stock under the 2015 ESPP in the years ended December 31, 2023 and 2022. As of December 31, 2023, there were 2,158,966 shares available for future purchase under the 2015 ESPP.

Inducement Awards

In the years ended December 31, 2023, 2022 and, 2021, the Company issued non-statutory stock options to purchase an aggregate of 573,000 , 390,000 , and 76,500 shares of the Company's common stock and restricted stock unit awards for an aggregate of 318,500 , 163,000 , and 13,000 shares of the Company's common stock, respectively, in each case outside of the Company's 2015 Stock Option Plan as an inducement material to certain individuals' acceptance of an offer of employment with the Company in accordance with Nasdaq Listing Rule 5635(c)(4).

The stock options will vest over a four-year period, with 25 % of the shares underlying the option award vesting on the first anniversary of the award and the remaining 75 % of the shares underlying the award vesting monthly thereafter over the subsequent 36 -month period. The restricted stock units vest over a three-year period, with 33 % of the restricted stock units vesting on the first anniversary of the award, 33 % of the restricted stock units vesting on the second anniversary, and the remaining restricted stock units vesting on the third anniversary.

Stock-based Compensation Expense

Total compensation cost recognized for all stock-based compensation awards in the statements of operations and comprehensive income (loss) is as follows:

	Year ended December 31,		
	2023	2022	2021
		(in thousands)	
General and administrative	$ 7,568	$ 6,398	$ 7,191
Research and development	3,585	2,946	4,133
Total stock-based compensation expense	$ 11,153	$ 9,344	$ 11,324

Stock-based compensation expense by type of award included within the consolidated statements of operations and comprehensive income (loss) was as follows:

	Year ended December 31,		
	2023	2022	2021
		(in thousands)	
Stock options	$ 7,627	$ 5,938	$ 7,438
Restricted stock awards and units	3,241	3,215	3,551
Employee stock purchase plan awards	285	191	335
Total stock-based compensation expense	$ 11,153	$ 9,344	$ 11,324

Restricted Stock Units

A summary of the status of and changes in unvested restricted stock unit activity under the Company's equity award plans for the year ended December 31, 2023 was as follows:

	Units	Weighted Average Grant Date Fair Value Per Unit
Unvested restricted stock units as of December 31, 2022	1,112,563	$ 5.27
Awarded	999,250	$ 7.58
Vested	(531,560)	$ 5.73
Forfeited	(209,356)	$ 6.08
Unvested restricted stock units as of December 31, 2023	1,370,897	$ 6.65

Stock-based compensation of restricted stock units is based on the fair value of the Company's common stock on the date of grant and is recognized over the vesting period. In the year ended December 31, 2023, the Company granted 999,250 restricted stock units vesting in equal amounts, annually over three years . The stock-based compensation expense was $ 3.1 million, $ 2.9 million, and $ 3.3 million for the years ended December 31, 2023, 2022, and 2021, respectively.

As of December 31, 2023, the Company had unrecognized stock-based compensation expense related to its unvested restricted stock units of $ 6.8 million which is expected to be recognized over the remaining weighted average vesting period of 1.1 years.

Stock Options

A summary of the status of, and changes in, stock options was as follows:

	Shares	Weighted Average Exercise Price	Remaining Contractual Life (in years)	Aggregate Intrinsic Value (in thousands)
Outstanding at December 31, 2022	6,199,571	$ 8.12		
Granted	2,326,800	$ 8.58		
Exercised	(385,655)	$ 4.98		
Cancelled or forfeited	(715,272)	$ 7.86		
Outstanding at December 31, 2023	7,425,444	$ 8.52	7.3	11,293
Exercisable at December 31, 2023	3,823,420	$ 9.43	5.9	$ 6,020

Using the Black-Scholes option pricing model, the weighted average fair value of options granted during the year ended December 31, 2023 was $ 6.12 . The stock-based compensation expense related to stock option awards granted was $ 7.2 million, $ 5.8 million, and $ 7.3 million for the years ended December 31, 2023, 2022, and 2021, respectively.

The fair value of each option was estimated at the date of grant using the Black-Scholes option pricing model with the following weighted-average assumptions:

	Year ended December 31,		
	2023	2022	2021
Risk-free interest rate	4.0 %	2.2 %	0.9 %
Expected dividend yield	— %	— %	— %
Expected term (in years)	6.0	6.0	6.0
Expected volatility	80.4 %	79.4 %	75.0 %

As of December 31, 2023, the Company had unrecognized stock-based compensation expense related to its unvested stock options of $ 16.6 million which is expected to be recognized over the remaining weighted average vesting period of 2.9 years.

12. 401(k) Savings plan

The Company has a defined-contribution savings plan under Section 401(k) of the Internal Revenue Code (the “401(k) Plan”). The 401(k) Plan covers all employees who meet defined minimum age and service requirements, and allows participants to defer a portion of their annual compensation on a pretax basis. The Company expensed approximately $ 1.1 million, $ 0.9 million, and $ 1.1 million related to employer contributions made during the years ended December 31, 2023, 2022, and 2021, respectively.

13. Income taxes

The Company evaluates its tax positions on an annual basis. The current tax expense recorded for the year ended December 31, 2023 is the residual tax due, after utilization of tax attributes, associated with revenue from collaboration agreements. The provision for incomes taxes is as follows:

	Year ended December 31,		
	2023	2022	2021
	(in thousands)		
Current			
Federal	$ 1,248	$ —	$ —
State	160	16	—
Total current	1,408	16	—
Deferred			
Federal	—	—	—
State	—	—	—
Total deferred	—	—	—
Total tax provision	$ 1,408	$ 16	$ —

A reconciliation of the expected income tax provision computed using the federal statutory income tax rate at the Company's effective tax rate for the years ended December 31, 2023, 2022, and 2021 is as follows:

	Year ended December 31,		
	2023	2022	2021
Income tax computed at federal statutory tax rate	21.0 %	21.0 %	21.0 %
State taxes, net of federal benefit	5.4 %	5.2 %	6.6 %
Provision to return	0.9 %	3.2 %	4.9 %
General business credit carryovers	(0.4)%	(3.5)%	3.2 %
Non-deductible expenses	0.5 %	(4.6)%	(3.8)%
Other	(0.4)%	— %	— %
Change in valuation allowance	(26.0)%	(21.3)%	(31.9)%
Total	1.00 %	— %	— %

The Company has historically incurred net operating losses ("NOLs"). As of December 31, 2023, the Company had federal and state net operating loss carryforwards of $ 55.3 million and $ 33.2 million, respectively. As of December 31, 2023, the Company had federal and state research and development tax credit carryforwards of $ 19.6 million and $ 10.8 million, respectively, which expire beginning in 2033. As of December 31, 2022, the Company had state investment credits of $ 0.4 million, which expire beginning in 2024.

The significant components of the Company's deferred tax assets and (liabilities) as of December 31, 2023 and 2022 are as follows:

	As of December 31,	
	2023	2022
	(in thousands)	
Deferred tax assets:		
Net operating loss carryforward	$ 13,724	$ 47,282
Tax credit carryforward	28,427	32,060
Lease liability	5,544	6,318
Deferred revenue	1,675	17,984
Stock compensation	5,157	4,630
Non-deductible accruals and reserves	2,613	1,603
Capitalized research expenses	31,347	14,351
Intangibles	554	610
Other temporary differences	—	(1)
Total deferred tax assets	89,041	124,837
Less valuation allowance	(82,612)	(117,416)
Net deferred tax assets	6,429	7,421
Deferred tax liabilities		
Right of use assets	(3,691)	(4,231)
Depreciation and amortization	(2,738)	(3,190)
Other temporary differences	—	—
Net deferred taxes	$ —	$ —

As required by ASC 740, management has evaluated the positive and negative evidence bearing upon the realizability of its deferred tax assets, which principally comprise NOL carryforwards, tax credit carryforwards, and capitalized research expenses. Management has determined that it is more likely than not that the Company will not recognize the benefits of its federal and state deferred tax assets, and as a result, a valuation allowance of $ 82.6 million and $ 117.4 million has been established at December 31, 2023 and 2022, respectively. The state NOLs will expire beginning in 2041 while the federal NOLs do not expire. The valuation allowance decreased by $ 34.8 million for the year ended December 31, 2023 primarily as a result of the utilization of the NOL carryforwards and tax credits in 2023.

At December 31, 2023 and 2022, the Company had no unrecognized tax benefits. The Company is in the process of completing a study of its research and development credit carryforwards. The Company has recorded an adjustment in the current year to reflect the preliminary results of the research and development study. The completion of the study may result in an additional adjustment to the Company's research and development credit carryforwards; however, until a study is completed, no amounts are being presented as an uncertain tax position. A full valuation allowance has been provided against the Company's research and development credits, and if an adjustment is required, this adjustment would be offset by an adjustment to the valuation allowance. Thus, there would be no impact to the balance sheets or statements of operations and comprehensive income (loss) if an adjustment were required.

Interest and penalty charges, if any, related to unrecognized tax benefits would be classified as income tax expense in the accompanying statements of operations. As of December 31, 2023 and 2022, the Company has no accrued interest related to uncertain tax positions. Since the Company is in a loss carryforward position, it is generally subject to examination by the U.S. federal, state, and local income tax authorities for all tax years in which a loss carryforward is available.

14. Related-party transactions

During the years ended December 31, 2023, 2022, and 2021, the Company received scientific advisory services from one of its prior executives, Dinah Sah, Ph.D., the Company's former Chief Scientific Officer. The total amount of fees paid to Dr. Sah for services provided during the years ended December 31, 2023, 2022 and 2021 was $ 0.7 million, $ 0.5 million, and $ 0.2 million, respectively.

During the year ended December 31, 2022, the Company received advisory services related to strategic planning, operations, and management from Alfred Sandrock, M.D., Ph.D., the Company's current President and Chief Executive Officer and a member of the Company's board of directors, before he commenced service in the capacity of President and Chief Executive Officer in March 2022. The total amount of fees paid to Dr. Sandrock for services provided was $ 60,000 for the year ended December 31, 2022.

Under both the 2019 Neurocrine Collaboration Agreement and the 2023 Neurocrine Collaboration Agreement, the Company and Neurocrine have agreed to conduct research, development and commercialization activities for certain of the Company's AAV gene therapy products (Note 9). Amounts due from Neurocrine are reflected as related party collaboration receivable. As of December 31, 2023, the Company recorded approximately $ 3.3 million in related party collaboration receivable relative to the 2019 Neurocrine Collaboration Agreement and the 2023 Neurocrine Collaboration Agreement.

15. Subsequent events

On January 4, 2024, the Company entered into an underwriting agreement (the "Underwriting Agreement") with Citigroup Global Markets Inc. and Guggenheim Securities, LLC, as representatives of the several underwriters named therein (the "Underwriters"), relating to an underwritten public offering of 7,777,778 shares of the Company's common stock, par value $ 0.001 per share, and, in lieu of common stock to certain investors, pre-funded warrants (the "Pre-Funded Warrants") to purchase up to 3,333,333 shares of common stock. The public offering price of the Company's common stock was $ 9.00 per share, and the public offering price of the Pre-Funded Warrants was $ 8.999 per share underlying each Pre-Funded Warrant. The Underwriters have agreed to purchase the Company's stock from the Company pursuant to the Underwriting Agreement at a price of $ 8.46 and the Pre-Funded Warrants from the Company pursuant to the Underwriting Agreement at a price of $ 8.459 per share underlying each Pre-Funded Warrant. Under the terms of the Underwriting Agreement, the Company also granted the Underwriters an option, exercisable for 30 days , to purchase up to an additional 1,666,665 shares of common stock at the public offering price less the underwriting discounts and commissions. This option was not exercised during the thirty-day exercise period, and the option expired on February 2, 2024.

The Company received net proceeds from the public offering of approximately $ 93.5 million after deducting underwriting discounts and commissions and estimated offering expenses.

The Company and Novartis entered into a stock purchase agreement ("the 2023 Novartis Stock Purchase Agreement") on December 28, 2023, for the sale and issuance of 2,145,002 shares of the Company's common stock (the "Novartis Shares") to Novartis at a price of $ 9.324 per share, for an aggregate purchase price of approximately $ 20.0 million. In accordance with the terms and conditions of the 2023 Novartis Stock Purchase Agreement, the Company issued and sold the Novartis Shares to Novartis on January 3, 2024.

In February 2024, the Company announced that the joint steering committee with its collaborator Neurocrine selected a lead development candidate for the FA Program, which triggered a $ 5.0 million milestone payment to the Company. The Company expects to receive the $ 5.0 million during the first quarter of 2024.

EXHIBIT INDEX

Exhibit No.	Description	Incorporated by Reference to: Form or Schedule	Exhibit No.	Filing Date with SEC	SEC File Number	Filed Herewith
3.1	Amended and Restated Certificate of Incorporation of the Registrant	8-K	3.1	11/16/2015	001-37625	
3.2	Amended and Restated By-Laws of the Registrant	8-K	3.2	11/16/2015	001-37625	
4.1	Specimen Common Stock Certificate of the Registrant	10-K	4.1	03/14/2018	001-37625	
4.2	Form of Pre-Funded Warrant	8-K	4.1	01/08/2024	001-37625	
4.4	Description of Registrant's Securities	10-K	4.4	03/03/2020	001-37625	
10.1#	2014 Stock Option and Grant Plan and forms of award agreements thereunder	S-1/A	10.1	10/28/2015	333-207367	
10.2#	2015 Stock Option and Incentive Plan and forms of award agreements thereunder	S-1/A	10.2	10/28/2015	333-207367	
10.3†	Collaboration Agreement, by and between the Registrant and Sanofi Genzyme Corporation, dated February 11, 2015	S-1/A	10.3	11/06/2015	333-207367	
10.4*	Termination Agreement, by and between the Registrant and Genzyme Corporation, dated June 14, 2019	10-Q	10.3	08/09/2019	001-37625	
10.5*	Amended and Restated Option and License Agreement, by and between the Registrant and Genzyme Corporation, dated June 14, 2019	10-Q	10.4	08/09/2019	001-37625	
10.6*	First Amendment to Amended and Restated Option and License Agreement with Genzyme Corporation, dated September 20, 2020	10-Q	10.1	11/09/2020	001-37625	
10.7†	Collaboration and License Agreement, by and between the Registrant and Neurocrine Biosciences, Inc., dated January 28, 2019	10-K	10.28	02/26/2019	001-37625	
10.8	Amendment No. 1 to the Collaboration and License Agreement, by and between the Registrant and Neurocrine Biosciences, Inc., dated June 14, 2019	10-Q	10.5	08/09/2019	001-37625	

10.09*	Option and License Agreement, by and between the Registrant and Pfizer Inc., dated October 1, 2021	10-Q	10.2	11/02/21	001-37625
10.10	Lease Agreement, by and between the Registrant and UP 64 Sidney Street, LLC, dated December 23, 2015	10-Q	10.6	05/12/2016	001-37625
10.11	First Amendment to the Lease Agreement, by and between the Registrant and UP 64 Sidney Street, LLC, dated June 1, 2018	8-K	10.2	06/05/2018	001-37625
10.12	Lease Agreement, by and between the Registrant and HCP/King 75 Hayden LLC, dated March 16, 2020	8-K	10.1	03/19/2020	001-37625
10.13	Form of Indemnification Agreement to be entered into between the Registrant and its directors	S-1/A	10.9	10/28/2015	333-207367
10.14	Form of Indemnification Agreement to be entered into between the Registrant and its executive officers	S-1/A	10.10	10/28/2015	333-207367
10.15#	2015 Employee Stock Purchase Plan	S-1/A	10.12	10/28/2015	333-207367
10.16#	Amendment No. 1 to the 2015 Employee Stock Purchase Plan	10-K	10.21	03/14/2018	001-37625
10.17#	Retirement Agreement, by and between the Registrant and Dinah Sah, Ph.D., dated May 20, 2019	8-K	10.1	05/21/2019	001-37625
10.18#	Employment Agreement, by and between the Registrant and Michael Higgins, dated May 19, 2021	8-K	10.2	05/19/2021	001-37625
10.19#	Employment Agreement, by and between the Registrant and Glenn Pierce, M.D., Ph.D., dated May 19, 2021	8-K	10.3	05/19/2021	001-37625
10.20#	Amendment No. 1 to Employment Agreement, by and between the Registrant and Glenn Pierce, dated June 7, 2021	8-K	10.1	06/08/2021	001-37625
10.21#	Employment Agreement, by and between the Registrant and Robert W. Hesslein, dated January 15, 2019	10-Q	10.5	05/07/2019	001-37625
10.22#	Amended and Restated Employment Agreement, by and between the Registrant and Robin Swartz, effective as of February 7, 2022	8-K	10.2	02/03/2022	001-37625

10.23#	Consulting Agreement, by and between the Registrant and Dinah Sah, Ph.D., dated June 28, 2019	10-Q	10.6	08/09/2019	001-37625
10.24#	Amendment No. 1 to the Consulting Agreement, by and between the Registrant and Dinah Sah, Ph.D., dated September 16, 2019	10-Q	10.2	11/06/2019	001-37625
10.25#	Consulting Agreement by and between the Registrant and Alfred Sandrock, effective as of February 7, 2022	8-K	10.1	02/03/2022	001-37625
10.26#	Form of Non-Qualified Stock Option Agreement for Inducement	10-K	10.27	02/26/2019	001-37625
10.27#	Form of Restricted Stock Unit Agreement for Inducement	10-K	10.33	02/26/2019	001-37625
10.28	Sales Agreement, by and between the Registrant and Cowen and Company, LLC, dated November 8, 2022	S-3	1.2	11/08/2022	333-268240
10.29*	Consulting Agreement by and between the Registrant and Alfred Sandrock, effective as of February 7, 2022	8-K	10.1	02/03/2022	001-37625
10.30*	Option and License Agreement by and between the Registrant and Novartis Pharma AG, dated March 4, 2022	10-K	10.36	03/07/2023	001-37625
10.31#	Employment Agreement, by and between the Registrant and Alfred Sandrock, M.D., Ph.D., effective as of March 22, 2022.	8-K	10.1	03/22/2022	001-37625
10.32#	Consulting Agreement by and between the Registrant and Glenn Pierce, M.D., Ph.D., effective as of June 6, 2022	8-K	10.1	06/07/2022	001-37625
10.33	Employment Agreement by and between the Registrant and Peter Pfreundschuh, effective as of September 7, 2022	8-K	10.1	09/07/2022	001-37625
10.34	Second Amended and Restated Employment Agreement by and between the Registrant and Todd Carter, Ph.D., effective as of September 7, 2022	8-K	10.2	09/07/2022	001-37625
10.35*	Patent and Know-How License between the Registrant and Touchlight IP Limited, dated as of November 3, 2022	10-K	10.43	03/07/2023	001-37625

10.36	Stock Purchase Agreement by and between the Registrant and Neurocrine Biosciences, Inc., dated as of January 8, 2023	10-K	10.44	03/07/2023	001-37625	
10.37*	Collaboration and License Agreement by and between the Registrant and Neurocrine Biosciences, Inc., dated as of January 8, 2023	10-K	10.45	03/07/2023	001-37625	
10.38	Amended and Restated Investor Agreement by and between the Registrant and Neurocrine Biosciences, Inc., dated as of January 8, 2023	10-K	10.46	03/07/2023	001-37625	
10.39#	Transition, Separation and Release of Claims Agreement, by and between the Company and Robert W. Hesslein, dated February 22, 2023.	8-K	10.1	02/23/2023	001-37625	
10.40	Consulting Agreement, by and between the Registrant and Robert W. Hesslein, dated April 28, 2023	10-Q	10.5	03/31/2023	001-37625	
10.41#	Employment Agreement by and between the Registrant and Jacquelyn Fahey Sandell, effective as of July 5, 2023	8-K	10.1	07/10/2023	001-37625	
10.42	First Amendment to Lease Agreement, by and between Registrant and LS 75 Hayden, LLC, dated August 11, 2023.	8-K	10.1	08/16/2023	001-37625	
10.43*	License and Collaboration Agreement by and between Registrant and Novartis Pharma AG, dated December 28, 2023					X
10.44	Stock Purchase Agreement by and between Registrant and Novartis Pharma AG, dated December 28, 2023					X
10.45	Investor Agreement by and between Registrant and Novartis Pharma AG, dated December 28, 2023					X
10.46	Amendment No. 2 to the Consulting Agreement, by and between the Registrant and Dinah Sah, Ph.D., dated June 27, 2022					X
10.47	Amendment No. 3 to the Consulting Agreement, by and between the Registrant and Dinah Sah, Ph.D., dated May 1, 2023					X
21.1	Subsidiaries of the Registrant.					X

23.1	Consent of Ernst & Young, Independent Registered Public Accounting Firm.	X
24.1	Power of Attorney (see signature page of this Annual Report on Form 10-K).	X
31.1	Certification of Principal Executive Officer pursuant to Exchange Act Rules 13a-14 or 15d-14.	X
31.2	Certification of Principal Financial Officer pursuant to Exchange Act Rules 13a-14 or 15d-14.	X
32.1+	Certifications of Principal Executive Officer and Principal Financial Officer pursuant to Exchange Act Rules 13a-14(b) or 15d-14(b) and 18 U.S.C. Section 1350.	X
97.1	Compensation Recovery Policy	X
101.INS	XBRL Instance Document - the instance document does not appear in the Interactive Data File because its XBRL tags are embedded within the Inline XBRL document.	X
101.SCH	Inline XBRL Taxonomy Extension Schema Document.	X
101.CAL	Inline XBRL Taxonomy Extension Calculation Document.	X
101.LAB	Inline XBRL Taxonomy Extension Definition Linkbase Document.	X
101.PRE	Inline XBRL Taxonomy Extension Labels Linkbase Document.	X
101.DEF	Inline XBRL Taxonomy Extension Presentation Link Document.	X
104	Cover Page Interactive Data File – The cover page interactive data file does not appear in the Interactive Data File because its XBRL tags are embedded within the Inline XBRL document	

Management contract or compensatory plan or arrangement filed in response to Item 15(a)(3) of the Instructions to the Annual Report on Form 10-K.

† Confidential treatment has been granted as to certain portions, which portions have been omitted and separately filed with the Securities and Exchange Commission.

* Portions of this exhibit have been omitted pursuant to Item 601(b)(10)(iv) of Regulation S-K

+ The certification furnished in Exhibit 32.1 hereto is deemed to be furnished with this Annual Report on Form 10-K and will not be deemed to be “filed” for purposes of Section 18 of the Securities Exchange Act of 1934, as amended, except to the extent that the Registrant specifically incorporates it by reference.

SIGNATURES

Pursuant to the requirements of Section 13 or 15(d) of the Securities Exchange Act of 1934, the registrant has duly caused this Form 10-K to be signed on its behalf by the undersigned, thereunto duly authorized.

Date: February 28, 2024

VOYAGER THERAPEUTICS, INC.
By: */s/ Alfred Sandrock, M.D., Ph.D.*
Alfred Sandrock, M.D., Ph.D.
Chief Executive Officer, President, and Director

SIGNATURES AND POWER OF ATTORNEY

We, the undersigned directors and officers of Voyager Therapeutics, Inc. (the "Company"), hereby severally constitute and appoint Alfred Sandrock and Peter Pfreundschuh, and each of them singly, our true and lawful attorneys, with full power to them, and to each of them singly, to sign for us and in our names in the capacities indicated below, any and all amendments to this Annual Report on Form 10-K, and to file or cause to be filed the same, with all exhibits thereto and other documents in connection therewith, with the Securities and Exchange Commission, granting unto said attorneys, and each of them, full power and authority to do and perform each and every act and thing requisite and necessary to be done in connection therewith, as fully to all intents and purposes as each of us might or could do in person, and hereby ratifying and confirming all that said attorneys, and each of them, or their substitute or substitutes, shall do or cause to be done by virtue of this Power of Attorney.

Pursuant to the requirements of the Securities Exchange Act of 1934, this Annual Report on Form 10-K has been signed by the following persons in the capacities and on the dates indicated.

Name	Title	Date
/s/Alfred Sandrock, M.D., Ph.D. Alfred Sandrock, M.D., Ph.D.	Chief Executive Officer, President, and Director *(Principal Executive Officer)*	February 28, 2024
/s/Peter P. Pfreundschuh Peter P. Pfreundschuh	Chief Financial Officer *(Principal Financial and Accounting Officer)*	February 28, 2024
/s/Michael Higgins Michael Higgins	Director (Chairman of the Board)	February 28, 2024
/s/Grace E. Colón, Ph.D. Grace E. Colón, Ph.D.	Director	February 28, 2024
/s/Jim Geraghty Jim Geraghty	Director	February 28, 2024
/s/Steven Hyman, M.D. Steven Hyman, M.D.	Director	February 28, 2024
/s/Catherine J. Mackey, Ph.D. Catherine J. Mackey, Ph.D.	Director	February 28, 2024
/s/Jude Onyia, Ph.D. Jude Onyia, Ph.D.	Director	February 28, 2024
/s/Glenn Pierce, M.D., Ph.D. Glenn Pierce, M.D., Ph.D.	Director	February 28, 2024
/s/George Scangos, Ph.D. George Scangos, Ph.D.	Director	February 28, 2024
/s/Nancy Vitale Nancy Vitale	Director	February 28, 2024

Exhibit 10.43
Execution Version

Certain identified information has been excluded from the exhibit because it is both (i) not material and (ii) is the type of information that the registrant treats as private or confidential. Double asterisks denote omissions.

LICENSE AND COLLABORATION AGREEMENT

By and between

VOYAGER THERAPEUTICS, INC.

AND

NOVARTIS PHARMA, AG

December 28, 2023

TABLE OF CONTENTS

	Page
ARTICLE 1 DEFINITIONS	1
ARTICLE 2 SMA PROGRAM	18
ARTICLE 3 HD PROGRAM	20
ARTICLE 4 MANAGEMENT OF THE HD PROGRAM	21
ARTICLE 5 GRANT OF LICENSES	24
ARTICLE 6 DEVELOPMENT, REGULATORY AND COMMERCIALIZATION ACTIVITIES	28
ARTICLE 7 UPFRONT CONSIDERATION; MILESTONES AND ROYALTIES; PAYMENTS	31
ARTICLE 8 INTELLECTUAL PROPERTY RIGHTS	39
ARTICLE 9 CONFIDENTIALITY	46
ARTICLE 10 REPRESENTATIONS AND WARRANTIES	49
ARTICLE 11 INDEMNIFICATION; INSURANCE	56
ARTICLE 12 TERM AND TERMINATION	59
ARTICLE 13 MISCELLANEOUS	65

List of Schedules:

Schedule 1.60: Existing In-License Agreements

Schedule 1.79: HD Program Plan

Schedule 7.1.1: Voyager Allocation Schedule

Schedule 7.2.2: Invoice Template

Schedule 7.3: Voyager Bank Account

Schedule 10.2.2: Relevant Patents

LICENSE AND COLLABORATION AGREEMENT

This LICENSE AND COLLABORATION AGREEMENT (the "Agreement") is entered into and made effective as of December 28, 2023 (the "Effective Date"), by and between Voyager Therapeutics, Inc., a Delaware corporation, having its principal place of business at 75 Hayden Ave, Lexington, MA 02421 ("Voyager"), and Novartis Pharma AG, a corporation organized and existing under the laws of Switzerland, having an address at Lichtstrasse 35, CH-4056 Basel, Switzerland ("Novartis"). Voyager and Novartis are referred to herein individually as a "Party" and collectively as the "Parties".

RECITALS

WHEREAS, Voyager Controls certain Patents, Know-How, scientific and technical information, and other proprietary rights and information relating to the generation and selection of Capsids (as defined below) and payloads for use in AAV Gene Therapy (as defined below);

WHEREAS, Novartis is engaged in the research, development and commercialization of certain AAV Gene Therapies; and

WHEREAS, the Parties are entering into this Agreement to: (a) provide rights to Novartis under certain Capsids discovered by Voyager for use in development by Novartis of AAV Gene Therapy Products (as defined below) comprising such Capsids and payloads intended for the treatment of spinal muscular atrophy ("SMA") that are directed to the SMA Target (as defined below); and (b) collaborate to develop AAV Gene Therapy Products intended for the treatment of Huntington's disease ("HD") that are directed to [**] of the HD Targets (as defined below); in each case, leveraging Capsids and other intellectual property Controlled (as defined below) by Voyager.

NOW, THEREFORE, in consideration of the premises and mutual covenants set forth in this Agreement, and for other good and valuable consideration, the receipt and sufficiency of which are hereby acknowledged, the Parties agree as follows:

ARTICLE 1
DEFINITIONS

As used in this Agreement, the following terms will have the meanings set forth in this ARTICLE 1 or otherwise ascribed to them elsewhere in this Agreement:

1.1 "AAV" means an adeno-associated virus, including its recombinant forms.

1.2 "AAV Gene Therapy Products" means therapies and products that use a viral vector, including an AAV vector, to deliver nucleic acid(s) into a patient's cells to treat a human disease, syndrome, disorder, illness or condition.

1.3 "Accounting Standards" means in the case of Voyager, United States Generally Accepted Accounting Principles ("US GAAP"), and in the case of Novartis, International Financial Reporting Standards ("IFRS"), in each case as generally and consistently applied throughout the applicable Party's organization. Each Party shall promptly notify the other in the

event that it changes the Accounting Standards pursuant to which its records are maintained, it being understood that each Party may only use internationally recognized accounting principles (e.g., IFRS, US GAAP, etc.).

1.4 "Acquired Affiliate" has the meaning set forth in Section 10.6.1.

1.5 "Acquired Competing Product" has the meaning set forth in Section 10.6.1.

1.6 "Acquiring Entity" has the meaning set forth in Section 1.41.

1.7 "Acquisition Party" has the meaning set forth in Section 10.6.1.

1.8 "Advance" shall mean the occurrence of any of the following with respect to an AAV Gene Therapy Product after the Effective Date: [**]

1.9 "Affiliate" means with respect to a Person, any other Person that (directly or indirectly) is controlled by, controls or is under common control with such Person as of any point in time and continuing for as long as such relationship continues to exist with respect to such other Person. For the purposes of this definition, the term "control" (including, with correlative meanings, the terms "controlled by" and "under common control with") as used with respect to a Person, will mean the possession, directly or indirectly, of the power to direct, or cause the direction of, the management or policies of such Person, whether through the ownership of voting securities, by contract or otherwise, and "control" will be presumed to exist if either of the following conditions is met: (a) in the case of a corporate entity, direct or indirect ownership of voting securities entitled to cast at least 50% (or in the case of entities organized under the laws of certain countries where the maximum percentage ownership permitted by law for a foreign investor is less than fifty percent (50%), the maximum ownership interest permitted by applicable Law) of the voting securities or other ownership or general partnership interest (whether directly or pursuant to any option, warrant or other similar arrangement) or other comparable equity interests of an entity; *provided*, *however*, that where an entity owns a majority of the voting power necessary to elect a majority of the board of directors or other governing board of another entity, but is restricted from electing such majority by contract or otherwise, such entity will not be considered to be in control of such other entity until such time as such restrictions are no longer in effect.

1.10 "Agreement" has the meaning set forth in the Preamble.

1.11 "Alliance Manager" has the meaning set forth in Section 4.5.

1.12 "Annual Net Sales" means, on a Licensed Product-by-Licensed Product basis, the total, aggregate Net Sales of such Licensed Product in the Territory in a particular Calendar Year.

1.13 "Arbitration Request" has the meaning set forth in Section 13.3.

1.14 "Arising Capsid IP" means: (a) Arising IP Created jointly by Representatives of Novartis and Representatives of Voyager that constitutes Capsid IP; and (b) Arising IP Created solely by Representatives of Novartis through the use of Voyager's Confidential Information, including unpublished sequence information for any Voyager Capsid that constitutes Capsid IP.

1.15 "Arising IP" means: (a) all Know-How Created by either or both Parties in the performance of the HD Program Plan or in the course of Development, Manufacture and Commercialization of HD Program Products; and (b) all Patents Covering such Know-How.

1.16 "Audited Party" has the meaning set forth in Section 7.8.2.

1.17 "Auditing Party" has the meaning set forth in Section 7.8.2.

1.18 "Biosimilar Product" means, with respect to a particular Licensed Product in a particular country in the Territory: (a) any pharmaceutical or biological product sold by a Third Party that is not a Sublicensee of Novartis or its Affiliates and that did not purchase such product in a chain of distribution that included Novartis or any of its Affiliates or Sublicensees; and (b) which pharmaceutical or biological product (i) is approved by the applicable Regulatory Authority as biosimilar to, or interchangeable with, such Licensed Product (including, with respect to the United States, a product that is the subject of an application submitted under Section 351(k) of the Public Health Services Act citing the Licensed Product as the reference product), (ii) for which the Regulatory Approval otherwise references or relies on such Licensed Product as a reference product or any corresponding foreign application in the Territory (including, with respect to the EU, a marketing authorization application for a biosimilar biological medicinal product pursuant to Article 10(4) of Directive 2001/83/EC, or (iii) otherwise utilizes the same Capsid, in combination with the same payload, or a payload substantially similar in structure and function to the payload of the Licensed Product, and is directed to the SMA Target or HD Target(s), as applicable; provided that such pharmaceutical or biological product is not the subject of an enforcement action brought by Voyager in accordance with Section 8.4.2.

1.19 "BLA" means (a) an application requesting permission from the FDA to introduce, or deliver for introduction, a biologic product into interstate commerce, or (b) any similar application or submission for Regulatory Approval of a biologic product filed with a Regulatory Authority in a country or group of countries.

1.20 "Business Day" means a day other than: (a) a Saturday or Sunday; (b) a holiday observed by the United States federal government or the Commonwealth of Massachusetts; or (c) a public holiday on which the banks are not open for business in Basel, Switzerland.

1.21 "Calendar Quarter" means a period of three (3) consecutive months ending on the last day of March, June, September, or December, respectively; provided that: (a) the first Calendar Quarter during the Term will begin on the Effective Date and end on the last day of the Calendar Quarter within which the Effective Date falls; and (b) the last Calendar Quarter during the Term will end upon the effective date of expiration or termination.

1.22 "Calendar Year" means a period of twelve (12) consecutive months beginning on January 1 and ending on December 31; provided that: (a) the first Calendar Year starts on the Effective Date and ends on the last day of the Calendar Year in which the Effective Date falls; and (b) the last Calendar Year starts on January 1 of such year and ends on the effective date of expiration or termination.

1.23 "Campaign" means (a) completion of at least [**] rounds of screening of Capsid candidates in a campaign directed to identification of Capsids useful for Development of AAV

Gene Therapy Products, excluding intermediate round screening results (other than such intermediate data that may be available and requested by Novartis within [**] before the expiration of the Capsid Evaluation Period), or (b) completion of subsequent rounds of screening associated with the evolution of Capsids of interest identified following the original multi-round screening specified in (a) above; excluding in each case ((a) and (b)) any such campaign conducted specifically for a Third Party.

1.24 "Capsid" means the protein shell of an AAV, consisting of oligomeric structural subunits made of certain proteins.

1.25 "Capsid Designation Notice" has the meaning set forth in Section 2.3.

1.26 "Capsid Evaluation Period" means the period beginning on the Effective Date and ending on the third (3rd) anniversary of the Effective Date.

1.27 "Capsid IP" means all Capsid Know-How and Capsid Patents.

1.28 "Capsid Know-How" means all Know-How that is related to any Voyager Capsid or any method of Manufacture or use of any Voyager Capsid; in each case, whether alone or in combination with any payload, including a Program Payload. Capsid Know-How shall be considered Voyager's Confidential Information except to the extent such Capsid Know-How relates to (a) any component of a Development Candidate other than the Voyager Capsid therein; (b) any Program Payload; or (c) any method of Manufacture or use of (i) a specific Development Candidate that is not specific to the Voyager Capsid therein or (ii) a Program Payload.

1.29 "Capsid Patent" means any Patent Controlled by Voyager as of the Effective Date or at any time during the Term with claims that Cover any Capsid Know-How.

1.30 "Change of Control" means, with respect to a Party, that: (a) any Third Party acquires directly or indirectly the beneficial ownership of any voting security of such Party, or if the percentage ownership of such Third Party in the voting securities of such Party is increased through stock redemption, cancellation, or other recapitalization, and immediately after such acquisition or increase such Third Party is, directly or indirectly, the beneficial owner of voting securities representing more than fifty percent (50%) of the total voting power of all of the then outstanding voting securities of such Party; (b) any merger, consolidation, recapitalization, or reorganization of such Party is consummated that would result in shareholders or equity holders of such Party immediately prior to such transaction owning less than fifty percent (50%) of the outstanding voting securities of the surviving entity (or its parent entity) immediately following such transaction; (c) the shareholders or equity holders of such Party approve any plan of complete liquidation of such Party, or an agreement for the sale or disposition by such Party of all or substantially all of such Party's assets, in each case, through one or more related transactions, other than to an Affiliate or pursuant to one or more related transactions that would result in shareholders or equity holders of such Party immediately prior to such transaction owning more than fifty percent (50%) of the outstanding voting securities of the surviving entity (or its parent entity) immediately following such transaction; or (d) the sale or transfer to any Third Party, in one or more related transactions, of all or substantially all of such Party's consolidated assets taken as a whole.

1.31 "[**]" means [**].

1.32 "Clinical Trial" means a human clinical study conducted on sufficient numbers of human subjects that is designed to: (a) establish that a biopharmaceutical product is reasonably safe for continued human testing; (b) investigate the safety and efficacy of the biopharmaceutical product for its intended use, and to define warnings, precautions and adverse reactions that may be associated with the pharmaceutical product in the dosage range to be prescribed; or (c) support Regulatory Approval of a biopharmaceutical product or label expansion of a pharmaceutical product.

1.33 "CMO" means contract manufacturing organization.

1.34 "Commercialization" means any and all activities directed to the marketing, promotion, distribution, offering for sale, sale, having sold, importing, having imported, exporting, having exported or other commercialization of a pharmaceutical or biologic product, but excluding activities directed to Manufacturing or Development. "Commercialize", "Commercializing", and "Commercialized" have correlating meanings.

1.35 "Commercially Reasonable Efforts" means, with respect to the efforts to be expended by a Party with respect to any objective, those reasonable, good faith efforts to accomplish such objective as such Party would normally use to accomplish a similar objective undersimilar circumstances. With respect to any efforts relating to the Development, Regulatory Approval, or Commercialization of a Licensed Product by Novartis, generally or with respect to any particular country in the Territory, [**]. It is anticipated that the level of effort may change over time, reflecting changes in the status of a Licensed Product. Further, to the extent that the performance of a Party's obligations hereunder is adversely affected by the other Party's failure to perform its obligations hereunder, the impact of such performance failure will be taken into account in determining whether such Party has used its Commercially Reasonable Efforts to perform any such affected obligations.

1.36 "Committee" means the JSC and/or any Subcommittee, as applicable.

1.37 "Competing HD Product" means: an AAV Gene Therapy Product comprising a Capsid for use with any payload intended to have a therapeutic effect on [**].

1.38 "Competitive Infringement" means infringement of any Licensed Patent by a Third Party by a product that is competitive with a Licensed Product.

1.39 "Competitive Program" has the meaning set forth in Section 10.6.1.

1.40 "Confidential Information" has the meaning set forth in Section 9.1.

1.41 "Control" means, with respect to a Person and any Know-How or Patent, the possession by such Person of the right (whether through ownership, license, or otherwise (other than by a license under this Agreement)) to grant the rights and licenses as provided herein, without violating the terms of any agreement with any Third Party. Notwithstanding the foregoing, in the event that a Third Party becomes an Affiliate or assignee of a Party after the Effective Date as a result of a Change of Control of such Party (such Third Party, together with its Affiliates other

than Affiliates who were Affiliates of the applicable Party prior to the Change of Control, the Acquiring Entities"), the following will be deemed to be not Controlled by such Party or any of its Affiliates: (a) any Patent, Know-How, Regulatory Filing, or Regulatory Approval owned or otherwise controlled by such Acquiring Entity immediately prior to the consummation of such Change of Control; and (b) any Patent, Know-How, Regulatory Filing, or Regulatory Approval developed by or on behalf of such Acquiring Entity outside the scope of activities under this Agreement or acquired by or on behalf of such Acquiring Entity after the consummation of such Change of Control.

1.42 "Cover" means with regard to a particular subject matter and a Valid Claim in a Patent, that in the absence of ownership of or a license granted under such Valid Claim in such Patent, the making, use, offer for sale, sale, importation, Development, Manufacture, or Commercialization of such subject matter, would infringe such Valid Claim in such Patent (with Valid Claims of pending patent applications treated as if issued).

1.43 "Created" means: (a) with respect to any Know-How constituting an Invention, invented in accordance with U.S. patent laws; or (b) with respect to any other Know-How, authored, discovered, developed or created.

1.44 "Debtor" has the meaning set forth in Section 12.4.1.

1.45 "Defense Proceeding" means an opposition, reexamination request, action for declaratory judgment, nullity action, interference or post-grant proceeding or other attack upon the validity, title or enforceability of a Patent that occurs in the context of litigation; excluding any such proceeding brought as a counterclaim to or defense of, or that accompanies a defense of, any enforcement action under Section 8.4.2.

1.46 "Designation Notice Date" has the meaning set forth in Section 2.3.

1.47 "Development" means all internal and external research, development, and regulatory activities related to pharmaceutical or biologic products, including: (a) research, non-clinical testing, toxicology, testing and studies, non-clinical and preclinical activities, and Clinical Trials; and (b) preparation, submission, review, and development of data or information for the purpose of submission to a Regulatory Authority to obtain authorization to conduct Clinical Trials and to obtain, support, or maintain Regulatory Approval of a pharmaceutical or biologic product and interacting with Regulatory Authorities following receipt of Regulatory Approval in the applicable country or region for such pharmaceutical or biologic product regarding the foregoing, but excluding activities directed to Manufacturing or Commercialization. "Development" includes development and regulatory activities for additional forms, formulations, or indications for a pharmaceutical or biologic product after receipt of Regulatory Approval of such product (including label expansion), including Clinical Trials initiated following receipt of Regulatory Approval or any Clinical Trial to be conducted after receipt of Regulatory Approval that was mandated by the applicable Regulatory Authority as a condition of such Regulatory Approval with respect to an approved formulation or indication (such as post-marketing studies, observational studies, implementation and management of registries and analysis thereof, in each case, if required by any Regulatory Authority in any region in the Territory to support or maintain Regulatory Approval

for a pharmaceutical or biologic product in such region). “Develop”, “Developing”, and “Developed” have correlating meanings.

1.48 “Development Budget” has the meaning set forth in Section 3.1.

1.49 “Development Candidate” means an HD Development Candidate or an SMA Development Candidate.

1.50 “Development Milestone Event” means any Milestone Event set forth in Section 7.2.1.

1.51 “Development Milestone Event Notice” has the meaning set forth in Section 7.2.1.

1.52 “Development Milestone Payment” has the meaning set forth in Section 7.2.2.

1.53 “Diligence Issue” has the meaning set forth in Section 6.2.5.

1.54 “Disclosing Party” has the meaning set forth in Section 9.1.

1.55 “Dollars” or “$” means the legal tender of the U.S.

1.56 “Effective Date” has the meaning set forth in the Preamble.

1.57 “EMA” means the European Medicines Agency, and any successor entity thereto.

1.58 “Evaluate” means evaluation conducted by or on behalf of Novartis during the Capsid Evaluation Period to assess any Voyager Capsid provided to Novartis by Voyager to determine Novartis’ interest in designating such Voyager Capsid as an SMA Program Capsid pursuant to Section 2.3. “Evaluation” and “Evaluating” have correlating meanings.

1.59 “Executive Officers” means: (a) with respect to Voyager, [**], or his or her designee; or (b) with respect to Novartis, [**], or his or her designee.

1.60 “Existing In-License Agreement” means each of the in-licenses of Voyager or any of its Affiliates listed in Schedule 1.60.

1.61 “Exploit” means to Develop, Manufacture, Commercialize, or otherwise exploit. “Exploitation” and “Exploiting” have correlating meanings.

1.62 “FDA” means the U.S. Food and Drug Administration, and any successor entity thereto.

1.63 “FD&C Act” means the U.S. Federal Food, Drug, and Cosmetic Act, 21 U.S.C. § 301*et seq.*, as amended from time to time, together with any rules, regulations and requirements promulgated thereunder (including all additions, supplements, extensions, and modifications thereto).

1.64 “Field” means all indications for therapeutic, diagnostic and prophylactic human and veterinary use.

1.65 "First Commercial Sale" means, with respect to a Licensed Product, the first sale in an arms'-length transaction of such Licensed Product to a Third Party (excluding Sublicensees or distributors) of such Licensed Product in such country after all Regulatory Approvals (excluding Price Approvals) have been granted by the applicable Regulatory Authority of such country or, if Regulatory Approval is not required, after the date on which sales are permitted by applicable Law. For clarity, the First Commercial Sale of a Licensed Product shall not include any distribution or other sale at or below cost solely for patient assistance, named patient use, compassionate use, or test marketing programs or non-registrational studies or similar programs or studies where the Licensed Product is supplied without charge or at or below the actual manufacturing cost thereof.

1.66 "FTE" means one (1) person (or the equivalent of one (1) person) working full time for one (1) twelve (12) month period in a Development, regulatory or other relevant capacity (excluding persons employed in general and administrative, non-technical management or other non-technical capacities) employed by Voyager or any of its Affiliates and assigned to perform specified work, with such commitment of time and effort to constitute one (1) employee performing such work on a full-time basis, which for purposes hereof shall be [**] hours per year. Any person who devotes less than [**] hours per year to work under this Agreement shall be treated as an FTE on a pro rata basis based upon the actual number of hours worked under this Agreement divided by [**].

1.67 "FTE Costs" means the FTE Rate multiplied by the applicable number of FTEs who perform a specified activity pursuant to this Agreement.

1.68 "FTE Rate" means [**] dollars ($[**]) per FTE for the period commencing on the Effective Date and ending December 31, 2024. On January 1, 2025 and on January 1st of each subsequent Calendar Year, the foregoing rate shall be increased for the Calendar Year then commencing by the percentage increase, if any, in the Consumer Price Index ("CPI") as of December 31 of the then most recently completed Calendar Year with respect to the level of the CPI on December 31, 2024. Consumer Price Index or CPI means the Consumer Price Index – Urban Wage Earners and Clerical Workers, US City Average, All Items, 1982-84 = 100, published by the United States Department of Labor, Bureau of Labor Statistics (or its successor equivalent index).

1.69 "Future In-License Agreement" means any agreement between Voyager (or any of its Affiliates), on the one hand, and a Third Party, on the other hand, entered into after the Effective Date, pursuant to which Voyager or any of its Affiliates acquires Control of any Know-How or Patent that, subject to Section 5.2, would be Voyager IP.

1.70 "Global Trade Control Laws" has the meaning set forth in Section 13.8.

1.71 "Governmental Authority" means any multinational, federal, national, state, provincial, local or other entity, office, commission, bureau, agency, political subdivision, instrumentality, branch, department, authority, board, court, arbitral or other tribunal exercising executive, judicial, legislative, police, regulatory, administrative or taxing authority or functions of any nature pertaining to government.

1.72 "HD" has the meaning set forth in the recitals.

1.73 "HD Candidate" means a product candidate that is Developed as a potential HD Program Product pursuant to the HD Program Plan, which product candidate comprises: (a) a Voyager Capsid; and (b) a polynucleotide sequence, whether single stranded or self-complementary, that is intended to have a therapeutic effect on the HD Target when packaged into a Voyager Capsid and delivered to the appropriate cells.

1.74 "HD DC Criteria" means the criteria developed by the JSC pursuant to Section 4.1.1(b) that must be achieved by an HD Candidate in order to be considered for selection as an HD Development Candidate.

1.75 "HD Development Candidate" means an HD Candidate that has been named by Novartis, in its sole discretion, as an HD Development Candidate pursuant to Section 3.4.

1.76 "HD Program" means the activities under this Agreement related to Development of AAV Gene Therapy Products directed to the HD Target intended and for treatment of HD.

1.77 "HD Program Capsid" means: (a) the Voyager Capsid component of an HD Program Product; or (b) any Capsid derived by or on behalf of Novartis or its Affiliates from a Capsid described in (a).

1.78 "HD Program Payload" means the payload component of an HD Program Product, which component comprises a polynucleotide sequence, whether single stranded or self-complementary, that is intended to have a therapeutic effect on the HD Target when packaged into a Voyager Capsid and delivered to the appropriate cells.

1.79 "HD Program Plan" means the plan for research and pre-clinical Development of HD Program Products from the Effective Date until the date when the first IND for an HD Program Product is filed, an initial draft of which is attached hereto as Schedule 1.79 and which may be updated by the JSC from time to time in accordance with Section 4.1.1.

1.80 "HD Program Product" means a product comprising an HD Development Candidate.

1.81 "HD Program Product Patent" means a Novartis HD Program Product Patent or Voyager HD Program Product Patent.

1.82 "HD Target" means the following genes, including the coding and non-coding regions affecting their function and regulation, and modifications thereof: [**].

1.83 "In-License Agreement" means: (a) any Existing In-License Agreement; and (b) any Future In-License Agreement.

1.84 "Inbound Licensor" has the meaning set forth in Section 5.2.1.

1.85 "IND" means an investigational new drug application (including any amendment or supplement thereto) submitted to the FDA pursuant to U.S. 21 C.F.R. Part 312, including any amendments thereto, or any comparable filing(s) outside the United States for the investigation of any product in any other country or group of countries.

1.86 "Indemnified Party" has the meaning set forth in Section 11.3.

1.87 "Indemnifying Party" has the meaning set forth in Section 11.3.

1.88 "Indirect Taxes" has the meaning set forth in Section 7.12.2.

1.89 "Infringement Notice" has the meaning set forth in Section 8.4.1.

1.90 "Invention" or "Invented" means the result or act of invention (whether patentable or not) as determined in accordance with U.S. patent laws.

1.91 "Invoice" means an invoice substantially in the form set forth in Schedule 7.2.2.

1.92 "[**]" means [**].

1.93 "Joint Arising IP" has the meaning set forth in Section 8.1.3(a).

1.94 "Joint Know-How" means any Know-How within the Joint Arising IP.

1.95 "Joint Patent" means any Patent within the Joint Arising IP.

1.96 "JSC" has the meaning set forth in Section 4.1.

1.97 "Know-How" means all proprietary information, know-how and data, including trade secrets, Inventions (whether patentable or not), discoveries, methods, specifications, processes, procedures, formulas, expertise, technology, data (including non-clinical, pre-clinical and clinical data), documentation, materials, and results (including pharmacological, toxicological, biological, chemical, physical, safety and Manufacturing data and results), analytical and quality control data and results, Manufacturing techniques, Regulatory Filings and other technical information. "Know-How" excludes in any event any Patents.

1.98 "Law" means any law, statute, rule, regulation, order, judgment or ordinance having the effect of law of any federal, national, multinational, state, provincial, county, city or other political subdivision.

1.99 "Licensed Capsid" means (a) any SMA Program Capsid or any HD Program Capsid; or (b) any Capsid derived by or on behalf of Novartis or its Affiliates from a Capsid described in (a) above where such derived Capsid (i) is Covered by a Capsid Patent, (ii) contains changes or improvements, and (iii) such changes or improvements are not Covered by a claim of a Capsid Patent that is patentably distinct from the claims of the Capsid Patent that Cover the initial Capsid set forth in (a) above.

1.100 "Licensed Patent" means any Patent Controlled by Voyager or any of its Affiliates as of the Effective Date or at any time during the Term (including Voyager's interest in the Joint Patents) that: (a) constitute Capsid Patents subject to the license under Section 5.1.1; (b) with respect SMA Program Products, Covers (i) the SMA Program Capsid component of the applicable SMA Program Product (including any such Patent Covering such SMA Program Capsid in combination with any payload, including an SMA Program Payload) or (ii) any SMA Program

Payload Developed by Voyager and made available by Voyager to Novartis for use in the SMA Program; and (c) with respect to HD Program Products, Covers any HD Program Product (including any HD Program Capsid or HD Program Payload included therein). Licensed Patents includes Voyager's or its Affiliate's interest in the Joint Patents. "Licensed Patents" expressly exclude [**].

1.101 "Licensed Product" means any SMA Program Product or any HD Program Product.

1.102 "Litigation Conditions" has the meaning set forth in Section 11.4.

1.103 "Losses" has the meaning set forth in Section 11.1.

1.104 "[**]" means [**].

1.105 "Manufacture" means activities directed to manufacturing, processing, packaging, labeling, filling, finishing, assembly, quality assurance, quality control, testing, and release, shipping, or storage of any pharmaceutical or biologic product (or any components or process steps involving any product or any companion diagnostic), placebo, or comparator agent, as the case may be, including process development, qualification, and validation, scale-up, pre-clinical, clinical, and commercial manufacture and analytic development, product characterization, and stability testing. "Manufacturing" has correlating meaning.

1.106 "Net Sales" means the net sales recorded by Novartis or any of its Affiliates or Sublicensees, excluding distributors and wholesalers, for any Licensed Product sold to Third Parties other than Sublicensees as determined in accordance with Novartis' Accounting Standards as consistently applied, less a deduction of [**] percent ([**]%) for direct expenses related to the sales of the Licensed Product, distribution and warehousing expenses and uncollectible amounts on previously sold products. The deductions booked on an accrual basis by Novartis and its Affiliates under its Accounting Standards to calculate the recorded net sales from gross sales include, without limitation, the following:

(a) normal trade and cash discounts;

(b) amounts repaid or credited by reasons of defects, rejections, recalls or returns;

(c) rebates and chargebacks to customers and third parties (including, without limitation, Medicare, Medicaid, Managed Healthcare and similar types of rebates);

(d) amounts provided or credited to customers through coupons and other discount programs;

(e) delayed ship order credits, discounts or payments related to the impact of price increases between purchase and shipping dates or retroactive price reductions;

(f) fee for service payments to customers for any non-separable services (including compensation for maintaining agreed inventory levels and providing information); and

(g) other reductions or specifically identifiable amounts deducted for reasons similar to those listed above in accordance with Novartis' Accounting Standards.

With respect to the calculation of Net Sales:

i. Net Sales only include the value charged or invoiced on the first arm's length sale to a Third Party;

ii. sales between or among Novartis and its Affiliates and Sublicensees shall be disregarded for purposes of calculating Net Sales; and

iii. If a Licensed Product is delivered to the Third Party before being invoiced (or is not invoiced), Net Sales will be calculated at the time the revenue recognition criteria under Novartis Accounting Standards are met.

1.107 "Non-Disclosing Party" has the meaning set forth in Section 9.6.

1.108 "Novartis" has the meaning set forth in the Preamble.

1.109 "Novartis Background IP" has the meaning set forth in Section 8.1.1.

1.110 "Novartis Evaluation Data" has the meaning set forth in Section 2.1.2.

1.111 "Novartis HD Program Product Patent" means, collectively, any Patent Controlled by Novartis at any time during the Term with claims directed to: (a) the combination of, or product including, (i) any HD Program Capsid and (ii) any HD Program Payload; (b) any HD Program Payload; or (c) any method of use or method of manufacture directed to the Inventions claimed in the Patents described in (a) or (b).

1.112 "Novartis IP" means the Novartis Know-How and the Novartis Patents.

1.113 "Novartis Know-How" means: (a) all Know-How that (i) is Controlled by Novartis or any of its Affiliates as of the Effective Date or during the Term, (ii) is disclosed or is required to be disclosed by or on behalf of Novartis to Voyager in connection with this Agreement, and (iii) is necessary or reasonably useful for Voyager to perform its obligations under the HD Program Plan or otherwise under this Agreement; and (b) Novartis' interest in the Joint Know-How.

1.114 "Novartis Patents" means (a) all Patents Controlled by Novartis or any of its Affiliates as of the Effective Date or during the Term that Cover any HD Candidate, SMA Development Candidate or Licensed Product and (b) Novartis' interest in the Joint Patents.

1.115 "Novartis Prosecuted Licensed Patents" has the meaning set forth in Section 8.3.8.

1.116 "Novartis SMA Program Product Patent" means, collectively, any Patent Controlled by Novartis at any time during the Term with claims directed to: (a) the combination of, or product including, (i) any SMA Program Capsid and (ii) any SMA Program Payload; (b) any SMA Program Payload; or (c) any method of use or method of manufacture directed to the Inventions claimed in the Patents described in (a) or (b).

1.117 "Novartis Third Party Code" has the meaning set forth in Section 10.7.3.

1.118 "Other Know-How" has the meaning set forth in Section 2.3.

1.119 "Out-of-Pocket Costs" means actual, documented out-of-pocket costs and expenses paid by a Party or any of its Affiliates to Third Parties, including to a consultant or contractor of such Party.

1.120 "Patent" means (a) any patent, patent application or utility models (including any provisional application, priority application, or international applications) in any country or multinational jurisdiction in the Territory (including any converted application, continuation, continuation-in-part, continued prosecution application or divisional of any such application, any reissue, renewal, extension, registration, confirmation, revalidation, restoration, substitution, reexamination, supplementary protection certificate, pediatric exclusivity period or the like of any such patent); (b) any foreign equivalent of any patent or patent application described in clause (a); and (c) all rights of priority in any of the foregoing.

1.121 "Parties" or "Party" has the meaning set forth in the Preamble.

1.122 "Person" means any individual, partnership, limited liability company, firm, corporation, association, trust, unincorporated organization or other similar entity or organization.

1.123 "Pivotal Clinical Trial" means a Clinical Trial of a Licensed Product that either: (a) would satisfy the requirements of 21 C.F.R. 312.21(c) or corresponding foreign regulations; or (b) is intended (as of the time the Clinical Trial is initiated) to obtain sufficient data to support the filing of a BLA for such Licensed Product. Pivotal Clinical Trial may include (i) a Clinical Trial that is designed to satisfy the requirements of both 21 C.F.R. 312.21(b) and 21 C.F.R. 312.21(c) or corresponding foreign regulations, or (ii) a Clinical Trial that is designed to satisfy the requirements of 21 C.F.R. 312.21(b) that is subsequently optimized or expanded to satisfy the requirements of 21 C.F.R. 312.21(c) or to provide sufficient data to support the filing of a BLA for such Licensed Product, as supported by a Regulatory Authority's formal meeting minutes or comparable documents, in which case such Pivotal Clinical Trial shall be deemed to have been initiated upon the first dosing of the first human subject under the optimized or expanded protocol for such Clinical Trial.

1.124 "Price Approval" means, in any country where a Governmental Authority authorizes reimbursement for, or approves or determines pricing for, pharmaceutical products, receipt (or, if required to make such authorization, approval or determination effective, publication) of such reimbursement authorization or pricing approval or determination (as the case may be).

1.125 "Program" means the HD Program or the SMA Program, as applicable. "Programs" means the HD Program and the SMA Program. In the event that the HD Program or SMA Program is terminated as permitted under this Agreement, such terminated HD Program or SMA Program will no longer be considered a Program under this Agreement.

1.126 "Program Payload" means the HD Program Payload and/or the SMA Program Payload, as applicable.

1.127 "Program Target(s)" means the SMA Target and/or the HD Target(s), as applicable.

1.128 "Program Capsid Patents" has the meaning set forth in Section 8.3.3.

1.129 "Program Patents" means the Patents described in subsection (b) and (c) of the definition of Licensed Patent.

1.130 "Prosecution and Maintenance" or "Prosecute and Maintain" means, with regard to a Patent: (a) the preparation, filing, prosecution, maintenance, and requests for patent term adjustments or patent term extensions, including terminally disclaiming an application or issued patent of or for such Patent, as well all appeals therefrom; and (b) any proceeding, other than routine *ex parte* prosecution, which challenges such Patent occurring independently of litigation of the Patent, including re-examinations, nullity actions, interferences, oppositions, derivation proceedings, post-grant reviews, reissues, and other similar proceedings with respect to such Patent and any appeals therefrom.

1.131 "Receiving Party" has the meaning set forth in Section 9.1.

1.132 "Redacted Version" has the meaning set forth in Section 9.5.2.

1.133 "Regulatory Approval" means the approval of the applicable Regulatory Authority necessary for the marketing and sale of a product in a country(ies), including any required Price Approval.

1.134 "Regulatory Approval Application" means a Regulatory Filing submitted to an applicable Regulatory Authority to obtain Regulatory Approval to market and sell a particular product in the country or countries that such Regulatory Authority is responsible for, including any amendments thereto and supplemental applications.

1.135 "Regulatory Authority" means the FDA in the United States or any Governmental Authority in another country in the Territory that is a counterpart to the FDA and holds responsibility for granting Regulatory Approval for a product in such country, including the EMA, and any successor(s) thereto.

1.136 "Regulatory Filing" means, with respect to a product, any documentation comprising or relating to or supporting any filing or application with any Regulatory Authority with respect to such product, or its use or potential use in the Field, including any document submitted to any Regulatory Authority, including any IND, any Regulatory Approval Application and any correspondence with any Regulatory Authority with respect to such product (including minutes of any meetings, telephone conferences or discussions with any Regulatory Authority).

1.137 "Related Third Party IP" has the meaning set forth in Section 5.2.2.

1.138 "Relevant Capsid Patents" has the meaning set forth in Section 10.2.2.

1.139 "Relevant Factors" means all relevant factors that may affect the Development, Regulatory Approval or Commercialization of a Licensed Product, including (as applicable): actual and potential issues of safety, efficacy or stability; product profile (including product

modality, category and mechanism of action); stage of development or life cycle status; actual and projected Development, Regulatory Approval, Manufacturing, and Commercialization costs; any issues regarding the ability to Manufacture or have Manufactured any Licensed Capsid or Licensed Product; the likelihood of obtaining Regulatory Approvals (including satisfactory or required Price Approvals); the timing of such approvals; the current guidance and requirements for Regulatory Approval for the Licensed Product and similar products and the current and projected regulatory status; labeling or anticipated labeling; the then-current competitive environment and the likely competitive environment at the time of projected entry into the market; past performance of the Licensed Product or similar products; present and future market potential; the ability to obtain adequate supply of any Licensed Capsid or Licensed Product, or any component thereof, from any Third Party as may be required to Develop, secure Regulatory Approval for or Commercialize any Licensed Capsid or Licensed Product; Patents of a Third Party; existing or projected pricing, sales, reimbursement and profitability; pricing or reimbursement changes in relevant countries; proprietary position, strength and duration of patent protection and anticipated exclusivity; and other relevant scientific, technical, operational and commercial factors.

1.140 "Relevant HD Payload Patents" has the meaning set forth in Section 10.2.2.

1.141 "Relevant Patents" has the meaning set forth in Section 10.2.2.

1.142 "Representatives" means: (a) with respect to Novartis, Novartis and its Affiliates and each of their respective officers, directors, employees, consultants, contractors, and agents; and (b) with respect to Voyager, Voyager and its Affiliates and each of their respective officers, directors, employees, consultants, contractors, and agents.

1.143 "Residual Knowledge" means knowledge, techniques, experience and Know-How that: (a) are, or are based on any Confidential Information Controlled by the Disclosing Party; and (b) are retained in the unaided memory of any authorized Representative of the Receiving Party after having access to such Confidential Information. An individual's memory will be considered to be unaided if the individual has not intentionally memorized the Confidential Information for the purpose of retaining and subsequently using or disclosing it. In no event, however, will Residual Knowledge include any knowledge, techniques, experience and Know-How to the extent (at any time, for such time) within the scope of any issued, valid, and enforceable patent claim Controlled by the Disclosing Party.

1.144 "Restricted Market" has the meaning set forth in Section 13.8.1.

1.145 "Restricted Party" has the meaning set forth in Section 13.8.2.

1.146 "Royalty Floor" has the meaning set forth in Section 7.5.5.

1.147 "Royalty Term" means, on a country-by-country and Licensed Product-by-Licensed Product basis, the period commencing on the First Commercial Sale of such Licensed Product in such country and terminating upon the latest to occur of: (a) expiration of the last Valid Claim of a Licensed Patent Covering such Licensed Product in such country; (b) termination or expiration of regulatory or data exclusivity for such Licensed Product in such country; and (c) [**] after the First Commercial Sale of such Licensed Product in such country.

1.148 "SMA" has the meaning set forth in the recitals.

1.149 "SMA Development Candidate" means a product candidate that is Developed as a potential SMA Program Product, which product candidate comprises (a) a Voyager Capsid and (b) an SMA Program Payload, that has been named by Novartis as an SMA Development Candidate pursuant to Section 2.4.

1.150 "SMA Program" means the activities under this Agreement related to Development of AAV Gene Therapy Products directed to the SMA Target intended for treatment of SMA.

1.151 "SMA Program Capsid" means a Voyager Capsid for which Novartis has submitted a Capsid Designation Notice pursuant to Section 2.3.

1.152 "SMA Program Payload" means a polynucleotide sequence, selected by Novartis, whether single stranded or self-complementary, that is intended to have a therapeutic effect on the SMA Target when packaged into a Voyager Capsid and delivered to the appropriate cells.

1.153 "SMA Program Product" means a product comprising an SMA Development Candidate.

1.154 "SMA Program Product Patent" means a Novartis SMA Program Product Patent or Voyager SMA Program Product Patent.

1.155 "SMA Target" means [**].

1.156 "Stock Purchase Agreement" has the meaning set forth in Section 7.1.1.

1.157 "Subcommittee" has the meaning set forth in Section 4.1.

1.158 "Sublicense" has the meaning set forth in Section 5.3.

1.159 "Sublicensee" has the meaning set forth in Section 5.3.

1.160 "Tax Action" has the meaning set forth in Section 7.12.3.

1.161 "Term" has the meaning set forth in Section 12.1.

1.162 "Territory" means worldwide.

1.163 "Third Party" means any Person that is neither a Party nor an Affiliate of a Party.

1.164 "Third Party Claims" has the meaning set forth in Section 11.1.

1.165 "Third Party Capsid License" has the meaning set forth in Section 7.5.2.

1.166 "Third Party HD Payload License" has the meaning set forth in Section 7.5.3.

1.167 "United States" or "U.S." means the United States of America and all of its territories and possessions.

1.168 "Valid Claim" means, with respect to a particular country and Licensed Patent: (a) a claim of an issued and unexpired Licensed Patent (i) that has not been revoked or held unenforceable, unpatentable or invalid by a decision of a court or other Governmental Authority of competent jurisdiction from which no appeal can be taken or has not been appealed within the time allowed for appeal and (ii) that has not been irrevocably abandoned, disclaimed, denied, or admitted to be invalid or unenforceable through reissue, re-examination, or disclaimer or otherwise; or (b) a claim of a pending patent application within the Licensed Patent(s) that has not been cancelled, withdrawn, abandoned or finally rejected by an administrative agency action from which no appeal can be taken, provided that any claim in any patent application pending for more than [**] from the earliest date on which such claim claims priority shall not be considered a Valid Claim for purposes of the Agreement from and after such [**] date.

1.169 "Voyager" has the meaning set forth in the Preamble.

1.170 "Voyager Capsid" means any Capsid that is: (a) made available by Voyager to Novartis for Evaluation under the SMA Program; (b) the subject of Development efforts under the HD Program; or (c) that is otherwise used by Voyager in, or is made available by Voyager to Novartis for use in, any Program during the Term. All Licensed Capsids are Voyager Capsids.

1.171 "Voyager Background IP" has the meaning set forth in Section 8.1.2.

1.172 "Voyager HD Program Costs" means all Out-of-Pocket Costs and FTE Costs incurred by Voyager to conduct its activities under the HD Program Plan.

1.173 "Voyager HD Program Product Patent" means, collectively, any Licensed Patent with claims directed to: (a) the combination of, or product including, (i) any HD Program Capsid and (ii) any HD Program Payload; (b) any HD Program Payload; or (c) any method of use or method of manufacture directed to the Inventions claimed in the Patents described in (a) or (b).

1.174 "Voyager IP" means the Capsid Patents, Licensed Patents and Voyager Know-How; in each case to the extent licensed to Novartis under Section 5.1.

1.175 "Voyager Know-How" means Know-How that: (a) is Controlled by Voyager or any of its Affiliates as of the Effective Date or that comes into the Control of Voyager or any of its Affiliates during the Term (other than through the grant of a license by Novartis); (b) is disclosed or is required to be disclosed by or on behalf of Voyager to Novartis in connection with this Agreement; and (c)(i) relates to any Voyager Capsid or the Exploitation of any Voyager Capsid for use in any SMA Program Product or HD Program Product, (ii) is necessary or reasonably useful to Exploit any SMA Program Payload provided to Novartis by Voyager for use in the SMA Program as part of an SMA Program Product, or (iii) is necessary or reasonably useful to Exploit any HD Candidate or HD Program Product (including any HD Program Capsid or HD Program Payload therein) in the Field and in the Territory. Voyager Know-How includes Voyager's interest in any Joint Know-How.

1.176 "Voyager's Knowledge" means the actual knowledge, as of the Effective Date, of Voyager's [**].

1.177 "Voyager [**] Platform Patents" means: (a) the Patents set forth in Section (b)(iv) of Schedule 10.2.2 (including any converted application, continuation, continuation-in-part, continued prosecution application or divisional of any such application, any reissue, renewal, extension, registration, confirmation, revalidation, restoration, substitution, reexamination, supplementary protection certificate, pediatric exclusivity period or the like of any such patent); and (b) any foreign equivalent of any patent or patent application described in clause (a); and (c) all rights of priority in any of the foregoing; in each case, solely to the extent such Patents Cover any HD Candidate, HD Development Candidate or HD Program Product.

1.178 "Voyager SMA Program Product Patent" means, collectively, any Licensed Patent with claims directed to: (a) the combination of, or product including, (i) any SMA Program Capsid and (ii) any SMA Program Payload; (b) any SMA Program Payload; or (c) any method of use or method of manufacture directed to the Inventions claimed in the Patents described in (a) or (b).

ARTICLE 2
SMA PROGRAM

2.1 Capsid Disclosure, Evaluation and Selection.

2.1.1 Campaigns and Disclosure. During the Capsid Evaluation Period, Voyager may (but will not be obligated to), at Voyager's sole discretion and expense, conduct Campaigns and identify Voyager Capsids that may be useful for AAV Gene Therapy Products. Voyager will disclose to Novartis, on a rolling basis (but no less frequently than [**]), the data relating to the performance characteristics for such Voyager Capsids that may be useful for the SMA Program and that (i) is or becomes Controlled by Voyager during the Capsid Evaluation Period, or (ii) arises from Campaigns conducted by Voyager during the Capsid Evaluation Period.

2.1.2 Evaluation of Voyager Capsids for the SMA Program. During the Capsid Evaluation Period, following the disclosure by Voyager to Novartis of a Voyager Capsid, Novartis will have the right, in its sole discretion, to select any number of such Voyager Capsids for Evaluation by written notice to Voyager. Upon receipt of such written notice, Voyager will promptly provide to Novartis plasmids for the production of each such Voyager Capsid selected by Novartis for such Evaluation. Novartis will promptly provide to Voyager all results of such Evaluation that are generated during the Capsid Evaluation Period that are related to biodistribution, expression level, and toxicity of the relevant Voyager Capsid(s) ("Novartis Evaluation Data"); provided that Novartis, in its sole discretion, may choose to redact, mask, or not provide any information related to an SMA Program Payload. Voyager will be free to use such Novartis Evaluation Data for its own internal research purposes, in support of Voyager's Patent filings, and as part of data packages shared under confidentiality in association with the applicable Voyager Capsid (without attribution of the source of such data to Novartis); provided, however, that (a) Voyager shall not include Novartis Evaluation Data in any Patent filing without Novartis' prior written consent, which consent shall not be unreasonably withheld and (b) Voyager shall only share the Novartis Evaluation Data with Third Parties who have similarly contracted with Voyager to make available the results of such Third Party's evaluation of Capsids, subject to similar confidentiality protections. In the event Novartis does not submit a Capsid Designation Notice for a particular Voyager Capsid pursuant to Section 2.3, Novartis will not: (x) disclose the data from the corresponding Evaluation of such Voyager Capsid to any Third Party; or (y) include

the data from the corresponding Evaluation of such Voyager Capsid in any Patent filing, except in each case of (x) or (y) where such data has become publicly available through no breach of this Agreement or with Voyager's prior written consent. Novartis may perform such Evaluation with respect to the SMA Target at any time during the Capsid Evaluation Period.

2.2 Reporting Obligations. During the Capsid Evaluation Period, Voyager shall provide written reports summarizing the Voyager Capsids disclosed pursuant to Section 2.1, and Novartis will provide written reports summarizing all results of the Evaluation(s) conducted pursuant to Section 2.1.2 with timing to be mutually agreed by the Parties. Without limiting the foregoing, Novartis shall, during the Term, until the First Commercial Sale of an SMA Program Product and on [**] basis (every [**]), provide a written report setting forth a non-binding overview of the anticipated timelines with respect to the Development and Commercialization of SMA Program Products over the next [**] period, which report shall set forth the anticipated timing for any milestone achievements and major inflection points (e.g., [**]), during such [**] period with respect to the SMA Program. The Alliance Managers will coordinate meetings to be held within [**] following receipt of such written reports to discuss the contents of such reports, with each Party providing the appropriate personnel to address any reasonable inquiries of the other Party.

2.3 Designation of Voyager Capsids as SMA Program Capsids. During the Term, after Evaluation of any Voyager Capsid, Novartis may designate any number of such Voyager Capsids as an SMA Program Capsid by providing written notice to Voyager, in accordance with Section 13.7, identifying such Voyager Capsid(s) (a "Capsid Designation Notice"). Upon Voyager's receipt of each Capsid Designation Notice (the "Designation Notice Date") each Voyager Capsid identified in the corresponding Capsid Designation Notice will be deemed an "SMA Program Capsid". Promptly following receipt of Novartis' Capsid Designation Notice, Voyager will provide Novartis with any Voyager Know-How for the corresponding SMA Program Capsid that has not been previously provided to Novartis as may be reasonably necessary or that the Parties mutually agree may be useful to enable Novartis to Exploit such SMA Program Capsid for use in an SMA Program Product; provided that Voyager shall not provide Novartis with any Know-How that is not reasonably necessary for Exploiting an SMA Program Capsid (such Know-How, "Other Know-How") without Novartis' prior written consent. In the event Voyager provides Other Know-How without Novartis' prior written consent, then (a) the provision of such Other Know-How will not be deemed a breach of this Agreement, and (b) Novartis shall have the right to use such Other Know-How for Development of SMA Program Products unless and until Voyager notifies Novartis of any inadvertent disclosure of Other Know-How, in which event Novartis shall destroy all such Other Know-How to the extent not previously relied upon in the Development of SMA Program Products.

2.4 SMA Development Candidates. On and after the Designation Notice Date, Novartis shall be solely responsible, in its sole discretion (but subject to its obligations under this Agreement (including Section 6.2.1)), for Developing SMA Development Candidates, including the selection of an SMA Program Payload to utilize with an SMA Program Capsid. Novartis shall notify Voyager in writing promptly upon (in any event, no greater than [**] after) the designation of any SMA Development Candidate.

2.5 SMA Program Costs. Each Party will conduct its activities under the SMA Program at its own cost and expense.

2.6 Alliance Managers. Alliance Managers will be appointed for the SMA Program in accordance with Section 4.5.

ARTICLE 3
HD PROGRAM

3.1 HD Program Plan. The HD Program Plan sets forth (and will at all times set forth): (a) a detailed summary of the anticipated activities for each Party over the next [**] period; (b) an overview of the anticipated activities and timeline with respect to Development and Commercialization of HD Program Product(s) over the next [**] period, which shall set forth the anticipated timing for all significant Development and Commercialization events, including milestone achievement (collectively, the "HD Program Timelines"); and (c) a budget for all of Voyager's activities under the HD Program (the "Development Budget"). Until completion of the HD Program Plan, the JSC will, prior to the end of each [**], review the HD Program Plan and determine whether to update such plan, and shall prepare a detailed budget under the HD Program Plan for the subsequent [**]. A Party may also develop and submit to the JSC from time-to-time proposed amendments to the HD Program Plan. The JSC shall review such proposed amendments and may approve such proposed amendments or any other proposed amendments that the JSC may consider from time to time in its discretion and, upon any such approval by the JSC, the HD Program Plan shall be deemed amended accordingly.

3.2 HD Program Responsibilities. Each Party shall have the respective responsibilities assigned to it under the HD Program Plan. Voyager will be primarily responsible for Developing Voyager Capsids for the HD Program and discovering HD Development Candidates in accordance with the HD Program Plan until the first IND for an HD Program Product is filed. After the first IND for an HD Program Product is filed, Novartis will be solely responsible for further Development and Commercialization of each HD Program Product and all HD Development Candidates. Novartis will be responsible for filing any IND for an HD Program Product, and Voyager will provide reasonable cooperation, including providing supporting activities as specified in the HD Program Plan. Notwithstanding a Party's responsibility, the Parties may collaborate on animal studies and certain other preclinical work with respect to the HD Program as specified in the HD Program Plan. The Parties shall conduct their respective activities as set forth in the HD Program Plan and shall use Commercially Reasonable Efforts to do so in accordance with the timelines and budgets set forth therein.

3.3 Reporting Obligations. On a [**] basis until the first IND for an HD Program Product is filed, in advance of each regularly-scheduled JSC meeting, each Party that conducted activities under the HD Program Plan in such [**] shall provide the JSC with reports describing the progress and results achieved by such Party since the last report and the overall progress with respect to such Party's activities under the HD Program Plan and HD Program Timelines. Following the first IND for an HD Program Product, Novartis shall, during the Term until the First Commercial Sale of an HD Program Product and on [**] basis (every [**]), provide a written report setting forth a non-binding overview of the anticipated timelines with respect to the Development and Commercialization of HD Program Products over the next [**] period, which

shall set forth the anticipated timing for all milestone achievements and major infection points during such [**] period with respect to the HD Program. The Alliance Managers will coordinate meetings to be held within [**] following receipt of such written reports to discuss the contents of such reports, with each Party providing the appropriate personnel to participate in such meeting.

3.4 HD Development Candidate(s). If Voyager determines that any HD Candidate arising out of the HD Program meets the HD DC Criteria, Voyager will notify the JSC. In such event, the JSC will meet to review and discuss the data regarding such HD Candidate, determine whether such HD Candidate meets the HD DC Criteria and determine whether to elect such HD Candidate as an HD Development Candidate. If [**], then such HD Candidate will thereafter be deemed to be an HD Development Candidate hereunder.

3.5 Voyager HD Program Costs. Novartis shall be responsible for all Voyager HD Program Costs in accordance with Section 7.7.

ARTICLE 4
MANAGEMENT OF THE HD PROGRAM

4.1 Joint Steering Committee. The Parties hereby establish a Joint Steering Committee (the "JSC") to serve as: (a) the oversight and decision-making body for the activities under the HD Program Plan for the HD Program; and (b) a forum for information exchange and discussion with respect to all other activities under the HD Program; in each case ((a) and (b)) as more fully described in this ARTICLE 4. The Parties anticipate that the JSC will not be involved in day-to-day implementation of the activities under the HD Program but shall have the responsibilities and decision-making authority set forth herein or as mutually agreed by the Parties in writing from time to time. The JSC may establish subcommittees as set forth in Section 4.1.2 (each a "Subcommittee").

4.1.1 Responsibilities. The JSC shall perform the following functions, subject to the limitations and final decision-making authority of the respective Parties as set forth in Section 4.4:

(a) serve as an information transfer vehicle to facilitate discussions regarding the Development of HD Candidates and progress under the HD Program Plan, including the HD Program Timelines, with respect to the HD Program;

(b) finalize the HD DC Criteria within [**] after the Effective Date;

(c) review and determine whether to update the HD Program Plan (including the Development Budget) at the end of each [**], or at other times, in accordance with Section 3.1;

(d) review and approve any substantive amendments to the HD Program Plan proposed by a Party, including any amendments to the Development Budget;

(e) review and discuss progress reports on the Parties' activities under the HD Program Plan as submitted by each Party, including the reports submitted under Section 3.3 and the progress with respect to the HD Program Timelines;

(f) address any issues or disputes arising between the Parties regarding the conduct of the Parties' activities under the HD Program Plan;

(g) review and discuss HD Program Product formulation and formulation optimization;

(h) form such Subcommittees as it deems necessary to achieve the objectives and intent of the HD Program in accordance with this Agreement; and

(i) perform such other responsibilities as may be expressly assigned to the JSC pursuant to this Agreement or as may be mutually agreed upon in writing by the Parties (through their authorized representatives outside of the JSC) in writing from time to time.

Notwithstanding anything to the contrary, the JSC shall not have any authority beyond the specific matters set forth in this Section 4.1.1, and the JSC's authority will be subject to the limitations set forth in Section 4.4.4.

4.1.2 Formation and Dissolution of Subcommittee(s). The JSC may, in its discretion, establish Subcommittees from time to time to handle specific matters within the scope of the JSC's area of authority and responsibility, and no Subcommittee's authority and responsibility may be greater than that of the JSC itself. Each Subcommittee shall have such authority and responsibility as determined by the JSC from time to time, and decisions and recommendations of any Subcommittee shall be made in accordance with Section 4.4. The JSC shall determine when each Subcommittee it forms shall be dissolved.

4.1.3 Membership. Each Committee shall be composed of an equal number of representatives appointed by each of Voyager and Novartis. The JSC shall be comprised of [**] representatives of each Party, and each other Committee shall be comprised of such number of representatives of each Party as is agreed upon by the Parties. Each individual appointed by a Party as a representative to the JSC shall be an employee of such Party. Each individual appointed by a Party as a representative to any Subcommittee shall be an employee of such Party, an employee of such Party's Affiliate or, upon the other Party's approval, a contractor to such Party or its Affiliate. Each Party may replace any of its Committee representatives at any time upon written notice to the other Party, which notice may be given by e-mail sent to the other Party's co-chairperson of such Committee. Each Committee shall be co-chaired by one designated representative of each Party. Any member of a Committee may designate a substitute who is an employee of the applicable Party to attend and perform the functions of that member at any meeting of such Committee, as applicable. Notwithstanding the foregoing, each Party shall ensure at all times during the existence of a Committee that its representatives (including any replacements or substitutes therefor) on such Committee are appropriate in terms of seniority, experience, expertise and decision-making authority and are subject to obligations of confidentiality and non-use with respect to the other Party's Confidential Information that are no less stringent than those set forth in ARTICLE 9.

4.2 JSC Meetings. The co-chairpersons shall be responsible, with respect to their Committee, for: (a) calling meetings; (b) preparing and circulating an agenda in advance of each meeting; provided that the co-chairpersons shall include any agenda items proposed by either Party

on such agenda; (c) ensuring that all decision-making is carried out in accordance with the voting and dispute resolution mechanisms set forth in this Agreement; and (d) preparing and issuing minutes of each meeting within [**] (or such shorter time as is agreed by the relevant Committee) thereafter. The location of regularly scheduled meetings shall alternate between Voyager's offices and Novartis' offices located in the Boston, Massachusetts area, unless otherwise agreed by such Committee. Such Committee may also determine that a meeting will instead be held telephonically, by video conference or by any other media; provided, however, that the JSC shall hold at least [**] in person each [**] unless the Parties mutually agree otherwise. Each Party may designate the same individual as a representative on more than one Committee. Each Party will bear all expenses it incurs in regard to participating in all meetings of each Committee, including all travel and lodging expenses.

4.3 Disbandment of the JSC. The JSC shall meet [**] until the first IND for an HD Program Product is filed. Following the filing of the first IND for an HD Program Product the JSC will automatically be fully dissolved and shall have no further responsibilities or authority under this Agreement (unless otherwise agreed by the Parties in writing). Thereafter, the exchange of information under this Agreement related to the progress of the HD Program shall be made through the Alliance Managers and in accordance with Section 3.3.

4.4 Decision-Making.

4.4.1 Escalation to the JSC. Except as otherwise expressly provided herein, all decisions of each Committee shall be made by consensus, with all of a Party's voting members collectively having one (1) vote. If a Subcommittee is incapable of reaching unanimous agreement on a matter before it within [**] after first attempting to decide such matter, the matter shall be referred to the JSC for resolution. Unless the Parties mutually agree otherwise, the JSC shall attempt to reach unanimous agreement on any matter within the scope of its authority within [**] after first attempting to decide such matter. If the JSC does not resolve such matter as set forth in this Section 4.4.1, then either Party may escalate the matter to the Executive Officers for resolution in accordance with Section 4.4.2. Notwithstanding the foregoing, matters in dispute for which greater exigency is required, may be escalated more quickly as agreed by the Parties.

4.4.2 Escalation to the Executive Officers. The Parties' respective Executive Officers shall meet within [**] after a matter within the scope of the JSC's authority is referred to them for resolution pursuant to Section 4.4.1, and shall negotiate in good faith to attempt to resolve the matter. If the Executive Officers are unable to resolve such matter within [**] after the matter is referred to them, then the matter will be determined in accordance with Section 4.4.3.

4.4.3 Final Decision Making Authority. With respect to any matter within the scope of the JSC's authority that remains unresolved after escalation to the Executive Officers under Section 4.4.2 [**].

4.4.4 Limitations on Scope and Final Decision-Making Authority. In no event shall any Committee or any Party alone have the power or authority to: (i) amend or modify this Agreement, or waive compliance with its terms; (ii) determine whether a Party has fulfilled or breached its obligations under this Agreement; (iii) impose any requirements on either Party to undertake obligations beyond those for which it is responsible, or to forgo any of its rights, under

this Agreement; (iv) make a decision that is expressly stated under this Agreement to require the mutual agreement of the Parties; (v) make a decision that could reasonably be expected to cause Voyager to breach an In-License Agreement or give rise to the right of the applicable Inbound Licensor to take any action under such In-License Agreement; or (vi) require any Party to perform any act that it reasonably believes to be inconsistent with any Law. In addition, Novartis will not have the right to exercise any final decision-making authority to: [**]. Any decision made by the Executive Officers in accordance with Section 4.4.2 or by a Party in accordance with this Section 4.4 shall be considered a decision made by the JSC.

4.5 Alliance Managers. Within [**] after the Effective Date, each Party will appoint an individual to act as an alliance manager for such Party (each, an "Alliance Manager") for each Program. A Party may appoint the same individual to be the Alliance Manager for both Programs. The Alliance Managers will be the primary point of contact for the Parties under this Agreement. The name and contact information for each Party's Alliance Manager, as well as any replacement chosen by such Party, in its sole discretion, from time to time, will be promptly provided to the other Party in writing. Each Party may change its designated Alliance Manager at any time upon written notice to the other Party; provided that each Party will maintain an Alliance Manager throughout the duration of the Term. The Parties may mutually agree in writing to eliminate the requirement to maintain an Alliance Manager at any time.

4.6 Authority. Each Party will retain the rights, powers and discretion granted to it under this Agreement and no such rights, powers or discretion will be delegated to or vested in the JSC or any other Subcommittee unless such delegation or vesting of rights is expressly provided for in this Agreement or the Parties expressly so agree in writing.

ARTICLE 5
GRANT OF LICENSES

5.1 Licenses to Novartis.

5.1.1 SMA Program Evaluation License. Subject to the terms and conditions of this Agreement, Voyager hereby grants to Novartis and its Affiliates, and Novartis hereby accepts, a non-exclusive (subject to Section 10.5), non-transferable (except in accordance with Section 13.4), non-sublicensable (except in the case of contractors performing services related to Evaluation for or on behalf of Novartis), worldwide, royalty-free right and license during the Term, under the Capsid Patents and Voyager Know-How, to Evaluate each Voyager Capsid during the Capsid Evaluation Period for use with the SMA Target and to determine whether to name a Voyager Capsid as an SMA Program Capsid in accordance with Section 2.3.

5.1.2 SMA Program Exclusive License from Voyager to Novartis.

(a) Subject to the terms and conditions of this Agreement, Voyager hereby grants to Novartis and its Affiliates, and Novartis hereby accepts, an exclusive (even as to Voyager), sublicensable (in accordance with Section 5.3), non-transferable (except in accordance with Section 13.4), royalty-bearing, right and license, under the Licensed Patents and Voyager Know-How, to Exploit SMA Program Capsid(s) and SMA Program Payloads as incorporated into SMA Program Products in the Field and in the Territory during the Term.

(b) In the event Novartis assigns any SMA Program Product Patent to Voyager in accordance with Section 8.3.7, Voyager hereby grants to Novartis and its Affiliates an exclusive (even as to Voyager), non-transferable (except in accordance with Section 13.4), perpetual, non-revocable, world-wide, sub-licensable license under such assigned SMA Program Product Patent for all purposes, including Exploitation of any Capsid. The foregoing license shall be royalty free, except with respect to any granted and valid independent claim of such assigned SMA Program Product Patent that recites the SMA Program Capsid sequence or that otherwise relies upon recitation of the SMA Program Capsid sequence for the novelty or non-obviousness of the claim (other than those SMA Program Product Patents assigned under Section 8.3.7(ii)), in which case Novartis shall be responsible for all payment obligations as would apply to a Licensed Patent.

Notwithstanding anything to the contrary, the exclusive licenses in this Section 5.1.2 are exclusive solely as each relates to the Exploitation of a Licensed Product in relation to the SMA Target.

5.1.3 HD Program Exclusive License from Voyager to Novartis.

(a) Subject to the terms and conditions of this Agreement, Voyager hereby grants to Novartis and its Affiliates, and Novartis hereby accepts, an exclusive (even as to Voyager), non-transferable (except in accordance with Section 13.4), sublicensable (in accordance with Section 5.3), royalty-bearing, right and license, under the Licensed Patents and Voyager Know-How, to Exploit HD Program Products in the Field in the Territory during the Term. The foregoing license shall be subject to Voyager's retained rights under the Voyager IP to conduct the activities allocated to Voyager under the HD Program Plan or otherwise under Section 5.4 through Section 5.5 and Section 10.5 of this Agreement.

(b) In the event Novartis assigns any HD Program Product Patent to Voyager in accordance with Section 8.3.7, Voyager hereby grants to Novartis and its Affiliates an exclusive (even as to Voyager), non-transferable (except in accordance with Section 13.4), perpetual, non-revocable, world-wide, sub-licensable license under such assigned HD Program Product Patent for all purposes, including Exploitation of any Capsid. The foregoing license shall be royalty free, except with respect to any granted and valid independent claim of such assigned HD Program Product Patent that recites the HD Program Capsid sequence or that otherwise relies upon recitation of the HD Program Capsid sequence for the novelty or non-obviousness of the claim (other than those HD Program Product Patents assigned under Section 8.3.7(ii)), in which case Novartis shall be responsible for all payment obligations as would apply to a Licensed Patent.

5.1.4 Right of Reference. Voyager hereby grants to Novartis a "Right of Reference," as that term is defined in 21 C.F.R. § 314.3(b) (or any analogous Law recognized outside of the United States), to all data Controlled by Voyager or its Affiliates that relates to any Licensed Capsid or Licensed Product solely for purposes of seeking Regulatory Approval for Licensed Products, and Voyager shall provide a signed statement to this effect, if requested by Novartis, in accordance with 21 C.F.R. § 314.50(g)(3) (or any analogous Law outside of the United States).

5.2 In-License Agreements.

5.2.1 Scope of Rights under In-License Agreements; Compliance. [**]. To the extent Patents under the In-License Agreements are sublicensed to Novartis hereunder, Novartis covenants to comply with, and to cause its Affiliates and Sublicensees to comply with the In-License Agreements pursuant to their terms. To the extent there is a conflict between any of the terms of any In-License Agreement and the rights granted to Novartis hereunder (including with respect to any sublicensing rights, Prosecution and Maintenance, enforcement and defense rights) the terms of such In-License Agreement shall control with respect to the Know-How and Patents licensed to Voyager under such In-License Agreement.

5.2.2 Related Third Party IP. If either Party becomes aware of any Third Party's Know-How that would be necessary or reasonably useful for the Exploitation of an HD Candidate or HD Program Product, or any Third Party's Patent that Covers any HD Candidate or HD Program Product in the Territory ("Related Third Party IP"), such Party shall promptly notify the other Party, and the Parties shall discuss whether to seek a license under such Related Third Party IP, subject to Section 5.2.3 and Section 5.2.4. Voyager shall have the first right (in Voyager's sole discretion) to enter into Third Party licenses for Related Third Party IP that Covers a Voyager Capsid with or without a payload. Nothing contained in this Section 5.2.2 creates an obligation for Voyager to obtain any license from a Third Party.

5.2.3 Future In-Licenses. If, after the Effective Date, Voyager or any of its Affiliates enters into a Future In-License Agreement with a Third Party pursuant to which Voyager or its Affiliate obtains Control over a Third Party's Know-How or Patents that would be included within Voyager IP, Voyager shall promptly provide such Future In-License Agreement to Novartis and provide any information reasonably requested by Novartis for Novartis to evaluate the rights licensed under such agreement. If Novartis agrees in writing to pay the share of the payments due to Inbound Licensors applicable to any Licensed Product (including any candidate to be a Licensed Product and any payload component of a Licensed Product, but excluding amounts payable with respect only to the Voyager Capsid component of the Licensed Product), as well as a reasonably allocable share of any other payments due to Inbound Licensors not specific to a compound or product, in each case only as and to the extent set forth in Section 5.2.4, then (and only then) will the Third Party's Know-How or Patents under the Future In-License Agreement be included in the license granted to Novartis under Section 5.1.3, in which case it will be considered Voyager IP hereunder.

5.2.4 Payments Related to In-License Agreements. As between the Parties, the amounts payable under all In-License Agreements shall be allocated as follows: [**].

5.2.5 Reporting Under the In-License Agreements. Novartis shall prepare and deliver to Voyager any additional reports required under the applicable In-License Agreements of Voyager, in each case to the extent requested by Voyager, and, provided that Voyager has notified Novartis reasonably sufficiently in advance of the applicable deadline, to enable Voyager to comply with its obligations under the applicable In-License Agreements.

5.2.6 Voyager Obligations under the In-License Agreements. Except as otherwise mutually agreed by the Parties in writing or contemplated by this Agreement, Voyager shall timely

perform its obligations under each of the Existing In-License Agreements and any Future In-License Agreements in all material respects and maintain them in full force and effect during the Term (subject to the expiration provisions of each Existing In-License Agreement and any Future In-License Agreements), including timely payment of all amounts due to be paid by Voyager thereunder. Voyager will not knowingly breach any Existing In-License Agreement or Future In-License Agreement. Except as otherwise mutually agreed by the Parties in writing or contemplated by this Agreement, Voyager shall not modify or amend any Existing In-License Agreement and any Future In-License Agreements during the Term in a manner that would or would reasonably be expected to adversely affect Novartis in any material respect without Novartis' advance, written consent. During the Term, Voyager will not exercise, waive, release, or assign any rights under any Existing In-License Agreement or any Future In-License Agreement in any manner that would limit, restrict or otherwise adversely affect in any material respect the rights granted to Novartis hereunder, in each case, without obtaining Novartis' prior written consent. As of the Effective Date, Voyager and its Affiliates are not parties to any agreement with a Third Party other than the Existing In-License Agreements pursuant to which Voyager or its Affiliates obtained rights to Voyager Capsids, Licensed Patents or Voyager Know-How.

5.3 Novartis' Sublicensing Rights. Novartis and its Affiliates will have the right to grant and authorize sublicenses through multiple tiers under the rights granted to it under this Agreement by Voyager, including Section 5.1.2 and Section 5.1.3, for the Exploitation of a Licensed Product (each such Third Party, a "Sublicensee"). Novartis will use Commercially Reasonable Efforts to include in each Sublicense, an obligation of the Sublicensee to provide Novartis with written notice of its achievement of a Development Milestone Event within [**] after such Sublicensee achieves the Development Milestone Event. Within [**] following execution of a sublicense with a Sublicensee (a "Sublicense"), Novartis will provide Voyager with a fully executed copy of the corresponding Sublicense, which copy may be redacted by Novartis to remove confidential or commercially sensitive information and any other information that is not necessary to demonstrate compliance with the terms of this Agreement. Each sublicense will be consistent with the terms of this Agreement. During the Term, Novartis will be responsible for any act or omission by a Sublicensee that would be a breach of this Agreement if such act or omission had been engaged in by Novartis. Novartis shall remain responsible for the payment to Voyager of all Development Milestone Payments, Sales Milestone Payments, and royalties that are payable with respect to the Development Milestone Event(s) achieved by, or the Net Sales of, a Licensed Product made by such Sublicensees.

5.4 Licenses to Voyager. Subject to the terms and conditions of this Agreement, Novartis hereby grants to Voyager, and Voyager accepts, a non-exclusive, royalty-free, non-transferable (except in accordance with Section 13.4), sublicensable right and license, under the Novartis IP disclosed or is required to be disclosed by or on behalf of Novartis to Voyager in connection with this Agreement, solely to conduct the Development activities allocated to Voyager under the HD Program Plan in accordance with this Agreement.

5.5 Scope of Exclusivity. Notwithstanding anything to the contrary set forth in this Agreement, (a) the exclusive licenses and exclusivity covenants set forth in this Agreement will not prevent Voyager from internal Development activities relating to the Voyager Capsids; and (b) nothing in this Agreement will prevent Voyager from Exploiting (or granting rights to an

Affiliate or Third Party to Exploit) any Licensed Capsid for use with payloads intended to have a therapeutic effect on targets other than the SMA Target and the HD Targets.

5.6 No Other Rights. Except as otherwise expressly provided in this Agreement, under no circumstances will a Party, as a result of this Agreement, obtain any ownership interest, license right or other right in any Know-How, Patent, or other intellectual property rights of the other Party or any of its Affiliates, including items owned, controlled, developed, or acquired by the other Party or any of its Affiliates, or provided by the other Party to the first Party at any time pursuant to this Agreement.

ARTICLE 6
DEVELOPMENT, REGULATORY AND COMMERCIALIZATION ACTIVITIES

6.1 Novartis Authority and Obligations.

6.1.1 SMA Program. Novartis will be solely responsible for, and have sole decision-making authority with respect to, at its own expense, the Exploitation of SMA Development Candidates, SMA Program Products and SMA Program Capsids and as they are used to Exploit an SMA Program Product.

6.1.2 HD Program. From and after the first IND filing for the HD Program, Novartis will be solely responsible for the Development and Commercialization of HD Program Products, including all further pre-clinical and clinical Development and any Commercialization of the HD Development Candidates and HD Program Products.

6.2 Diligence.

6.2.1 SMA Program Diligence. Novartis will use Commercially Reasonable Efforts to Develop and obtain Regulatory Approval for, and after receipt of Regulatory Approval Commercialize, at least one (1) SMA Program Product in (a) the United States, (b) [**]. Except for the foregoing, Novartis will have no other diligence obligations with respect to the Development or Commercialization of SMA Program Products under this Agreement.

6.2.2 HD Program Diligence. Upon completion of the HD Program Plan,Novartis will use Commercially Reasonable Efforts to Develop and obtain Regulatory Approval for, and after receipt of Regulatory Approval Commercialize, at least one (1) HD Program Product in (a) the United States and (b) [**]. Except for the foregoing, Novartis will have no other diligence obligations with respect to the Development and Commercialization of HD Program Products under this Agreement.

6.2.3 Exceptions to Diligence Obligations. Notwithstanding any provision of this Agreement to the contrary, Novartis will be relieved of its diligence obligations under this Agreement with respect to any Licensed Product to the extent that any of the following occurs with respect to such Licensed Product:

(a) Novartis or Voyager receives, generates, or otherwise becomes aware of, any safety, tolerability, or other data reasonably indicating or signaling that a Licensed

Capsid or Licensed Product has or would have an unacceptable risk-benefit profile or is otherwise not reasonably suitable for initiation or continuation of Clinical Trials; or

(b) Novartis or Voyager receive any notice, information or correspondence from any applicable Regulatory Authority, or any applicable Regulatory Authority takes any action, that reasonably indicates that a Licensed Product is unlikely to receive Regulatory Approval.

6.2.4 Deemed Satisfaction of Novartis's Diligence Obligations. Without in any way expanding Novartis's obligations under this Agreement:

(a) Novartis's achievement of any Development Milestone Event entitling Voyager to receive a specific Development Milestone Payment described in Section 7.2.2 will be conclusive evidence that Novartis has satisfied all of its diligence obligations under this Agreement for the corresponding Licensed Product, up to the point such Development Milestone Event is achieved; and

(b) Novartis's payment, and Voyager's acceptance, of any Sales Milestone Payment as set forth in Section 7.2.3 will be conclusive evidence that Novartis has satisfied all its diligence obligations under this Agreement for the corresponding Licensed Product up to the date of the achievement of such milestone and if Voyager does not return in full a Sales Milestone Payment by Novartis with a written rejection of such payment within [**] of receipt, Voyager shall be deemed to have accepted such Sales Milestone Payment.

(c) For the avoidance of doubt, the provisions of Section 6.2.4 are intended only as examples of diligence constituting satisfaction of Novartis's diligence obligations. Novartis may fully satisfy its diligence obligations without achieving any of the specific diligence examples set forth in Section 6.2.4, *provided that* Novartis otherwise complies with the provisions of Section 6.2.1 or Section 6.2.2, as applicable.

6.2.5 Assertion of Novartis Diligence Obligation Claims. If Voyager is or becomes aware of facts that might form a reasonable basis to allege that Novartis has failed to meet any of its diligence obligations, then Voyager will promptly notify Novartis in writing of such potential alleged performance failure (each such potential alleged performance failure, a "Diligence Issue"). Promptly upon Novartis' receipt of any notice of a Diligence Issue pursuant to this Section 6.2.5, the Novartis Alliance Manager will contact the Voyager Alliance Manager to discuss the specific nature of such Diligence Issue and seek to identify an appropriate corrective course of action. If, no later than [**] after Novartis' receipt of such a notice, (i) the Parties have not reached consensus regarding whether Novartis has failed to satisfy its obligations pursuant to Section 6.2.1 or Section 6.2.2 and (ii) the Parties have not agreed upon an appropriate corrective course of action for such Diligence Issue, then such Diligence Issue will be escalated and resolved pursuant to the dispute resolution provisions set forth in Section 13.2. If Voyager fails to notify Novartis of a Diligence Issue (which notification may accomplished through JSC or Alliance Manager discussions) pursuant to this Section 6.2.5 within [**] after the date that Voyager first discovers such Diligence Issue, then Novartis will be deemed to have satisfied its obligations under Section 6.2.1 or Section 6.2.2 (as applicable) with respect to such Diligence Issue.

6.2.6 Remedies for Breach of Novartis Diligence Obligations. If Novartis materially breaches any of its diligence obligations under Section 6.2.1 or Section 6.2.2, and Novartis fails to timely remedy such breach within [**] after Novartis' receipt of notice of such breach from Voyager (or if such breach is not reasonably curable within [**] and if Novartis is making a *bona fide* effort to cure such breach, within a time period to be agreed by both Parties in order to permit Novartis a reasonable period of time to cure such breach, the time to remedy such breach shall be extended for a time period to be agreed by both Parties in order to permit Novartis a reasonable period of time to cure such breach), then Voyager may, in its sole discretion, elect to either: (i) terminate this Agreement pursuant to the provisions of Section 12.2.1 on a Program-by-Program or country-by-country basis; or (ii) convert any exclusive license or sublicense granted to Novartis under this Agreement with respect to the applicable terminated Program in any applicable country into a non-exclusive license (in which event the exclusivity obligations with respect to the applicable Program in the applicable country under Section 10.5 will immediately terminate).

6.2.7 Cooperation. Upon Novartis' request and at Novartis' expense, Voyager will provide Novartis with reasonable assistance in connection with Novartis' preparation of any portion(s) of the relevant Regulatory Filings that relate to the Licensed Products, including by providing relevant data in Voyager's possession and participating in meetings between the Parties to prepare documents to be filed.

6.3 Remedy for Novartis Deprioritizing a SMA Program Capsid. Without limiting Novartis' obligations under Section 6.2.1, if, (a) during the period beginning on the Designation Notice Date of an SMA Program Capsid and ending on the date of First Commercial Sale by Novartis of an SMA Program Product in the United States and at least [**] (whichever is later), Novartis Advances an AAV Gene Therapy Product candidate incorporating an SMA Program Payload for Development with a Capsid other than an SMA Program Capsid, and (b) Novartis does not Advance an AAV Gene Therapy Product candidate incorporating an SMA Program Capsid and an SMA Program Payload in its Development efforts for any continuous [**] period (including efforts aimed at continuing Development through an Affiliate or Sublicensee), as indicated in Novartis' [**] report made under Section 2.2, then, within [**] after receiving such report, Voyager shall notify Novartis in accordance with Section 13.7 of any objection it has to Novartis' decision to not Advance an AAV Gene Therapy Product candidate incorporating an SMA Program Capsid and an SMA Program Payload. If Voyager timely notifies Novartis of its objection, Novartis shall, by written notice to Voyager within [**] after Novartis' receipt of Voyager's notice, elect in Novartis' sole discretion to either (i) promptly Advance an AAV Gene Therapy Product candidate that incorporates the SMA Program Capsid and an SMA Program Payload, or (ii) [**] to Novartis under this Agreement [**], in which event (A) all subsequent development obligations under Section 6.2.1 will terminate for the corresponding SMA Program Product, (B) the exclusivity obligations with respect to the SMA Target under Section 10.5 will immediately terminate, and (C) all amounts for the corresponding SMA Program Product that would be due hereunder shall be reduced by [**] percent ([**]%). For the avoidance of doubt, nothing in this Section 6.3 shall be deemed to apply to any AAV Gene Therapy Product for which the First Commercial Sale has already occurred in the United States and at least [**] (whichever is later).

6.4 Compliance. All activities to be conducted by a Party under this Agreement will be conducted in compliance with applicable Laws.

ARTICLE 7
UPFRONT CONSIDERATION; MILESTONES AND ROYALTIES; PAYMENTS

7.1 Upfront Consideration.

7.1.1 Upfront Fee. Novartis will pay Voyager an initial, one-time, non-refundable, non-creditable payment of Eighty Million Dollars ($80,000,000) within ten (10) Business Days after receipt of an Invoice from Voyager to be issued no earlier than the Effective Date. Such upfront cash payment shall be allocated by Voyager as set forth on Schedule 7.1.1.

7.1.2 Equity Purchase. As of the Effective Date, Voyager and Novartis entered into a stock purchase agreement (the "**Stock Purchase Agreement**") whereby Novartis agreed to purchase Twenty Million Dollars ($20,000,000) of Voyager common stock.

7.2 Milestone Payments.

7.2.1 Generally. Novartis will provide Voyager with written notice (a "Development Milestone Event Notice") of the achievement of a development milestone event specified in Section 7.2.2 for each of the first SMA Program Product and the first HD Program Product to achieve such milestone event (each, a "Development Milestone Event"). Such notice will be provided within [**] after such Development Milestone Event is achieved; provided that in the case such Development Milestone Event is achieved by a Sublicensee, Novartis' notice shall be provided within [**] after Novartis receives notice from the corresponding Sublicensee of achieving the Development Milestone Event.

7.2.2 Development Milestone Events and Payments. Novartis will pay the milestone payments set forth below for the first SMA Program Product and the first HD Program Product to achieve such Development Milestone Event (each, a "Development Milestone Payment"). After receipt of a Development Milestone Event Notice, Voyager shall submit an Invoice to Novartis with respect to the corresponding Development Milestone Payment. If for any reason a Development Milestone Event (c) below does not occur prior to the occurrence of Development Milestone Event (d) below, then Development Milestone Event (c) will be deemed to occur concurrently with the occurrence of Development Milestone Event (d), and the Development Milestone Payments associated with both Development Milestone Events will be paid following the achievement of Development Milestone Event (d).

	Development Milestone Event	**Development Milestone Payment**	
		SMA Program	**HD Program**
(a)	[**]	[**] Dollars ($[**])	[**] Dollars ($[**])
(b)	[**]	[**] Dollars ($[**])	[**] Dollars ($[**])

	Development Milestone Event	Development Milestone Payment	
		SMA Program	HD Program
(c)	[**]	[**] Dollars ($[**])	[**] Dollars ($[**])
(d)	[**]	[**] Dollars ($[**])	[**] Dollars ($[**])
(e)	[**]	[**] Dollars ($[**])	[**] Dollars ($[**])
(f)	[**]	[**] Dollars ($[**])	[**] Dollars ($[**])
	Totals	**Two Hundred Million Dollars ($200,000,000)**	**Two Hundred Twenty Five Million Dollars ($225,000,000)**

*The Development Milestones in (e) and (f) above will be deemed to have been achieved upon the occurrence of [**]. Each of the Development Milestone Payments set forth above will be payable one time only.

7.2.3 Sales Milestones. Novartis will pay to Voyager sales milestones with respect to the aggregate Annual Net Sales of all Licensed Products for the first occurrence of each milestone event as follows:

	Milestone Event	Sales Milestone Payment	
		SMA Product Program	HD Product Program
(a)	First Calendar Year with aggregate Annual Net Sales exceeding [**] Dollars ($[**])	[**] Dollars ($[**])	[**] Dollars ($[**])
(b)	First Calendar Year with aggregate Annual Net Sales exceeding [**] Dollars ($[**])	[**] Dollars ($[**])	[**] Dollars ($[**])
(c)	First Calendar Year with aggregate Annual Net Sales exceeding [**] Dollars ($[**])	[**] Dollars ($[**])	[**] Dollars ($[**])
(d)	First Calendar Year with aggregate Annual Net Sales exceeding [**] Dollars ($[**])	[**] Dollars ($[**])	[**] Dollars ($[**])

	Milestone Event	Sales Milestone Payment	
		SMA Product Program	**HD Product Program**
	Totals	**Four Hundred Million Dollars ($400,000,000)**	**Three Hundred Seventy Five Million Dollars ($375,000,000)**

Upon receipt of a royalty report under Section 7.6 indicating that a sales milestone has been achieved with respect to a Program, Voyager shall submit an Invoice to Novartis for the applicable sales milestone. Upon receipt of such Invoice, Novartis will pay Voyager the foregoing sales milestones in accordance with Section 7.3.

7.3 Invoicing and Payment Procedure. Voyager shall submit an Invoice to Novartis for all amounts due to it under this Agreement. Unless otherwise noted, all fees owed to Voyager will be payable within [**] after Novartis' receipt of an Invoice from Voyager. All invoices will be delivered to Novartis by email to [**] or other email address as specified by Novartis. All invoice or billing related questions should be referred to Novartis' finance department at [**] or other email address as specified by Novartis. All payments due from Novartis to Voyager pursuant to this Agreement shall be made in U.S. dollars by wire transfer to the bank account of Voyager set forth in Schedule 7.3 (subject to confirmation) or to another bank account of Voyager specified in writing to Novartis and, in each case, in accordance with such instructions as are provided by Voyager to Novartis from time to time.

7.4 Royalties.

7.4.1 Royalties on Licensed Products Sold. Subject to the adjustments under Section 7.5, Novartis will make tiered royalty payments to Voyager in respect of Annual Net Sales, on a Licensed Product-by-Licensed Product basis, at the following royalty rates during the applicable Royalty Term:

	Annual Net Sales of an SMA Program Product in the Territory during a Calendar Year during the Royalty Term	Royalty Rate
(a)	Annual Net Sales of an SMA Program Product less than or equal to [**] Dollars ($[**])	[**]%
(b)	Annual Net Sales of an SMA Program Product greater than [**] Dollars ($[**]) but less than or equal to [**] Dollars ($[**])	[**]%

	Annual Net Sales of an SMA Program Product in the Territory during a Calendar Year during the Royalty Term	Royalty Rate
(c)	Annual Net Sales of an SMA Program Product greater than [**] Dollars ($[**])	[**]%

	Annual Net Sales of an HD Program Product in the Territory during a Calendar Year during the Royalty Term	Royalty Rate
(a)	Annual Net Sales of an HD Program Product less than or equal to [**] Dollars ($[**])	[**]%
(b)	Annual Net Sales of an HD Program Product greater than [**] Dollars ($[**]) but less than or equal [**] Dollars ($[**])	[**]%
(c)	Annual Net Sales of an HD Program Product greater than [**] Dollars ($[**]) but less than or equal to [**] Dollars ($[**])	[**]%
(d)	Annual Net Sales of an HD Program Product greater than [**] Dollars ($[**]) but less than or equal to [**] Dollars ($[**])	[**]%
(e)	Annual Net Sales of an HD Program Product greater than [**] Dollars ($[**])	[**]%

7.4.2 Calculation of Royalties. Royalties on Annual Net Sales of each Licensed Product in a Calendar Year during the Royalty Term will be paid at the rate applicable to the portion of Net Sales within each of the Annual Net Sales tiers during such Calendar Year. For example, if, during a Calendar Year during the Royalty Term, Annual Net Sales of a HD Program Product in the Territory are equal to $ [**], then the royalties payable by Novartis would be calculated by [**].

7.5 Royalty Adjustments.

7.5.1 Valid Claim Expiration. If, during any Calendar Quarter during the Royalty Term, on a country-by-country and Licensed Product-by-Licensed Product basis, there is no Valid Claim within the Licensed Patents that Covers: (a) the applicable SMA Program Product in such country or (b) the applicable HD Program Product in such country, then the royalty rate for such Licensed Product in such country will be reduced by [**] percent ([**]%) from the average rate(s) otherwise applicable as set forth in Section 7.4.1.

7.5.2 Third Party Licenses Pertaining to Voyager Capsids. In the event that, during the Royalty Term on a Licensed Product-by-Licensed Product basis, Novartis, its Affiliates

or Sublicensees are required to pay royalties to a Third Party in consideration for a license under Patents Controlled by such Third Party that are reasonably necessary, with respect to any Licensed Product, to Exploit the SMA Program Capsid or HD Program Capsid (as applicable) as part of such Licensed Product in such country, (each, a "Third Party Capsid License"), then Novartis may deduct up to [**] percent ([**]%) of the royalties payable to such Third Party for such Third Party Capsid License(s) from royalties owed by Novartis to Voyager under Section 7.4.1 for Net Sales of the applicable Licensed Product with such reduction continuing in future Calendar Quarters until all such amounts have been expended In cases where royalties under the Third Party Capsid License are not readily attributable to a country, Novartis may allocate such royalties to countries using a reasonable methodology.

7.5.3 Third Party Licenses Pertaining to HD Program Payloads. In the event that, during the Royalty Term on a Licensed Product-by-Licensed Product basis, Novartis, its Affiliates or Sublicensees are required to pay royalties to a Third Party in consideration for a license under Patents Controlled by such Third Party that are reasonably necessary, with respect to any HD Program Product, to Exploit the HD Program Payload as part of such HD Program Product in such country, (each, a "Third Party HD Payload License"), then Novartis may deduct up to [**] percent ([**]%) of the royalties payable to such Third Party for such Third Party HD Payload License(s) from royalties owed by Novartis to Voyager under Section 7.4.1 for Net Sales of the applicable HD Program Product; provided that any such deduction will not reduce royalties owed to Voyager for the applicable Licensed Product in the applicable country to less than [**] percent ([**]%) of the royalty rate that would be apply for the HD Program Product under Section 7.4.1 (with no deductions) in such country with such reduction continuing in future Calendar Quarters until all such amounts have been expended. In cases where royalties under the Third Party HD Payload License are not readily attributable to a country, Novartis may allocate such royalties to countries using a reasonable methodology.

7.5.4 Biosimilar Products. If (a) for any Calendar Year in the applicable Royalty Term for a Licensed Product in a country in the Territory where (i) at least one (1) Biosimilar Product with respect to such Licensed Product is being sold in such country, and (ii) the Net Sales of such Licensed Product sold in such country in such Calendar Year are less than [**] percent ([**]%) as compared with the Net Sales of such Licensed Product in that country in the [**] preceding the marketing or sale of the first Biosimilar Product, then (b), subject to Section 7.5.5, the royalty rate payable on Net Sales of such Licensed Product in such country in such Calendar Year would be reduced by [**] percent ([**]%) of the amounts of royalties otherwise applicable on such Net Sales pursuant to Section 7.4.1 for the remainder of the applicable Royalty Term, such reduction to be prorated appropriately in aggregate for the then-current Calendar Year.

7.5.5 Limit on Deductions. On a Licensed Product-by-Licensed Product basis, In no event will the cumulative effect of the adjustments in Sections 7.5.1 through Section 7.5.4 reduce the royalties payable to Voyager under Section 7.4.1 by more than [**] percent ([**]%) of the amounts that would otherwise have been payable with respect to the applicable Licensed Product in the applicable country in the applicable Calendar Quarter (the "Royalty Floor"). In the event that a reduction would be permitted under this Section 7.5 but for the fact that such reduction would reduce the applicable royalties payable in accordance with Section 7.4.1 by more than the Royalty Floor, then Novartis may carry over such royalty reduction to payments payable hereunder with respect to any royalty payments owed in any future Calendar Quarter for the applicable

Licensed Product, in each case with such reduction continuing until all such amounts have been expended.

7.6 Reports; Payment of Royalty. During the Royalty Term, Novartis will furnish to Voyager a written report within [**] after the end of each Calendar Quarter showing, on a Licensed Product-by-Licensed Product and country-by-country basis, the Net Sales of each Licensed Product in each country of the Territory and the royalties payable under this Agreement. Upon receipt of a royalty report under this Section 7.6, Voyager shall submit an Invoice to Novartis for the applicable royalty payments. Novartis will pay Voyager the foregoing royalties in accordance with Section 7.3.

7.7 Payment of Voyager HD Program Costs.

7.7.1 Novartis shall reimburse Voyager in accordance with this Section 7.7 for the Voyager HD Program Costs, without mark-up. FTEs shall be reimbursed at the FTE Rate based on FTEs actually utilized, on a Calendar Quarter basis, in arrears. For the avoidance of doubt, the FTE Rate is intended to cover the cost of salaries, benefits, infrastructure costs, travel, general laboratory or office supplies, postage, insurance, training and all other general expenses and overhead items. Indirect personnel (including support functions such as financial, legal or business development) shall not constitute reimbursable FTEs. If additional research activities are requested by Novartis and agreed to by Voyager under the HD Program Plan, an increased Development Budget shall be agreed upon by the JSC in accordance with Section 4.1.1(c) prior to the commencement of such activities and such costs shall be deemed "Voyager HD Program Costs". Novartis will not be required to reimburse any amounts in excess of the amount which is [**] percent ([**]%) greater than the Development Budget in the HD Program Plan or reimburse any amounts in excess of the amount which is [**] percent ([**]%) greater than the Development Budget in the HD Program Plan without Novartis' prior written consent.

7.7.2 Within [**] following the end of each Calendar Quarter until all activities under the HD Program Plan are complete, Voyager shall prepare and deliver to Novartis (A) a report, in reasonable detail, of all Voyager HD Program Costs reasonably incurred in the prior Calendar Quarter and supporting documentation (including electronic copies of invoices Voyager has paid for such Voyager HD Program Costs) with respect thereto; and (B) an Invoice for such Voyager HD Program Costs reasonably incurred by Voyager during such period. Novartis shall pay all invoiced amounts within [**] after its receipt of an Invoice except that Novartis may withhold any amounts reasonably disputed in good faith by Novartis in writing within such period pending resolution of such dispute.

7.8 Accounting; Audit.

7.8.1 Records. Novartis agrees to keep, and to require its Affiliates and Sublicensees to keep, full, clear and accurate records for a minimum period of [**] after the end of the Calendar Year to which they pertain, in sufficient detail to enable royalties and other compensation payable to Voyager hereunder to be determined. In addition, Voyager agrees to keep, and to require its Affiliates to keep, full, clear and accurate records for a minimum period of [**] after the end of the Calendar Year to which they pertain, in sufficient detail to enable Voyager HD Program Costs to be determined.

7.8.2 Audits. Each Party (the "Audited Party") agrees, upon not less than [**] prior written notice, to permit, and to require its Affiliates to permit, the books and records specified in Section 7.8.1 to be examined during regular business hours at such place or places where such records are customarily kept by an independent internationally-recognized accounting firm selected by the other Party (the "Auditing Party") and reasonably acceptable to Audited Party for the purpose of verifying reports provided (or required to be provided) by Novartis under this ARTICLE 7 or otherwise verifying the amounts payable hereunder. Any such audit will not be performed more frequently than [**] and not more frequently than [**] with respect to records covering any specific period of time and will be conducted under appropriate confidentiality provisions, for the sole purpose of verifying the accuracy and completeness of all financial, accounting and numerical information and calculations provided under this Agreement. In addition, the Auditing Party shall only be entitled to audit the books and records of the Audited Party from the [**] prior to the calendar year in which the audit request is made. Before beginning its audit, the accounting firm shall execute an agreement reasonably acceptable to the audited Party pursuant to which the accounting firm agrees to keep confidential all information reviewed during the audit. The independent accounting firm will only share the results of the audit, not the underlying records, with the auditing party. The Auditing Party agrees to treat as the Audited Party' Confidential Information all information received and all information learned in the course of any audit or inspection, except to the extent necessary to enforce its rights under this Agreement or to the extent required to comply with any law, regulation or judicial order. Upon request from Voyager, Novartis will conduct an audit on the Sublicensees under the same terms of audit in this Section 7.8.2 and share the result with Voyager.

7.8.3 Audit Reports and Disputes. The independent accounting firm will provide its audit report and the basis for any determination to the Audited Party at the time such report is provided to the Auditing Party before such report is considered to be final. The Audited Party will have the right to request a further determination by such accounting firm as to matters which the Audited Party disputes within [**] following the Audited Party' receipt of such report. The Audited Party will provide the Auditing Party and the accounting firm with a reasonably detailed statement of the grounds upon which it disputes any findings in the audit report and the accounting firm will undertake to complete such further determination, at the Audited Party's expense, within [**] after the dispute notice is provided, which determination will be limited to the disputed matters. Any matter that remains unresolved shall be resolved in accordance with the dispute resolution procedures contained in Section 13.2. In the event that the final result of the inspection reveals an undisputed underpayment or overpayment by Novartis, the underpaid or overpaid amount shall be settled promptly.

7.8.4 Audit Expenses. Except as provided in Section 7.8.3, any audit conducted by the Auditing Party is to be made at the expense of the Auditing Party, except if the results of the audit reveal (a) if Voyager is the Auditing Party, an underpayment of royalties, milestones or other payments to Voyager under this Agreement of [**] percent ([**]%) or more in the audit period, in which case (i) Novartis will promptly remit to Voyager the amount of such underpayment and (ii) the audit fees charged by the auditor for such audit will be paid by Novartis; or (b) if Novartis is the Auditing Party, an overpayment of [**] percent ([**]%) or more with respect to Voyager HD Program Costs in the audit period, in which case (i) Voyager will promptly remit to Novartis the amount of such over payment and (ii) the audit fees charged by the auditor

for such audit will be paid by Voyager. Voyager will be deemed to be the Auditing Party with respect to any audits of Sublicensees requested by Voyager under Section 7.8.2.

7.9 Currency Conversion. Notwithstanding anything to the contrary in the Agreement, conversion of sales recorded in local currencies to U.S. dollars will be performed in a manner consistent with Novartis' normal practices used to prepare its audited financial statements for external reporting purposes.

7.10 Books and Records. Any books and records to be maintained under this Agreement by a Party or its Affiliates or Sublicensees will be maintained in accordance with Accounting Standards.

7.11 Methods of Payments. All payments due from Novartis to Voyager under this Agreement will be paid in Dollars by wire transfer to a bank in the United States designated in writing by Voyager.

7.12 Taxes.

7.12.1 General. Each Party will be solely responsible for the payment of all taxes imposed on its share of income arising directly or indirectly from the activities of the Parties under this Agreement.

7.12.2 Indirect Tax. All amounts mentioned in this Agreement are exclusive of any value added, goods and services, sales, use, excise, consumption and other similar indirect Taxes ("Indirect Taxes"). Where the prevailing legislation requires the recipient to self-account for Indirect Taxes (for example, but not limited to, the reverse charge mechanism), then Novartis covenants that it will correctly account for Indirect Taxes in respect of the services received. Voyager shall issue all invoices in full compliance with the Indirect Tax laws and regulations applicable at Voyager's place of business. If any Indirect Taxes are due based on local law, Voyager will be allowed to add the amount of Indirect Taxes to the amounts mentioned in this Agreement and invoice Novartis the net amount plus the applicable Indirect Taxes. Both parties agree that Voyager is in general allowed to issue tax exempt invoices in case of cross-border supply of services as agreed in this contract. Each Party will be responsible for reporting its own transactions to the local tax authorities if required for Indirect Tax purposes. There will be no shared, mutual or otherwise collective Indirect Tax filings that may suggest that the Parties are anything other than separately operational entities for Indirect Tax purposes.

7.12.3 Tax Action. Notwithstanding anything in this Agreement to the contrary, if an action (including any assignment or sublicense of its rights or obligations under this Agreement, relocation of a Party to a different jurisdiction, or any failure to comply with applicable Laws or filing or record retention requirements) by a Party (a "Tax Action") leads to the imposition of withholding tax liability or Indirect Tax on the other Party that would not have been imposed in the absence of such Tax Action or in an increase in such liability above the liability that would have been imposed in the absence of such Tax Action, then the sum payable by that Party (in respect of which such deduction or withholding is required to be made) shall be increased to the extent necessary to ensure that the other Party receives a sum equal to the sum which it would have received had no such Tax Action occurred.

7.12.4 Subject to Section 7.12.3, in the event any payments to be made to Voyager or its Affiliates under this Agreement are subject to withholding tax under applicable Laws, including extra-territorial taxation, or if it is unclear whether the requirements of applicable Laws, including extra-territorial taxation, are met, Novartis or its Affiliates shall be authorized to deduct the withholding tax from the payments, and shall pay all such withholding tax to the relevant tax authority, so that only the correspondingly reduced amount of payments (i.e. the full amount payable less withholding tax) is paid out to Voyager. Novartis shall provide Voyager with proof of the withholding tax payment. Any such withholding taxes required under applicable Law to be paid or withheld shall be an expense of, and borne solely by, Voyager. If a Party believes that it is required to withhold taxes on a payment to the other Party, the paying Party shall use Commercially Reasonable Efforts to notify the other Party of such determination no less than [**] prior to making such payment (but notice shall not be required for subsequent payments except in case of changes to the expected withholding). Novartis will provide Voyager with reasonable assistance to enable Voyager to recover such taxes as permitted by Law.

7.12.5 Voyager and Novartis shall make all reasonable efforts to obtain relief or reduction of withholding tax under the applicable tax treaties, including but not limited to the submission or issuance of requisite forms and information. If a special procedure is required for treaty relief by Law, a treaty relief based on a tax treaty will only be taken into account if Voyager submits any exemption certificate requested by Novartis to Novartis in accordance with legal requirements on or prior to the time of the payment to Voyager.

7.12.6 If no withholding tax deduction has been made on the payments to Voyager or its Affiliates under this Agreement, but tax authorities subsequently take the position that a withholding tax deduction should have been made, including extra-territorial taxation, Voyager shall provide, at its own expense, all reasonable support to Novartis to obtain any available relief or reduction of withholding under the applicable Laws, including but not limited to the submission or issuance of requisite forms and information, and the Parties will bear such liability (reimburse one another as necessary) in a manner consistent with that which would have resulted had the tax been originally withheld. Any refunds of withholding taxes that are granted to Voyager by the competent tax authority and which would cause Voyager to receive payments in excess of that which Novartis would owe under this Agreement, including related interest, shall be paid to Novartis by Voyager.

7.12.7 Late Payments. Any amount required to be paid by a Party hereunder which is not paid on the date due shall bear interest compounded daily, to the extent permitted by law, at the Federal Funds Effective Rate EFFR or any successor to such rate) for the date such payment was due, as reported by the Federal Reserve of New York (https://apps.newyorkfed.org/markets/autorates/fed%20funds).

ARTICLE 8
INTELLECTUAL PROPERTY RIGHTS

8.1 Ownership; Disclosure.

8.1.1 Novartis Background IP. As between the Parties, Novartis will own and Control all right, title and interest in and to all Patents or Know-How: (a) Controlled by Novartis

and existing as of or before the Effective Date; or (b) Created or acquired solely by or on behalf of Novartis (including through its Representatives) after the Effective Date outside of its activities under this Agreement ((a) and (b), collectively, "Novartis Background IP").

8.1.2 Voyager Background IP. As between the Parties, Voyager will own and Control all right, title and interest in and to all Patents or Know-How: (a) Controlled by Voyager and existing as of or before the Effective Date; or (b) Created or acquired solely by or on behalf of Voyager (including through its Representatives) after the Effective Date outside of its activities under this Agreement ((a) and (b), collectively, "Voyager Background IP").

8.1.3 Arising IP.

(a) Arising IP will be owned as follows: (i) Voyager will solely own all Arising Capsid IP; and (ii) with respect to all Arising IP other than Arising Capsid IP: (A) Voyager will solely own all such Arising IP Created solely by Representatives of Voyager; (B) Novartis will solely own all such Arising IP Created solely by Representatives of Novartis; and (C) the Parties will jointly own all such Arising IP Created jointly by Representatives of Novartis and Representatives of Voyager ("Joint Arising IP"). For clarity, Novartis will solely own all Know-How relating to Capsids not constituting Capsid IP that is Created solely by Representatives of Novartis without use of or reference to Voyager Confidential Information and all Patents Covering such Know-How.

(b) Except as expressly provided in this Agreement, each Party may (subject to the licenses and exclusivity provisions of this Agreement) use the Joint Arising IP for internal research activities, provided that neither Party may: (i) Exploit the Joint Arising IP to Develop, Manufacture or Commercialize a product incorporating or Covered by the Joint Arising IP or (ii) grant licenses or otherwise encumber its ownership interest in any Joint Arising IP, in each case ((i) and (ii)) without the prior written consent of the other Party.

(c) Novartis, on behalf of itself and its Affiliates, hereby assigns, and to the extent such present assignment is not possible, agrees to assign, to Voyager all of Novartis' right, title and interest in and to all Arising Capsid IP. Novartis will, at its sole cost and expense, provide Voyager all reasonable assistance and cooperation in connection with effecting with the foregoing ownership allocation, including providing any necessary powers of attorney and executing any other required documents or instruments as requested by Voyager.

8.2 Disclosure.

8.2.1 During the Term, the Parties shall promptly disclose to each other any Arising IP that Covers or is otherwise necessary for the Exploitation of any HD Program Product.

8.2.2 During the Term, Novartis shall promptly disclose to Voyager any Arising Capsid IP made solely by Novartis or jointly by the Parties.

8.2.3 During the Term, each Party shall promptly disclose to the other Party any Joint Arising IP of which it becomes aware that is not otherwise captured by Section 8.2.1.

8.3 Patent Prosecution and Maintenance; Defense Proceedings.

8.3.1 Licensed Patents. Subject to the terms of any applicable In-License Agreement, and except as expressly set forth in Section 8.3.2 through Section 8.3.5 below, Voyager shall have the sole right, at its sole cost and expense, to Prosecute and Maintain the Licensed Patents and for conducting any Defense Proceeding relating thereto. Notwithstanding anything herein to the contrary, Voyager shall not include any data or information related to any HD Development Candidate or SMA Development Candidate (other than related solely to the Voyager Capsid therein) or Program Target (or any non-human homolog thereof) in any Licensed Patents (or disclose any such data or information in connection with the Prosecution and Maintenance thereof) without Novartis's prior written consent, which Novartis may grant or withhold in its sole discretion.

8.3.2 Capsid Patents. Voyager will: (i) allow Novartis a reasonable opportunity and reasonable time to review and provide comment to Voyager's in-house counsel regarding relevant substantive communications by Voyager and drafts of any responses or other proposed substantive filings by Voyager in relation to Capsid Patents that Cover Voyager Capsids under Evaluation by Novartis before any applicable filings are submitted to any relevant patent office and (ii) reasonably consider any reasonable and timely comments offered by Novartis in any final filings submitted by Voyager to any relevant patent office in relation to such Capsid Patents; provided that Novartis will not have any right to review or comment on any Capsid Patent application prior to filing of such application with the relevant patent office. Voyager will not disclose in, or in connection with Prosecution of, any Capsid Patent any of Novartis' Confidential Information without the prior written consent of Novartis.

8.3.3 Program Capsid Patents and Voyager [**] Platform Patents. With regard to the Program Patents that Cover the SMA Program Capsid or HD Program Capsid (as applicable) component of a Licensed Product ("Program Capsid Patents") and the Voyager [**] Platform Patents, Voyager will: (i) allow Novartis a reasonable opportunity and reasonable time to review and provide comment to Voyager's in-house counsel regarding relevant substantive communications by Voyager and drafts of any responses or other proposed substantive filings by Voyager before any applicable filings are submitted to any relevant patent office and (ii) give due consideration to any reasonable and timely comments offered by Novartis in any final filings, including terminal disclaimers, submitted by Voyager to any relevant patent office. Voyager will not disclose in, or in connection with Prosecution of, any Program Capsid Patent or Voyager [**] Platform Patent any of Novartis' Confidential Information without the prior written consent of Novartis. Upon Novartis' request, Voyager will reasonably consider filing, Prosecuting and Maintaining the Program Capsid Patents and Voyager [**] Platform Patents in any jurisdiction reasonably requested by Novartis including consideration of an arrangement in which Novartis pays Voyager for all of its costs, or a pro-rata share of costs as applicable, for such activity if Voyager would not, but for the Novartis request, otherwise ordinarily perform the activity in such jurisdiction. The Parties will coordinate to develop a patent strategy designed to maximize the value and coverage of the Program Capsid Patents and Voyager [**] Platform Patents for the associated Licensed Products.

8.3.4 SMA Program Product Patents.

(a) As between the Parties, Voyager will own and Control all rights, title and interest in all Voyager SMA Program Product Patents. Novartis shall have the first right, at its sole cost and expense, for Prosecuting and Maintaining the Voyager SMA Program Product Patents and for conducting any Defense Proceeding relating thereto. After the Effective Date, the Parties will coordinate to transfer any Prosecution and Maintenance activities with respect to the Voyager SMA Program Product Patents to Novartis' patent counsel (reasonably acceptable to Voyager and with no conflict of interest with Voyager or the applicable technology).

(b) As between the Parties, and subject to Section 8.3.6, Novartis will own and Control all right, title, and interest in and to all Novartis SMA Program Product Patents. Novartis shall not file a Novartis SMA Program Product Patent prior to the first publication of any Capsid Patent that first discloses the sequence for the Licensed Capsid that is the subject of the corresponding Licensed Product, without first receiving Voyager's written approval, not to be unreasonably withheld, conditioned or delayed, to make such filing.

8.3.5 HD Program Product Patents.

(a) As between the Parties, Voyager will own and Control all right, title and interest in and to all Voyager HD Program Product Patents. Novartis shall have the first right, at its sole cost and expense, for Prosecuting and Maintaining the Voyager HD Program Product Patents other than the Voyager [**] Platform Patents. and for conducting any Defense Proceeding relating thereto. After the Effective Date, the Parties will coordinate to transfer any Prosecution and Maintenance activities with respect to such Voyager HD Program Product Patents to Novartis' patent counsel (reasonably acceptable to Voyager and with no conflict of interest with Voyager or the applicable technology).

(b) As between the Parties, and subject to Section 8.3.7, Novartis will own and Control all right, title, and interest in and to all Novartis HD Program Product Patents. Novartis shall not file a Novartis HD Program Product Patent prior to the first publication of any Capsid Patent that first discloses the sequence for the Licensed Capsid that is the subject of the corresponding Licensed Product, without first receiving Voyager's written approval, not to be unreasonably withheld, conditioned or delayed, to make such filing.

8.3.6 Patent Term Extension. Novartis will have sole authority to make decisions for a patent term extension (e.g., selection of which patents to apply for patent term extension) in respect to any Licensed Products pursuant to rights under the Drug Price Competition and Patent Term Restoration Act, 21 U.S.C. §355, as amended (or any successor statute or regulation) in the U.S., and pursuant to any analogous Law in a foreign jurisdiction; provided, however, that Novartis may not elect to file for such patent term extension on a Program Capsid Patent Controlled by Voyager without Voyager's prior written consent, not to be unreasonably withheld, conditioned or delayed. Notwithstanding the foregoing, Novartis may not elect to file for patent term extension on any other Capsid Patent or any Voyager [**] Platform Patent without Voyager's prior written consent which may be granted or denied in Voyager's sole discretion. For the avoidance of doubt, Patents assigned to Voyager pursuant to Section 8.3.7 are not considered Controlled by Voyager for this purpose.

8.3.7 Assignment of Certain Patents to Voyager. In addition to other provisions that the Parties may agree are appropriate to implement, in the event that: (i) a Novartis SMA Program Product Patent or Novartis HD Program Product Patent is filed after Voyager's approval in accordance with the second sentence of each of Section 8.3.4(b) or Section 8.3.5(b) or (ii) any other SMA Program Product Patent or HD Program Product Patent filed by Novartis creates an obviousness-type double patenting (OTDP) rejection or challenge against any Capsid Patent that requires filing of a terminal disclaimer to obviate such rejection or challenge (and cannot otherwise be overcome by other approaches as agreed to by the Parties), Novartis will assign its right, title, and interest in such Patent to Voyager in the United States only, subject to Novartis receiving the exclusive licenses set forth in Sections 5.1.2(b) and 5.1.3(b); provided that Novartis will retain the right (x) to Prosecute and Maintain the assigned SMA Program Product Patent or HD Program Product Patents in all countries and (y) to enforce or defend all assigned SMA Program Product Patents or HD Program Product Patents; in each case ((x) and (y)) in the same manner it had pursuant to this Agreement prior to such assignment.

8.3.8 Novartis Prosecuted Licensed Patents. With respect to Voyager SMA Program Product Patents and Voyager HD Program Product Patents for which Novartis is responsible for Prosecution and Maintenance and conducting Defense Proceedings pursuant to Sections 8.3.4(a) or 8.3.5(a) (the "Novartis Prosecuted Licensed Patents"), the following will apply:

(i) Novartis shall keep Voyager fully informed with respect to: (A) the issuance of a Novartis Prosecuted Licensed Patents; and (B) the abandonment of any Novartis Prosecuted Licensed Patents.

(ii) Without limiting the foregoing, Novartis shall: (A) provide Voyager with copies of the text of the applications for any Novartis Prosecuted Licensed Patents it Prosecutes or Maintains as soon as practicable but at least [**] before filing, except for urgent filings, in which case Novartis shall provide copies as soon as practicable before, simultaneously with or immediately after filing; (B) provide Voyager with a copy of each submission made to and material or substantive document received from a patent authority, court or other tribunal regarding any Novartis Prosecuted Licensed Patents reasonably promptly after making such filing or receiving such document, including a copy of each application as filed together with notice of its filing date and application number; (C) keep Voyager advised of the status of all substantive communications, actual and prospective filings or submissions regarding any Novartis Prosecuted Licensed Patents, and give Voyager copies of any such communications, filings and submissions proposed to be sent to any patent authority or judicial body; and (D) consider in good faith Voyager's comments on such communications, filings and submissions for any such Novartis Prosecuted Licensed Patents and shall reasonably incorporate such comments unless their incorporation would reasonably be expected to have a material adverse effect on the scope of any Novartis Prosecuted Licensed Patents.

(b) Novartis shall notify Voyager as to any decision to abandon, to cease Prosecution and Maintenance of, or not to continue to pay the expenses of Prosecution and Maintenance of, any Novartis Prosecuted Licensed Patents in any country in which it was filed. Novartis will provide such notices at least [**] prior to any filing or payment due date, or any other due date that requires action, in connection with such Novartis Prosecuted Licensed Patents.

Notwithstanding the foregoing, if Novartis has provided notice of termination under Section 12.2.2, Section 12.2.2(c), or Section 12.3, Novartis will not discontinue the Prosecution and Maintenance of any Novartis Prosecuted Licensed Patents during the applicable notice period until such Prosecution and Maintenance is assumed by Voyager pursuant to this Section 8.3.5(b); provided that Voyager will be responsible for all Out-of-Pocket Costs incurred by Novartis to conduct any Prosecution and Maintenance activities during such notice period that are requested by Voyager. Promptly after notice of termination is provided under Section 12.2.2, Section 12.2.2(c), or Section 12.3, the Parties will coordinate to transfer any Prosecution and Maintenance activities with respect to any Novartis Prosecuted Licensed Patents to Voyagers' patent counsel (reasonably acceptable to Voyager and with no conflict of interest with Voyager or the applicable technology).

8.3.9 Novartis Patents. Except as set forth in Section 8.3.4(b) and Section 8.3.5(b), Novartis shall be solely responsible, at its sole cost and expense, and shall have the exclusive right, but not the obligation, for Prosecuting and Maintaining the Novartis Patents and for conducting Defense Proceedings relating thereto.

8.3.10 Joint Patents. Neither Party will file any Patent application for any Joint Arising IP without mutual consent. If the Parties decide to seek patent protection for any Joint Arising IP, the Parties will cooperate in good faith to determine, on a case-by-case basis, which Party will have the responsibility for Prosecuting and Maintaining, and conducting Defense Proceedings relating to any Joint Patents, and how the cost for such activities will be shared.

8.3.11 Cooperation. Each Party will reasonably cooperate with and assist the other Party in connection with the activities of such Party under this Section 8.3 upon the reasonable request of the other Party, including by making patent attorneys, scientists and scientific records reasonably available and the execution of all such documents and instruments and the performance of such acts as may be reasonably necessary in order to continue any Prosecution and Maintenance or conduct any Defense Proceedings of such Patents.

8.4 Enforcement. Subject to the terms of any applicable In-License Agreement:

8.4.1 Notice. Each Party will promptly notify the other Party in writing (the "Infringement Notice") of any knowledge it acquires of any actual or potential Competitive Infringement.

8.4.2 Capsid Patents and Voyager [**] Platform Patents. Voyager will have the sole right (but not the obligation) to institute litigation or take other steps to remedy any Competitive Infringement in connection with any Capsid Patents or Voyager [**] Platform Patents in the Territory, and any such litigation or steps will be at Voyager's expense and all recoveries will be retained by Voyager. In the event that (a)(i) Voyager (A) does not institute litigation or take other steps to remedy such Competitive Infringement in connection with any such Capsid Patents or Voyager [**] Platform Patents within [**] after the corresponding Competitive Infringement is first notified in accordance with Section 8.4.1, (B) does not continue its litigation to a final, unappealable decision, or (C) does not remedy the Competitive Infringement through other means within such [**] period, and (ii) such Competitive Infringement has (or reasonably threatens to have) a direct and material adverse impact on Novartis's Commercialization of

Licensed Products, then (b) the royalties due to Voyager pursuant to Section 7.4 and payable as of the date of the Infringement Notice shall be reduced by [**] percent ([**]%), but only in the country in which the infringing activity exists with no right of offset with regard to royalties payable for other jurisdictions.

8.4.3 HD Program Product Patents. Novartis will have the first right (but not the obligation) to institute litigation or take other steps to remedy anyCompetitive Infringement in connection with any HD Program Product Patents excluding the Voyager [**] Platform Patents anywhere in the Territory. If Novartis fails to institute litigation or take other steps to remedy anyCompetitive Infringement in connection with any such HD Program Product Patents in the Territory within [**] after the corresponding Competitive Infringement is first notified in accordance with Section 8.4.1, then Voyager may, upon written notice to Novartis, institute litigation or take other steps to remedy theCompetitive Infringement in its sole discretion, at its sole cost and expense using counsel of its choice. At the request and sole expense of the Party bringing an action under this Section 8.4.3, the other Party shall provide reasonable assistance in connection therewith, including by executing reasonably appropriate documents, cooperating in discovery and joining as a party to the action if required by Law to pursue such action. The controlling Party shall have the right to settle any Competitive Infringement pursuant to this Section 8.4.3; provided, that neither Party shall have the right to settle any such action in a manner that adversely impacts any HD Program Product Patents or Licensed Patents, or imposes any liability on, or involves any admission by, the other Party, without the express written consent of such other Party (which consent shall not be unreasonably withheld, conditioned, or delayed). Any recovery realized as a result of any Competitive Infringement described in this Section 8.4.3 (whether by way of settlement or otherwise) shall be first allocated to reimburse the Parties for their costs and expenses, which amounts shall be allocated pro rata if insufficient to cover the totality of such expenses. Any remainder after such reimbursement is made shall be retained by the Party that has exercised its right to bring the enforcement action and will be treated as Net Sales of the HD Program Product in the calendar year in which the amount is actually received.

8.4.4 SMA Program Product Patents. Novartis will have the sole right (but not the obligation) to take action to obtain a discontinuance of infringement or bring suit against a Third Party infringing or challenging the validity or enforceability of any SMA Program Product Patents in the Territory, and any such litigation or steps will be at Novartis's expense and all recoveries will be retained by Novartis.

8.4.5 Joint Patents. Immediately after an infringement of a Joint Patent is first identified, the Parties shall meet and cooperate in good faith to determine, on a case-by-case basis, (i) what action, if any, the Parties will take to obtain a discontinuance of such infringement or bring suit against a Third Party infringing or challenging the validity or enforceability of any Joint Patent, and (ii) how the costs for and any recoveries from such activities will be shared.

8.5 Infringement Claimed by Third Parties.

8.5.1 Notice. If a Third Party commences, or threatens to commence, any proceeding against a Party alleging infringement of such Third Party's intellectual property by the Exploitation by a Party, its Affiliates, subcontractors or Sublicensees of any HD Candidate or

Licensed Product, the Party against whom such proceeding is threatened or commenced will give prompt notice to the other Party.

8.5.2 Control of Proceeding. Unless the Party against whom such proceeding is filed seeks indemnification for such claim under ARTICLE 11, such Party will control the defense and settlement of any such proceeding described in Section 8.5.1 at its own cost and expense, using counsel of its choice, in its sole discretion. If the Party against whom such proceeding is filed does seek indemnification for such claim, then the provisions of ARTICLE 11 will govern the Parties' rights and responsibilities with respect to such claim.

ARTICLE 9
CONFIDENTIALITY

9.1 Confidentiality; Exceptions. Except to the extent expressly authorized by this Agreement, the Parties agree that the receiving Party (the "Receiving Party") will keep confidential and will not publish or otherwise disclose or use for any purpose other than to perform its obligations and exercise its rights as provided for in this Agreement any Know-How or other confidential and proprietary information and materials, patentable or otherwise, in any form (written, oral, photographic, electronic, magnetic, or otherwise) that is disclosed to it by the other Party (the "Disclosing Party"), including trade secrets, Know-How, inventions or discoveries, proprietary information, formulae, processes, techniques and information relating to the Disclosing Party's past, present or future marketing, financial, or Exploitation activities of any product or potential product or technology of the Disclosing Party or the pricing thereof (collectively, "Confidential Information"). For clarity, any data, information, or Patent filings provided by one Party to the other Party will constitute the Disclosing Party's Confidential Information. Without limiting the foregoing, the Receiving Party will treat all Confidential Information provided by the Disclosing Party with the same degree of care as the Receiving Party uses for its own similar information, but in no event less than a reasonable degree of care. Notwithstanding the foregoing, "Confidential Information" will exclude information to the extent that it can be established by the Receiving Party that such information:

9.1.1 was in the lawful knowledge or possession of the Receiving Party prior to the time it was first disclosed to the Receiving Party by the Disclosing Party, or was otherwise developed independently by the Receiving Party without reference to any of the Disclosing Party's Confidential Information, as evidenced by written records kept in the ordinary course of business, or other documentary proof of knowledge or possession by the Receiving Party;

9.1.2 was generally available to the public or otherwise part of the public domain at the time of its first disclosure to the Receiving Party by the Disclosing Party;

9.1.3 became generally available to the public or otherwise part of the public domain after its disclosure to the Receiving Party by the Disclosing Party and other than through any act or omission of the Receiving Party in breach of this Agreement; or

9.1.4 was disclosed to the Receiving Party, other than under an obligation of confidentiality, by a Third Party who had no obligation to the Disclosing Party not to disclose such information to others.

9.2 The existence and terms of this Agreement will be considered the Confidential Information of both Parties. Any reports, Know-How, and other proprietary or sensitive information disclosed or shared by one Party with the other Party pursuant to the activities contemplated by this Agreement will be the Confidential Information of the Party that first shared such report, Know-How or other proprietary or sensitive information with the other Party.

9.3 Authorized Disclosure.

9.3.1 Disclosure to a Party's Representatives. Notwithstanding the foregoing provisions of Section 9.1, the Receiving Party may disclose Confidential Information belonging to the Disclosing Party to the Receiving Party's Representatives who (a) have a need to know such Confidential Information in connection with the performance of the Receiving Party's obligations or the exercise of the Receiving Party's rights under this Agreement and (b) have agreed in writing to non-disclosure and non-use provisions with respect to such Confidential Information that are at least as restrictive as those set forth in this ARTICLE 9.

9.3.2 Disclosure to Third Parties. Notwithstanding Section 9.1, each Party may disclose Confidential Information belonging to the other Party to the extent such disclosure is reasonably necessary:

(a) to Governmental Authorities (i) to the extent desirable to obtain or maintain INDs or Regulatory Approvals for any Licensed Product within the Territory and (ii) in order to respond to inquiries, requests or investigations relating to Licensed Products or this Agreement;

(b) to existing or prospective outside consultants, contractors, advisory boards, investors, collaboration partners, professional advisors, managed care organizations, and non-clinical and clinical investigators, in each case to the extent desirable to develop, register or market any Licensed Product or otherwise as reasonably necessary to perform such Party's obligations under this Agreement; provided that the Receiving Party shall obtain the same confidentiality obligations from such Third Parties as it obtains with respect to its own similar types of confidential information;

(c) in connection with filing or Prosecuting Patents or trademark rights as permitted by this Agreement;

(d) in connection with Prosecuting or defending litigation as permitted by this Agreement;

(e) subject to the provisions of Section 9.6, in connection with or included in scientific presentations and publications relating to Capsids or Licensed Products, including abstracts, posters, journal articles and the like, and posting results of and other information about clinical trials to clincialtrials.gov or PhRMA websites;

(f) to a court or arbitrator, to the extent reasonably necessary in order to enforce its rights under this Agreement;

(g) in communication with existing or prospective investors, lenders, professional advisors, acquirers, merger partners, collaboration partners, subcontractors, Sublicensees, or licensees on a need to know basis, in each case under appropriate confidentiality obligations substantially equivalent to those of this Agreement; or

(h) to the extent mutually agreed to in writing by the Parties.

9.4 Residual Knowledge Exception. Notwithstanding any provision of this Agreement to the contrary, Residual Knowledge shall not be considered Confidential Information for purposes of this ARTICLE 9.

9.5 Press Release; Disclosure of Agreement.

9.5.1 Press Releases. On or promptly after the Effective Date, Voyager anticipates issuing a public announcement regarding the signing of this Agreement in a form to be agreed in advance in writing by the Parties. Except as may be expressly permitted under Section 9.5.2, neither Party will make any public announcement regarding this Agreement without the prior written approval of the other Party; provided that to the extent information regarding this Agreement has already been publicly disclosed, except as a result of a breach of this Agreement, each Party may subsequently disclose the same information to the public without the consent of the other Party, provided that such information remains true, accurate, and up to date. In addition, nothing in this Agreement shall prevent Novartis from making any scientific publication or public announcement with respect to any Licensed Product under and during the Term of this Agreement; *provided, however*, that, except as permitted under Section 9.3.1, Novartis shall not disclose any of Voyager's Confidential Information in any such publication or announcement without obtaining Voyager's prior written consent to do so.

9.5.2 SEC Filings and other Disclosures of this Agreement. Notwithstanding Section 9.5.1, each Party will be permitted to disclose the existence and terms of this Agreement to the extent required to comply with applicable Laws including the rules or regulations of the U.S. Securities and Exchange Commission, or similar agency in any country other than the United States, or of any stock exchange, including Nasdaq, provided that (a) prior to disclosing this Agreement or any of the terms hereof as permitted under this Section 9.5.2, the Parties will coordinate in advance with each other in connection with the redaction of certain provisions of this Agreement prior to such disclosure (the "Redacted Version"), (b) to the extent permitted by applicable Laws, the Parties will use reasonable efforts to file redacted versions with such agencies and stock exchanges that are consistent with the Redacted Version, and (c) each Party will, at its own expense, use reasonable efforts to seek confidential treatment for such terms as may be reasonably requested by the other Party.

9.6 Publications. Novartis will not publish or publicly disclose the scientific results of any Evaluation conducted by it for any Voyager Capsid, without the prior written consent of Voyager. Nothing in this Agreement shall prevent Novartis from making any scientific publication or public announcement with respect to any Licensed Product during the Term; provided, however, that, except as permitted under Section 9.3, Novartis shall not disclose any of Voyager's Confidential Information in any such publication or announcement without obtaining Voyager's prior written consent to do so. In addition, (i) Voyager shall not publish or make any public

announcement regarding a Licensed Product without Novartis' prior written approval, and (ii) Novartis shall provide Voyager a copy of each publication or other public disclosure relating to a Licensed Product that contains unpublished information relating to a Licensed Capsid. During the Term, each Party will provide the other Party (the "Non-Disclosing Party") for review and approval any proposed abstract, manuscript, or presentation that contains the Non-Disclosing Party's Confidential Information. Written copies of each proposed publication that are required to be submitted hereunder shall be provided to the Non-Disclosing Party no less than [**] ([**] with respect to disclosures in a patent application) prior to its intended submission for publication or presentation. The Non-Disclosing Party will respond in writing promptly and in no event later than [**] after receipt of the proposed publication or presentation, with one or more of the following: (a) comments on the proposed publication or presentation, which the publishing Party will consider in good faith and use reasonable efforts to incorporate, (b) a specific statement of concern, based upon the need to delay publication if the Non-Disclosing Party determines that the proposed publication or presentation contains or describes intellectual property that needs to be incorporated into a Patent application; provided that such delay shall not exceed an additional [**] unless agreed in writing by the Parties, or (c) an identification of the Non-Disclosing Party's Confidential Information that needs to be removed from the proposed publication or presentation.

9.7 Remedies. Each Party will be entitled to seek, in addition to any other right or remedy it may have, at Law or in equity, a temporary injunction, without the posting of any bond or other security, enjoining or restraining the other Party from any violation or threatened violation of this ARTICLE 9.

ARTICLE 10
REPRESENTATIONS AND WARRANTIES

10.1 Representations and Warranties of Both Parties. Each Party hereby represents and warrants to the other Party, as of the Effective Date, that:

10.1.1 such Party is duly organized, validly existing and in good standing under the Laws of the jurisdiction of its incorporation and has full corporate power and authority to enter into this Agreement and to carry out the provisions hereof;

10.1.2 such Party has taken all necessary action on its part to authorize the execution and delivery of this Agreement and the performance of its obligations hereunder and does not require any action or approval by any of its shareholders or other holders of its voting securities or voting interests;

10.1.3 this Agreement has been duly executed and delivered on behalf of such Party, and constitutes a legal, valid, binding obligation, enforceable against it in accordance with the terms hereof;

10.1.4 the execution, delivery and performance of this Agreement by such Party does not conflict with any agreement or any provision thereof, or any instrument or understanding, oral or written, to which it is a party or by which it is bound, nor violate any law or regulation of any court, governmental body or administrative or other agency having jurisdiction over such Party;

10.1.5 neither such Party nor any of its Affiliates has been debarred or is subject to debarment pursuant to Section 306 of the FD&C Act, as amended, or that is the subject of a conviction described in such section; and

10.1.6 no government authorization, consent, approval, license, exemption of or filing or registration with any court or governmental department, commission, board, bureau, agency or instrumentality, domestic or foreign, under any applicable Laws currently in effect, is or will be necessary for, or in connection with, the transaction contemplated by this Agreement or any other agreement or instrument executed in connection herewith, or for the performance by it of its obligations under this Agreement and such other agreements, except as may be required to conduct Clinical Trials, to Manufacture Licensed Products, or to seek or obtain Regulatory Approvals.

10.2 Representations and Warranties, as applicable, of Voyager. Voyager hereby represents, warrants, and covenants to Novartis, as of the Effective Date that:

10.2.1 Voyager has disclosed to Novartis all material scientific and technical information and all material information that, to Voyager's Knowledge, are relevant to safety and efficacy with respect to the Capsids;

10.2.2 Section (a) of Schedule 10.2.2 sets forth a true and complete list of all Capsid Patents as of the Effective Date that Cover the Voyager Capsids that will be provided to Novartis for Evaluation or included in the HD Program Product (the "Relevant Capsid Patents"), (b) Section (b) of Schedule 10.2.2 sets forth a true and complete list of all Patents as of the Effective Date that Cover the payload that is anticipated as of the Effective Date to be the HD Program Payload under the HD Program Plan ("Relevant HD Payload Patents", and collectively with the Relevant Capsid Patents, the "Relevant Patents"), (c) each such Patent remains in full force and effect and (d) Voyager or its Affiliates have timely paid all filing and renewal fees payable with respect to such Patents;

10.2.3 Voyager is the sole and exclusive owner of the Relevant Patents and Voyager Know-How, all of which is free and clear of any claims, liens, charges, or encumbrances that would conflict with the rights granted to Novartis hereunder;

10.2.4 Voyager has and will have the right, power, and authority to grant all rights, title, and interests in the licenses granted or to be granted to Novartis under this Agreement;

10.2.5 Voyager has not granted any right or license, to any Third Party relating to any of the Relevant Patents that would conflict with the rights or licenses granted to Novartis hereunder as of the Effective Date;

10.2.6 [**] no claim, demand, suit, proceeding, arbitration, inquiry, investigation, litigation, or other legal action of any nature, civil, criminal, regulatory or otherwise, is pending, has been brought, or to Voyager's Knowledge, threatened against Voyager or any Affiliate of Voyager, or, to Voyager's Knowledge, any Third Party, alleging that the Exploitation of Voyager's Background IP is infringing or, if practiced or commercialized, will infringe the rights of any Third Party;

10.2.7 [**] there is no judgment or settlement against or owed by Voyager or any of its Affiliates, in each case in connection with the Relevant Patents or Voyager Know-How relating to the transactions contemplated by this Agreement;

10.2.8 to Voyager's Knowledge, no Third Party has challenged or threatened to challenge the scope, validity or enforceability of any Relevant Patents (including, by way of example, through the institution or written threat of institution of interference, nullity or similar invalidity proceedings before the United States Patent and Trademark Office or any analogous foreign Governmental Authority);

10.2.9 to Voyager's Knowledge: (a) the Relevant Patents, are, or, upon issuance, will be, valid and enforceable patents; and (b) as of the Effective Date no Person is infringing or threatening to infringe, or misappropriating or threatening to misappropriate, the Relevant Patents in a manner that would affect Novartis' rights under this Agreement;

10.2.10 all of its employees, officers, and consultants have executed (a) valid and enforceable agreements assigning or (b) have existing obligations under applicable Laws requiring assignment to Voyager of all Inventions made during the course of and as the result of their association with Voyager and obligating the individual to maintain as confidential Voyager's Confidential Information as well as confidential information of other Persons (including Novartis and its Affiliates) which such individual may receive;

10.2.11 Voyager has taken reasonable precautions to preserve the confidentiality of any Know-How that constitutes Voyager's Background IP existing as of the Effective Date and that is, or would be, licensed to Novartis pursuant to this Agreement, including requiring each Person having access to any Know-How within such Voyager's Background IP to be subject to confidentiality, non-use and non-disclosure obligations protecting such Know-How as the confidential, proprietary materials and information of Voyager;

10.2.12 to Voyager's Knowledge, Voyager has complied with all applicable Laws, including any disclosure requirements, in connection with the filing, Prosecution and Maintenance of the Relevant Patents;

10.2.13 to Voyager's Knowledge, Voyager has independently developed all Voyager Know-How or otherwise has a valid right to use, and to permit Novartis, Novartis' Affiliates, and Novartis' Sublicensees to use, the Voyager Know-How for all permitted purposes under this Agreement;

10.2.14 no Relevant Patent is subject to any funding agreement with any government or Governmental Authority;

10.2.15 neither Voyager nor any of its Affiliates are party to or otherwise subject to any agreement or arrangement that would conflict with Novartis' rights or Voyager's obligations under this Agreement;

10.2.16 to Voyager's Knowledge, [**] the Exploitation by Voyager or Novartis (or their respective Affiliates or Sublicensees) of any Voyager Capsid or HD Program

Payload (as identified in the HD Program Plan summaryattached hereto as Schedule 1.79) does not infringe any claim of an issued patent of any Third Party as of the Effective Date;

10.2.17 Voyager, its Affiliates, and to Voyager's Knowledge all Third Parties and Representatives acting on Voyager's behalf, have complied in all material respects with all applicable Law and accepted pharmaceutical industry business practices with regard to the subject matter of this Agreement, including, to the extent applicable, the FD&C Act (21 U.S.C. § 301, et seq.), the Anti-Kickback Statute (42 U.S.C. § 1320a-7b), Civil Monetary Penalty Statute (42 U.S.C. § 1320a-7a), the False Claims Act (31 U.S.C. § 3729 et seq.), comparable state statutes, the regulations promulgated under all such statutes, and the regulations issued by the FDA, consistent with the 'Compliance Program Guidance for Pharmaceutical Manufacturers' published by the Office of Inspector General, U.S. Department of Health and Human Services;

10.2.18 with respect to any Licensed Capsids, Licensed Products, payments, or services provided under this Agreement, Voyager, its Affiliates, and to Voyager's Knowledge all Third Parties and Representatives acting on Voyager's behalf, have not taken and will not during the Term take any action directly or indirectly to offer, promise or pay, or authorize the offer or payment of, any money or anything of value in order to improperly or corruptly seek to influence any government official or any other person in order to gain an improper advantage, and has not accepted, and will not accept in the future such payment;

10.2.19 The Existing In-License Agreements are in full force and effect in accordance with their terms; Voyager is not in breach of any Existing In-License Agreement and knows of no breach of any Existing In-License Agreement by the other party(ies) thereto; and Voyager has neither sent, provided, nor received any notice of breach or intent to terminate any Existing In-License Agreement. The redacted copies of the Existing In-License Agreements that were made available to Novartis by Voyager in due diligence in connection with this Agreement were true, accurate and complete (subject to the redactions), including all amendments thereto; and

10.2.20 Voyager, its Affiliates, and to Voyager's Knowledge all Third Parties and Representatives acting on Voyager's behalf, have complied with the laws and regulations of the countries where it operates, including anti-bribery and anti-corruption laws, including, to the extent applicable, the U.S. Foreign Corrupt Practices Act of 1977 and the U.K. Bribery Act 2010, accounting and record keeping laws, and laws relating to interactions with healthcare professionals or healthcare providers and government officials.

10.3 Representation and Warranty of Novartis. Novartis hereby represents and warrants to Voyager, as of the Effective Date, that Novartis has determined in good faith and in accordance with 16 CFR § 801.10(c)(3), that the fair market value of the U.S. assets to be licensed hereunder are not greater than One Hundred Eleven Million Four Hundred Thousand Dollars ($111,400,000). This determination is made solely for the purpose of determining the applicability of the Hart-Scott-Rodino Antitrust Improvements Act of 1976, as amended from time to time, to the transaction.

10.4 Mutual Covenants. Each Party hereby covenants to the other Party that, during the Term of this Agreement:

10.4.1 it will perform its obligations under this Agreement in compliance with applicable Laws;

10.4.2 all individuals who are employees or independent contractors of such Party or any of its Affiliates working under this Agreement will be under the obligation to assign or exclusively license all right, title and interest in and to their Know-How, and all intellectual property rights therein, to such Party or its Affiliate as the sole owner or exclusive licensee thereof;

10.4.3 such Party will not knowingly (a) employ, or use any contractor or consultant that employs or uses, any Person debarred or disqualified by the FDA (or subject to a similar sanction of EMA or any other Governmental Authority) or, (b) employ any Person that is the subject of an FDA debarment investigation or proceeding (or similar proceeding of EMA or any other Governmental Authority), in each of clauses (a) and (b) in the conduct of its activities under this Agreement; and

10.4.4 in performing its obligations or exercising its rights under this Agreement, such Party, its Affiliates, and, with respect to Novartis, its Sublicensees, will comply with all applicable Law, including all anti-corruption Laws.

10.5 Exclusivity Covenants.

10.5.1 Voyager. During the Term, other than the conduct of the Campaigns and the performance of the HD Program Plans and Voyager's other obligations under this Agreement, Voyager agrees that Voyager and its Affiliates will not:

(a) conduct any internal program or program on behalf of a Third Party that is directed to Development or Commercialization of any Capsids for use in any therapeutic product comprising a Capsid in combination with a payload intended to have a therapeutic effect on the SMA Target when packaged into a Capsid and delivered to the appropriate cells;

(b) Develop or Commercialize any Competing HD Product; or

(c) grant any Third Party any right, license, option, covenant not to assert or similar right, under any Patents or Know-How Controlled by Voyager or its Affiliates (excluding an Acquiring Entity) as of the Effective Date or during the Term, that would enable a Third Party to do any of the foregoing.

10.5.2 Novartis. During the Term, other than the performance of the HD Program Plans and the exercise of Novartis' rights or performance of Novartis's obligations under this Agreement, Novartis agrees that Novartis and its Affiliates will not:

(a) Develop or Commercialize any Competing HD Product, other than a Licensed Product; or

(b) grant any Third Party any right, license, option, covenant not to assert or similar right, under any Patents or Know-How Controlled by Novartis or its Affiliates (excluding an Acquiring Entity) as of the Effective Date or during the Term, that would enable a Third Party to do any of the foregoing.

10.5.3 Exception For Orthogonal Research Activities. The restrictions set forth in Section 10.5.1 and 10.5.2 shall not prevent either Party or any of its Affiliates, alone or with, for, or through any Third Party, from conducting any non-clinical Development activity that has, as its specified goal, as evidenced by laboratory notebooks or other relevant documents contemporaneously kept, taken as a whole, to Develop a product that is not directed to any Program Target.

10.6 Acquisitions.

10.6.1 If a Party or any of its Affiliates (such Party and its Affiliates existing as of the closing of the applicable transaction, the "Acquisition Party") acquires or is acquired by a Third Party (such Third Party and any of its Affiliates other than the Acquisition Party, each, an "Acquired Affiliate") (whether such acquisition occurs by way of a purchase of assets, merger, consolidation, change of control or otherwise) that is, at the time of such acquisition, engaging in any activities that would violate Section 10.5.1 or 10.5.2 (as applicable) if conducted by such Acquisition Party (such activities a "Competitive Program" and any product Developed, Commercialized or otherwise Exploited thereunder, an "Acquired Competing Product") then (y) the Acquisition Party or its Acquired Affiliate shall, no later than [**] following the date of consummation of the relevant acquisition, notify the other Party in writing that the Acquisition Party or its Acquired Affiliate has elected one of the following:

(a) to divest, whether by license or otherwise, its interest in the Competitive Program to a Third Party, to the extent necessary to be in compliance with Section 10.5.1 or 10.5.2 (as applicable), with no rights in such Competitive Program (other than a financial interest) retained by the Acquisition Party, its Acquired Affiliate or any of their Affiliates;

(b) to terminate Development, Manufacture and Commercialization under the Competitive Program, to the extent necessary to be in compliance with Section 10.5.1 or 10.5.2 (as applicable); or

(c) to continue the activities solely through Acquired Affiliates, in which case: (i) the Acquisition Party and any Acquired Affiliate engaged in such activities will: (A) implement and enforce firewalls with respect to such Competitive Program; (B) ensure that no Patents or Know-How Controlled by the Acquisition Party (that are not otherwise Controlled by the Acquired Affiliates outside of their Affiliate relationship with the Acquisition Party) are used in such Competitive Program; and (C) ensure that no Confidential Information of the other Party to this Agreement is used in such Competitive Program; and (ii) if Voyager is the Acquisition Party and the closing of the applicable transaction under Section 10.6.1 occurs prior to the completion of Voyager's activities under the HD Program Plan, then Novartis may (at its option), upon written notice to Voyager within [**] after Novartis receives notice from Voyager that an Acquired Affiliate has a Competitive Program to the HD Program, elect to: (A) assume all further Development under the HD Program Plan, in which case Voyager will provide all information and reasonable assistance necessary to effectively transfer such activities to Novartis to enable Novartis to complete the HD Program Plan: and (B) limit Voyager's rights to receive information with respect to the HD Program to the reports set forth in Section 3.3, Section 5.2.5 and Section 7.6.

10.6.2 If the Acquisition Party or its Acquired Affiliate notifies the other Party in writing that it intends to divest such Competitive Program or terminate Development, Manufacture and Commercialization under the Competitive Program as provided in Section 10.5.1(a) or 10.5.2(b), then the Acquisition Party or its Acquired Affiliate, as applicable, shall effect the consummation of such divestiture within [**] or effect such termination within [**] after the consummation of the relevant acquisition, subject to compliance with applicable Laws, and shall confirm to the other Party in writing when such divestiture or termination has been completed.

10.6.3 The Acquisition Party shall keep the other Party reasonably informed of its and its Affiliates' efforts and progress in effecting such divestiture or termination until it is completed. Until such divestiture or termination occurs, the Acquisition Party shall keep its and its Affiliates' activities with respect to such Competitive Program separate from their activities under this Agreement and shall (a) implement and enforce firewalls with respect to such Competitive Program (b) ensure that that no Patents or Know-How Controlled by the Acquisition Party (that are not otherwise Controlled by the Acquired Affiliates outside of their Affiliate relationship with the Acquisition Party) are used in such Competitive Program and (c) ensures that no Confidential Information of the other Party to this Agreement is used in such Competitive Program.

10.6.4 Subject to the Acquisition Party's compliance with this Section 10.6, the activities of the Acquisition Party or its Acquired Affiliate with respect to any Competitive Program shall not be a breach of this Agreement.

10.7 Other Covenants of Voyager. In addition to the covenants made by the Parties elsewhere in this Agreement, Voyager hereby covenants to Novartis that:

10.7.1 During the Term, Voyager shall not, and shall cause its Affiliates not to: (a) license, sell, assign or otherwise transfer to any Person (other than Novartis or its Affiliates or Sublicensees pursuant to the terms of this Agreement) any rights under the Licensed Patents to use a Licensed Capsid with the corresponding an SMA Target or an HD Target (or agree to do any of the foregoing) or (b) incur or permit to exist, with respect to any Licensed Patents, any lien, encumbrance, charge, security interest, mortgage, liability, assignment, grant of license or other binding obligation that is or would be inconsistent with the licenses and other rights granted to Novartis or its Affiliates under this Agreement;

10.7.2 During the Term: (a) Voyager will not enter into any agreement with a Third Party that conflicts with (i) the rights granted to Novartis under this Agreement or (ii) Voyager's ability to fully perform its obligations hereunder; (b) Voyager will not amend or otherwise modify any agreements with a Third Party or consent or waive rights with respect thereto in any manner that conflicts with (i) the rights granted to Novartis under this Agreement or (ii) Voyager's ability to fully perform its obligations hereunder; and

10.7.3 Novartis has put in place a third party risk management framework that is aimed at promoting the societal and environmental values of the United Nations Global Compact with specific Third Parties that Novartis deals with (the "Novartis Third Party Code"). In connection with the foregoing, during the Term Voyager shall use Commercially Reasonable

Efforts to comply with the ethical business practices and related terms set forth in the Novartis Third Party Code found at https://www.novartis.com/about-us/corporate-responsibility/resources-news/codes-policies-guidelines, provide true and accurate information/documentation reasonably requested by Novartis to allow Novartis to verify compliance with the Novartis Third Party Code in the form requested, use Commercially Reasonable Efforts to rectify identified non-compliances with the Novartis Third Party Code (where capable of remedy) and report remediation progress to Novartis on request.

10.8 Other Covenants of both Parties. In addition to the covenants made by the Parties elsewhere in the Agreement, each Party will maintain valid and enforceable agreements with all Persons acting by or on behalf of such Party or its Affiliates under this Agreement that enable such Party to comply with its obligations under this Agreement and which require such Persons to assign to such Party their entire right, title and interest in and to all Patents and Know-How that such Party is required to assign to the other Party under this Agreement.

10.9 Representation by Legal Counsel. Each Party represents that it has been represented by legal counsel in connection with this Agreement and acknowledges that it has participated in the drafting hereof. In interpreting and applying the terms and provisions of this Agreement, the Parties agree that no presumption shall exist or be implied against the Party which drafted such terms and provisions.

10.10 Disclaimer. Except as otherwise expressly set forth in this Agreement, NEITHER PARTY MAKES ANY REPRESENTATION OR EXTENDS ANY WARRANTY OF ANY KIND, EITHER EXPRESS OR IMPLIED, INCLUDING ALL IMPLIED WARRANTIES OF MERCHANTABILITY, FITNESS FOR A PARTICULAR PURPOSE, ALL OF WHICH ARE HEREBY SPECIFICALLY EXCLUDED AND DISCLAIMED.

ARTICLE 11
INDEMNIFICATION; INSURANCE

11.1 Indemnification by Novartis. Novartis will indemnify, hold harmless and defend Voyager and its Affiliates, and its or their respective directors, officers, employees, agents, consultants and Representatives (each a "Voyager Indemnified Party"), from and against any and all liabilities, damages, losses, costs and expenses, including the reasonable fees of attorneys (collectively, "Losses") that the Voyager Indemnified Party may be required to pay to one or more Third Parties to the extent arising out of or resulting from any Third Party suits, claims, actions, proceedings, hearings, investigations, judgments, orders, decrees, stipulations, or injunctions or demands ("Third Party Claims") arising out of or resulting from:

11.1.1 the gross negligence, recklessness or wrongful intentional acts or omissions of Novartis or any of its Affiliates or Sublicensees, or its or their respective directors, officers, employees, agents, consultants or Representatives, in connection with performance by or on behalf of Novartis or exercise of Novartis' rights under this Agreement;

11.1.2 any material breach of this Agreement, including any representation, warranty, or covenant, by Novartis; or

11.1.3 the Exploitation of any Licensed Product conducted by or on behalf of Novartis, any of its Affiliates or any Sublicensee hereunder, including: (a) any product liability, personal injury, property damage or other damage; and (b) infringement of any Patent or other intellectual property right of any Third Party; except, in each case, to the extent such Losses arise from (x) the negligence, recklessness, or intentional acts of any Voyager Indemnified Party, or (y) any Third Party Claim for which Voyager is responsible for indemnifying Novartis pursuant to Section 11.2.

11.2 Indemnification by Voyager. Voyager will indemnify, hold harmless and defend, Novartis and its Affiliates, and its or their respective directors, officers, employees, consultants, agents, and Representatives (each a "Novartis Indemnified Party"), from and against any and all Losses that the Novartis Indemnified Party may be required to pay to one or more Third Parties, to the extent arising out of or resulting from any Third Party Claims arising out of or resulting from:

11.2.1 the gross negligence, recklessness or wrongful intentional acts or omissions of Voyager or any of its Affiliates or subcontractors, or its or their respective directors, officers, employees, agents, consultants or Representatives, in connection with performance by or on behalf of Voyager or exercise of Voyager's rights under this Agreement; or

11.2.2 any material breach of this Agreement, including any representation, warranty, or covenant, by Voyager; except, in each case, to the extent such Losses arise from (x) the negligence, recklessness, or intentional acts of any Novartis Indemnified Party or (y) any Third Party Claim for which Novartis is responsible for indemnifying Voyager pursuant to Section 11.1.

11.3 Notice. Each Party will notify the other Party in writing in the event it becomes aware of a claim for which indemnification may be sought hereunder. In the event that any Third Party asserts a claim or other proceeding (including any governmental investigation) with respect to any Third Party Claim for which a Party (the "Indemnified Party") is entitled to indemnification hereunder, then the Indemnified Party shall promptly notify the Party obligated to indemnify the Indemnified Party (the "Indemnifying Party") thereof; *provided, however,* that no delay on the part of the Indemnified Party in notifying the Indemnifying Party shall relieve the Indemnifying Party from any obligation hereunder unless (and then only to the extent that) the Indemnifying Party is prejudiced thereby.

11.4 Control. Subject to each Party's right to control certain actions described in Sections 8.4 and 8.5 (even where such Party is the Indemnifying Party), the Indemnifying Party shall have the right, exercisable by notice to the Indemnified Party within [**] after receipt of notice from the Indemnified Party of the commencement of or assertion of any Third Party Claim, to assume direction and control of the defense, litigation, settlement, appeal, or other disposition of the Third Party Claim (including the right to settle the claim solely for monetary consideration) with counsel selected by the Indemnifying Party and reasonably acceptable to the Indemnified Party; provided that (a) the Indemnifying Party has sufficient financial resources, in the reasonable judgment of the Indemnified Party, to satisfy the amount of any adverse monetary judgment that is sought, (b) the Third Party Claim seeks solely monetary damages, and (c) the Indemnifying Party expressly agrees in writing that as between the Indemnifying Party and the Indemnified Party, the Indemnifying Party shall be solely obligated to satisfy and discharge the Third Party

Claim in full (the conditions set forth in clauses (a), (b), and (c) above are collectively referred to as the "Litigation Conditions"). Within [**] after the Indemnifying Party has given notice to the Indemnified Party of its exercise of its right to defend a Third Party Claim, the Indemnified Party shall give notice to the Indemnifying Party of any objection thereto based upon the Litigation Conditions. If the Indemnified Party reasonably so objects, the Indemnified Party shall continue to defend the Third Party Claim, at the expense of the Indemnifying Party, until such time as such objection is withdrawn. If no such notice is given, or if any such objection is withdrawn, the Indemnifying Party shall be entitled, at its sole cost and expense, to assume direction and control of such defense, with counsel selected by the Indemnifying Party and reasonably acceptable to the Indemnified Party. During such time as the Indemnifying Party is controlling the defense of such Third Party Claim, the Indemnified Party shall cooperate, and shall cause its Affiliates and agents to cooperate upon request of the Indemnifying Party, in the defense or prosecution of the Third Party Claim, including by furnishing such records, information, and testimony and attending such conferences, discovery proceedings, hearings, trials, or appeals as may reasonably be requested by the Indemnifying Party. In the event that the Indemnifying Party does not satisfy the Litigation Conditions or does not notify the Indemnified Party of the Indemnifying Party's intent to defend any Third Party Claim within [**] after notice thereof, the Indemnified Party may (without further notice to the Indemnifying Party) undertake the defense thereof with counsel of its choice and at the Indemnifying Party's expense (including reasonable, out-of-pocket attorneys' fees and costs and expenses of enforcement or defense). The Indemnifying Party or the Indemnified Party, as the case may be, shall have the right to join in (including the right to conduct discovery, interview, and examine witnesses and participate in all settlement conferences), but not control, at its own expense, the defense of any Third Party Claim that the other Party is defending as provided in this Agreement.

11.5 Settlement. The Indemnifying Party shall not, without the prior written consent of the Indemnified Party (not to be unreasonably withheld, conditioned or delayed), enter into any compromise or settlement that commits the Indemnified Party to take, or to forbear to take, any action (other than the payment of money which will be fully satisfied by the Indemnifying Party). The Indemnified Party shall have the sole and exclusive right to settle any Third Party Claim, on such terms and conditions as it deems reasonably appropriate, to the extent such Third Party Claim involves equitable or other non-monetary relief, but shall not have the right to settle such Third Party Claim to the extent such Third Party Claim involves monetary damages for which the Indemnifying Party would be responsible without the prior written consent of the Indemnifying Party (not to be unreasonably withheld). Each of the Indemnifying Party and the Indemnified Party shall not make any admission of liability in respect of any Third Party Claim without the prior written consent of the other Party, and the Indemnified Party shall use reasonable efforts to mitigate liabilities arising from such Third Party Claim.

11.6 Insurance. Each Party agrees to obtain and maintain, during the Term, commercial general liability insurance, including products liability insurance (or clinical trials insurance, if applicable), with minimum "A-" A.M. Best rated insurance carriers to cover its indemnification obligations under Section 11.1 or Section 11.2, as applicable, in each case with limits of not less than $[**] ([**] U.S. dollars) per occurrence and in the aggregate. All deductibles and retentions will be the responsibility of the named insured. Novartis and its Affiliates will be an additional insured on the Voyager's commercial general liability and products liability policies (or clinical trials insurance, if applicable), and be provided with a waiver of subrogation. For U.S. exposures,

additional insured status Voyager's commercial general liability and products liability policies shall be via form CG20101185 or its equivalent. Licensed Products liability coverage shall be maintained for [**] following termination of this Agreement. To the extent of its culpability or negligence, all coverages of Voyager will be primary and non-contributing with any similar insurance, carried by Novartis. Notwithstanding any provision of this Section 11.6 to the contrary, Novartis may meet its obligations under this Section 11.6 through any combination of insurance and self-insurance. Neither Party's insurance will be construed to create a limit of liability with respect to its indemnification obligations under this ARTICLE 11. Each Party will furnish the other Party with a certificate of such insurance promptly following request.

11.7 Limitation of Liability. EXCEPT FOR A BREACH OF SECTION 10.5, 10.7 OR ARTICLE 9 OR FOR CLAIMS OF A THIRD PARTY THAT ARE SUBJECT TO INDEMNIFICATION UNDER THIS ARTICLE 11, NEITHER VOYAGER NOR NOVARTIS, NOR ANY OF THEIR RESPECTIVE AFFILIATES, LICENSORS, LICENSEES OR SUBLICENSEES, WILL BE LIABLE UNDER THIS AGREEMENT TO THE OTHER PARTY, ITS AFFILIATES OR REPRESENTATIVES FOR ANY INDIRECT, INCIDENTAL, CONSEQUENTIAL, SPECIAL OR PUNITIVE DAMAGES OR LOST PROFITS OR ROYALTIES, LOST DATA OR COST OF PROCUREMENT OF SUBSTITUTE GOODS OR SERVICES, WHETHER LIABILITY IS ASSERTED IN CONTRACT, TORT (INCLUDING NEGLIGENCE AND STRICT LIABILITY), INDEMNITY OR CONTRIBUTION, AND IRRESPECTIVE OF WHETHER THAT PARTY OR ANY REPRESENTATIVE OF THAT PARTY HAS BEEN ADVISED OF, OR OTHERWISE MIGHT HAVE ANTICIPATED THE POSSIBILITY OF, ANY SUCH LOSS OR DAMAGE. Without limiting the generality of the foregoing, "consequential damages" will be deemed to include, and neither Party will be liable to the other Party or any of such other Party's Representatives or stockholders for any damages based on or measured by loss of projected or speculative future sales of the Licensed Products, any payment due upon any unachieved Development Milestone Event, any Sales Milestone Payment due upon any unachieved total annual Net Sales level, any unearned royalties or any other unearned, speculative or otherwise contingent payments provided for in this Agreement.

ARTICLE 12
TERM AND TERMINATION

12.1 Term. This Agreement will commence as of the Effective Date and, unless terminated earlier, this Agreement will continue in full force and effect until, with respect to any Licensed Product(s), on a country-by-country basis, the expiration of the last to expire Royalty Term with respect to such Licensed Product in such country in the Territory (the "Term"). Upon expiration of the Royalty Term for any Licensed Product in any country, the licenses granted with respect to the applicable Licensed Product in such country will become fully paid-up and irrevocable.

12.2 Termination for Breach.

12.2.1 Termination by Voyager. Subject to Section 6.2.6 and the dispute resolution provisions of Section 13.2 and 13.3 (to the extent applicable), this Agreement may be terminated by Voyager (a) on a Licensed Product-by-Licensed Product basis, if Novartis is in material breach of its obligations under this Agreement with respect to such Licensed Product, (b) on Program-by-

Program basis, if Novartis is in material breach of its obligations under this Agreement with respect to a Program, or (c) in its entirety, if Novartis is in material breach of is obligations under this Agreement with respect to all Licensed Products; in each case ((a) through (c)), by providing written notice that includes the particulars of the alleged material breach, and provided the material breach remains uncured for [**] (or [**] in the case of nonpayment), measured from the date written notice of such material breach is given to Novartis (except if any breach is not reasonably curable within [**] (or [**] in the case of a nonpayment) and if Novartis is making a *bona fide* effort to cure such breach, such termination shall be delayed for a time period to be agreed by both Parties in order to permit Novartis a reasonable period of time to cure such breach). If the alleged material breach relates to non-payment of any amount due under this Agreement other than the upfront fee payable under Section 7.1, the cure period shall be tolled pending resolution of any *bona fide*, good faith dispute between the Parties as to whether such payment is due.

12.2.2 Termination by Novartis.

(a) Subject to the dispute resolution provisions of Sections 13.2 and 13.3 (to the extent applicable), this Agreement may be terminated by Novartis (a) on a Licensed Product-by-Licensed Product basis, if Voyager is in material breach of its obligations under this Agreement with respect to such Licensed Product, (b) on Program-by-Program basis, if Voyager is in material breach of its obligations under this Agreement with respect to a Program, or (c) in its entirety, if Voyager is in material breach of is obligations under this Agreement with respect to all Licensed Products; in each case ((a) through (c)), by providing written notice that includes the particulars of the alleged material breach, and provided the material breach remains uncured for [**], measured from the date written notice of such material breach is given to Voyager; provided, however, that if any breach is not reasonably curable within [**] and if Voyager is making a *bona fide* effort to cure such breach, such termination shall be delayed for a time period to be agreed by both Parties in order to permit Voyager a reasonable period of time to cure such breach.

(b) Notwithstanding anything to the contrary in this Agreement and subject to the dispute resolution provisions of Section 13.2 and 13.3 (to the extent applicable), Novartis may terminate this Agreement in whole or relevant part, immediately and without regard to any cure period, if, in Novartis' reasonable opinion, a violation of Global Trade Control Laws has occurred. Any such termination will be deemed for cause under this Section 12.2.2(b), under which Novartis will not be responsible for any related payments due, even if activities have already occurred. Voyager will be responsible for reimbursing Novartis for any payments due to Novartis under this Agreement that are blocked due to violation of Global Trade Control Laws.

(c) Subject to the dispute resolution provisions of Section 13.2 and 13.3 (to the extent applicable), Novartis may terminate this Agreement if Voyager breaches any of the representations or warranties set forth in Sections 10.2.17 through 10.2.18 or if Novartis learns that improper payments are being or have been made to government officials by Voyager with respect to services performed in connection with this Agreement. Further, in the event of such termination, Voyager shall not be entitled to any further payment, regardless of any activities undertaken or agreements with additional Third Parties entered into prior to termination, and Voyager shall be liable for damages or remedies as provided by law.

12.3 Termination by Novartis for Convenience. Novartis may terminate this Agreement in its entirety or on a Licensed Product-by-Licensed Product and country-by-country basis for any or no reason upon ninety (90) days' written notice to Voyager.

12.4 Provisions for Insolvency.

12.4.1 Termination by Novartis for Insolvency Event. If Voyager is deemed a Debtor, then Novartis may terminate this Agreement by providing written notice to Voyager. If Novartis terminates this Agreement pursuant to this Section 12.4.1, then, in addition to all other rights that it may have at law, Novartis will have the right to offset, against any payment owing to Voyager hereunder, any damages found or agreed by the Parties to be owed by Voyager to Novartis. Voyager will be deemed a "Debtor" under this Agreement if, at any time during the Term (a) a case is commenced by or against Voyager under the Bankruptcy Code, (b) Voyager files for or is subject to the institution of bankruptcy, reorganization, liquidation or receivership proceedings (other than a case under the Bankruptcy Code), (c) Voyager assigns all or a substantial portion of its assets for the benefit of creditors, (d) a receiver or custodian is appointed for Voyager's business or (e) a substantial portion of Voyager's business is subject to attachment or similar process; provided, however, that in the case of any involuntary case under the Bankruptcy Code, Voyager will not be deemed a Debtor if the case is dismissed within [**] after the commencement thereof.

12.4.2 Rights to Intellectual Property. All rights and licenses now or hereafter granted by Voyager to Novartis under or pursuant to any Section of this Agreement, including ARTICLE 2 and ARTICLE 3 hereof, are rights to "intellectual property" (as defined in the Bankruptcy Code). The Parties acknowledge and agree that the payments provided for under Section 7.1 and all other payments by Novartis to Voyager hereunder, other than royalty payments pursuant to Section 7.4, do not constitute royalties within the meaning of Section 365(n) of the Bankruptcy Code or relate to licenses of intellectual property hereunder. If: (a) (i) a case under the Bankruptcy Code is commenced by or against Voyager, (ii) this Agreement is rejected as provided in the Bankruptcy Code and (iii) Novartis elects to retain its rights hereunder as provided in Section 365(n) of the Bankruptcy Code; then (b) Voyager (in any capacity, including debtor-in-possession) and its successors and assigns (including any trustee) will provide to Novartis all intellectual property licensed hereunder, and agrees to grant and hereby grants to Novartis and its Affiliates a right to access and to obtain possession of and to benefit from and, in the case of any chemical or biological material or other tangible item of which there is a fixed or limited quantity, to obtain a pro rata portion of, any "embodiments" of intellectual property pursuant to Section 365(n) of the Bankruptcy Code (which will be deemed to include the Voyager Know-How), and all other embodiments of the intellectual property licensed hereunder. Voyager will not interfere with the exercise by Novartis or its Affiliates of rights and licenses to intellectual property licensed hereunder and embodiments thereof in accordance with this Agreement and agrees to use Commercially Reasonable Efforts to assist Novartis and its Affiliates to obtain such intellectual property and embodiments thereof in the possession or control of Third Parties as reasonably necessary or desirable for Novartis or its Affiliates or Sublicensees to exercise such rights and licenses in accordance with this Agreement.

12.4.3 No Limitation of Rights. All rights, powers and remedies of Novartis provided in this Section 12.4 are in addition to and not in substitution for any and all other rights,

powers and remedies now or hereafter existing at Law or in equity (including the Bankruptcy Code) in the event of the commencement of a case under the Bankruptcy Code involving Voyager.

12.5 Effects of Termination.

12.5.1 Termination of the SMA Program or an SMA Program Product. If this Agreement is terminated with respect to the SMA Program, an SMA Program Product (including as to one or more countries) or this Agreement in its entirety, the following will apply with respect to the applicable SMA Program Product(s) upon the effective date of termination:

(a) except as otherwise expressly provided herein, all rights and obligations of each Party hereunder will cease with regard to the terminated SMA Program Product or the SMA Program (including as to one or more countries, as applicable), including, for the avoidance of doubt, all rights to conduct the Evaluation and any and all rights and licenses and sublicenses granted by either Party to the other Party hereunder and in the case of termination of the SMA Program, the exclusivity covenants in Section 10.5.1(a).

(b) the Parties shall discuss and determine, based on mutual consent, whether to seek or continue to seek Patent protection with respect to any data generated from the Evaluation and, if applicable, each Party's rights and obligations with respect to such activities. If the Parties cannot reach mutual written agreement on the course of action to take with respect to the filing, Prosecution and Maintenance of such Patent, neither Party will have any responsibility to file, Prosecute or Maintain such Patent or share in the costs thereof.

12.5.2 Termination of the HD Program or an HD Program Product If this Agreement is terminated with respect to the HD Program, an HD Program Product (including as to one or more countries) or this Agreement in its entirety, the following will apply with respect to the applicable HD Program Product(s) upon the effective date of termination:

(a) except as otherwise expressly provided herein, all rights and obligations of each Party hereunder will cease with regard to the terminated HD Program Product or the HD Program (including as to one or more countries, as applicable), including, for the avoidance of doubt, any and all rights and licenses and sublicenses granted by either Party to the other Party hereunder, and in the case of termination of the HD Program, the exclusivity covenants in Section 10.5.1(b) and Section 10.5.2 will cease;

(b) if Novartis has achieved at least one First Commercial Sale of a terminated HD Program Product, Novartis will have the right to sell or otherwise dispose of any inventory of any such terminated HD Program Product on hand at the time of such termination or in the process of Manufacturing for a period of [**] following the effective date of termination; provided, however, that any revenue obtained from such disposal will be treated as Net Sales and the provisions of ARTICLE 7 will apply to such Net Sales and, in the event that such sales result in the achievement of a Development Milestone Event or a Sales Milestone Event, the Development Milestone Payment or the Sale Milestone Payment due upon achievement of such Development Milestone Event or Sales Milestone Event (as applicable) will be payable;

(c) if Novartis has not achieved at least one First Commercial Sale of a terminated HD Program Product, Novartis will not have the right to sell or otherwise dispose of

any inventory of any terminated HD Program Product on hand at the time of such termination or in the process of Manufacturing and shall transfer any such terminated HD Program Product to Voyager at cost;

(d) if Novartis has terminated the HD Program or an HD Program Product in accordance with Section 12.3 (Convenience) or if Voyager terminates under Section 12.2.1 (For Breach), Novartis shall and hereby does, effective upon such termination, grant to Voyager a worldwide, sublicensable (through multiple tiers), royalty-bearing license under all Patents and Know-How Controlled by Novartis that Cover the terminated HD Program Candidate(s) or HD Program Product(s) (but excluding such Patents and Know-How to the extent of proprietary manufacturing technology of Novartis) for Voyager to Exploit the applicable terminated HD Program Candidate(s) or HD Program Product(s), which license will be (i) non-exclusive or exclusive as requested by Voyager, and (ii) subject to a reasonable royalty to be negotiated in good faith by the Parties within [**] after the effective date of termination, and if the Parties cannot agree to such reasonable royalty within such time period, such reasonable royalty will be determined in accordance with Section 13.2 and Section 13.3.

(e) if Novartis has terminated the HD Program or an HD Program Product in accordance with Section 12.2.2 (For Breach), upon Voyager's request, the Parties shall [**] commercially reasonably financial terms, on which Novartis [**] all Patents and Know-How Controlled by Novartis that Cover the terminated HD Program Candidate(s) or HD Program Product(s), in each case to the extent necessary for Voyager to Exploit the applicable HD Program Candidate(s) or HD Program Product(s), provided that the foregoing [**] shall exclude any access or license to any proprietary manufacturing technology of Novartis;

(f) if Novartis has terminated the HD Program or an HD Program Product in accordance with Section 12.3 (Convenience) or if Voyager terminates under Section 12.2.1 (For Breach), to the extent Novartis or any of its Affiliates or their Sublicensees is engaged in commercial Manufacture of HD Program Product(s) as of the date notice of termination is given, Novartis or such Affiliate or Sublicensee shall, as requested by Voyager, within [**] of such termination, (i) use Commercially Reasonable Efforts to Manufacture and supply (whether directly by Novartis or indirectly through its CMO) such HD Program Product(s) from the effective date of termination until such time as Voyager secures an alternative commercial Manufacturing source reasonably satisfactory to Voyager or until [**] after the effective date of termination, whichever is earlier and (ii) provide reasonable assistance to Voyager (or its designated CMO) to enable Voyager (or its designated CMO) to Manufacture such HD Program Product(s), provided that such assistance shall not require Novartis to disclose or provide access to any of its proprietary manufacturing technology;

(g) if Voyager so requests, and subject to the relevant Third Party's consent if required, Novartis shall assign to Voyager any Third Party contract that solely relates the Exploitation of the terminated HD Program Product, or to the extent any such Third Party contract is not assignable to Voyager, reasonably cooperate with Voyager to arrange for such Third Party to continue performance of such Third Party contract for a reasonable time after termination upon terms reasonably acceptable to Voyager, at Voyager's cost and expense;

(h) if Voyager so requests, Novartis shall transfer all right, title and interest to any Regulatory Filings (including all Regulatory Approvals), pricing and reimbursement approvals in the Territory and any data or information included with or related to the Regulatory Approvals or approvals with respect to the applicable Licensed Products to Voyager; and

(i) Novartis shall, and shall cause its Affiliates and shall use Commercially Reasonable Efforts to cause its Sublicensees to, execute all documents as may be reasonably requested by Voyager in order to give effect to this Section 12.5.2(i).

12.5.3 Novartis Special Remedy in Lieu of Termination. In the event that Novartis has the right to terminate this Agreement for Voyager's uncured material breach with respect to one or more Licensed Product(s) in one or more country(ies) in the Territory or in its entirety pursuant to Section 12.2.2, and provided the event that gave rise to the right of termination materially impairs the ability to Exploit the applicable Licensed Product(s) in the applicable terminated country(ies), then in lieu of such termination, Novartis may, in addition to all other rights that it may have at law, taking into account the permitted reduction in payment below, elect for this Agreement to remain in full force and effect with respect to such Licensed Product(s) in such country(ies), in which case all future obligations to pay any milestones and royalties under ARTICLE 7 with respect to the applicable Licensed Product(s) in the applicable terminated country(ies) shall be reduced by an amount equal to [**] percent ([**]%) of the amount that would otherwise have been payable under this Agreement, such amount to be paid in accordance with and subject to the other terms of this Agreement governing such payments. Novartis may elect the foregoing remedy by providing Voyager with written notice of such election within [**] after the right to terminate accrues. The foregoing will not be construed to limit Voyager's right to receive the full amount of any payments that accrued before the effective date of such election.

12.6 Ongoing Obligation for Confidentiality. Upon early termination of this Agreement for any reason, each Party and its Affiliates shall immediately return to the other Party or destroy any Confidential Information disclosed by the other Party or any of its Affiliates, except for one (1) copy which may be retained in its confidential files for archive or compliance purposes. If the material is destroyed, such Party shall provide the other Party written certification of such destruction. Notwithstanding the foregoing, such Party also shall be permitted to retain such additional copies of or any computer records or files containing such Confidential Information that have been created solely by such Party's automatic archiving and back-up procedures, to the extent created and retained in a manner consistent with such other Party's standard archiving and back-up procedures, but not for any other use or purpose.

12.7 Accrued Rights. Termination or expiration of this Agreement for any reason will be without prejudice to any payments that accrued before the effective date of such termination or expiration or rights that will have accrued to the benefit of any Party prior to such termination or expiration, and any and all damages or remedies arising from any breach hereunder. Such termination or expiration will not relieve any Party from obligations which are expressly indicated to survive expiration or termination of this Agreement.

12.8 Survival. The provisions of ARTICLE 1 (for purposes of interpreting any other surviving provision of this Agreement), Section 5.1.2(b), Section 5.1.3(b), ARTICLE 7 (to the

extent of accrued but unpaid payment obligations, and, with respect to Section 7.8 for a period of three (3) years after termination), Section 8.1, Section 8.3.10, Section 8.3.11, Section 8.4.5, ARTICLE 9, Sections 11.1 through 11.5, Section 11.7, Section 12.4.1, Section 12.5, Section 12.6, Section 12.7, Section 12.8 and ARTICLE 13 (excluding Section 13.8 and Section 13.14), together with any sections that expressly survive (including any perpetual licenses and sublicenses granted hereunder) and remedies for breach of this Agreement, will survive the termination of this Agreement in its entirety or expiration of this Agreement for any reason, in accordance with their respective terms and conditions, and for the duration stated, and where no duration is stated, will survive indefinitely.

ARTICLE 13
MISCELLANEOUS

13.1 Governing Law. This Agreement and any dispute arising from the performance or breach hereof will be governed by and construed and enforced in accordance with the Laws of the Commonwealth of Massachusetts without reference to conflicts of laws principles; provided that with respect to matters involving the enforcement of intellectual property rights, the Laws of the applicable country will apply. The provisions of the United Nations Convention on Contracts for the International Sale of Goods will not apply to this Agreement or any subject matter hereof.

13.2 Dispute Resolution. If a dispute between the Parties arises under this Agreement, either Party will have the right to refer such dispute in writing to the respective Executive Officers, and such Executive Officers will attempt in good faith to resolve such dispute. If the Parties are unable to resolve a given dispute pursuant to this Section 13.2 within [**] after referring such dispute to the Executive Officers, either Party may have the given dispute settled by binding arbitration pursuant to Section 13.3 (subject to the exceptions specified therein).

13.3 Arbitration Request. If a Party intends to begin an arbitration to resolve a dispute arising under this Agreement, such Party will provide written notice (the "Arbitration Request") to the other Party of such intention and a statement of the issues for resolution. From the date of the Arbitration Request and until such time as the dispute has become finally settled, the running of the time periods as to which Party must cure a breach of this Agreement becomes suspended as to any breach that is the subject matter of the dispute.

13.3.1 Additional Issues. Within [**] after the receipt of the Arbitration Request, the other Party may, by written notice, add additional issues for resolution in a statement of counter-issues.

13.3.2 No Arbitration of Patent Issues. Notwithstanding anything to the contrary in this Agreement, any disputes, claims or controversies arising out of, or for which resolution depends in whole or in part on a determination of the ownership, inventorship, interpretation, validity, enforceability, or infringement of United States Patents will not be subject to arbitration under this Agreement, but instead may be brought by either Party in the United States District Court for the District of Delaware, before the United States Patent & Trademark Office, or before United States appellate courts as applicable.

13.3.3 Arbitration Procedure. Any arbitration pursuant to this Section 13.3 will be held in Boston, Massachusetts unless another location is mutually agreed by the Parties. The arbitration will be governed under the rules of the International Chamber of Commerce, to the exclusion of any inconsistent state Law. The Parties will mutually agree on the rules to govern discovery and the rules of evidence for the arbitration within [**] after the Arbitration Request. If the Parties fail to timely agree to such rules, the United States Federal Rules of Civil Procedure will govern discovery and the United States Federal Rules of Evidence will govern evidence for the arbitration. The arbitration will be conducted by three (3) arbitrators, of which each Party will appoint one, and the arbitrators so appointed will select the third and final arbitrator. The arbitrators will have experience of biotechnology and therapeutics licensing disputes. The arbitrators may proceed to an award, notwithstanding the failure of either Party to participate in the proceedings. The arbitrators will, within [**] after the conclusion of the arbitration hearing, issue a written award and statement of decision describing the essential findings and conclusions on which the award is based, including the calculation of any damages awarded. The arbitrators will be limited in the scope of their authority to resolving only the specific matters which the Parties have referred to arbitration for resolution and will not have authority to render any decision or award on any other issues. Subject to Section 11.7, the arbitrators will be authorized to award compensatory damages, but will not be authorized to award punitive, special, consequential, or any other similar form of damages, or to reform, modify, or materially change this Agreement. The arbitrators also will be authorized to grant any temporary, preliminary or permanent equitable remedy or relief the arbitrators deem just and equitable and within the scope of this Agreement, including an injunction or order for specific performance. The award of the arbitrators will be the sole and exclusive remedy of the Parties, except for those remedies that are set forth in this Agreement or which apply to a Party by operation of the applicable provisions of this Agreement, and the Parties hereby expressly agree to waive the right to appeal from the decisions of the arbitrators, and there will be no appeal to any court or other authority (government or private) from the decision of the arbitrators. Judgment on the award rendered by the arbitrator may be enforced in any court having competent jurisdiction thereof, subject only to revocation of the award on grounds set forth in the United Nations Convention on the Recognition and Enforcement of Foreign Arbitral Awards.

13.3.4 Arbitration Expenses. Each Party will bear its own attorneys' fees, costs, and disbursements arising out of the arbitration, and will pay an equal share of the fees and costs of the arbitrator; provided, however, that the arbitrators, in their award, will be authorized to determine whether a Party is the prevailing Party, and if so, to award to that prevailing Party reimbursement for its reasonable attorneys' fees, costs and disbursements (including, for example, expert witness fees and expenses, transcripts, photocopy charges and travel expenses).

13.3.5 Preliminary Injunctions. Notwithstanding anything in this Agreement to the contrary, a Party may seek any remedy at law or in equity, including the issuance of a temporary restraining order or a preliminary, temporary, or permanent injunction from any court of competent jurisdiction in order to preserve or enforce its rights under this Agreement or to prevent immediate and irreparable injury, loss, or damage on a provisional basis, pending the award of the arbitrator on the ultimate merits of any dispute.

13.3.6 Confidentiality. All proceedings and decisions of the arbitrator will be deemed Confidential Information of each of the Parties and will be subject to ARTICLE 9. For clarity, no information concerning an arbitration, beyond the names of the Parties and the relief

requested, may be unilaterally disclosed to a Third Party by any Party unless required by law. In addition, any documentary or other evidence given by a Party or witness in the arbitration shall be treated as Confidential Information by any Party whose access to such evidence arises exclusively as a result of its participation in the arbitration, and shall not be disclosed to any Third Party (other than a witness or expert), except as may be required by applicable Law.

13.4 Assignment. This Agreement may not be assigned or otherwise transferred, nor may any right, interest, or obligation hereunder be assigned or transferred, by either Party without the written consent of the other Party; provided, however, that either Party may assign this Agreement or its rights and obligations under this Agreement to (a) a Third Party that acquires all or substantially all of the business or assets of such Party to which this Agreement relates (whether by merger, reorganization, acquisition, sale or otherwise), and agrees in writing to be bound by the terms of this Agreement, or (b) an Affiliate; provided that in each case of (a) and (b) the assignee will expressly agree to be bound by such Party's obligations under this Agreement and that such Party will remain liable for all of its rights and obligations under this Agreement. Any purported assignment in violation of this Section 13.4 will be void. All terms of this Agreement will remain in full force and effect in the event of a Change of Control of either Party and will be binding upon any Acquiring Entity of either Party. In addition, Novartis may assign its rights and obligations under this Agreement to a Third Party where Novartis or its Affiliate is required or makes a good-faith determination based on advice of legal counsel, to divest a Licensed Product in order to comply with Law or the order of any Governmental Authority as a result of a merger or acquisition, provided that the assignee will expressly agree to be bound by Novartis' obligations under this Agreement. Each Party will promptly notify the other Party of any assignment or transfer under the provisions of this Section 13.4.

13.5 Performance by Affiliates and Sublicensees. Each Party may perform some or all of its obligations or exercise some or all of its rights under this Agreement through Affiliates or Sublicensees; provided that each Party hereby acknowledges and agrees that it will be responsible for the full and timely performance and observance of all the covenants, terms, conditions and agreements set forth in this Agreement by its Affiliate(s) and Sublicensees.

13.6 Force Majeure. No Party will be held liable or responsible to the other Party nor be deemed to be in default under, or in breach of any provision of, this Agreement for failure or delay in fulfilling or performing any obligation (other than a payment obligation) of this Agreement when such failure or delay is due to force majeure, whether or not foreseeable as of the Effective Date or thereafter. For purposes of this Agreement, force majeure is defined as any cause beyond the control of the affected Party and without the fault or negligence of such Party, which may include acts of God; material changes in Law; war; civil commotion; destruction of production facilities or materials by fire, flood, earthquake, explosion or storm; labor disturbances; epidemics, pandemics, and the spread of infectious diseases, including COVID-19 (as defined by the World Health Organization and any of the strains, variants, or mutations thereof); quarantines; and failure of public utilities or common carriers. In such event the Party affected by such force majeure will immediately notify the other Party of such inability and a good faith estimate of the period for which such inability is expected to continue based on currently available information. The Party giving such notice will thereupon be excused from such of its obligations under this Agreement as it is thereby disabled from performing for so long as the condition constituting force majeure continues and the non-performing Party takes Commercially Reasonable Efforts to remove the

condition, after which time the Parties will promptly meet to discuss in good faith how to best proceed in a manner consistent with this Agreement. To the extent possible, each Party will use reasonable efforts to minimize the duration of any force majeure.

13.7 Notices. Any notice or request required or permitted to be given under or in connection with this Agreement will be deemed to have been sufficiently given if in writing and personally delivered or sent by certified mail (return receipt requested), or overnight express courier service (signature required), prepaid, to the Party for which such notice is intended, at the address set forth for such Party below:

If to Voyager,

addressed to:

Voyager Therapeutics, Inc.
75 Hayden Ave, Lexington, MA 02421
Attention: Chief Executive Officer

with a copy to (which will not constitute notice):

Voyager Therapeutics, Inc.
75 Hayden Ave, Lexington, MA 02421
Attention: Chief Legal Officer
Email: [**]

Wilmer Cutler Pickering Hale and Dorr LLP
60 State Street
Boston, MA 02109
Attention: Brian A. Johnson, Esq.
Sarah T. Hogan, Esq.

Email: brian.johnson@wilmerhale.com
sarah.hogan@wilmerhale.com

and an email copy to (which will not constitute notice): Voyager's Alliance Manager, to the contact information provided in accordance with Section 4.5.

If to Novartis,

addressed to:

Novartis Pharma AG
Lichtstrasse 35
CH-4056 Basel
Switzerland
Attn: Head of NIBR General Legal, Europe
And an email copy to: [**]

with a copy to (which will not constitute notice):

Novartis Institutes for BioMedical Research, Inc.
250 Massachusetts Avenue
Cambridge, MA 02139 USA
Attn: General Counsel

and

Arnold & Porter Kaye Scholer LLP
250 W 55th Street
New York, NY 10019
Attn: Eric Rothman
Email: eric.rothman@arnoldporter.com

and an email copy to (which will not constitute notice): Novartis's Alliance Manager, to the contact information provided in accordance with Section 4.5.

Copies of notices may be provided to such other address for such Party as it will have specified by like notice to the other Party, provided that notices of a change of address will be effective only upon receipt thereof. If delivered personally, the date of delivery will be deemed to be the date on which such notice or request was given. If sent by overnight express courier service, the date of delivery will be deemed to be the next Business Day after such notice or request was deposited with such service. If sent by certified mail, the date of delivery will be deemed to be the third (3rd) Business Day after such notice or request was deposited with the U.S. Postal Service.

13.8 Global Trade Control Laws. The Parties acknowledge that certain activities covered by or performed under this Agreement may be subject to laws, regulations, or orders regarding economic sanctions, import controls, or export controls ("Global Trade Control Laws"). Each of the Parties will perform all activities under this Agreement in compliance with all applicable Global Trade Control Laws. Furthermore, with respect to the activities performed under this Agreement, each of the Parties represents, warrants, and covenants that:

13.8.1 It will not, for activities under this Agreement, (i) engage in any such activities in a Restricted Market; (ii) involve individuals ordinarily resident in a Restricted Market; or (iii) include companies, organizations or Governmental Entities from or located in a Restricted Market. "Restricted Market" for purposes of this Agreement means the Crimean Peninsula, Cuba, the Donbass Region, Iran, North Korea, Sudan, and Syria, or any other country or region sanctioned by the United States or European Union.

13.8.2 It is not a Restricted Party and is not owned or controlled by a Restricted Party. With respect to activities performed under this Agreement, neither Party will engage or delegate to any Restricted Party for any activities under this Agreement. Each Party will screen all relevant third parties involved by such Party in the activities under this Agreement under the relevant Restricted Party Lists. "Restricted Party" for purposes of this Agreement means any individual or entity on any of the following "Restricted Party Lists": the list of sanctioned entities maintained by the United Nations; the Specially Designated Nationals List and the Sectoral Sanctions Identifications List of the U.S. Treasury Department's Office of Foreign Assets Control;

the U.S. Denied Persons List, the U.S. Entity List, and the U.S. Unverified List of the U.S. Department of Commerce; entities subject to restrictive measures and the Consolidated List of Persons, Groups and Entities Subject to E.U. Financial Sanctions, as implemented by the E.U. Common Foreign & Security Policy; the List of Excluded Individuals / Entities published by the U.S. Health and Human Services' Office of Inspector General; any lists of prohibited or debarred parties established under the U.S. Federal Food Drug and Cosmetic Act; the list of parties suspended or debarred from contracting with the U.S. government; and similar lists of restricted parties maintained by the Governmental Authorities of the countries that have jurisdiction over the activities conducted under this Agreement.

13.8.3 It will not knowingly transfer to the other Party any goods, software, technology or services that are (i) controlled under the U.S. International Traffic in Arms Regulations or at a level other than EAR99 under the U.S. Export Administration Regulations; or (ii) specifically identified as an E.U. Dual Use Item or on an applicable export control list of another country.

13.9 Waiver. No provision of this Agreement will be waived by any act, omission or knowledge of a Party or its agents or employees except by an instrument in writing expressly waiving such provision and signed by a duly authorized officer of the waiving Party. The failure of either Party to assert a right hereunder or to insist upon compliance with any term of this Agreement will not constitute a waiver of that right or excuse a similar subsequent failure to perform any such term or condition. No waiver by either Party of any condition or term in any one or more instances will be construed as a continuing waiver of such condition or term or of another condition or term except to the extent set forth in writing.

13.10 Severability. If any provision hereof should be invalid, illegal or unenforceable in any jurisdiction, the Parties will negotiate in good faith a valid, legal and enforceable substitute provision that most nearly reflects the original intent of the Parties and all other provisions hereof will remain in full force and effect in such jurisdiction and will be construed in order to maintain this Agreement's existence, validity and enforceability to the greatest extent possible and to carry out the intentions of the Parties as nearly as may be possible. Such invalidity, illegality or unenforceability will not affect the validity, legality or enforceability of such provision in any other jurisdiction.

13.11 Entire Agreement. This Agreement, together with the Schedules hereto, constitutes and contains the complete, final and exclusive understanding and agreement of the Parties and sets forth all the covenants, promises, agreements, warranties, representations, conditions and understandings between the Parties and supersede and terminate all prior agreements negotiations, correspondence, agreements, and understanding, whether oral or written, between the Parties with respect to the subject matter of this Agreement. The Parties agree that the Option and License Agreement between the Parties, effective as of March 4, 2022, is a separate and distinct agreement from this Agreement, and neither this Agreement nor such Option and License Agreement will have any effect on the other. No subsequent alteration, amendment, change or addition to this Agreement will be valid or effective unless reduced to writing and signed by the respective authorized officers of each Party.

13.12 Independent Contractors. Nothing herein will be construed to create any relationship of employer and employee, agent and principal, partnership or joint venture or any other legal arrangement that would impose liability upon one Party for the act or failure to act of the other Party between the Parties. Each Party is an independent contractor under this Agreement. Neither Party will have the authority to enter into any contracts or commitments or to incur any liabilities in the name of, or on behalf of, the other Party, or to bind or obligate the other Party and neither Party will represent that it has such authority.

13.13 Headings; Construction; Interpretation. Headings and any table of contents used herein are for convenience only and will not in any way affect the construction of or be taken into consideration in interpreting this Agreement. The terms of this Agreement represent the results of negotiations between the Parties and their Representatives, each of which has been represented by counsel of its own choosing, and neither of which has acted under duress or compulsion, whether legal, economic or otherwise. Accordingly, the terms of this Agreement will be interpreted and construed in accordance with their usual and customary meanings, and each of the Parties hereby waives the application in connection with the interpretation and construction of this Agreement of any rule of Law to the effect that ambiguous or conflicting terms or provisions contained in this Agreement will be interpreted or construed against the Party whose attorney prepared the executed draft or any earlier draft of this Agreement. Any reference in this Agreement to an Article, Section, subsection, paragraph, clause, Schedule will be deemed to be a reference to any Article, Section, subsection, paragraph, clause, Schedule, of or to, as the case may be, this Agreement. Except where the context otherwise requires, (a) any definition of or reference to any agreement, instrument or other document refers to such agreement, instrument other document as from time to time amended, supplemented or otherwise modified (subject to any restrictions on such amendments, supplements or modifications set forth herein or therein), (b) any reference to any Law includes all rules and regulations thereunder and any successor Law, in each case as from time to time enacted, repealed or amended, (c) the words “herein,” “hereof” and “hereunder,” and words of similar import, refer to this Agreement in its entirety and not to any particular provision hereof, (d) the words “include,” “includes,” “including,” “exclude,” “excludes,” and “excluding,” will be deemed to be followed by the phrase “but not limited to,” “without limitation” or words of similar import, (e) the word “or” is used in the inclusive sense (and/or), (f) words in the singular or plural form include the plural and singular form, respectively, (g) references to any gender refer to each other gender, (h) references to a particular Person include such Person’s successors and assigns to the extent not prohibited by this Agreement, and (i) a capitalized term not defined herein but reflecting a different part of speech than a capitalized term which is defined herein will be interpreted in a correlative manner.

13.14 Further Actions. Each Party will execute, acknowledge and deliver such further instruments, and do all such other acts, as may be necessary or appropriate in order to carry out the expressly stated purposes and the clear intent of this Agreement.

13.15 Parties in Interest. All of the terms and provisions of this Agreement will be binding upon, and will inure to the benefit of and be enforceable by the parties hereto and their respective successors, heirs, administrators and permitted assigns.

13.16 Counterparts. This Agreement may be signed in counterparts, each and every one of which will be deemed an original, notwithstanding variations in format or file designation which

may result from the electronic transmission, storage and printing of copies from separate computers or printers. Facsimile signatures and signatures transmitted via PDF will be treated as original signatures.

[Signature page follows.]

IN WITNESS WHEREOF, and intending to be legally bound hereby, the Parties have caused this Agreement to be executed by their duly authorized representatives as of the Effective Date.

Voyager Therapeutics, Inc.

By: /s/ Alfred Sandrock
Name: Alfred Sandrock, M.D., Ph.D.
Title: Chief Executive Officer and President

Novartis Pharma AG

By: /s/ Ian James Hiscock
Name: Ian James Hiscock
Title: Head Global IP Lit. Transactions

By: /s/ Marc Ceulemans
Name: Marc Ceulemans
Title: Head Capital Venture Fund Management

[Signature page to License and Collaboration Agreement]

Exhibit 10.44

EXECUTIBE VERSION

STOCK PURCHASE AGREEMENT

By and Between

NOVARTIS PHARMA AG

AND

VOYAGER THERAPEUTICS, INC.

Dated as of December 28, 2023

TABLE OF CONTENTS

			Page
1.	Definitions		1
	1.1	Defined Terms	1
	1.2	Additional Defined Terms	4
2.	Purchase and Sale of Common Stock		5
3.	Closing Date; Deliveries		6
	3.1	Closing Date	6
	3.2	Deliveries	6
4.	Representations and Warranties of the Company		6
	4.1	Organization, Good Standing and Qualification	7
	4.2	Capitalization and Voting Rights	7
	4.3	Subsidiaries	8
	4.4	Authorization	8
	4.5	No Defaults	9
	4.6	No Conflicts	9
	4.7	No Governmental Authority or Third-Party Consents	9
	4.8	Valid Issuance of Shares	10
	4.9	Litigation	10
	4.10	Licenses and Other Rights; Compliance with Laws	10
	4.11	Company SEC Documents; Financial Statements; Nasdaq Stock Market	10
	4.12	Absence of Certain Changes	12
	4.13	Offering	13
	4.14	No Integration	13
	4.15	Brokers' or Finders' Fees	13
	4.16	Investment Company	13
	4.17	No General Solicitation	13
	4.18	Foreign Corrupt Practices	14
	4.19	Regulation M Compliance	14
	4.20	Office of Foreign Assets Control	14
	4.21	Development Matters	14
	4.22	Intellectual Property	15
	4.23	Real and Personal Property	16

	4.24	Labor and Employment	16
	4.25	ERISA Matters	17
	4.26	Environmental Matters	17
	4.27	Taxes	17
	4.28	Insurance	18
	4.29	Cybersecurity	18
	4.30	Related Party Transactions	18
	4.31	CFIUS Representations	18
	4.32	No Disqualification Events	19
5.		Representations and Warranties of the Investor	19
	5.1	Organization; Good Standing	19
	5.2	Authorization	19
	5.3	No Conflicts	19
	5.4	No Governmental Authority or Third-Party Consents	20
	5.5	Purchase Entirely for Own Account	20
	5.6	Disclosure of Information	20
	5.7	Investment Experience and Accredited Investor Status	20
	5.8	Acquiring Person	20
	5.9	No “Bad Actor” Disqualification	21
	5.10	Restricted Securities	21
	5.11	Legends	21
	5.12	Financial Assurances	21
	5.13	SEC Reports	21
	5.14	HSR Act	21
6.		Investor’s Conditions to Closing	22
	6.1	Representations and Warranties	22
	6.2	Representations and Warranties in the Collaboration Agreement	22
	6.3	Covenants	22
	6.4	Investor Agreement	22
	6.5	Collaboration Agreement	22
	6.6	No Material Adverse Effect	22
	6.7	Listing	22
	6.8	Closing Deliverables	23
7.		Company’s Conditions to Closing	23

7.1 Representations and Warranties 23
7.2 Covenants 23
7.3 Investor Agreement 23
7.4 Collaboration Agreement 23
8. Mutual Conditions to Closing 23
8.1 Absence of Litigation 23
8.2 No Prohibition 23
9. Termination 23
9.1 Ability to Terminate 24
9.2 Effect of Termination 24
10. Additional Covenants and Agreements 24
10.1 Market Listing 24
10.2 Assistance and Cooperation 25
10.3 Legend Removal 25
10.4 Conduct of Business 26
10.5 Right to Conduct Activities 26
11. Miscellaneous 27
11.1 Governing Law; Submission to Jurisdiction 27
11.2 Waiver 27
11.3 Notices 27
11.4 Entire Agreement 27
11.5 Headings; Nouns and Pronouns; Section References 28
11.6 Severability 28
11.7 Assignment 28
11.8 Parties in Interest 28
11.9 Counterparts 28
11.10 Third-Party Beneficiaries 28
11.11 No Strict Construction 28
11.12 Survival of Warranties 28
11.13 Remedies 29
11.14 Expenses 29
11.15 No Publicity 29

Exhibit A – Notices
Exhibit B – Wire Instructions

STOCK PURCHASE AGREEMENT

THIS STOCK PURCHASE AGREEMENT (this "**Agreement**") is dated as of December 28, 2023 (the "**Signing Date**"), by and between Novartis Pharma AG (the "**Investor**"), a corporation organized and existing under the laws of Switzerland, with its principal business office at Lichtstrasse 35, CH-4056 Basel, Switzerland, and Voyager Therapeutics, Inc. (the "**Company**"), a Delaware corporation, with its principal place of business at 75 Hayden Avenue, Lexington, MA 02421.

WHEREAS, pursuant to the terms and subject to the conditions set forth in this Agreement, the Company desires to issue and sell to the Investor, and the Investor desires to subscribe for and purchase from the Company, certain shares of common stock, par value $0.001 per share, of the Company (the "**Common Stock**"); and

WHEREAS, simultaneously with the execution of this Agreement, the Company and the Investor are entering into the Collaboration Agreement and the Investor Agreement (in each case, as defined below).

NOW, THEREFORE, in consideration of the following mutual promises and obligations, and for good and valuable consideration, the adequacy and sufficiency of which are hereby acknowledged, the Investor and the Company agree as follows:

1. Definitions.

1.1 Defined Terms. When used in this Agreement, the following terms shall have the respective meanings specified therefor below:

"**2014 Stock Option and Grant Plan**" shall mean the Company's 2014 Stock Option and Grant Plan, as amended to date and as the same may be amended and/or restated from time to time.

"**2015 Employee Stock Purchase Plan**" shall mean the Company's 2015 Employee Stock Purchase Plan, as amended to date and as the same may be amended and/or restated from time to time.

"**2015 Stock Option and Incentive Plan**" shall mean the Company's 2015 Stock Option and Incentive Plan, as amended to date and as the same may be amended and/or restated from time to time.

"**Affiliate**" shall mean, with respect to any Person, another Person that, directly or indirectly through one or more intermediaries, controls, is controlled by or is under common control with such Person. A Person shall be deemed to control another Person if such Person possesses, directly or indirectly, the power to direct or cause the direction of the management and policies of such other Person, whether through the ownership of voting securities, by contract or otherwise. Without limiting the generality of the foregoing, a Person shall be deemed to control another Person if such Person (i) owns, directly or indirectly, beneficially or legally, more than fifty percent (50%) of the outstanding voting securities or capital stock of such other Person, or has other comparable ownership interest(s) with respect to any Person other than a corporation; or (ii) has the power, whether pursuant to contract, ownership of securities or otherwise, to direct the

management and policies of such other Person. For the purposes of this Agreement, in no event shall the Investor or any of its Affiliates be deemed Affiliates of the Company or any of the Company's Affiliates, nor shall the Company or any of the Company's Affiliates be deemed Affiliates of the Investor or any of its Affiliates.

"**Aggregate Purchase Price**" shall mean the product of the number of Shares issuable hereunder and the Per-Share Purchase Price.

"**Agreement**" shall have the meaning set forth in the Preamble, including all Exhibits attached hereto.

"**Board**" shall mean the Board of Directors of the Company.

"**Business Day**" shall mean a day on which banking institutions in Boston, Massachusetts, United States and Basel, Switzerland are open for business, excluding any Saturday or Sunday.

"**Closing Conditions**" shall mean the conditions to Closing set forth in Sections 6, 7, and 8 hereof.

"**Collaboration Agreement**" shall mean the License and Collaboration Agreement, of even date herewith, between the Investor and the Company, as the same may be amended and/or restated from time to time.

"**Company Covered Person**" shall mean, with respect to the Company as an "issuer" for purposes of Rule 506 promulgated under the Securities Act, any Person listed in the first paragraph of Rule 506(d)(1).

"**Company Financial Advisors**" shall mean Chestnut Securities, Inc.

"**Disqualification Event**" shall mean a "bad actor" disqualifying event described in Rule 506(d)(1)(i)-(viii) promulgated under the Securities Act.

"**DOJ**" shall mean the U.S. Department of Justice.

"**Effect**" shall have the meaning set forth in the definition of "Material Adverse Effect."

"**Exchange Act**" shall mean the Securities Exchange Act of 1934, as amended, and the rules and regulations promulgated thereunder.

"**Forward-Looking Disclosures**" shall mean any general or categorical forward-looking statements that are primarily predictive or cautionary in nature, that do not include a reference to a specific event or circumstance that is currently impacting or has previously impacted the Company, and that are set forth in any "risk factors" section or "forward-looking statements" section of the Company SEC Documents. For the avoidance of doubt, no statement of historical or current fact shall be considered a Forward-Looking Disclosure for the purposes of this Agreement.

"**FTC**" shall mean the U.S. Federal Trade Commission.

"**FTC Act**" shall mean the Federal Trade Commission Act, as amended.

"**GAAP**" shall mean generally accepted accounting principles in the United States.

"**Governmental Authority**" shall mean any multinational, federal, national, state, provincial, local or other entity, office, commission, bureau, agency, political subdivision, instrumentality, branch, department, authority, board, court, arbitral or other tribunal exercising executive, judicial, legislative, police, regulatory, administrative or taxing authority or functions of any nature pertaining to government.

"**HSR Act**" shall mean the Hart-Scott-Rodino Antitrust Improvements Act of 1976, as amended from time to time.

"**Investor Agreement**" shall mean that certain Investor Agreement, of even date herewith, between the Investor and the Company, as the same may be amended and/or restated from time to time.

"Knowledge" shall mean the actual knowledge after reasonable investigation of the following individuals and assuming such knowledge as would be obtained from the reasonable performance of such individual's duties in the ordinary course: the Company's Chief Executive Officer, Chief Financial Officer, Chief Patent Counsel, Chief Scientific Officer, and Chief Legal Officer.

"LAS" shall mean the Nasdaq Notification Form: Listing of Additional Shares.

"**Law"** shall mean any law, statute, rule, regulation, order, judgment or ordinance having the effect of law of any federal, national, multinational, state, provincial, county, city or other political subdivision.

"**Material Adverse Effect**" shall mean any change, event or occurrence (each, an "**Effect**") that, individually or when taken together with all other Effects that have occurred prior to the date of determination of the occurrence of the Material Adverse Event, has had a material adverse effect on the business, properties, management, financial position, stockholders' equity or results of operations of the Company and its Subsidiaries taken as a whole or on the performance by the Company of its obligations under the Transaction Agreements, except to the extent that any such Effect results from or arises out of: (A) changes in conditions in the United States or global economy or capital or financial markets generally, including changes in interest or exchange rates, (B) changes in general legal, regulatory, political, economic or business conditions or changes in generally accepted accounting principles in the United States or interpretations thereof, (C) acts of war, sabotage or terrorism, or any escalation or worsening of any such acts of war, sabotage or terrorism, (D) earthquakes, hurricanes, floods or other natural disasters, (E) any epidemic, pandemic, or disease outbreak (including the COVID-19 virus) or any escalation or worsening thereof, (F) the announcement of the Transaction Agreements, the Collaboration Agreement or the Transaction, (G) any change in the Company's stock price or trading volume or any failure to meet internal projections or forecasts or published revenue or earnings projections of industry analysts (provided that the underlying events giving rise to any such change shall not be excluded) or (H) any breach, violation or non-performance by the Investor or any of its Affiliates under the Collaboration Agreement, provided, however, that the Effects excluded in clauses (A), (B), (C),

(D) and (E) shall only be excluded to the extent such Effects are not disproportionately adverse on the Company and its Subsidiaries as compared to other companies operating in the Company's industry.

"**Per-Share Purchase Price**" shall mean $9.324.

"**Person"** shall mean any individual, partnership, joint venture, limited liability company, corporation, firm, trust, association, unincorporated organization, Governmental Authority or other entity, as well as any syndicate or group that would be deemed to be a Person under Section 13(d)(3) of the Exchange Act.

"**Rule 144**" shall mean Rule 144 promulgated under the Securities Act.

"**Sales Agreement**" shall mean that certain Sales Agreement, by and between the Company and Cowen and Company, LLC, dated as of November 8, 2022.

"**SEC**" shall mean the U.S. Securities and Exchange Commission.

"**Securities Act**" shall mean the Securities Act of 1933, as amended, and the rules and regulations promulgated thereunder.

"**Termination Date**" shall mean the date that is three (3) months after the Signing Date.

"**Third Party"** shall mean any Person other than the Investor, the Company or any Affiliate of the Investor or the Company.

"**Transaction"** shall mean the issuance and sale of the Shares by the Company, and the purchase of the Shares by the Investor, in accordance with the terms hereof.

"**Transaction Agreements"** shall mean this Agreement and the Investor Agreement.

"**Transfer Agent**" shall mean the Company's transfer agent.

1.2 Additional Defined Terms. In addition to the terms defined in Section 1.1 hereof, the following terms shall have the respective meanings assigned thereto in the sections indicated below:

Defined Term	Section
By-laws	Section 3.2(a)
Charter	Section 3.2(a)
Closing	Section 3.1
Closing Date	Section 3.1

Defined Term	Section
Code	Section 4.25
Common Stock	Recitals
Company	Preamble
Company SEC Documents	Section 4.11(a)
Company Studies and Trials	Section 4.21(b)
DPA	Section 4.31
ERISA	Section 4.25
Enforceability Exceptions	Section 4.4(b)
Environmental Laws	Section 4.26
Existing Business	Section 10.5
FDA	Section 4.21(b)
Investor	Preamble
Investor Group	Section 10.5
Intellectual Property Rights	Section 4.22
Intellectual Property Assets	Section 4.22
IT Systems and Data	Section 4.29
Modified Clause	Section 11.6
OFAC	Section 4.20
Shares	Section 2
Signing Date	Preamble
Subsidiary/Subsidiaries	Section 4.3

2. Purchase and Sale of Common Stock.

Subject to the terms and conditions of this Agreement, at the Closing, the Company shall issue and sell to the Investor, and the Investor shall purchase from the Company, 2,145,002 shares of Common Stock (the "**Shares**").

3. Closing Date; Deliveries.

3.1 Closing Date. The closing of the purchase and sale of the Shares hereunder (the "**Closing**") shall take place remotely via the exchange of documents and signatures at 9:00 a.m. New York City time on the third (3rd) Business Day following the satisfaction or waiver of all of the Closing Conditions (other than those conditions that by their nature are to be satisfied at the Closing, but subject to the satisfaction at such time of such conditions), or at such other time, date, and location as the parties may agree. The date the Closing occurs is hereinafter referred to as the "**Closing Date**."

3.2 Deliveries.

(a) Deliveries by the Company. At the Closing, the Company shall deliver, or cause to be delivered, to the Investor the Shares, registered in the name of the Investor, and the Company shall instruct its transfer agent to register such issuance at the time of such issuance. The Company shall also deliver at the Closing: (i) a certificate in form and substance reasonably satisfactory to the Investor and duly executed on behalf of the Company by an authorized executive officer of the Company, certifying that the conditions to Closing set forth in Sections 6 and 8.1 hereof have been fulfilled and (ii) a certificate of the secretary or assistant secretary of the Company dated as of the Closing Date certifying (A) that attached thereto is a true and complete copy of the Amended and Restated By-laws of the Company as in effect at the time of the actions by the Board referred to in clause (B) below and on the Closing Date (the "**By-laws**"); (B) that attached thereto is a true and complete copy of all resolutions adopted by the Board authorizing the execution, delivery and performance of the Transaction Agreements, the Collaboration Agreement and the transactions contemplated hereunder and thereunder and that all such resolutions are in full force and effect and are all the resolutions adopted in connection with the transactions contemplated hereby and thereby as of the Closing Date; (C) that attached thereto is a true and complete copy of the Company's Fifth Amended and Restated Certificate of Incorporation as in effect at the time of the actions by the Board referred to in clause (B) above and on the Closing Date (the "**Charter**"); and (D) as to the incumbency and specimen signature of any officer of the Company executing a Transaction Agreement or the Collaboration Agreement on behalf of the Company.

(b) Deliveries by the Investor. At the Closing, the Investor shall deliver, or cause to be delivered, to the Company the Aggregate Purchase Price by wire transfer of immediately available United States funds to an account designated by the Company in accordance with the wire instructions attached as Exhibit A hereto. The Investor shall also deliver, or cause to be delivered, at the Closing: (i) a certificate in form and substance reasonably satisfactory to the Company duly executed by an authorized executive officer or authorized representative of the Investor certifying that the conditions to Closing set forth in Section 7 hereof have been fulfilled and (ii) a certificate of the secretary or assistant secretary or authorized representative of the Investor dated as of the Closing Date certifying as to the incumbency and specimen signature of any officer or representative executing a Transaction Agreement or the Collaboration Agreement on behalf of the Investor.

4. Representations and Warranties of the Company. The Company hereby represents and warrants to the Investor that:

4.1 Organization, Good Standing and Qualification.

(a) The Company has been duly organized and is validly existing and in good standing under the Laws of Delaware, is duly qualified to do business and is in good standing in each jurisdiction in which its ownership or lease of property or the conduct of its businesses requires such qualification, and has all power and authority necessary to own or hold its properties and to conduct the businesses in which it is engaged, except where the failure to be so qualified or in good standing or have such power or authority would not, individually or in the aggregate, have a Material Adverse Effect. The Charter and the Bylaws are each filed or incorporated by reference as exhibits to the Company SEC Documents.

(b) The Company has all requisite corporate power and corporate authority to enter into the Transaction Agreements and the Collaboration Agreement, to issue and sell the Shares and to perform its obligations hereunder and to carry out the other transactions contemplated by the Transaction Agreements and the Collaboration Agreement.

4.2 Capitalization and Voting Rights.

(a) As of the Signing Date, the authorized capital of the Company consists of: (i) 120,000,000 shares of Common Stock, of which (A) 44,003,425 shares are issued and outstanding, (B) 9,306,676 shares are issuable upon the exercise of outstanding stock options or upon the settlement of outstanding equity awards issued pursuant to the 2014 Stock Option and Grant Plan, the 2015 Stock Option and Incentive Plan, or inducement awards in accordance with Nasdaq Listing Rule 5635(c)(4), (C) 3,062,527 shares are reserved for future issuance pursuant to the 2015 Stock Option and Incentive Plan, and (D) 2,208,281 shares are reserved for future issuance pursuant to the 2015 Employee Stock Purchase Plan, and (ii) 5,000,000 shares of preferred stock, par value $0.001 per share, of which no shares are issued and outstanding. The Company is also party to the Sales Agreement pursuant to which the Company may issue and sell shares of its Common Stock having an aggregate offering price of up to $75,000,000 through Cowen and Company, LLC, from time to time, in “at-the-market” offerings or certain negotiated transactions. All of the issued and outstanding shares of Common Stock have been duly authorized and validly issued and are fully paid and non-assessable, were issued in compliance with federal and state securities Laws, and are not subject to any pre-emptive rights.

(b) Except as described or referred to in Section 4.2(a) above, as of the Signing Date, there are no outstanding rights (including, without limitation, pre-emptive rights), warrants or options to acquire, or instruments convertible into or exchangeable for, any shares of capital stock or other equity interest in the Company, or any contract, commitment, agreement, understanding or arrangement of any kind relating to the issuance of any capital stock of the Company, any such convertible or exchangeable securities or any such rights, warrants or options. Neither the execution of this Agreement nor the issuance of the Shares will give rise to any preemptive rights, rights of first refusal or similar rights on behalf of any Person. There are no obligations (contingent or otherwise) on the part of the Company to repurchase, redeem or otherwise acquire any of the Company’s equity securities or any interests therein or to pay any dividend or make any distribution in respect thereof.

(c) Except as disclosed in the Company SEC Documents, no Person has any right to cause the Company to effect the registration under the Securities Act of any securities of the Company.

(d) The Common Stock is registered pursuant to Section 12(b) or 12(g) of the Exchange Act, and the Company has taken no action designed to terminate, or which to its Knowledge is likely to have the effect of terminating, the registration of the Common Stock under the Exchange Act nor has the Company received any notification that the SEC is contemplating terminating such registration.

(e) Except as disclosed in the Company SEC Documents, the Company is not a party to or subject to any agreement or understanding relating to the voting of shares of capital stock of the Company. All of the authorized shares of Common Stock are entitled to one vote per share.

(f) The Company does not have outstanding any stockholder rights plans or "poison pill" or any similar arrangement in effect giving any person the right to purchase any equity interest in the Company upon the occurrence of certain events.

4.3 Subsidiaries. As of the Signing Date, the Company does not own or control, directly or indirectly, any corporation, association or other entity other than the subsidiaries listed in Schedule 1 hereto (each, a "**Subsidiary**" and collectively, the "**Subsidiaries**"). All the outstanding shares of capital stock or other equity interests of each subsidiary owned, directly or indirectly, by the Company have been duly authorized and validly issued, are fully paid and non-assessable (except, in the case of any foreign subsidiary, for directors' qualifying shares) and are owned directly or indirectly by the Company, free and clear of any lien, charge, encumbrance, security interest, restriction on voting or transfer or any other claim of any Third Party. Each Subsidiary that owns any assets material to the Company has been duly incorporated or organized, as the case may be, and is validly existing as a corporation, partnership or limited liability company, as applicable, in good standing under the Laws of the jurisdiction of its incorporation or organization and has the power and authority (corporate or other) to own, lease and operate its properties and to conduct its business as presently conducted, except where the failure to be in good standing would not reasonably be expected to have a Material Adverse Effect. Each Subsidiary is duly qualified as a foreign corporation, partnership or limited liability company, as applicable, to do business and is in good standing in each jurisdiction in which such qualification is required, whether by reason of the ownership or leasing of property or the conduct of business, except where the failure to so qualify or be in good standing would not reasonably be expected to have, individually or in the aggregate, a Material Adverse Effect.

4.4 Authorization.

(a) The Company has full right, power and authority to execute and deliver the Transaction Agreements and the Collaboration Agreement and to perform its obligations hereunder and thereunder; and all action required to be taken for the due and proper authorization, execution and delivery by it of each of the Transaction Agreements and the Collaboration Agreement and the consummation by it of the transactions contemplated thereby has been duly and validly taken.

(b) The Transaction Agreements and the Collaboration Agreement have been duly executed and delivered by the Company and, upon the due execution and delivery of the Transaction Agreements and the Collaboration Agreement by the Investor, will constitute valid and legally binding obligations of the Company, enforceable against the Company in accordance with their respective terms, except, with respect to the Investor Agreement and the Collaboration Agreement, as enforceability may be limited by applicable bankruptcy, insolvency, reorganization, moratorium or similar Laws affecting creditors' rights generally or by equitable principles relating to enforceability (collectively, the "**Enforceability Exceptions**").

(c) No stop order or suspension of trading of the Common Stock has been imposed by the Nasdaq Stock Market, the SEC or any other Governmental Authority and remains in effect.

4.5 No Defaults. The Company is not (i) in violation of its Charter or By-laws or similar organizational documents; (ii) in default, and no event has occurred that, with notice or lapse of time or both, would constitute such a default, in the due performance or observance of any term, covenant or condition contained in any indenture, mortgage, deed of trust, loan agreement or other agreement or instrument to which the Company is a party, by which the Company is bound or to which any of the property or assets of the Company is subject; or (iii) in violation of any Law or any judgment, order, rule or regulation of any Governmental Authority having jurisdiction over the Company or any of its Subsidiaries, except, in the case of clauses (ii) and (iii) above, for any such default or violation that would not, individually or in the aggregate, have a Material Adverse Effect.

4.6 No Conflicts. The execution, delivery and performance of the Transaction Agreements and the Collaboration Agreement, the issuance and sale of the Shares and the consummation of the transactions contemplated by the Transaction Agreements and the Collaboration Agreement will not (i) conflict with or result in a breach or violation of any of the terms or provisions of, or constitute a default under, or result in the creation or imposition of any lien, charge or encumbrance upon any property or assets of the Company pursuant to, any indenture, mortgage, deed of trust, loan agreement or other agreement or instrument to which the Company is a party, by which the Company is bound or to which any of the property or assets of the Company is subject, (ii) result in any violation of the provisions of the Charter or By-laws or similar organizational documents of the Company or (iii) result in the violation of any Law or any judgment, order, rule or regulation of any Governmental Authority having jurisdiction over the Company or any of its Subsidiaries, except, in the case of clauses (i) and (iii) above, for any such conflict, breach, violation or default that would not, individually or in the aggregate, have a Material Adverse Effect.

4.7 No Governmental Authority or Third-Party Consents. No consent, approval, authorization, order, license, registration or qualification of or with any court or arbitrator or governmental or regulatory authority is required for the execution, delivery and performance by the Company of each of the Transaction Agreements or the Collaboration Agreement or the issuance and sale of the Shares, except (i) such filings as may be required to be made with the SEC and with any state blue sky or securities regulatory authority, which filings shall be made in a timely manner in accordance with all applicable Laws and (ii) with respect to the Shares, the filing with the Nasdaq Stock Market of, and the absence of unresolved issues with

respect to, an LAS and a Nasdaq Shares Outstanding Change Form, in each case to the extent required.

4.8 Valid Issuance of Shares. When issued, sold and delivered at the Closing in accordance with the terms hereof for the Aggregate Purchase Price, the Shares shall be duly authorized, validly issued, fully paid and nonassessable and free from any liens, encumbrances or restrictions on transfer, including pre-emptive rights, rights of first refusal or other similar rights, other than restrictions on transfer under the Transaction Agreements, as a result of any action by the Investor or under federal or state securities Laws.

4.9 Litigation. There are no legal, governmental or regulatory investigations, actions, suits or proceedings pending to which the Company is a party or to which any property of the Company is subject, or any legal actions, suits or proceedings which the Company intends to initiate, that, individually or in the aggregate, would reasonably be expected to have a Material Adverse Effect; and no such investigations, actions, suits or proceedings are, to the Knowledge of the Company, threatened or contemplated by any governmental or regulatory authority or others.

4.10 Licenses and Other Rights; Compliance with Laws The Company and its Subsidiaries possess or are in the process of obtaining all licenses, certificates, permits and other authorizations issued by, and have made all declarations and filings with, the appropriate federal, state, local or foreign governmental or regulatory authorities that are necessary for the ownership or lease of their respective properties or the conduct of their respective businesses as described in the Company SEC Documents (excluding any Forward-Looking Disclosures), except where the failure to possess or make the same would not, individually or in the aggregate, have a Material Adverse Effect; and except as described in the Company SEC Documents (excluding any Forward-Looking Disclosures), neither the Company nor any of its Subsidiaries has received notice of any revocation or modification of any such license, certificate, permit or authorization or has any reason to believe that any such license, certificate, permit or authorization will not be renewed, except where the failure to possess or make the same would not, individually or in the aggregate, have a Material Adverse Effect. The Company and its Subsidiaries are, and at all times since January 1, 2023, have been, in compliance with all statutes, rules and regulations applicable to the ownership, packaging, processing, use, distribution, import, or export of any product manufactured or distributed by the Company or its Subsidiaries, except where such noncompliance would not, individually or in the aggregate, reasonably be expected to have a Material Adverse Effect.

4.11 Company SEC Documents; Financial Statements; Nasdaq Stock Market

(a) Since January 1, 2023, the Company has timely filed all required reports, schedules, forms, statements and other documents (including exhibits and all other information incorporated therein) required to be filed by it under the Securities Act and the Exchange Act, and any required amendments to any of the foregoing, with the SEC (the "**Company SEC Documents**"). As of its respective filing date, each of the Company SEC Documents complied in all material respects with the requirements of the Securities Act, the Exchange Act, and the rules and regulations of the SEC promulgated thereunder applicable to such Company SEC Documents, and no Company SEC Documents when filed, declared effective or mailed, as applicable, contained any untrue statement of a material fact or omitted to state a

material fact required to be stated therein or necessary in order to make the statements therein, in light of the circumstances under which they were made, not misleading.

(b) As of the Signing Date, there are no outstanding or unresolved comments in comment letters received from the SEC or its staff.

(c) The financial statements of the Company included in its Annual Report on Form 10-K for the fiscal year ended December 31, 2022 and in its quarterly reports on Form 10-Q for the quarterly periods ended March 31, 2023; June 30, 2023; and September 30, 2023 fairly present the financial position of the Company and its consolidated Subsidiaries as of the dates indicated and the results of their operations and the changes in their cash flows for the periods specified; such financial statements have been prepared in conformity with GAAP applied on a consistent basis throughout the periods covered thereby, except as otherwise disclosed therein and, in the case of unaudited, interim financial statements, subject to normal year-end audit adjustments and the exclusion of certain footnotes, and any supporting schedules included in the Company SEC Documents present fairly the information required to be stated therein.

(d) The Common Stock is listed on the Nasdaq Stock Market, and the Company has taken no action designed to, or which is likely to have the effect of, terminating the registration of the Common Stock under the Exchange Act or delisting the Common Stock from the Nasdaq Stock Market. The Company has not received any notification that, and has no Knowledge that, the SEC or the Nasdaq Stock Market is contemplating terminating such listing or registration.

(e) The Company and its Subsidiaries have established and maintain systems of "internal control over financial reporting" (as defined in Rule 13a-15(f) of the Exchange Act) that have been designed by, or under the supervision of, their respective principal executive and principal financial officers, or persons performing similar functions, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with GAAP, including, but not limited to, internal accounting controls sufficient to provide reasonable assurance that (i) transactions are executed in accordance with management's general or specific authorizations; (ii) transactions are recorded as necessary to permit preparation of financial statements in conformity with GAAP and to maintain asset accountability; (iii) access to assets is permitted only in accordance with management's general or specific authorization; (iv) the recorded accountability for assets is compared with the existing assets at reasonable intervals and appropriate action is taken with respect to any differences and (v) interactive data in eXtensible Business Reporting Language included in the Company SEC Documents fairly presents the information called for in all material respects and is prepared in accordance with the SEC's rules and guidelines applicable thereto. Since January 1, 2023, to the Knowledge of the Company, there have been (1) no material weaknesses in the Company's internal control over financial reporting (whether or not remediated), (2) no fraud, whether or not material, that involves management or other employees who have a role in the internal control over financial reporting of the Company or its Subsidiaries, and (3) no change in the Company's internal control over financial reporting that has materially affected, or is reasonably likely to materially affect, the Company's internal control over financial reporting. The Company's auditors and the Audit Committee of the Board have been advised of: (i) all significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which have

adversely affected or are reasonably likely to adversely affect the Company's ability to record, process, summarize and report financial information; and (ii) any fraud, whether or not material, that involves management or other employees who have a significant role in the Company's internal control over financial reporting.

(f) The Company maintains disclosure controls and procedures (as defined in Rule 13a-15(e) of the Exchange Act) that comply with the requirements of the Exchange Act; such disclosure controls and procedures have been designed to ensure that information required to be disclosed by the Company in reports that it files or submits under the Exchange Act is recorded, processed, summarized and reported within the time periods specified in the SEC's rules and forms, including controls and procedures designed to ensure that such information is accumulated and communicated to the Company's management to allow timely decisions regarding disclosures. The Company has conducted evaluations of the effectiveness of its disclosure controls and procedures as required by Rule 13a-15 of the Exchange Act and, since January 1, 2023, such disclosure controls and procedures have been, based on such evaluations, effective at the reasonable assurance level to perform the functions for which they were established.

(g) There is and has been no material failure on the part of the Company or, to the Knowledge of the Company, any of the Company's directors or officers, in their capacities as such, to comply with any applicable provision of the Sarbanes-Oxley Act of 2002 and the rules and regulations promulgated in connection therewith, including Section 402 related to loans and Sections 302 and 906 related to certifications.

4.12 Absence of Certain Changes.

(a) Except as disclosed in the Company SEC Documents (excluding any Forward-Looking Disclosures), since September 30, 2023, (i) there has not been any (1) material change in the capital stock (other than (y) the issuance of shares of Common Stock upon exercise of stock options, the settlement of equity awards and the exercise of warrants described as outstanding in, and the grant of options and awards under existing equity incentive plans described in, the Company SEC Documents and (z) the issuance of shares of Common Stock, options and equity awards granted to new employees of the Company as inducement awards pursuant to Nasdaq Listing Rule 5635(c)(4)), (2) material change in short-term debt or long-term debt of the Company or any of its Subsidiaries, (3) dividend or distribution of any kind declared, set aside for payment, paid or made by the Company on any class of capital stock, (4) change or development, or the imposition of any material lien upon or adversely affecting any material property or other material assets of the Company or any of its Subsidiaries, that has had or would reasonably be expected to result in, individually or in the aggregate, a material adverse change in or affecting the business, properties, management, financial position, stockholders' equity, results of operations of the Company and its Subsidiaries, taken as a whole, (5) waiver of any material rights or claims of the Company and its Subsidiaries, taken as a whole, or (6) material loans or advances made by the Company or any of its Subsidiaries to any of its or their other Affiliates, other than in the ordinary course of business; (ii) neither the Company nor any of its Subsidiaries has entered into any transaction or agreement (whether or not in the ordinary course of business) that is material to the Company and its Subsidiaries taken as a whole or incurred any liability or obligation, direct or contingent, that is material to the Company and its Subsidiaries taken as a

whole; and (iii) neither the Company nor any of its Subsidiaries has sustained any loss or interference with its business that is material to the Company and its Subsidiaries taken as a whole and that is either from fire, explosion, flood or other calamity, whether or not covered by insurance, or from any labor disturbance or dispute or any action, order or decree of any court or arbitrator or governmental or regulatory authority.

4.13 Offering. Subject to the accuracy of the Investor's representations set forth in Sections 5.5, 5.6, 5.7, 5.9, 5.10 and 5.11 hereof, the offer, sale and issuance of the Shares to be issued in conformity with the terms of this Agreement constitute transactions which are exempt from the registration requirements of the Securities Act and from all applicable state registration or qualification requirements. Neither the Company nor any Person acting on its behalf will take any action that would cause the loss of such exemption.

4.14 No Integration. The Company has not, directly or through any agent, sold, offered for sale, solicited offers to buy or otherwise negotiated in respect of, any security (as defined in the Securities Act), that is or will be integrated with the sale of the Shares in a manner that would require registration of the Shares under the Securities Act or cause this offering of Shares to be aggregated with any prior offering of securities of the Company such that the shareholder approval provisions of the Nasdaq Stock Market would require the Company to obtain stockholder approval of the issuance of the Shares, nor will the Company take any action that would cause the offering or issuance of the Shares to be integrated or aggregated, as applicable, with future offerings such that the Shares would be required to be registered under the Securities Act or that the Company would be required to obtain stockholder approval of the issuance of the Shares pursuant to the shareholder approval provisions of the Nasdaq Stock Market.

4.15 Brokers' or Finders' Fees. Except with respect to the Company Financial Advisors, neither the Company nor any of its Subsidiaries is a party to any contract, agreement or understanding with any Person that would give rise to a valid claim against the Company or any of its Subsidiaries for a brokerage commission, finder's fee or like payment in connection with the transactions contemplated by the Transaction Agreements and the Collaboration Agreement. No Person will have, as a result of the transactions contemplated by the Transaction Agreements and the Collaboration Agreement, any valid right, interest or claim against or upon the Investor or any of its Affiliates for any brokerage commission, finder's fee or like payment pursuant to any agreement, arrangement or understanding entered into by or on behalf of the Company, including without limitation, under any agreement, arrangement or understanding the Company has with Company Financial Advisors.

4.16 Investment Company. The Company is not and, immediately after giving effect to the offering and sale of the Shares and the application of the proceeds thereof, will not be required to register as an "investment company" or an entity "controlled" by an "investment company" within the meaning of the Investment Company Act of 1940, as amended, and the rules and regulations of the SEC thereunder.

4.17 No General Solicitation. Neither the Company nor any Person acting on behalf of the Company has offered or sold any of the Shares by any form of general solicitation or general advertising. The Company has offered the Shares for sale only to the Investor.

4.18 Foreign Corrupt Practices. Neither the Company nor, to the Knowledge of the Company, any agent or other Person acting on behalf of the Company or any of its Subsidiaries, has: (i) directly or indirectly used any funds for unlawful contributions, gifts, entertainment or other unlawful expenses related to foreign or domestic political activity, (ii) made any unlawful payment to foreign or domestic government officials or employees or to any foreign or domestic political parties or campaigns from corporate funds, (iii) failed to disclose fully any contribution made by the Company (or made by any person acting on its behalf of which the Company is aware) which is in violation of Law or (iv) violated in any material respect any provision of the Foreign Corrupt Practices Act of 1977, as amended, or any applicable non-U.S. anti-bribery Law.

4.19 Regulation M Compliance. The Company has not taken, directly or indirectly, any action designed to or that would reasonably be expected to cause or result in stabilization or manipulation of the price of the Common Stock to facilitate the sale or resale of the Shares.

4.20 Office of Foreign Assets Control. Neither the Company nor any of its Subsidiaries nor, to the Company's Knowledge, any director, officer, agent, employee or Affiliate of the Company is currently subject to any U.S. sanctions administered by the Office of Foreign Assets Control of the U.S. Treasury Department ("**OFAC**"). The Company will not directly or, to the Company's Knowledge, indirectly use the proceeds from the sale of the Shares, or lend, contribute or otherwise make available such proceeds to any Subsidiary or any joint venture partner or other Person, for the purpose of financing the activities of or business with any Person, or in any country or territory, that currently is subject to any U.S. sanctions administered by OFAC or in any other manner that will result in a violation by the Company or any of its Subsidiaries of U.S. sanctions administered by OFAC.

4.21 Development Matters.

(a) All preclinical and clinical studies described in the Company SEC Documents and conducted by or on behalf of the Company to support approval for commercialization of the Company's products or product candidates have been conducted by the Company, or to the Company's Knowledge by Third Parties, in compliance with all applicable federal, state or foreign laws, rules, orders and regulations, except for such failure or failures to be in compliance which would not reasonably be expected to have, singularly or in the aggregate, a Material Adverse Effect.

(b) The studies, tests and preclinical or clinical trials conducted by or on behalf of the Company that are described in the Company SEC Documents (the "**Company Studies and Trials**") were and, if still pending, are being, conducted in all material respects in accordance with experimental protocols, procedures and controls pursuant to, where applicable, accepted professional scientific standards for products or product candidates comparable to those being developed by the Company; the descriptions of the results of the Company Studies and Trials contained in the Company SEC Documents are accurate in all material respects; the Company has no Knowledge of any other studies or trials not described in the Company SEC Documents, the results of which are materially inconsistent with or call in question the results described or referred to in the Company SEC Documents when viewed in the context in which such results are described and the stage of development of the applicable product or product candidate; and, except as

disclosed in the Company SEC Documents, the Company has not received any notices or correspondence from the United States Food and Drug Administration (the "**FDA**") or any foreign, state or local governmental authority exercising comparable authority requiring the termination, suspension or material modification of any Company Studies and Trials, other than ordinary course communications with respect to modifications in connection with the design and implementation of such trials, which such termination, suspension or material modification would reasonably be expected to have a Material Adverse Effect, and, to the Company's Knowledge, there are no reasonable grounds for the same. The Company has obtained (or caused to be obtained) informed consent by or on behalf of each human subject who participated in the Company Studies and Trials. To the Company's Knowledge, none of the Company Studies and Trials involved any investigator who has been disqualified as a clinical investigator or has been found by the FDA to have engaged in scientific misconduct. To the Company's Knowledge, the manufacturing facilities and operations of its suppliers are operated in compliance in all material respects with all applicable statutes, rules, regulations and policies of the FDA and comparable governmental authorities outside of the United States to which the Company is subject.

4.22 Intellectual Property. The Company owns, possesses, or can acquire on reasonable terms the right to use all (i) patents, patent applications, trademarks, trademark registrations, service marks, service mark registrations, Internet domain name registrations, copyrights, copyright registrations, licenses and trade secret rights (collectively, "**Intellectual Property Rights**") and (ii) inventions, software, works of authorships, trademarks, service marks, trade names, databases, formulae, know how, Internet domain names and other intellectual property (including trade secrets and other unpatented and/or unpatentable proprietary confidential information, systems, or procedures) (collectively, "**Intellectual Property Assets**") necessary to conduct its business as currently conducted, and as proposed to be conducted and described in the Company SEC Documents. The Company has not received any opinion from its legal counsel concluding that any activities of its business infringes, misappropriates, or otherwise violates, valid and enforceable Intellectual Property Rights of any other Person, and has not received written notice of any challenge, which is to its Knowledge still pending, by any other Person to the rights of the Company with respect to any Intellectual Property Rights or Intellectual Property Assets owned or used by the Company that, in each case, singularly or in the aggregate would reasonably be expected to have a Material Adverse Effect. To the Company's Knowledge, the Company's business as now conducted does not give rise to any infringement of, any misappropriation of, or other violation of, any valid and enforceable Intellectual Property Rights or Intellectual Property Assets of any other Person in any material respect. All licenses for the use of the Intellectual Property Rights or Intellectual Property Assets described in the Company SEC Documents or otherwise necessary to conduct the Company's business as currently conducted are valid, binding upon, and enforceable by or against the Company, and to the Company's Knowledge, by or against the parties thereto in accordance with their terms. The Company has complied in all material respects with, and is not in material breach of, nor has it received any asserted or threatened claim of breach of any intellectual property licenses for the use of the Intellectual Property Rights or Intellectual Property Assets, and the Company has no Knowledge of any breach or anticipated breach by any other Person of any such intellectual property licenses, in each case which claim remains outstanding and has not been dismissed, settled or otherwise resolved or which breach remains uncured. Except as disclosed in the Company SEC Documents (excluding any Forward-Looking Disclosures), no claim has been made or is pending against the Company alleging the infringement by the Company of any Intellectual Property Asset, Intellectual Property Right or

franchise right of any Person. To the Company's Knowledge, there is no infringement by any Person of any Intellectual Property Rights or Intellectual Property Assets owned or used by the Company, and no such claims have been made against any Person by the Company that singularly or in the aggregate would reasonably be expected to have a Material Adverse Effect. The Company has taken reasonable steps to protect, maintain and safeguard its Intellectual Property Rights and Intellectual Property Assets, including the execution of appropriate nondisclosure and confidentiality agreements. The consummation of the transactions contemplated by this Agreement will not result in the loss or impairment of or payment of any additional amounts with respect to, nor require the consent of any other Person in respect of, the Company's right to own, use, or hold for use any of the Intellectual Property Rights or Intellectual Property Assets as owned, used or held for use in the conduct of the business as currently conducted, in each case except as would not reasonably be expected to have a Material Adverse Effect. The Company has at all times complied in all material respects with all applicable Laws relating to privacy, data protection, and the collection and use of personal information collected, used, or held for use by the Company in the conduct of the Company's business. No claims have been asserted or, to the Company's Knowledge, threatened against the Company alleging a violation of any Person's privacy or personal information or data rights and the consummation of the transactions contemplated hereby will not breach or otherwise cause any violation of any applicable Law related to privacy, data protection, or the collection and use of personal information collected, used, or held for use by the Company in the conduct of the Company's business. The Company takes reasonable measures to ensure that such information is protected against unauthorized access, use, modification or other misuse. The Company has taken all necessary actions to secure and record its ownership of all works of authorship and inventions made by its employees, consultants and contractors with an obligation of assignment during the time they were employed by or under contract with the Company and which relate to the Company's business. All founders and key employees have signed confidentiality and invention assignment agreements with the Company.

4.23 Real and Personal Property. The Company has good and marketable title in fee simple (in the case of real property) to, or has valid and marketable rights to lease or otherwise use, all items of real or personal property which are material to the business of the Company taken as a whole, in each case free and clear of all liens, encumbrances, security interests, claims and defects that do not, singularly or in the aggregate, materially affect the value of such property and do not materially interfere with the use made and proposed to be made of such property by the Company; and all of the leases and subleases material to the business of the Company, and under which the Company holds properties described in the Company SEC Documents, are in full force and effect other than as described in the Company SEC Documents, and the Company has not received any notice of any material claim of any sort that has been asserted by anyone adverse to the rights of the Company under any of the leases or subleases mentioned above, or affecting or questioning the rights of the Company to the continued possession of the leased or subleased premises under any such lease or sublease.

4.24 Labor and Employment. There is (a) no unfair labor practice complaint pending against the Company, nor to the Company's Knowledge, threatened against it, before the National Labor Relations Board, any state or local labor relations board or any foreign labor relations board, and no significant grievance or significant arbitration proceeding arising out of or under any collective bargaining agreement is so pending against the Company, or, to the Company's Knowledge, threatened against it and (b) no labor disturbance by or dispute with,

employees of the Company exists or, to the Company's Knowledge, is contemplated or threatened, and the Company is not aware of any existing or imminent labor disturbance by the employees of any of its principal suppliers, manufacturers, customers or contractors, in the case of each clause (a) and (b) that would reasonably be expected, singularly or in the aggregate, to have a Material Adverse Effect. The Company is not aware that any key employee or significant group of employees of the Company plans to terminate employment with the Company.

4.25 ERISA Matters. Each employee benefit plan of the Company is in compliance in all material respects with applicable Law, including the Employee Retirement Income Security Act of 1974, as amended, including the regulations and published interpretations thereunder ("**ERISA**") and the Internal Revenue Code of 1986, as amended from time to time (the "**Code**"). The Company has not incurred and would not reasonably be expected to incur liability under Title IV of ERISA with respect to the funding of, termination of, or withdrawal from, any pension plan (as defined in ERISA). Each pension plan for which the Company would have any liability that is intended to be qualified under Section 401(a) of the Code is so qualified, and nothing has occurred, whether by action or by failure to act, which would, singularly or in the aggregate, reasonably be expected to cause the loss of such qualification.

4.26 Environmental Matters. The Company is in compliance in all material respects with all Laws relating to the use, treatment, storage and disposal of hazardous or toxic substances or waste and protection of health and safety or the environment which are applicable to its businesses (the "**Environmental Laws**"). There has been no storage, generation, transportation, handling, treatment, disposal, discharge, emission, or other release of any kind of toxic or other wastes or other hazardous substances by, due to, or caused by the Company (or, to the Company's Knowledge, any other entity for whose acts or omissions the Company is or may otherwise be liable) upon any of the property now or previously owned or leased by the Company, or upon any other property, in violation of any Law, decree or permit or which would, under any Law (including rule of common law), decree or permit, give rise to any liability; and there has been no disposal, discharge, emission or other release of any kind on to such property or into the environment surrounding such property of any toxic or other wastes or other hazardous substances that in each case would, singularly or in the aggregate, reasonably be expected to have a Material Adverse Effect.

4.27 Taxes. The Company (i) has timely filed all federal, state, local and foreign tax returns (or timely filed extensions with respect to such returns) required to be filed by it, and all such returns were true, complete and correct, (ii) has paid all federal, state, local and foreign taxes, assessments, governmental or other charges due and payable for which it is liable, including, without limitation, all sales and use taxes and all taxes which the Company is obligated to withhold from amounts owing to employees, creditors and Third Parties (except as currently being contested in good faith and for which reserves required by GAAP have been created in the financial statements), and (iii) does not have any tax deficiency or claims outstanding or assessed or, to its Knowledge, proposed against it, except those, in each of the cases described in clauses (i), (ii) and (iii) above, that would not, singularly or in the aggregate, reasonably be expected to have a Material Adverse Effect. The Company has not engaged in any transaction which is a corporate tax shelter or which could be reasonably characterized as such by the Internal Revenue Service or any other taxing authority. The accruals and reserves on the books and records of the Company in respect of tax liabilities for any taxable period not yet finally determined are adequate to meet any

assessments and related liabilities for any such period, and since January 1, 2023, the Company has not incurred any liability for taxes other than in the ordinary course.

4.28 Insurance. The Company carries or is covered by insurance in such amounts and covering such risks as is adequate for the conduct of its business and the value of its properties and as is customary for companies engaged in similar businesses, at a similar stage of development, in similar industries. The Company has no reason to believe that it will not be able to renew its existing insurance coverage as and when such coverage expires or to obtain similar coverage from similar insurers as may be necessary to continue its business at a cost that would not reasonably be expected to have a Material Adverse Effect. All policies of insurance owned by the Company are, to the Company's Knowledge, in full force and effect and the Company is in compliance in all material respects with the terms of such policies. The Company has not received written notice from any insurer, agent of such insurer or the broker of the Company that any material capital improvements or any other material expenditures (other than premium payments) are required or necessary to be made in order to continue such insurance. Except for customary deductibles, the Company does not insure risk of loss through any captive insurance, risk retention group, reciprocal group or by means of any fund or pool of assets specifically set aside for contingent liabilities other than as described in the Company SEC Documents.

4.29 Cybersecurity. Except as disclosed in the Company SEC Documents (excluding any Forward-Looking Disclosures), (a) to the Company's Knowledge, there has been no material security breach or other material compromise of or relating to any of the Company's information technology and computer systems, networks, hardware, software, data (including the data of their respective customers, employees, suppliers, vendors and any Third Party data maintained by or on behalf of them), equipment or technology (collectively, "**IT Systems and Data**"); (b) the Company has not been notified of, and has no Knowledge of any event or condition that would reasonably be expected to result in, any material security breach or other material compromise to its IT Systems and Data; (c) the Company is presently in compliance with all applicable Laws and all orders, rules and regulations of any Governmental Authority, internal policies and contractual obligations relating to the privacy and security of IT Systems and Data and to the protection of such IT Systems and Data from unauthorized use, access, misappropriation or modification, except as would not, in the case of this clause (c), individually or in the aggregate, have a Material Adverse Effect; and (d) the Company has implemented and maintains reasonable backup, disaster recovery and support arrangements for its IT Systems and Data consistent with industry standards and practice for companies of similar size.

4.30 Related Party Transactions. There are no business relationships or related-party transactions involving the Company, its Subsidiaries or any other Person required by the Securities Act to be described in the Company SEC Documents that have not been described as required.

4.31 CFIUS Representations. The Company does not engage in (a) the design, fabrication, development, testing, production or manufacture of one (1) or more "critical technologies" within the meaning of the Defense Production Act of 1950, as amended, including all implementing regulations thereof (the "**DPA**"); (b) the ownership, operation, maintenance, supply, manufacture, or servicing of "covered investment critical infrastructure" within the meaning of the DPA (where such activities are covered by column 2 of Appendix A to 31 C.F.R.

Part 800); or (c) the maintenance or collection, directly or indirectly, of "sensitive personal data" of U.S. citizens within the meaning of the DPA. The Company has no current intention of engaging in such activities in the future.

4.32 No Disqualification Events. No Disqualification Event is applicable to the Company or, to its Knowledge, any Company Covered Person, except for a Disqualification Event as to which Rule 506(d)(2) or (d)(3) promulgated under the Securities Act is applicable.

5. Representations and Warranties of the Investor. The Investor hereby represents and warrants to the Company that:

5.1 Organization; Good Standing. The Investor is a corporation duly organized, validly existing and in good standing under the Laws of Switzerland. The Investor has all requisite corporate power and corporate authority to enter into the Transaction Agreements, to purchase the Shares and to perform its obligations under and to carry out the other transactions contemplated by the Transaction Agreements.

5.2 Authorization.

(a) The Investor has full right, power and authority to execute and deliver the Transaction Agreements and the Collaboration Agreement and to perform its obligations hereunder and thereunder; and all action required to be taken for the due and proper authorization, execution and delivery by it of each of the Transaction Agreements and the Collaboration Agreement and the consummation by it of the transactions contemplated thereby has been duly and validly taken.

(b) The Transaction Agreements and the Collaboration Agreement have been duly executed and delivered by the Investor and, upon the due execution and delivery of the Transaction Agreements and the Collaboration Agreement by the Company, will constitute valid and legally binding obligations of the Investor, enforceable against the Investor in accordance with their respective terms, except with respect to the Enforceability Exceptions.

5.3 No Conflicts. The execution, delivery and performance of the Transaction Agreements and the Collaboration Agreement, the subscription for and purchase of the Shares and the consummation of the transactions contemplated by the Transaction Agreements and the Collaboration Agreement will not (i) conflict with or result in a breach or violation of any of the terms or provisions of, or constitute a default under, or result in the creation or imposition of any lien, charge or encumbrance upon any property or assets of the Investor pursuant to, any indenture, mortgage, deed of trust, loan agreement or other agreement or instrument to which the Investor is a party, by which the Investor is bound or to which any of the property or assets of the Investor is subject, (ii) result in any violation of the provisions of the charter or by-laws or similar organizational documents of the Investor or (iii) result in the violation of any Law or any judgment, order, rule or regulation of any Governmental Authority having jurisdiction over the Investor or any of its subsidiaries, except, in the case of clauses (i) and (iii) above, for any such conflict, breach, violation or default that would not, individually or in the aggregate, have a material adverse effect on the Investor's ability to perform its obligations or consummate the Transaction in accordance with the terms of this Agreement.

5.4 No Governmental Authority or Third-Party Consents. No consent, approval, authorization, order, license, registration or qualification of or with any court or arbitrator or governmental or regulatory authority is required for the execution, delivery and performance by the Investor of each of the Transaction Agreements or the Collaboration Agreement or with the subscription for and purchase of the Shares.

5.5 Purchase Entirely for Own Account. The Investor acknowledges that the Shares shall be acquired for investment for the Investor's own account, not as a nominee or agent, and not with a view to the resale or distribution of any part thereof, and the Investor has no present intention of selling, transferring or otherwise distributing the Shares. The Investor can bear the economic risk of an investment in the Shares indefinitely and a total loss with respect to such investment. The Investor does not have and will not have as of the Closing any contract, undertaking, agreement, arrangement or understanding with any Person to sell, transfer or otherwise distribute any of the Shares.

5.6 Disclosure of Information. The Investor has received or has had full access to all the information from the Company and its management that the Investor considers necessary or appropriate for deciding whether to purchase the Shares hereunder. The Investor further represents that it has had an opportunity to ask questions and receive answers from the Company regarding the Company, its financial condition, results of operations and prospects and the terms and conditions of the offering of the Shares sufficient to enable it to evaluate its investment.

5.7 Investment Experience and Accredited Investor Status. The Investor is an "accredited investor" (as defined in Regulation D under the Securities Act). The Investor has such knowledge and experience in financial or business matters that it is capable of evaluating the merits and risks of the investment in the Shares to be purchased hereunder.

5.8 Acquiring Person. As of the Signing Date, to the Investor's knowledge after reasonable inquiry, the Investor beneficially owns (as determined pursuant to Rule 13d-3 under the Exchange Act without regard for the number of days in which a Person has the right to acquire such beneficial ownership, and without regard to Investor's rights under this Agreement) no shares of the Common Stock, except for Common Stock that may be beneficially owned by pension or employee benefit plans or trusts of either the Investor or any of its Affiliates and Common Stock held by any diversified index, mutual funds or similar entities that either the Investor or any of its Affiliates own or have an interest in. As of the Signing Date, to the Investor's knowledge after reasonable inquiry, neither the Investor nor any of its Affiliates beneficially owns, and immediately prior to the Closing, neither the Investor nor any of its Affiliates will beneficially own (in each case, as determined pursuant to Rule 13d-3 under the Exchange Act without regard for the number of days in which a Person has the right to acquire such beneficial ownership, and without regard to Investor's rights under this Agreement), any securities of the Company, except for securities that may be beneficially owned by pension or employee benefit plans or trusts of either the Investor or any of its Affiliates and securities held by any diversified index, mutual funds or similar entities that either the Investor or any of its Affiliates own or have an interest in. All securities of the Company owned by the Investor or any of its Affiliates that are required to be reported in accordance with the reporting requirements of Section 16 of the Exchange Act and the rules and regulations promulgated thereunder have been duly reported in such filings.

5.9 No "Bad Actor" Disqualification. The Investor is not subject to the disqualification provisions of Rule 506(d)(1) of the Securities Act.

5.10 Restricted Securities. The Investor understands that the Shares, when issued, shall be "restricted securities" under the federal securities Laws inasmuch as they are being acquired from the Company in a transaction not involving a public offering and that under such Laws the Shares may be resold without registration under the Securities Act only if an exemption from such registration requirements is available. The Investor represents that it is familiar with Rule 144, as presently in effect. The Investor understands that the Shares are being offered and sold to it in reliance on specific exemptions from the registration requirements of United States federal and state securities Laws and the Company is relying in part upon the truth and accuracy of, and the Investor's compliance with, the representations, warranties, agreements, acknowledgements and understandings of the Investor set forth in this Agreement in order to determine the availability of such exemptions and the eligibility of the Investor to acquire the Shares.

5.11 Legends. The Investor understands that any certificates or ledger entries representing the Shares shall bear the following legends:

(a) "THESE SECURITIES HAVE NOT BEEN REGISTERED UNDER THE SECURITIES ACT OF 1933. THEY MAY NOT BE SOLD, OFFERED FOR SALE, PLEDGED OR HYPOTHECATED IN THE ABSENCE OF A REGISTRATION STATEMENT IN EFFECT WITH RESPECT TO THE SECURITIES UNDER THE SECURITIES ACT OR AN OPINION OF COUNSEL REASONABLY SATISFACTORY TO THE COMPANY THAT SUCH REGISTRATION IS NOT REQUIRED OR UNLESS SOLD PURSUANT TO RULE 144 OF THE SECURITIES ACT.";

(b) "THESE SECURITIES ARE SUBJECT TO AND SHALL BE TRANSFERABLE ONLY UPON THE TERMS AND CONDITIONS OF AN INVESTOR AGREEMENT DATED AS OF DECEMBER 28, 2023, BY AND BETWEEN VOYAGER THERAPEUTICS, INC. AND NOVARTIS PHARMA AG, A COPY OF WHICH IS ON FILE WITH THE SECRETARY OF VOYAGER THERAPEUTICS, INC."; and

(c) any legend required by applicable state securities Laws or the other Transaction Agreements.

5.12 Financial Assurances. As of the Signing Date, the Investor has, and as of the Closing Date, the Investor will have, access to cash in an amount sufficient to pay to the Company the Aggregate Purchase Price.

5.13 SEC Reports. The Investor acknowledges that the Company has made the Company SEC Documents available (by filing on the SEC's electronic data gathering and retrieval system (EDGAR)) to the Investor.

5.14 HSR Act. The Investor has determined, in good faith and in accordance with 16 CFR § 801.10(c)(3), that the fair market value of the U.S. assets to be acquired pursuant to the Transaction Agreements and the Collaboration Agreement are not greater than $111.4 million. This determination is made solely for the purpose of determining the applicability of the

HSR Act to the transactions contemplated under the Transaction Agreements and Collaboration Agreement.

6. Investor's Conditions to Closing. The Investor's obligation to purchase the Shares at the Closing is subject to the fulfillment as of the Closing of the following conditions (unless waived in writing by the Investor):

6.1 Representations and Warranties. The representations and warranties made by the Company in Section 4 hereof shall be true and correct as of the Signing Date and as of the Closing Date as though made on and as of such Closing Date, except to the extent such representations and warranties are specifically made as of a particular date, in which case such representations and warranties shall be true and correct as of such date; provided, however, that for purposes of this Section 6.1, all such representations and warranties of the Company (other than Sections 4.1, 4.2, 4.3, 4.4, 4.5, 4.6, 4.7, 4.8, 4.11 and 4.15 hereof) shall be deemed to be true and correct for purposes of this Section 6.1 unless the failure or failures of such representations and warranties to be so true and correct, without regard to any "material," "materiality" or "Material Adverse Effect" qualifiers set forth therein, constitute a Material Adverse Effect.

6.2 Representations and Warranties in the Collaboration Agreement. The representations and warranties made by the Company in Section 10.2 of the Collaboration Agreement shall be true and correct as of the Closing Date as though made on and as of such Closing Date, except to the extent such representations and warranties are specifically made as of a particular date, in which case such representations and warranties shall be true and correct as of such date; provided, however, that for purposes of this Section 6.2, all such representations and warranties of the Company shall be deemed to be true and correct for purposes of this Section 6.2 unless the failure or failures of such representations and warranties to be so true and correct, without regard to any "material" or "materiality" qualifiers set forth therein, individually or in the aggregate, has had or would reasonably be expected to have a Material Adverse Effect.

6.3 Covenants. All covenants and agreements contained in this Agreement to be performed or complied with by the Company on or prior to the Closing Date shall have been performed or complied with in all material respects.

6.4 Investor Agreement. The Investor Agreement shall not have been terminated in accordance with its terms and shall be in full force and effect.

6.5 Collaboration Agreement. The Collaboration Agreement shall not have been terminated in accordance with its terms and shall be in full force and effect.

6.6 No Material Adverse Effect. From and after the Signing Date until the Closing Date, there shall not have occurred any change, event or occurrence that would constitute a Material Adverse Effect.

6.7 Listing. The Shares shall be eligible and approved for listing on the Nasdaq Stock Market.

6.8 Closing Deliverables. All Closing deliverables as required to be delivered by the Company (or its transfer agent) to the Investor under Section 3.2(a) hereof shall have been so delivered.

7. Company's Conditions to Closing. The Company's obligation to issue and sell the Shares at the Closing is subject to the fulfillment as of the Closing of the following conditions (unless waived in writing by the Company):

7.1 Representations and Warranties. The representations and warranties made by the Investor in Section 5 hereof shall be true and correct as of the Signing Date and as of the Closing Date as though made on and as of such Closing Date, except to the extent such representations and warranties are specifically made as of a particular date, in which case such representations and warranties shall be true and correct as of such date, except in each case (other than Sections 5.1 through 5.7 and Sections 5.9 through 5.11 hereof) where the failure of such representations and warranties to be so true and correct (without regard to any "material," "materiality" or "material adverse effect" qualifiers set forth therein) would not reasonably be expected to have a material adverse effect on the Investor's ability to perform its obligations hereunder or consummate the transactions contemplated hereby.

7.2 Covenants. All covenants and agreements contained in this Agreement to be performed or complied with by the Investor on or prior to the Closing Date shall have been performed or complied with in all material respects.

7.3 Investor Agreement. The Investor Agreement shall not have been terminated in accordance with its terms and shall be in full force and effect.

7.4 Collaboration Agreement. The Collaboration Agreement shall not have been terminated in accordance with its terms and shall be in full force and effect.

8. Mutual Conditions to Closing. The obligations of the Investor and the Company to consummate the Closing are subject to the fulfillment as of the Closing Date of the following conditions:

8.1 Absence of Litigation. There shall be no action, suit, proceeding or investigation by a Governmental Authority pending or currently threatened in writing against the Company or the Investor (i) that questions (A) the validity of any Transaction Agreement or the Collaboration Agreement or (B) the right of the Company or the Investor to enter into any Transaction Agreement or the Collaboration Agreement or to consummate the transactions contemplated hereby or thereby or (ii) which, if determined adversely, would impose substantial monetary damages on the Company or the Investor as a result of the consummation of the transactions contemplated by any Transaction Agreement.

8.2 No Prohibition. No provision of any applicable Law and no judgment, injunction (preliminary or permanent), order or decree that prohibits, makes illegal or enjoins the consummation of the Transaction or the transactions contemplated under the Transaction Agreements or the Collaboration Agreement shall be in effect.

9. Termination.

9.1 Ability to Terminate. This Agreement may be terminated at any time prior to the Closing by:

(a) mutual written consent of the Company and the Investor;

(b) either the Company or the Investor, upon written notice to the other, if any of the mutual conditions to the Closing set forth in Section 8 hereof shall have become incapable of fulfillment by the Termination Date and shall not have been waived in writing by the each party within ten (10) Business Days after receiving receipt of written notice of an intention to terminate pursuant to this clause (b); provided, however, that the right to terminate this Agreement under this Section 9.1(b) shall not be available to any party whose failure to fulfill any obligation under this Agreement has been the cause of, or resulted in, the failure to consummate the transactions contemplated hereby prior to the Termination Date;

(c) the Company, upon written notice to the Investor, so long as the Company is not then in breach of its representations, warranties, covenants or agreements under this Agreement such that any of the conditions set forth in Section 6.1, 6.2, 6.3, 6.4 or 6.5 hereof, as applicable, could not be satisfied by the Termination Date, (i) upon a material breach of any covenant or agreement on the part of the Investor set forth in this Agreement or (ii) if any representation or warranty of the Investor shall have been or become untrue, in each case such that any of the conditions set forth in Section 7.1, 7.2, 7.3 or 7.4 hereof, as applicable, could not be satisfied by the Termination Date;

(d) the Investor, upon written notice to the Company, so long as the Investor is not then in breach of its representations, warranties, covenants or agreements under this Agreement such that any of the conditions set forth in Section 7.1, 7.2, 7.3, or 7.4 hereof, as applicable, could not be satisfied by the Termination Date, (i) upon a material breach of any covenant or agreement on the part of the Company set forth in this Agreement, (ii) if any representation or warranty of the Company shall have been or become untrue, in each case such that any of the conditions set forth in Section 6.1, 6.2, 6.3, 6.4 or 6.5 hereof, as applicable, could not be satisfied by the Termination Date, or (iii) if there has been any change, event or occurrence since the Signing Date that has constituted a Material Adverse Effect.

9.2 Effect of Termination. In the event of the termination of this Agreement pursuant to Section 9.1 hereof, (i) this Agreement (except for this Section 9.2 and Section 11 hereof (other than Section 11.12), and any definitions set forth in this Agreement and used in such sections) shall forthwith become void and have no effect, without any liability on the part of any party hereto or its Affiliates, and (ii) all filings, applications and other submissions made pursuant to this Agreement, to the extent practicable, shall be withdrawn from the agency or other Person to which they were made or appropriately amended to reflect the termination of the transactions contemplated hereby; provided, however, that nothing contained in this Section 9.2 shall relieve any party from liability for fraud or any intentional or willful breach of this Agreement.

10. Additional Covenants and Agreements.

10.1 Market Listing. From the Signing Date through the Closing Date, the Company shall use all commercially reasonable efforts to (i) maintain the listing and trading of

the Common Stock on the Nasdaq Stock Market and (ii) effect the listing of the Shares on the Nasdaq Stock Market, including submitting an LAS to the Nasdaq Stock Market, if and as required in connection herewith, no later than fifteen (15) calendar days prior to the Closing Date.

10.2 Assistance and Cooperation. Prior to the Closing, upon the terms and subject to the conditions set forth in this Agreement, each of the parties agrees to use all reasonable efforts to take, or cause to be taken, all actions and to do, or cause to be done, and to assist and cooperate with the other party in doing, all things necessary, proper or advisable to consummate and make effective, in the most expeditious manner practicable, the transactions contemplated by this Agreement, including using all reasonable efforts to accomplish the following: (i) taking all reasonable acts necessary to cause the conditions precedent set forth in Sections 6, 7 and 8 hereof to be satisfied (including, in the case of the Company, promptly notifying the Investor of any notice from the Nasdaq Stock Market with respect to any LAS filed in connection herewith); (ii) taking all reasonable actions necessary to obtain all necessary actions or non-actions, waivers, consents, approvals, orders and authorizations from Governmental Authorities and the making of all necessary registrations, declarations and filings (including registrations, declarations and filings with Governmental Authorities, if any); (iii) taking all reasonable actions necessary to obtain all necessary consents, approvals or waivers from Third Parties; and (iv) defending any suits, claims, actions, investigations or proceedings, whether judicial or administrative, challenging this Agreement or the consummation of the transactions contemplated hereby, including seeking to have any stay or temporary restraining order entered by any court or other Governmental Authority vacated or reversed.

10.3 Legend Removal.

(a) Certificates or ledger entries evidencing the Shares shall not contain the legend set forth in Section 5.11(a) hereof: (i) following a sale of such Shares pursuant to a registration statement covering the resale of such Shares, while such registration statement is effective under the Securities Act, (ii) following any sale of such Shares pursuant to Rule 144, (iii) if such Shares are eligible for sale under Rule 144, without the requirement for the Company to be in compliance with the current public information required under Rule 144 as to such Shares and without volume or manner-of-sale restrictions under Rule 144 or (iv) if such legend is not required under applicable requirements of the Securities Act (including judicial interpretations and pronouncements issued by the staff of the SEC).

(b) Certificates or ledger entries evidencing the Shares shall not contain the legend set forth in Section 5.11(b) hereof following: (i) a sale of such Shares pursuant to a registration statement covering the resale of such Shares, while such registration statement is effective under the Securities Act, (ii) any sale of such Shares pursuant to Rule 144 or (iii) the expiration of the Standstill Term (as defined in the Investor Agreement) and the Lock-Up Term (as defined in the Investor Agreement); provided that any transfer described in clause (i) or (ii) above shall have been in compliance with all applicable provisions of the Investor Agreement. The Company and the Investor further agree that, if the Shares are eligible for sale under Rule 144 prior to the expiration of the Lock-Up Term and the Standstill Term, without the requirement for the Company to be in compliance with the current public information required under Rule 144 as to such Shares and without volume or manner-of-sale restrictions under Rule 144, the Investor shall be entitled, at its option, to request the Company to remove, and the Company agrees to

remove or cause to be removed, any legend set forth in Section 5.11(b) on the certificates or ledger entries evidencing the Shares in accordance with the procedures specified in Section 10.3(c) provided that such legend removal request is made in connection with a proposed transfer of the Shares to a brokerage or investment account in the name or for the benefit of the Investor and is accompanied by the delivery to the Company by the Investor of representation letters and any other documentation that may be reasonably requested by the Company and/or its counsel, in a form reasonably satisfactory to the Company, acknowledging any contractual restrictions on the transfer of the Shares then in effect and providing customary representations as to, among other things, the sufficiency of the Investor's internal policies and procedures in observance thereof.

(c) The Company agrees that at such time as any legend set forth in Section 5.11 hereof is no longer required under this Section 10.3, the Company will, no later than three (3) Business Days following the delivery by the Investor to the Company of notice of either (i) the delivery by the Investor to the Transfer Agent of a certificate representing Shares issued with such legend or (ii) in the event such Shares are uncertificated, notice of the Investor's desire to remove such legend(s) that are no longer required, deliver or cause to be delivered to the Investor (together with any legal opinion required by the Transfer Agent) a certificate representing such Shares that is free from such legend, or, in the event that such Shares are uncertificated, remove or cause to be removed any such legend in the Company's stock records. The Company may not make any notation on its records or give instructions to the Transfer Agent that enlarge the restrictions on transfer set forth in Section 5.11 hereof.

10.4 Conduct of Business. During the period from the Signing Date until the Closing, except as consented to in writing by the Investor, the Company shall not (i) declare, set aside or pay any dividend or make any other distribution or payment (whether in cash, stock or property or any combination thereof) in respect of its capital stock, or establish a record date for any of the foregoing, or (ii) make any other actual, constructive or deemed distribution in respect of any shares of its capital stock or otherwise make any payments to stockholders in their capacity as such, except pursuant to repurchases of equity pursuant to the terms of its equity compensation plans.

10.5 Right to Conduct Activities. The Company hereby agrees and acknowledges that the Investor and its Affiliates have numerous business lines (the "**Existing Business**") and an active investment and acquisition program. The Company hereby agrees that none of the Investor or any of its Affiliates (together, the "**Investor Group**") shall be liable to the Company or any of its Affiliates for any claim arising out of, or based upon, (a) the investment by the Investor Group in any entity competitive with the Company, (b) actions taken by any partner, officer or other representative of the Investor Group to assist any such competitive company, whether or not such action was taken as a member of the board of directors of such competitive company or otherwise, and whether or not such action has a detrimental effect on the Company, or (c) with respect to the Investor Group, the Investor Group engaging in the Existing Business; provided, however, that the foregoing shall not limit any of the Investor's or any of its Affiliates' obligations under the Transactions Agreement or the Collaboration Agreement or otherwise relieve the Investor or any Affiliate of the Investor from liability associated with the breach by the Investor of any representation, warranty, covenant, agreement or obligation set forth in the Transaction Agreements or the Collaboration Agreement.

11. Miscellaneous.

11.1 Governing Law; Submission to Jurisdiction. This Agreement shall be governed by and construed in accordance with the Laws of the State of Delaware, without regard to the conflict of laws principles thereof that would require the application of the Law of any other jurisdiction. Any action brought, arising out of, or relating to this Agreement shall be brought in the Court of Chancery of the State of Delaware. Each party hereby irrevocably submits to the exclusive jurisdiction of said Court in respect of any claim relating to the validity, interpretation and enforcement of this Agreement, and hereby waives, and agrees not to assert, as a defense in any action, suit or proceeding in which any such claim is made that it is not subject thereto or that such action, suit or proceeding may not be brought or is not maintainable in such courts, or that the venue thereof may not be appropriate or that this agreement may not be enforced in or by such courts. The parties hereby consent to and grant the Court of Chancery of the State of Delaware jurisdiction over such parties and over the subject matter of any such claim and agree that mailing of process or other papers in connection with any such action, suit or proceeding in the manner provided in Section 11.3 hereof or in such other manner as may be permitted by Law, shall be valid and sufficient thereof.

11.2 Waiver. Neither party may waive or release any of its rights or interests in this Agreement except in writing. The failure of either party to assert a right hereunder or to insist upon compliance with any term of this Agreement shall not constitute a waiver of that right or excuse a similar subsequent failure to perform any such term or condition. No waiver by either party of any condition or term in any one or more instances shall be construed as a continuing waiver of such condition or term or of another condition or term except to the extent set forth in writing.

11.3 Notices. All notices, instructions and other communications hereunder or in connection herewith shall be in writing, shall be sent to the address of the relevant party set forth on Exhibit B attached hereto and shall be (i) delivered personally; (ii) sent by certified mail (return receipt requested), postage prepaid; or (iii) sent via a reputable nationwide overnight express courier service (signature required). Any such notice, instruction or communication shall be deemed to have been delivered (A) upon receipt if delivered by hand; (B) three (3) Business Days after it is sent by certified mail, return receipt requested, postage prepaid; or (C) one (1) Business Day after it is sent via a reputable nationwide overnight courier service. Either party may change its address by giving notice to the other party in the manner provided above; provided that notices of a change of address shall be effective only upon receipt thereof.

11.4 Entire Agreement. This Agreement, the Investor Agreement and the Collaboration Agreement, in each case together with the schedules and exhibits thereto, set forth all the covenants, promises, agreements, warranties, representations, conditions and understandings between the parties and supersede and terminate all prior agreements and understanding between the parties. There are no covenants, promises, agreements, warranties, representations, conditions or understandings, either oral or written, between the parties other than as set forth herein and therein. No subsequent alteration, amendment, change or addition to this Agreement shall be binding upon the parties unless reduced to writing and signed by the respective authorized officers of the parties.

11.5 Headings; Nouns and Pronouns; Section References. Headings and any table of contents used in this Agreement are for convenience only and shall not in any way affect the construction of or be taken into consideration in interpreting this Agreement. Whenever the context may require, any pronouns used herein shall include the corresponding masculine, feminine or neuter forms, and the singular form of names and pronouns shall include the plural and vice-versa. References in this Agreement to a section or subsection shall be deemed to refer to a section or subsection of this Agreement unless otherwise expressly stated.

11.6 Severability. If, under applicable Laws, any provision hereof is invalid or unenforceable, or otherwise directly or indirectly affects the validity of any other material provision(s) of this Agreement in any jurisdiction ("**Modified Clause**"), then, it is mutually agreed that this Agreement shall endure and that the Modified Clause shall be enforced in such jurisdiction to the maximum extent permitted under applicable Laws in such jurisdiction; provided that the parties shall consult and use all reasonable efforts to agree upon, and hereby consent to, any valid and enforceable modification of this Agreement as may be necessary to avoid any unjust enrichment of either party and to match the intent of this Agreement as closely as possible, including the economic benefits and rights contemplated herein.

11.7 Assignment. Except for an assignment of this Agreement or any rights hereunder by the Investor to an Affiliate, neither this Agreement nor any of the rights or obligations hereunder may be assigned by either the Investor or the Company without (i) the prior written consent of Company in the case of any assignment by the Investor or (ii) the prior written consent of the Investor in the case of an assignment by the Company.

11.8 Parties in Interest. All of the terms and provisions of this Agreement shall be binding upon, and shall inure to the benefit of and be enforceable by the parties hereto and their respective successors, heirs, administrators and permitted assigns.

11.9 Counterparts. This Agreement may be signed in counterparts, each and every one of which shall be deemed an original, notwithstanding variations in format or file designation which may result from the electronic transmission, storage and printing of copies from separate computers or printers. Facsimile signatures and signatures transmitted via PDF shall be treated as original signatures.

11.10 Third-Party Beneficiaries. None of the provisions of this Agreement shall be for the benefit of or enforceable by any Third Party, including any creditor of any party hereto. No Third Party shall obtain any right under any provision of this Agreement or shall by reason of any such provision make any claim in respect of any debt, liability or obligation (or otherwise) against any party hereto.

11.11 No Strict Construction. This Agreement has been prepared jointly and will not be construed against either party.

11.12 Survival of Warranties. The representations and warranties of the Company and the Investor contained in this Agreement shall survive the Closing and the delivery of the Shares.

11.13 Remedies. The rights, powers and remedies of the parties under this Agreement are cumulative and not exclusive of any other right, power or remedy which such parties may have under any other agreement or Law. No single or partial assertion or exercise of any right, power or remedy of a party hereunder shall preclude any other or further assertion or exercise thereof.

11.14 Expenses. Each party shall pay its own fees and expenses in connection with the preparation, negotiation, execution and delivery of the Transaction Agreements.

11.15 No Publicity. The parties hereto agree that the provisions of Section 9.5 of the Collaboration Agreement shall be applicable to the parties to this Agreement with respect to any public disclosures regarding the proposed transactions contemplated by the Transaction Agreements and the Collaboration Agreement or regarding the parties hereto or their Affiliates (it being understood that the provisions of Section 9.5 of the Collaboration Agreement shall be read to apply to disclosures of information relating to this Agreement and the transactions contemplated hereby).

(Signature Page Follows)

IN WITNESS WHEREOF, the parties have executed and delivered this Agreement as of the date first above written.

NOVARTIS PHARMA AG

By: */s/ Ian James Hiscock*
Name: Ian James Hiscock
Title: Head Global IP Lit. Transactions

By: /s/ Marc Ceulemans
Name: Marc Ceulemans
Title: Head Capital Venture Fund Management

VOYAGER THERAPEUTICS, INC.

By: */s/ Alfred Sandrock, M.D., Ph.D.*
Name: Alfred Sandrock, M.D., Ph.D.
Title: Chief Executive Officer and President

(Signature Page to Stock Purchase Agreement)

SCHEDULE 1

LIST OF SUBSIDIARIES

1. Voyager Securities Corporation, a Massachusetts corporation

EXHIBIT B

NOTICES

If to the Investor:

Novartis Pharma AG
Lichtstrasse 35
CH-4056 Basel
Switzerland
Attention: Head of NIBR General Legal, Europe

with an e-mail copy (which shall not constitute notice) to:

nibr.generalcounsel@novartis.com

with copies (which shall not constitute notice) to:

Novartis Institutes for BioMedical Research, Inc.
250 Massachusetts Avenue
Cambridge, MA 02139 USA
Attn: General Counsel

Arnold & Porter Kaye Scholer LLP
Three Embarcadero Center, 10th Floor
San Francisco, CA 94111
Attention: Stephanie Coutu, Esq.
E-mail: stephanie.coutu@arnoldporter.com

If to the Company:

Voyager Therapeutics, Inc.
75 Hayden Avenue
Lexington, MA 02421
Attention: Chief Executive Officer

with copies (which shall not constitute notice) to:

Voyager Therapeutics, Inc.
75 Hayden Avenue
Lexington, MA 02421
Attention: Chief Legal Officer

Wilmer Cutler Pickering Hale and Dorr LLP
7 World Trade Center
250 Greenwich Street
New York, NY 10007
Attention: Brian A. Johnson, Esq.

Exhibit 10.45

EXECUTION VERSION

INVESTOR AGREEMENT

By and Between

NOVARTIS PHARMA AG

AND

VOYAGER THERAPEUTICS, INC.

Dated as of December 28, 2023

TABLE OF CONTENTS

1.	Definitions		1
2.	Restrictions on Beneficial Ownership		5
3.	Restrictions on Dispositions		6
	3.1	Lock-Up	6
	3.2	Certain Tender Offers	7
	3.3	Sale Limitations	7
	3.4	Offering Lock-Up	7
	3.5	Transactions for Personal Account; Change of Control	7
4.	Termination of Certain Rights and Obligations		8
	4.1	Termination of Standstill Period	8
	4.2	Termination of Lock-Up Term	8
	4.3	Termination of Agreement	8
	4.4	Effect of Termination	8
5.	Company Disclosure of Material Non-Public Information		9
6.	Compliance with Rule 144		9
7.	Miscellaneous		9
	7.1	Governing Law; Submission to Jurisdiction	9
	7.2	Waiver	9
	7.3	Notices	10
	7.4	Entire Agreement	10
	7.5	Headings; Nouns and Pronouns; Section References	10
	7.6	Severability	10
	7.7	Assignment	10
	7.8	Parties in Interest	11
	7.9	Counterparts	11
	7.10	Third-Party Beneficiaries	11
	7.11	No Strict Construction	11
	7.12	Remedies	11
	7.13	Specific Performance	11
	7.14	No Conflicting Agreements	11
	7.15	Use of Proceeds	12
	7.16	No Publicity	12

Exhibit A – Notices

INVESTOR AGREEMENT

THIS INVESTOR AGREEMENT (this "**Agreement**") is dated as of December 28, 2023 and effective as of the Closing (as defined below), by and between Novartis Pharma AG (the "**Investor**"), a corporation organized and existing under the laws of Switzerland, with its principal business office at Lichtstrasse 35, CH-4056 Basel, Switzerland, and Voyager Therapeutics, Inc. (the "**Company**"), a Delaware corporation, with its principal place of business at 75 Hayden Avenue, Lexington, MA 02421.

WHEREAS, the Stock Purchase Agreement, of even date herewith, by and between the Investor and the Company (the "**Purchase Agreement**"), provides for the issuance and sale by the Company to the Investor, and the purchase by the Investor, of 2,145,002 shares (the "**Purchased Shares**") of the Company's common stock, par value $0.001 per share (the "**Common Stock**");

WHEREAS, as a condition to consummating the transactions contemplated by the Purchase Agreement, the Investor and the Company have agreed upon certain rights and restrictions as set forth herein with respect to the Purchased Shares, and the Investor and the Company acknowledge that it is a condition to the closing under the Purchase Agreement (the "**Closing**") that this Agreement be in full force and effect; and

WHEREAS, simultaneously with the execution of the Purchase Agreement and this Agreement, the Company and the Investor entered into the Collaboration Agreement.

NOW, THEREFORE, in consideration of the premises and mutual agreements hereinafter set forth, and for other valuable consideration, the receipt and sufficiency of which are hereby acknowledged, the parties hereto agree as follows:

1. Definitions. As used in this Agreement, the following terms shall have the following meanings:

(a) "**Affiliate**" shall mean, with respect to any Person, another Person that, directly or indirectly through one or more intermediaries, controls, is controlled by or is under common control with such Person. A Person shall be deemed to control another Person if such Person possesses, directly or indirectly, the power to direct or cause the direction of the management and policies of such Person, whether through the ownership of voting securities, by contract or otherwise. Without limiting the generality of the foregoing, a Person shall be deemed to control another Person if such Person (ii) owns, directly or indirectly, beneficially or legally, more than fifty percent (50%) of the outstanding voting securities or capital stock of such other Person, or has other comparable ownership interest with respect to any Person other than a corporation; or (ii) has the power, whether pursuant to contract, ownership of securities or otherwise, to direct the management and policies of such other Person. For the purposes of this Agreement, in no event shall the Investor or any of its Affiliates be deemed Affiliates of the Company or any of its Affiliates, nor shall the Company or any of its Affiliates be deemed Affiliates of the Investor or any of its Affiliates.

(b) "**Agreement**" shall have the meaning set forth in the

Preamble to this Agreement, including all Exhibits attached hereto.

(c) "**Beneficial owner**," "**beneficially owns**," "**beneficial ownership**" and terms of similar import used in this Agreement shall, with respect to a Person, have the meaning set forth in Rule 13d-3 under the Exchange Act (i) assuming the full conversion into, and exercise and exchange for, shares of Common Stock of all Common Stock Equivalents beneficially owned by such Person and (ii) determined without regard for the number of days in which such Person has the right to acquire such beneficial ownership.

(d) "**Business Day**" shall mean a day on which banking institutions in Boston, Massachusetts, United States and Basel, Switzerland are open for business, excluding any Saturday or Sunday.

(e) "**Change of Control**" shall mean (i) the acquisition of beneficial ownership, directly or indirectly, by any Third Party of securities or other voting interests of the Company representing a majority of the combined voting power of the Company's then outstanding securities or other voting interests; (ii) any merger, consolidation or business combination involving the Company with a Third Party that results in the holders of beneficial ownership (other than by virtue of obtaining irrevocable proxies) of voting securities or other voting interests of the Company immediately prior to such merger, consolidation or other business combination ceasing to hold beneficial ownership of more than fifty percent (50%) of the combined voting power of the surviving entity immediately after such merger, consolidation or business combination; (iii) any sale, exclusive or sole license, lease, exchange, contribution or other transfer to a Third Party (in one transaction or a series of related transactions) of all or substantially all of the Company's assets; or (iv) individuals who, as of the date hereof, constitute the Board of Directors of the Company (the "**Incumbent Board**") cease for any reason to constitute at least a majority of the Board of Directors of the Company (provided, however, that any individual becoming a director subsequent to the date hereof whose election, or nomination for election by the Company's shareholders, was recommended or approved by a vote of at least a majority of the directors then comprising the Incumbent Board shall be considered as though such individual were a member of the Incumbent Board, but excluding, for this purpose, any such individual whose initial assumption of office occurs as a result of an actual or threatened election contest with respect to the election or removal of directors or other actual or threatened solicitation of proxies or consents by or on behalf of any person other than the Board of Directors of the Company).

(f) "**Closing**" shall have the meaning set forth in the Recitals to this Agreement.

(g) "**Closing Date**" shall have the meaning set forth in the Purchase Agreement.

(h) "**Collaboration Agreement**" shall mean the License and Collaboration Agreement, of even date herewith, between the Investor and the Company.

(i) "**Common Stock**" shall have the meaning set forth in the Recitals to this Agreement.

(j) "**Common Stock Equivalents**" shall mean any options, restricted stock units, warrants or other securities or rights convertible into or exercisable, exchangeable or settleable for, whether directly or following conversion into or exercise, exchange or settlement for other options, restricted stock units, warrants or other securities or rights, shares of Common Stock or any swap, hedge or similar agreement or arrangement that transfers in whole or in part, the economic risk of ownership of, or voting or other rights of, the Common Stock.

(k) "**Company**" shall have the meaning set forth in the Preamble to this Agreement.

(l) "**Competitor**" shall mean any operating company with a biopharmaceutical business involved in the development and/or commercialization of gene therapies, antibodies or other non-viral modalities, and/or treatments or platforms that involve genetic or neurologic medicines, or any other Person that directly or indirectly beneficially owns a majority of the voting securities of or voting interests in such a company, or any direct or indirect majority-owned subsidiary of such a company or of such a Person.

(m) "**Disposition**," "**Dispose of**" or "**Disposing**" shall mean any (i) pledge, sale, contract to sell, sale of any option or contract to purchase, purchase of any option or contract to sell, grant of any option, right or warrant for the sale of, or other disposition of or transfer of any shares of Common Stock, or any Common Stock Equivalents, including, without limitation, any "short sale" or similar arrangement, or (ii) swap or any other agreement or any transaction that transfers, in whole or in part, directly or indirectly, the economic consequence of ownership of shares of Common Stock, whether any such swap or transaction is to be settled by delivery of securities, in cash or otherwise.

(n) "**Exchange Act**" shall mean the Securities Exchange Act of 1934, as amended, and the rules and regulations promulgated thereunder.

(o) "**Governmental Authority**" shall mean any multinational, federal, national, state, provincial, local or other entity, office, commission, bureau, agency, political subdivision, instrumentality, branch, department, authority, board, court, arbitral or other tribunal exercising executive, judicial, legislative, police, regulatory, administrative or taxing authority or functions of any nature pertaining to government.

(p) "**Incumbent Board**" shall have the meaning set forth in the definition of "Change of Control."

(q) "**Investor**" shall have the meaning set forth in the Preamble to this Agreement.

(r) "**Law**" shall mean any law, statute, rule, regulation, order,

judgment or ordinance having the effect of law of any federal, national, multinational, state, provincial, county, city or other political subdivision.

(s) "**Lock-Up Agreement**" shall have the meaning set forth in Section 3.4 hereof.

(t) "**Lock-Up Term**" shall mean the period from and after the date of this Agreement until the occurrence of any event set forth in Section 4.2 hereof.

(u) "**Modified Clause**" shall have the meaning set forth in Section 7.6 hereof.

(v) "**Permitted Transferee**" shall mean (i) a controlled Affiliate of the Investor that is wholly owned, directly or indirectly, by the Investor, or (ii) a controlling Affiliate of the Investor that wholly owns, directly or indirectly, the Investor (or any controlled Affiliate of such controlling Affiliate), or the acquiring Person in the case of a Change of Control of the Investor (replacing references to "Company" with "Investor" in the definition of "Change of Control"); it being understood that for purposes of this definition "wholly owned" shall mean an Affiliate in which the Investor owns, or an Affiliate that owns, as applicable, directly or indirectly, at least ninety-nine percent (99%) of the outstanding capital stock of such Affiliate or the Investor, as applicable.

(w) "**Person**" shall mean any individual, partnership, joint venture, limited liability company, corporation, firm, trust, association, unincorporated organization, Governmental Authority or other entity, as well as any syndicate or group that would be deemed to be a Person under Section 13(d)(3) of the Exchange Act.

(x) "**Purchase Agreement**" shall have the meaning set forth in the Recitals to this Agreement and shall include all Exhibits attached thereto.

(y) "**Purchased Shares**" shall have the meaning set forth in the Recitals to this Agreement, and shall be adjusted for (i) any stock split, stock dividend, share exchange, merger, consolidation or similar recapitalization and (ii) any Common Stock issued as (or issuable upon the exercise of any warrant, right or other security that is issued as) a dividend or other distribution with respect to, or in exchange or in replacement of, the Purchased Shares.

(z) "**Rule 144**" shall mean Rule 144 promulgated under the Securities Act.

(aa) "**SEC**" shall mean the U.S. Securities and Exchange Commission.

(bb) "**Securities Act**" shall mean the Securities Act of 1933, as amended, and the rules and regulations promulgated thereunder.

(cc) "**Shares of Then-Outstanding Common Stock**" shall mean, at any time, the issued and outstanding shares of Common Stock at such time, as

well as all capital stock issued and outstanding as a result of any stock split, stock dividend, or reclassification of Common Stock distributable, on a pro rata basis, to all holders of Common Stock.

(dd) "**Standstill and Lock-Up Relaxation Date**" shall mean the second anniversary of the Closing Date.

(ee) "**Standstill Parties**" shall have the meaning set forth in Section 21 hereof.

(ff) "**Standstill Period**" shall mean the period from and after the date of this Agreement until the occurrence of any event set forth in Section 4.1 hereof.

(gg) "**Third Party**" shall mean any Person other than the Investor, the Company or any Affiliate of the Investor or the Company.

2. Restrictions on Beneficial Ownership.

2.1 For the duration of the Standstill Period, unless the Company or its Affiliates or representatives have specifically invited or approved the Investor to do so in writing, neither the Investor nor any of its Affiliates (collectively, the "**Standstill Parties**") will in any manner, directly or indirectly (or instruct, encourage or facilitate any representatives acting on behalf of the Investor to): (i) effect or seek, offer or propose (whether publicly or otherwise) to effect, or cause or knowingly participate in or in any way advise, assist or knowingly encourage any other Person to effect or seek, offer or propose (whether publicly or otherwise) to effect or participate in, (A) any acquisition of any equity securities (or beneficial ownership thereof) or a material portion of the assets of the Company, or any rights to acquire any such securities (including derivative securities representing the right to vote or economic benefit of any such securities) or such assets; (B) any tender or exchange offer, merger or other business combination involving the Company; (C) any recapitalization, restructuring, liquidation, dissolution or other extraordinary transaction with respect to the Company; or (D) any "solicitation" of "proxies" (as such terms are used in the proxy rules of the SEC) or consents to vote any voting securities of the Company; (ii) form, join or in any way participate in a "group" (as defined under the Exchange Act) with respect to any securities of the Company; (iii) otherwise act, alone or in concert with others, in a manner primarily intended to seek to control or influence (A) the members of management or the Board of Directors of the Company, in each case in such individuals' capacities as members of management and/or the Board of Directors of the Company, respectively, or (B) the policies of the Company; (iv) take any action that would reasonably be expected to require the Company to make a public announcement regarding any of the types of matters set forth in clause (i) above; (v) enter into any discussions or arrangements with any Third Party other than the Investor's or any of its Affiliates' respective advisors or representatives with respect to any of the foregoing; or (vi) publicly disclose any intention, plan or arrangement regarding any of the foregoing. Notwithstanding anything to the contrary contained in this Agreement, the Investor and its Affiliates shall not be precluded from owning or acquiring interests in any diversified index, mutual funds or similar entities that own capital stock of the Company, and nothing herein shall prohibit investments by pension or employee benefit plans or trusts of the Investor. None of (x) the direct or indirect acquisition by the Investor or any of its Affiliates of securities or assets of the

Company in connection with the Investor's or its Affiliate's acquisition of a Third Party that holds such securities or assets of the Company so long as such acquisition of such Third Party is not consummated for the purpose of circumventing this Section 2, (y) the acquisition of assets or securities of the Company or any of its Affiliates, as debtor, that are acquired in a transaction subject to the approval of the United States Bankruptcy Court pursuant to proceedings under the United States Bankruptcy Code, or (z) transfers or resales of any securities of the Company by the Investor to any other Person in compliance with the express terms and conditions of this Agreement, will be deemed to be a breach of the Investor's standstill obligations under this Section 2.

2.2 The Investor also agrees during the Standstill Period not to request the Company (or its directors, officers, employees or agents), directly or indirectly, to amend or waive any provision of this Section 2 (including this sentence), other than by means of a confidential communication to the Company's Chairman of the Board or Chief Executive Officer in a manner reasonably believed not to require the Company to make a public announcement of such amendment or waiver; provided, however, that the Investor not publicly disclose its interest or intention to request any such amendment or waiver.

2.3 Notwithstanding anything to the contrary contained in this Agreement, if, at any time (i) a Third Party enters into a definitive agreement with the Company contemplating the acquisition (by way of merger, tender offer or otherwise) of more than fifty percent (50%) of the then-outstanding Common Stock of the Company, of securities representing more than fifty percent (50%) of the voting power of all then-outstanding securities of the Company or all or substantially all of the consolidated assets of the Company or publicly announces its intention to do so, then the restrictions set forth in Section 2.1 shall terminate and cease to be of any further force or effect or (ii) a Third Party commences, or publicly announces an intention to commence, a tender or exchange offer that, if consummated, would make such Third Party the beneficial owner (within the meaning of Section 13(d)(1) of the Exchange Act) of at least 50% of the voting power of all then-outstanding securities of the Company, then until the expiration or termination of a tender or exchange offer that has been commenced or until the public announcement of a withdrawal or abandonment of an intention to commence a tender or exchange offer, the restrictions set forth in Section 2.1 shall be suspended and of no force or effect.

2.4 Notwithstanding anything to the contrary contained in this Agreement, the Investor shall not be precluded from making any confidential offers or proposals to the Board of Directors of the Company in a manner reasonably believed not to require the Company to make a public announcement of such offer or proposal; provided, however, that the Investor not publicly disclose its interest or intention to make, or the actual making of, any such offer or proposal.

3. Restrictions on Dispositions.

3.1 Lock-Up. During the Lock-Up Term, without the prior approval of the Company, the Investor shall not, and shall cause its Affiliates not to, Dispose of any of the Purchased Shares; provided, however, that the foregoing shall not prohibit the Investor from (i) transferring any of the Purchased Shares in accordance with the terms hereof or (ii) Disposing of any of the Purchased Shares to reduce the beneficial ownership of the Standstill Parties to nineteen and ninety-nine hundredths percent (19.99%) of the Shares of Then-Outstanding

Common Stock; and provided further that, notwithstanding anything in this Section 3.1, the Investor shall not be precluded from the Disposition of Purchased Shares through open market sales effected through one or more "brokers' transactions" (as such term is used in Rule 144) on or after the Standstill and Lock-Up Relaxation Date in an amount not to exceed one percent (1%) of the Shares of Then-Outstanding Common Stock in any three (3) month period.

3.2 Certain Tender Offers. Subject to the restrictions set forth in Section 3.3 hereof, this Section 3 shall not prohibit or restrict any Disposition of the Purchased Shares by the Standstill Parties into (i) a tender offer by a Third Party or (ii) an issuer tender offer by the Company.

3.3 Sale Limitations. Until the expiration or earlier valid termination of the Collaboration Agreement, subject to the restrictions set forth in Section 3.1 hereof, the Investor agrees that, except for any Disposition of the Purchased Shares by the Investor to a Permitted Transferee in accordance with the terms hereof or to the Company and Dispositions contemplated under Sections 3.2 and 3.5 hereof, it (i) shall not, and shall cause its Affiliates not to, Dispose of any of the Purchased Shares in a "block trade" private placement transaction, at any time to any Person that such Investor or Affiliate knows (after a reasonable inquiry) is a Competitor of the Company and (ii) shall, and shall cause its Affiliates to, instruct the broker(s) in any such "block trade" not to Dispose of the Purchased Shares to a Competitor (unless the identity of the Person purchasing the Purchased Shares is not known to the broker(s) or such Person Disposing of the Purchased Shares).

3.4 Offering Lock-Up. Until the expiration or earlier valid termination of the Collaboration Agreement, the Investor shall, if requested by the Company and an underwriter of Common Stock of the Company in connection with any public offering involving an underwriting of Common Stock of the Company (whether such public offering takes place before or after the expiration of the Lock-Up Term), agree not to Dispose of any Shares of Then-Outstanding Common Stock and/or Common Stock Equivalents for a specified period of time immediately following the launch of such offering, such period of time not to exceed ninety (90) days following the pricing of such offering (a "**Lock-Up Agreement**"), provided that all officers and directors of the Company are subject to substantially similar restrictions, and provided, further, that such Lock-Up Agreement shall not restrict the Investor's ability to Dispose of any Shares of Then-Outstanding Common Stock and/or Common Stock Equivalents in accordance with Section 3.2 or 3.5 hereof, as applicable, during the Lock-Up Term. Any Lock-Up Agreement shall be in writing in a form reasonably satisfactory to the Company and the underwriter(s) in such offering. The Company may impose stop transfer instructions with respect to the Shares of Then-Outstanding Common Stock and/or Common Stock Equivalents subject to the foregoing restrictions until the end of the Lock-Up Term. Any discretionary waiver or termination of the restrictions of any or all of such Lock-Up Agreements by the Company or the underwriters shall apply pro rata to the Investor based on the number of shares subject to such Lock-Up Agreements, excluding any waivers granted that fall within a customary de minimis exemption set forth in the associated Lock-Up Agreement.

3.5 Transactions for Personal Account; Change of Control. For the avoidance of doubt, nothing in this Section 3 (including, without limitation, under Section 3.3) will restrict any Disposition of any Shares of Then-Outstanding Common Stock and/or Common Stock

Equivalents (i) held by an executive officer or director of the Investor for his or her personal account, (ii) that may occur (or be deemed to occur) in connection with a Change of Control of the Investor (replacing references to "Company" with "Investor" in the definition of "Change of Control") or (iii) pursuant to any Change of Control of the Company, subject to the Investor's compliance with Section 2 hereof.

4. Termination of Certain Rights and Obligations.

4.1 Termination of Standstill Period. Section 2 hereof shall terminate and have no further force or effect upon the earliest to occur of:

(a) the expiration or earlier valid termination of the Collaboration Agreement;

(b) the date that is the third anniversary of the Closing Date;

(c) a liquidation or dissolution of the Company; and

(d) the date on which the Common Stock ceases to be registered pursuant to Section 12 of the Exchange Act.

4.2 Termination of Lock-Up Term. Section 3.1 hereof shall terminate and have no further force or effect upon the earliest to occur of:

(a) the expiration or earlier valid termination of the Collaboration Agreement;

(b) the date that is the third anniversary of the Closing Date;

(c) the beneficial ownership of the Standstill Parties falls below three percent (3%) of the Shares of Then-Outstanding Common Stock;

(d) a Change of Control of the Company;

(e) a liquidation or dissolution of the Company; and

(f) the date on which the Common Stock ceases to be registered pursuant to Section 12 of the Exchange Act.

4.3 Termination of Agreement. This Agreement shall terminate and have no further force or effect upon any termination of the Purchase Agreement prior to the Closing pursuant to Section 9.1 thereof.

4.4 Effect of Termination. No termination pursuant to any of Sections 4.1, 4.2, or 4.3 hereof shall relieve any of the parties (or the Permitted Transferee, if any) for liability for breach of or default under any of their respective obligations or restrictions under any terminated provision of this Agreement, which breach or default arose out of events or circumstances

occurring or existing prior to the date of such termination.

5. Company Disclosure of Material Non-Public Information. Without the prior written consent of the Investor, the Company shall not disclose to the Investor any information following the Closing the Company believes to constitute material non-public information regarding the Company except as may be advisable, desirable or required in connection with the Collaboration Agreement and the activities in connection therewith.

6. Compliance with Rule 144. With a view to making available to the Investor the benefits of Rule 144 and any other rule or regulation of the SEC that may at any time permit the Investor to sell the Purchased Shares to the public without registration, the Company agrees to use its commercially reasonable efforts to: (a) for a period of twelve (12) months following the Closing Date or such shorter period as the Investor or any of its Permitted Transferees hold any portion of the Purchased Shares, (i) make and keep current public information available, as those terms are understood and defined in Rule 144 and (ii) file with the Commission in a timely manner all reports required of the Company under the Exchange Act (or obtain extensions in respect thereof and file within the applicable grace period); and (b) furnish to the Investor or its Permitted Transferees forthwith upon request, as long as the Investor or such Permitted Transferee owns any Purchased Shares, (i) a written statement by the Company that it has complied with the reporting requirements of Rule 144 and the Exchange Act and (ii) such other information as may be reasonably requested to avail the Investor or any of its Permitted Transferees of any rule or regulation of the SEC that permits the selling of any such securities without registration under Rule 144.

7. Miscellaneous.

7.1 Governing Law; Submission to Jurisdiction. This Agreement shall be governed by and construed in accordance with the Laws of the State of Delaware, without regard to the conflict of laws principles thereof that would require the application of the Law of any other jurisdiction. Except as set forth in Section 7.13, any action brought, arising out of, or relating to this Agreement shall be brought in the Court of Chancery of the State of Delaware. Each party hereby irrevocably submits to the exclusive jurisdiction of said Court in respect of any claim relating to the validity, interpretation and enforcement of this Agreement, and hereby waives, and agrees not to assert, as a defense in any action, suit or proceeding in which any such claim is made that it is not subject thereto or that such action, suit or proceeding may not be brought or is not maintainable in such courts, or that the venue thereof may not be appropriate or that this agreement may not be enforced in or by such courts. The parties hereby consent to and grant the Court of Chancery of the State of Delaware jurisdiction over such parties and over the subject matter of any such claim and agree that mailing of process or other papers in connection with any such action, suit or proceeding in the manner provided in Section 7.3 hereof or in such other manner as may be permitted by Law, shall be valid and sufficient thereof.

7.2 Waiver. Neither party may waive or release any of its rights or interests in this Agreement except in writing. The failure of either party to assert a right hereunder or to insist upon compliance with any term of this Agreement shall not constitute a waiver of that right or excuse a similar subsequent failure to perform any such term or condition. No waiver by either party of any condition or term in any one or more instances shall be construed as a continuing waiver of such condition or term or of another condition or term except to the extent set forth in

writing.

7.3 Notices. All notices, instructions and other communications hereunder or in connection herewith shall be in writing, shall be sent to the address of the relevant party set forth on Exhibit A attached hereto and shall be (i) delivered personally; (ii) sent by certified mail (return receipt requested), postage prepaid; or (iii) sent via a reputable nationwide overnight express courier service (signature required). Any such notice, instruction or communication shall be deemed to have been delivered (A) upon receipt if delivered by hand; (B) three (3) Business Days after it is sent by certified mail, return receipt requested, postage prepaid; or (C) one (1) Business Day after it is sent via a reputable nationwide overnight courier service. Either party may change its address by giving notice to the other party in the manner provided above; provided that notices of a change of address shall be effective only upon receipt thereof.

7.4 Entire Agreement. This Agreement, the Purchase Agreement and the Collaboration Agreement, in each case together with the schedules and exhibits thereto, set forth all the covenants, promises, agreements, warranties, representations, conditions and understandings between the parties and supersede and terminate all prior agreements and understanding between the parties. There are no covenants, promises, agreements, warranties, representations, conditions or understandings, either oral or written, between the parties other than as set forth herein and therein. No subsequent alteration, amendment, change or addition to this Agreement shall be binding upon the parties unless reduced to writing and signed by the respective authorized officers of the parties.

7.5 Headings; Nouns and Pronouns; Section References. Headings and any table of contents used in this Agreement are for convenience only and shall not in any way affect the construction of or be taken into consideration in interpreting this Agreement. Whenever the context may require, any pronouns used herein shall include the corresponding masculine, feminine or neuter forms, and the singular form of names and pronouns shall include the plural and vice-versa. References in this Agreement to a section or subsection shall be deemed to refer to a section or subsection of this Agreement unless otherwise expressly stated.

7.6 Severability. If, under applicable Laws, any provision hereof is invalid or unenforceable, or otherwise directly or indirectly affects the validity of any other material provision(s) of this Agreement in any jurisdiction ("**Modified Clause**"), then, it is mutually agreed that this Agreement shall endure and that the Modified Clause shall be enforced in such jurisdiction to the maximum extent permitted under applicable Laws in such jurisdiction; provided that the parties shall consult and use all reasonable efforts to agree upon, and hereby consent to, any valid and enforceable modification of this Agreement as may be necessary to avoid any unjust enrichment of either party and to match the intent of this Agreement as closely as possible, including the economic benefits and rights contemplated herein.

7.7 Assignment. Except for an assignment of this Agreement or any rights hereunder by the Investor to a Permitted Transferee, neither this Agreement nor any of the rights or obligations hereunder may be assigned by either the Investor or the Company without (i) the prior written consent of the Company in the case of any assignment by the Investor; or (ii) the prior written consent of the Investor in the case of an assignment by the Company.

7.8 Parties in Interest. All of the terms and provisions of this Agreement shall be binding upon and shall inure to the benefit of and be enforceable by the parties hereto and their respective successors, heirs, administrators and permitted assigns.

7.9 Counterparts. This Agreement may be signed in counterparts, each and every one of which shall be deemed an original, notwithstanding variations in format or file designation which may result from the electronic transmission, storage and printing of copies from separate computers or printers. Facsimile signatures and signatures transmitted via PDF shall be treated as original signatures.

7.10 Third-Party Beneficiaries. None of the provisions of this Agreement shall be for the benefit of or enforceable by any Third Party, including any creditor of any party hereto. No Third Party shall obtain any right under any provision of this Agreement or shall by reason of any such provision make any claim in respect of any debt, liability or obligation (or otherwise) against any party hereto.

7.11 No Strict Construction. This Agreement has been prepared jointly and will not be construed against either party.

7.12 Remedies. The rights, powers and remedies of the parties under this Agreement are cumulative and not exclusive of any other right, power or remedy which such parties may have under any other agreement or Law. No single or partial assertion or exercise of any right, power or remedy of a party hereunder shall preclude any other or further assertion or exercise thereof.

7.13 Specific Performance. The Company and the Investor hereby acknowledge and agree that the rights of the parties hereunder are special, unique and of extraordinary character, and that if any party refuses or otherwise fails to act, or to cause its Affiliates to act, in accordance with the provisions of this Agreement, such refusal or failure would result in irreparable injury to the Company or the Investor, as the case may be, the exact amount of which would be difficult to ascertain or estimate and the remedies at law for which would not be reasonable or adequate compensation. Accordingly, if any party refuses or otherwise fails to act, or to cause its Affiliates to act, in accordance with the provisions of this Agreement, then, in addition to any other remedy which may be available to any damaged party at law or in equity, such damaged party will be entitled to seek specific performance and injunctive relief, without posting bond or other security, and without the necessity of proving actual or threatened damages, which remedy such damaged party will be entitled to seek in any court of competent jurisdiction.

7.14 No Conflicting Agreements. The Investor hereby represents and warrants to the Company that neither it nor any of its Affiliates is, as of the date of this Agreement, a party to, and agrees that neither it nor any of its Affiliates shall, on or after the date of this Agreement, enter into any agreement that conflicts with the rights granted to the Company in this Agreement. The Company hereby represents and warrants to the Investor that it is not, as of the date of this Agreement, a party to, and agrees that it shall not, on or after the date of this Agreement, enter into any agreement or approve any amendment to its charter or by-laws or similar organizational documents of the Company with respect to its securities that conflicts with the rights granted to the Investor in this Agreement which have not expired or been terminated in accordance with the

terms hereof. The Company further represents and warrants that the rights granted to the Investor hereunder do not in any way conflict with the rights granted to any other holder of the Company's securities under any other agreements.

7.15 Use of Proceeds. The Company shall use the proceeds from the sale of the Purchased Shares for research and development and other working capital purposes and shall not use such proceeds for the redemption of any shares of Common Stock or for the payment of any dividends on shares of Common Stock or in any manner in violation of the Purchase Agreement.

7.16 No Publicity. The parties hereto agree that the provisions of Section 9.5 of the Collaboration Agreement shall be applicable to the parties to this Agreement with respect to any public disclosures regarding the proposed transactions contemplated by the Purchase Agreement and the Collaboration Agreement or regarding the parties hereto or their Affiliates (it being understood that the provisions of Section 9.5 of the Collaboration Agreement shall be read to apply to disclosures of information relating to this Agreement and the transactions contemplated hereby).

(Signature Page Follows)

IN WITNESS WHEREOF, the parties have executed and delivered this Agreement as of the date first above written.

NOVARTIS PHARMA AG

By: */s/ Ian James Hiscock*
Name: Ian James Hiscock
Title: Head Global IP Lit. Transactions

By: */s/ Marc Ceulemans*
Name: Marc Ceulemans
Title: Head Capital Venture Fund Management

VOYAGER THERAPEUTICS, INC.

By: */s/ Alfred Sandrock, M.D., Ph.D.*
Name: Alfred Sandrock, M.D., Ph.D.
Title: Chief Executive Officer and President

EXHIBIT A

NOTICES

If to the Investor:

Novartis Pharma AG
Lichtstrasse 35
CH-4056 Basel
Switzerland
Attention: Head of NIBR General Legal, Europe

with an e-mail copy (which shall not constitute notice)to:

nibr.generalcounsel@novartis.com

with copies (which shall not constitute notice) to:

Novartis Institutes for BioMedical Research, Inc.
250 Massachusetts Avenue
Cambridge, MA 02139 USA
Attn: General Counsel

Arnold & Porter Kaye Scholer LLP
Three Embarcadero Center, 10th Floor
San Francisco, CA 94111
Attention: Stephanie Coutu, Esq.
E-mail: stephanie.coutu@arnoldporter.com

If to the Company:

Voyager Therapeutics, Inc.
75 Hayden Avenue
Lexington, MA 02421
Attention: Chief Executive Officer

with copies (which shall not constitute notice) to:

Voyager Therapeutics, Inc.
75 Hayden Avenue
Lexington, MA 02421
Attention: Chief Legal Officer

Wilmer Cutler Pickering Hale and Dorr LLP
7 World Trade Center
250 Greenwich Street
New York, NY 10007
Attention: Brian A. Johnson, Esq.

Exhibit 10.46

AMENDMENT NO. 2

CONSULTING AGREEMENT

Amendment No. 2 (this "Amendment") to the Consulting Agreement, between Dinah Sah ("Consultant") and Voyager Therapeutics, Inc. ("Voyager") effective as of June 28, 2019 (the "Effective Date") as amended by Amendment No. 1 to the Consulting Agreement, effective as of September 16, 2019 (together, the "Agreement"), is entered into as of June 27, 2022 (the "Amendment Effective Date"). Capitalized terms used in this Amendment and not otherwise defined shall have the meanings given such terms in the Agreement.

WHEREAS, the parties previously entered the Agreement; and

WHEREAS, the parties desire to amend the Agreement as set forth herein.

NOW, THEREFORE, in consideration of the covenants and obligations set forth below, and other good and valuable consideration, the receipt and sufficiency of which is hereby acknowledged, the parties agree as follows:

1. Section 2 – Term & Termination shall be amended by deleting the first sentence and inserting in substitution thereof the following sentence:

   "The term of this Agreement shall be from the Effective Date through June 30, 2024, unless earlier terminated in accordance with this Agreement or extended by mutual agreement (the "Term")."

2. Exhibit A: Section 2 – Compensation shall be amended by adding at the end of the subsection entitled "Fees" the following:

   "Beginning June 27, 2022, $600 per hour, subject to the following.

   Consultant and Voyager acknowledge that separate and independent from this Agreement, Consultant and Voyager have entered into the Scientific Advisory Board and Consulting Agreement, effective March 1, 2020, as amended (the "SAB Consulting Agreement"), pursuant to which Consultant may provide consulting services that are outside the scope of the Services. For purposes of this Agreement and the SAB Consulting Agreement, the services provided under this Agreement and the SAB Consulting Agreement, together, shall constitute "Combined Services."

   Consultant will be paid a retainer for the services performed under the SAB Consulting Agreement, which retainer shall be due and owing to Consultant regardless of the number of hours Consultant works under the SAB Consulting Agreement or this Agreement. However, if Consultant dedicates more than 50 hours of Combined Services to Voyager in any calendar year (including attendance at and travel to meetings of the SAB), Consultant shall be entitled to receive additional compensation at a rate of $600 per hour for each hour of service over and above 50 hours of Combined Services. Consultant shall invoice Voyager for each hour of Service over and above 50 hours of Combined Services. For the avoidance of doubt,

Consultant and Voyager confirm that for services rendered under both this Agreement and the SAB Consulting Agreement, Consultant shall be paid nothing extra beyond the amount of the retainer specified in the SAB Consulting Agreement, unless and until the amount of Combined Services exceeds 50 hours, at which point Consultant shall be compensated at a rate of $600 per hour for each hour of service in excess of 50 hours, regardless of whether the service is performed under this Agreement or the SAB Consulting Agreement."

3. Exhibit A: Section 3 – Period of Performance shall be amended by deleting the only sentence in the Section and inserting in substitution thereof the following sentence:

   "Services are anticipated to commence on the Effective Date and be completed no later than June 30, 2024."

4. Except as expressly modified by this Amendment, all terms and conditions of the Agreement shall remain unmodified and in full force and effect.

5. This Amendment may be executed and delivered by facsimile or electronically transmitted signature and in two or more counterparts, all of which together shall constitute one and the same instrument. The parties agree that upon being signed and delivered by the parties, this Amendment shall become effective and binding upon the parties, and that such signed copies will constitute evidence of the existence of this Amendment.

IN WITNESS WHEREOF, the parties hereto entered into this Amendment by their duly authorized representatives intending it to be effective as of the Amendment Effective Date.

VOYAGER THERAPEUTICS, INC.	**DINAH SAH**
By: */s/ Robert Hesslein*	By: */s/ Dinah Sah*
Name: Robert Hesslein	
Title: Senior Vice President & General Counsel	

Exhibit 10.47

AMENDMENT NO. 3 TO
CONSULTING AGREEMENT

This Amendment No. 3 to Consulting Agreement (this "Amendment") effective as of May 1, 2023 ("Amendment Effective Date") is entered into by and between (i) **Voyager Therapeutics, Inc.**, a Delaware corporation with an office located at 64 Sidney Street, Cambridge, MA 02139 ("Voyager") and (ii) **Dinah Sah, Ph.D.**, an individual residing at 15 Huckleberry Road, Hopkinton, MA 01748 ("Consultant").

WHEREAS, Voyager and Consultant are parties to that certain Consulting Agreement effective as of June 28, 2019, as amended by (i) Amendment No. 1 effective as of September 16, 2019 and (ii) Amendment No. 2 effective as of June 27, 2022 (as amended, the "Original Agreement"); and

WHEREAS, Voyager and Consultant now wish to amend the Original Agreement as set forth herein.

NOW, THEREFORE, in consideration of the covenants and obligations set forth below, and other good and valuable consideration, the receipt and sufficiency of which is hereby acknowledged, the parties agree as follows:

1. **Amendment to Section 2 (Term & Termination)**. The first sentence of Section 2 of the Original Agreement (Term & Termination) is hereby deleted in its entirety and replaced with the following:

> The term of this Agreement shall be from June 28, 2019 through June 30, 2024, unless earlier terminated in accordance with this Agreement or extended by mutual written agreement (the "Term").

2. **Amendment to Exhibit A, Section 2 (Compensation)**. Exhibit A, Section 2 of the Original Agreement (Compensation), Subsection entitled "Fees" is hereby deleted in its entirety and replaced with the following:

> **Fees**: During the Term, Voyager will pay Consultant fees for Services as follows:
>
> A. For the period May 1, 2023 to June 30, 2024,**$50,000** per month subject to the following:
>
> · If Consultant provides 83.3 hours or more hours of Services in any calendar month after May 1, 2023, Consultant will invoice a flat fee to Voyager of **$50,000** for such calendar month.
>
> · If Consultant provides less than 83.3 hours in any calendar month after May 1, 2023, then Consultant will invoice Voyager as follows:

1. If the “monthly running average” of hours of Services performed by Consultant between May 1, 2023 and such calendar month is equal to or greater than 83.3 hours, then Consultant will invoice a flat fee to Voyager of **$50,000** for such calendar month; and

2. If the “monthly running average” of hours of Services performed by Consultant between May 1, 2023 and such calendar month is less than 83.3 hours, then Consultant will invoice Voyager for the actual number of hours of Services provided for such month at an hourly rate of **$600** per hour.

· Consultant and Voyager acknowledge that separate and independent from this Agreement, Consultant and Voyager have entered into the Scientific Advisory Board and Consulting Agreement, effective March 1, 2020, as amended (the “SAB Consulting Agreement”), pursuant to which Consultant may provide consulting services that are outside the scope of the Services. For purposes of this Agreement and the SAB Consulting Agreement, the Services provided under this Agreement and the SAB Consulting Agreement, together, shall constitute “Combined Services”. Consultant will be paid a retainer for the services performed under the SAB Consulting Agreement, which retainer shall be due and owing to Consultant regardless of the number of hours Consultant works under the SAB Consulting Agreement or this Agreement. However, if Consultant dedicates more than 50 hours of Combined Services to Voyager in any calendar year (including attendance at and travel to meetings of the SAB), Consultant shall be entitled to receive additional compensation at the rates indicated above for hours over and above 50 hours of Combined Services. Consultant shall invoice Voyager for each hour of Service over and above 50 hours of Combined Services. For the avoidance of doubt, Consultant and Voyager confirm that for services rendered under both this Agreement and the SAB Consulting Agreement, Consultant shall be paid nothing extra beyond the amount of the retainer specified in the SAB Consulting Agreement, unless and until the amount of Combined Services exceeds 50 hours, at which point Consultant shall be compensated at the rates indicated above for each hour of service in excess of 50 hours, regardless of whether the service is performed under this Agreement or the SAB Consulting Agreement. These additional fees will exist as long as a SAB Consulting Agreement is in place.

B. For the period June 27, 2022 to April 30, 2023,**$600** per hour subject to the following:

Consultant and Voyager acknowledge that separate and independent from this Agreement, Consultant and Voyager have entered into the Scientific Advisory Board and Consulting Agreement, effective March 1, 2020, as amended (the “SAB Consulting Agreement”),

pursuant to which Consultant may provide consulting services that are outside the scope of the Services. For purposes of this Agreement and the SAB Consulting Agreement, the Services provided under this Agreement and the SAB Consulting Agreement, together, shall constitute "Combined Services". Consultant will be paid a retainer for the services performed under the SAB Consulting Agreement, which retainer shall be due and owing to Consultant regardless of the number of hours Consultant works under the SAB Consulting Agreement or this Agreement. However, if Consultant dedicates more than 50 hours of Combined Services to Voyager in any calendar year (including attendance at and travel to meetings of the SAB), Consultant shall be entitled to receive additional compensation at a rate of **$600** per hour for each hour of service over and above 50 hours of Combined Services. Consultant shall invoice Voyager for each hour of Service over and above 50 hours of Combined Services. For the avoidance of doubt, Consultant and Voyager confirm that for services rendered under both this Agreement and the SAB Consulting Agreement, Consultant shall be paid nothing extra beyond the amount of the retainer specified in the SAB Consulting Agreement, unless and until the amount of Combined Services exceeds 50 hours, at which point Consultant shall be compensated at a rate of **$600** per hour for each hour of service in excess of 50 hours, regardless of whether the service is performed under this Agreement or the SAB Consulting Agreement.

C. For the period January 1, 2020 to June 26, 2022,**$450** per hour.

D. For the months of October 2019 through December 2019,**$2,500** per day, provided, that regardless of the number of days worked in any month, Consultant (i) shall receive as a retainer a minimum monthly payment of **$20,0000** per month and (ii) shall not be entitled to receive a payment in excess of **$40,000** per month, For purposes of calculating the number of days worked in any month, Consultant shall (i) aggregate the number of days worked during the month, (ii) divide by eight (8) and (iii) invoice the Company in full and half-day increments.

E. For the months of August 2019 and September 2019,**$35,000** per month.

F. For the month of July 2019,**$20,000** per month.

3. Amendment to Exhibit A, Section 3 (Period of Performance) Exhibit A, Section 3 of the Original Agreement (Period of Performance) is hereby deleted in its entirety and replaced with the following:

"Services are anticipated to commence on June 28, 2019 and be completed no later than June 30, 2024."

4. **Trading in Securities.** Consultant is aware that the United States and other applicable securities laws prohibit any person who has material, non-public information about a company from purchasing or selling securities of such company or from communicating such information to any other person under circumstances in which it is reasonably foreseeable that such person is likely to purchase or sell such securities. Consultant shall comply with all relevant laws, rules, and regulations respecting any trading in public securities. Consultant acknowledges and agrees that, effective as of the Amendment Effective Date, Consultant will comply with any requirements applicable to Consultant under Voyager's Insider Trading Policy (as amended from time to time, the "Policy"), which may include (a) a requirement to obtain preclearance for any trading in Voyager securities in accordance with the requirements applicable to "Insiders" under the Policy, and (b) compliance with restrictions on trading in Voyager securities that apply during regular or special blackout periods under the Policy.

5. **No Other Modifications.** Any terms and conditions of the Original Agreement not expressly amended by this Amendment shall remain in full force and effect.

6. **Complete Understanding; Counterparts.** This Amendment constitutes the entire agreement between the parties with respect to the specific subject matter of this Amendment and all prior agreements, oral or written, with respect to such subject matter, including the Original Agreement, are superseded. If there is any conflict, discrepancy or inconsistency between the terms of this Amendment and the terms of the Original Agreement, the terms of this Amendment will control. This Amendment may be executed in any number of counterparts, each of which will be deemed to be an original and all of which together will constitute one and the same instrument. A facsimile or portable document format (".pdf") copy of this Amendment, including the signature pages, will be deemed an original.

[Remainder of this page is intentionally left blank]

SIGNATURE PAGE TO
AMENDMENT NO. 3 TO CONSULTING AGREEMENT

IN WITNESS WHEREOF, each of the parties has caused this Amendment to be executed under seal effective as of the Amendment Effective Date.

VOYAGER:

VOYAGER THERAPEUTICS, INC.

By: */s/ Alfred W. Sandrock, Jr., M.D., Ph.D.*
Name: Alfred W. Sandrock, Jr., M.D., Ph.D.
Title: President and CEO

CONSULTANT:

/s/ Dinah Sah, Ph.D.
(SIGNATURE)
Print Name: Dinah Sah, Ph.D.

Exhibit 21.1

SUBSIDIARIES OF THE REGISTRANT

Name of Entity	State/Country of Organization
Voyager Securities	Corporation Massachusetts

Exhibit 23.1

Consent of Independent Registered Public Accounting Firm

We consent to the incorporation by reference in the following Registration Statements:

- Registration Statement (Form S-3 No. 333-268240) and related Prospectus of Voyager Therapeutics, Inc. for the registration of common stock, preferred stock, debt securities, depositary shares, subscription rights, warrants, purchase contracts, and units,
- Registration Statement (Form S-8 No. 333-207958) pertaining to the 2014 Stock Option and Grant Plan, the 2015 Stock Option and Incentive Plan, and the 2015 Employee Stock Purchase Plan of Voyager Therapeutics, Inc.,
- Registration Statement (Form S-8 Nos. 333-210258, 333-216699, 333-223638, 333-236870 and 333-263356) pertaining to the 2015 Stock Option and Incentive Plan and the 2015 Employee Stock Purchase Plan of Voyager Therapeutics, Inc., and
- Registration Statement (Form S-8 Nos. 333-229891, 333-253549, and 333-270317) pertaining to the 2015 Stock Option and Incentive Plan, the 2015 Employee Stock Purchase Plan, the Inducement Stock Option Grant Awards and the Inducement Restricted Stock Unit Awards of Voyager Therapeutics, Inc.;

of our report dated February 28, 2024, with respect to the consolidated financial statements of Voyager Therapeutics, Inc. included in this Annual Report (Form 10-K) of Voyager Therapeutics, Inc. for the year ended December 31, 2023.

/s/ Ernst & Young LLP

Boston, Massachusetts
February 28, 2024

Exhibit 31.1

Certification

I, Alfred Sandrock, certify that:

1. I have reviewed this Annual Report on Form 10-K of Voyager Therapeutics, Inc.;

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:

   a. Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

   b. Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

   c. Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

   d. Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

   a. All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

   b. Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: February 28, 2024

/s/Alfred Sandrock, M.D., Ph.D.

Alfred Sandrock, M.D., Ph.D.
Chief Executive Officer, President, and Director
(Principal Executive Officer)

Exhibit 31.2

Certification

I, Peter P. Pfreundschuh, certify that:

1. I have reviewed this Annual Report on Form 10-K of Voyager Therapeutics, Inc.;

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:

   a. Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

   b. Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

   c. Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

   d. Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

   a. All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

   b. Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: February 28, 2024

/s/Peter P. Pfreundschuh

Peter P. Pfreundschuh
Chief Financial Officer
(Principal Financial and Accounting Officer)

Exhibit 32.1

CERTIFICATION PURSUANT TO
18 U.S.C. SECTION 1350,
AS ADOPTED PURSUANT TO
SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002

In connection with the Annual Report on Form 10-K of Voyager Therapeutics, Inc. (the "Company") for the year ended December 31, 2023 as filed with the Securities and Exchange Commission on the date hereof (the "Report"), each of the undersigned officers hereby certifies, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that to the best of his or her knowledge:

1) the Report fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934, as amended; and

2) the information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

Date: February 28, 2024

/s/ Alfred Sandrock, M.D., Ph.D.

Alfred Sandrock, M.D., Ph.D.
Chief Executive Officer, President, and Director
(Principal Executive Officer)

Date: February 28, 2024

/s/Peter P. Pfreundschuh

Peter P. Pfreundschuh
Chief Financial Officer
(Principal Financial and Accounting Officer)

Exhibit 97.1

VOYAGER THERAPEUTICS, INC.

Compensation Recovery Policy

This Compensation Recovery Policy (this "**Policy**") is adopted by Voyager Therapeutics, Inc. (the "**Company**") in accordance with Nasdaq Listing Rule 5608 ("**Rule 5608**"). This Policy is effective as of October 2, 2023 (the "**Effective Date**").

1. Definitions

(a) **"Accounting Restatement"** means a requirement that the Company prepare an accounting restatement due to the material noncompliance of the Company with any financial reporting requirement under the U.S. federal securities laws, including any required accounting restatement to correct an error in previously issued financial statements that is material to the previously issued financial statements, or that would result in a material misstatement if the error were corrected in the current period or left uncorrected in the current period. Changes to the Company's financial statements that do not represent error corrections are not an Accounting Restatement, including: (A) prospective or retrospective application of a change in accounting principle; (B) prospective or retrospective revision to reportable segment information due to a change in the structure of the Company's internal organization; (C) retrospective reclassification due to a discontinued operation; (D) retrospective application of a change in reporting entity, such as from a reorganization of entities under common control; and (E) retrospective revision for stock splits, reverse stock splits, stock dividends or other changes in capital structure.

(b) **"Committee"** means the Compensation Committee of the Company's Board of Directors (the "Board").

(c) **"Covered Person"** means a person who served as an Executive Officer (see definition below) at any time during the performance period for the applicable Incentive-Based Compensation.

(d) **"Erroneously Awarded Compensation"** means the amount of Incentive-Based Compensation that was Received that exceeds the amount of Incentive-Based Compensation that otherwise would have been Received had the amount of Incentive-Based Compensation been determined based on the restated amounts, computed without regard to any taxes paid by the Covered Person or by the Company on the Covered Person's behalf. For Incentive-Based Compensation based on stock price or total shareholder return, where the amount of Erroneously Awarded Compensation is not subject to mathematical recalculation directly from the information in an Accounting Restatement, the amount of Erroneously Awarded Compensation will be based on a reasonable estimate by the Committee of the effect of the Accounting Restatement on the stock price or total shareholder return upon which the Incentive-Based Compensation was Received. The Company will maintain documentation of the determination of that reasonable estimate and provide such documentation to Nasdaq.

(e) **"Executive Officer"** means the Company's officers as defined in Rule 16a-1(f) under the Securities Exchange Act of 1934, as amended.

(f) **"Financial Reporting Measures"** means (A) measures that are determined and presented in accordance with the accounting principles used in preparing the Company's financial statements, and any measures that are derived wholly or in part from such measures (whether or not such measures are presented within the Company's financial statements or included in a filing made with the U.S. Securities and Exchange Commission), (B) stock price and (C) total shareholder return.

(g) **"Incentive-Based Compensation"** means any compensation that is granted, earned, or vested based wholly or in part upon the attainment of a Financial Reporting Measure.

(h) Incentive-Based Compensation is deemed to be **"Received"** in the Company's fiscal period during which the Financial Reporting Measure specified in the applicable Incentive-Based Compensation award is attained, even if the payment or grant of the Incentive-Based Compensation occurs after the end of that period or is subject to additional time-based vesting requirements.

(i) **"Recovery Period"** means the three completed fiscal years immediately preceding the earlier of: (A) the date the Board, a committee of the Board, or the officer or officers of the Company authorized to take such action if Board action is not required, concludes, or reasonably should have concluded, that the Company is required to prepare an Accounting Restatement; or (B) the date a court, regulator, or other legally authorized body directs the Company to prepare an Accounting Restatement. In addition, if there is a change in the Company's fiscal year end, the Recovery Period will also include any transition period to the extent required by Rule 5608.

2. Recovery of Erroneously Awarded Compensation

Subject to the terms of this Policy and the requirements of Rule 5608, if the Company is required to prepare an Accounting Restatement, the Company will attempt to recover, reasonably promptly from each Covered Person, any Erroneously Awarded Compensation that was Received by such Covered Person during the Recovery Period pursuant to Incentive-Based Compensation that is subject to this Policy.

3. Interpretation and Administration

(a) Role of the Committee. This Policy will be interpreted by the Committee in a manner that is consistent with Rule 5608 and any other applicable law and will otherwise be interpreted in the business judgment of the Committee. All decisions and interpretations of the Committee will be final and binding.

(b) Compensation Not Subject to this Policy. This Policy does not apply to Incentive-Based Compensation that was Received before the Effective Date. With respect to any Covered Person, this Policy does not apply to Incentive-Based Compensation that was Received by such Covered Person before beginning service as an Executive Officer.

(c) Determination of Means of Recovery. Subject to the requirement that recovery be made reasonably promptly, the Committee will determine the appropriate means of recovery, which may vary between Covered Persons or based on the nature of the applicable Incentive-Based Compensation, and which may involve, without limitation, establishing a deferred repayment plan or setting off against current or future compensation otherwise payable to the Covered Person. Recovery of Erroneously Awarded Compensation will be made without regard to income taxes paid by the Covered Person or by the Company on the Covered Person's behalf in connection with such Erroneously Awarded Compensation.

(d) Determination That Recovery is Impracticable. The Company is not required to recover Erroneously Awarded Compensation if a determination is made by the Committee that either (A) after the Company has made and documented a reasonable attempt to recover such Erroneously Awarded Compensation, the direct expense paid to a third party to assist in enforcing this Policy would exceed the amount to be recovered or (B) recovery of such Erroneously Awarded Compensation would likely cause an otherwise tax-qualified retirement plan, under which benefits are broadly available to employees of the registrant, to fail to meet the requirements of Section 401(a)(13) or 411(a) of the Internal Revenue Code and regulations thereunder.

(e) No Indemnification or Company-Paid Insurance. The Company will not indemnify any Covered Person against the loss of Erroneously Awarded Compensation and will not pay or reimburse any Covered Person for the purchase of a third-party insurance policy to fund potential recovery obligations.

(f) Interaction with Other Clawback Provisions. The Company will be deemed to have recovered Erroneously Awarded Compensation in accordance with this Policy to the extent the Company actually receives such amounts pursuant to any other Company policy, program or agreement, pursuant to Section 304 of the Sarbanes-Oxley Act or otherwise.

(g) No Limitation on Other Remedies. Nothing in this Policy will be deemed to limit the Company's right to terminate employment of any Covered Person, to seek recovery of other compensation paid to a Covered Person, or to pursue other rights or remedies available to the Company under applicable law.

Adopted by the Board on September 22, 2023.