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DELTA REPORT

10-Q

VYGR - VOYAGER THERAPEUTICS, INC

10-Q - MARCH 31, 2024 COMPARED TO 10-Q - SEPTEMBER 30, 2023

The following comparison report has been automatically generated

TOTAL DELTAS	2220
CHANGES	148
DELETIONS	720
ADDITIONS	1352

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UNITED STATES
SECURITIES AND EXCHANGE COMMISSION

Washington, D.C. 20549

FORM 10-Q

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

For the quarterly period ended September 30, 2023 March 31, 2024

☐ **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)	**(I.R.S. 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)

64 Sidney Street, Cambridge, Massachusetts 02139 Not Applicable

(Former name, former address and former fiscal year, if changed since last report)

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 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 is a shell company (as defined in Rule 12b-2 of the Exchange Act). Yes ☐ No ☒

The number of outstanding shares of the registrant's common stock, par value $0.001 per share, as of October 30, 2023 May 8, 2024 was 43,995,363. 54,393,628.

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Forward-Looking Statements

This Quarterly Report on Form 10-Q contains forward-looking statements that involve substantial risks and uncertainties. All statements other than statements of historical facts contained in this Quarterly Report on Form 10-Q, 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 proprietary antibodies; 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 partner, partners Neurocrine Biosciences, Inc. and Novartis Pharma AG, or Novartis, and our licensees licensee Alexion, AstraZeneca Rare Disease or Alexion (successor-in-interest to former licensee Pfizer Inc.), and Novartis Pharma AG; ;

- our planned clinical trials and 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 capital requirements; 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;

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- 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.

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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 our Annual Report on Form 10-K filed with the Securities and Exchange Commission on March 7, 2023February 28, 2024, particularly in "Part I, Item 1A — Risk Factors," and, if applicable, our Quarterly Reports on Form 10-Q, particularly in "Part II, 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 Quarterly Report on Form 10-Q and the documents that we have filed as exhibits to this Quarterly Report on Form 10-Q 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 Quarterly Report on Form 10-Q and the documents we have filed as exhibits to the Quarterly Report on Form 10-Q 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 Quarterly Report on Form 10-Q and the documents we have filed as exhibits to the Quarterly Report on Form 10-Q 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 Quarterly Report on Form 10-Q. 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 Quarterly Report on Form 10-Q, 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 Quarterly Report on Form 10-Q and the documents filed as exhibits to the Quarterly Report on Form 10-Q contain references to trademarks, service marks and trade names referred to in this Quarterly Report on Form 10-Q and the information incorporated herein, including logos, artwork, and other visual displays, that may appear without the ® or ™ 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 Quarterly Report on Form 10-Q and the documents filed as exhibits to the Quarterly Report on Form 10-Q are the property of their respective owners.

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VOYAGER THERAPEUTICS, INC.

FORM 10-Q

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PART I. FINANCIAL INFORMATION

Voyager Therapeutics, Inc.
Condensed Consolidated Balance Sheets
(amounts in thousands, except share and per share data)

(unaudited)

	September 30, 2023	December 31, 2022
Assets		
Current assets:		
Cash and cash equivalents	$ 65,269	$ 98,959
Marketable securities, current	187,667	19,889
Related party collaboration receivable	3,253	257
Prepaid expenses and other current assets	5,736	5,394
Total current assets	261,925	124,499
Property and equipment, net	17,109	17,857
Deposits and other non-current assets	1,593	1,515
Operating lease, right-of-use assets	14,026	15,485
Total assets	$ 294,653	$ 159,356
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable	$ 3,270	$ 2,566
Accrued expenses	11,051	7,816
Other current liabilities	3,106	2,832
Deferred revenue, current	44,261	59,377
Total current liabilities	61,688	72,591
Deferred revenue, non-current	37,826	6,450
Other non-current liabilities	18,919	21,295
Total liabilities	118,433	100,336
Commitments and contingencies (see note 8)		
Stockholders' equity:		
Preferred stock, $0.001 par value: 5,000,000 shares authorized at September 30, 2023 and December 31, 2022; no shares issued and outstanding at September 30, 2023 and December 31, 2022	—	—
Common stock, $0.001 par value: 120,000,000 shares authorized at September 30, 2023 and December 31, 2022; 43,909,161 and 38,613,891 shares issued and outstanding at September 30, 2023 and December 31, 2022, respectively	44	38
Additional paid-in capital	494,001	452,713
Accumulated other comprehensive loss	(248)	(219)
Accumulated deficit	(317,577)	(393,512)
Total stockholders' equity	176,220	59,020
Total liabilities and stockholders' equity	$ 294,653	$ 159,356

	March 31, 2024	December 31, 2023
Assets		
Current assets:		
Cash and cash equivalents	$ 143,078	$ 68,802
Marketable securities, current	256,490	162,073
Accounts receivable	837	80,150
Related party collaboration receivable	2,620	3,341

Prepaid expenses and other current assets	6,112	5,318
Total current assets	409,137	319,684
Property and equipment, net	17,381	16,494
Deposits and other non-current assets	2,890	1,593
Marketable securities, non-current	980	—
Operating lease, right-of-use assets	39,204	13,510
Total assets	$ 469,592	$ 351,281
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable	$ 7,579	$ 1,604
Accrued expenses	7,595	16,823
Other current liabilities	5,940	3,200
Deferred revenue, current	51,439	42,881
Total current liabilities	72,553	64,508
Deferred revenue, non-current	13,157	32,359
Other non-current liabilities	42,996	18,094
Total liabilities	128,706	114,961
Commitments and contingencies (see note 7)		
Stockholders' equity:		
Preferred stock, $0.001 par value: 5,000,000 shares authorized at March 31, 2024 and December 31, 2023; no shares issued and outstanding at March 31, 2024 and December 31, 2023	—	—
Common stock, $0.001 par value: 120,000,000 shares authorized at March 31, 2024 and December 31, 2023; 54,318,133 and 44,038,333 shares issued and outstanding at March 31, 2024 and December 31, 2023, respectively	54	44
Additional paid-in capital	613,850	497,506
Accumulated other comprehensive loss	(506)	(48)
Accumulated deficit	(272,512)	(261,182)
Total stockholders' equity	340,886	236,320
Total liabilities and stockholders' equity	$ 469,592	$ 351,281

The accompanying notes are an integral part of these condensed consolidated financial statements.

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Voyager Therapeutics, Inc.
Condensed Consolidated Statements of Operations and Comprehensive (Loss) Income
(amounts in thousands, except share and per share data)
(unaudited)

Three Months Ended	Nine Months Ended	Three Months Ended

	September 30,		September 30,		March 31,	
	2023	2022	2023	2022	2024	2023
Collaboration revenue	$ 4,614	$ 41,086	$ 159,947	$ 42,457	$ 19,516	$ 150,480
Operating expenses:						
Research and development	25,863	19,337	66,416	46,213	27,092	18,568
General and administrative	8,258	7,307	25,580	22,518	8,607	9,028
Total operating expenses	34,121	26,644	91,996	68,731	35,699	27,596
Operating (loss) income	(29,507)	14,442	67,951	(26,274)	(16,183)	122,884
Other income:						
Interest income	3,429	545	8,567	816	4,867	1,864
Other income	—	2,637	3	2,676		
Total other income, net	3,429	3,182	8,570	3,492		
Total other income					4,867	1,864
(Loss) income before income taxes	(26,078)	17,624	76,521	(22,782)	(11,316)	124,748
Income tax (benefit) provision	(177)	—	586	—		
Income tax provision					14	704
Net (loss) income	$ (25,901)	$ 17,624	$ 75,935	$ (22,782)	$ (11,330)	$ 124,044
Other comprehensive (loss) income:						
Net unrealized (loss) gain on available-for-sale securities	(115)	27	(29)	(199)	(458)	87
Total other comprehensive (loss) income	(115)	27	(29)	(199)	(458)	87
Comprehensive (loss) income	$ (26,016)	$ 17,651	$ 75,906	$ (22,981)	$ (11,788)	$ 124,131
Net (loss) income per share, basic	$ (0.59)	$ 0.46	$ 1.85	$ (0.59)	$ (0.20)	$ 3.05
Net (loss) income per share, diluted	$ (0.59)	$ 0.45	$ 1.78	$ (0.59)	$ (0.20)	$ 2.94
Weighted-average common shares outstanding, basic	43,864,838	38,507,542	40,962,116	38,292,497	57,117,046	40,632,087
Weighted-average common shares outstanding, diluted	43,864,838	39,570,394	42,610,724	38,292,497	57,117,046	42,161,326

The accompanying notes are an integral part of these condensed consolidated financial statements.

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Voyager Therapeutics, Inc.
Condensed Consolidated Statements of Stockholders' Equity
(amounts in thousands, except share data)
(unaudited)

	Common Stock Shares	Common Stock Amount	Additional Paid-In Capital	Accumulated Other Comprehensive (Loss) Income	Accumulated Deficit	Stockholders' Equity	Common Stock	Additional Paid-In	Accumulated Other Comprehensive (Loss)	Accumulated	Stockholder
Balance at December 31, 2021	37,918,395	$ 38	$ 442,259	$ (138)	$ (347,104)	$ 95,055					
Exercises of vested stock options	11,484	—	12	—	—	12					
Vesting of restricted stock units	312,090	—	—	—	—	—					
Stock-based compensation expense	—	—	2,268	—	—	2,268					
Unrealized loss on available-for-sale securities, net of tax	—	—	—	(85)	—	(85)					
Net loss	—	—	—	—	(21,319)	(21,319)					
Balance at March 31, 2022	38,241,969	$ 38	$ 444,539	$ (223)	$ (368,423)	$ 75,931					
Exercises of vested stock options	63,012	—	575	—	—	575					

Vesting of restricted stock units	32,165	—	—	—	—	—
Issuance of common stock under ESPP	102,105	—	313	—	—	313
Stock-based compensation expense	—	—	2,460	—	—	2,460
Unrealized loss on available-for-sale securities, net of tax	—	—	—	(141)	—	(141)
Net loss	—	—	—	—	(19,087)	(19,087)
Balance at June 30, 2022	38,439,251	$ 38	$ 447,887	$ (364)	$ (387,510)	$ 60,051
Exercises of vested stock options	5,341	—	15	—	—	15
Vesting of restricted stock units	75,744	—	—	—	—	—
Stock-based compensation expense	—	—	2,106	—	—	2,106
Unrealized gain on available-for-sale securities, net of tax	—	—	—	27	—	27
Net income	—	—	—	—	17,624	17,624
Balance at September 30, 2022	38,520,336	$ 38	$ 450,008	$ (337)	$ (369,886)	79,823

							Shares	Amount	Capital	Income	Deficit	Equity
Balance at December 31, 2022	38,613,891	$ 38	$ 452,713	$ (219)	$ (393,512)	59,020	38,613,891	$ 38	$ 452,713	$ (219)	$ (393,512)	59,02
Exercises of vested stock options	51,993	—	185	—	—	185	51,993	—	185	—	—	18

Vesting of restricted stock units	374,417	—	—	—	—	—	374,417	—	—	—	—	–
Issuance of common stock in connection with the 2023 Neurocrine Collaboration Agreement	4,395,588	5	31,116	—	—	31,121	4,395,588	5	31,116	—	—	31,12
Stock-based compensation expense	—	—	2,504	—	—	2,504	—	—	2,504	—	—	2,50
Unrealized gain on available-for-sale securities, net of tax	—	—	—	87	—	87	—	—	—	87	—	8
Net income	—	—	—	—	124,044	124,044	—	—	—	—	124,044	124,04
Balance at March 31, 2023	43,435,889	$ 43	$ 486,518	$ (132)	$ (269,468)	$ 216,961	43,435,889	$ 43	$ 486,518	$ (132)	$ (269,468)	$ 216,96
Balance at December 31, 2023							44,038,333	$ 44	$ 497,506	$ (48)	$ (261,182)	$ 236,32
Exercises of vested stock options	198,348	1	1,228	—	—	1,229	32,500	—	78	—	—	7
Vesting of restricted stock units	62,828	—	—	—	—	—	324,520	—	—	—	—	–
Issuance of common stock under ESPP	62,344		418			418						
Issuance of common stock in connection with the 2023 Novartis Stock Purchase Agreement							2,145,002	2	19,303	—	—	19,30

Issuance of common stock and pre-funded warrants in connection with underwritten public offering							7,777,778	8	93,465	—	—	93,47
Stock-based compensation expense	—	—	2,627	—	—	2,627	—	—	3,498	—	—	3,49
Unrealized loss on available-for-sale securities, net of tax	—	—	—	(1)	—	(1)	—	—	—	(458)	—	(45
Net loss	—	—	—	—	(22,208)	(22,208)	—	—	—	—	(11,330)	(11,33
Balance at June 30, 2023	43,759,409	$ 44	$ 490,791	$ (133)	$ (291,676)	$ 199,026						
Exercises of vested stock options	127,252	—	415	—	—	415						
Vesting of restricted stock units	22,500	—	—	—	—	—						
Stock-based compensation expense	—	—	2,795	—	—	2,795						
Unrealized loss on available-for-sale securities, net of tax	—	—	—	(115)	—	(115)						
Net loss	—	—	—	—	(25,901)	(25,901)						
Balance at September 30, 2023	43,909,161	$ 44	$ 494,001	$ (248)	$ (317,577)	$ 176,220						
Balance at March 31, 2024							54,318,133	$ 54	$ 613,850	$ (506)	$ (272,512)	$ 340,88

The accompanying notes are an integral part of these condensed consolidated financial statements.

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Voyager Therapeutics, Inc.
Condensed Consolidated Statements of Cash Flows
(amounts in thousands)
(unaudited)

	Nine Months Ended September 30,	
	2023	**2022**
Cash flow from operating activities		
Net income (loss)	$ 75,935	$ (22,782)
Adjustments to reconcile net income (loss) to net cash provided by (used in) operating activities:		
Stock-based compensation expense	8,108	6,998
Depreciation	3,106	5,357
Amortization of premiums and discounts on marketable securities	(1,832)	(23)
Loss on disposal of fixed assets	143	—
Other non-cash items	—	(2,468)
Changes in operating assets and liabilities:		
Accounts receivable	—	(10,000)
Related party collaboration receivable	(2,996)	507
Prepaid expenses and other current assets	(342)	(1,652)
Operating lease, right-of-use asset	1,459	3,005
Accounts payable	704	763
Accrued expenses	3,236	1,536
Operating lease liabilities	(2,102)	(3,280)
Other non-current liabilities	—	(151)
Deferred revenue	16,260	21,925
Net cash provided by (used in) operating activities	101,679	(265)
Cash flow from investing activities		
Purchases of property and equipment	(2,501)	(1,558)
Purchases of marketable securities	(194,975)	(54,848)
Proceeds from sales and maturities of marketable securities	29,000	35,000
Net cash used in investing activities	(168,476)	(21,406)
Cash flow from financing activities		
Proceeds from the exercise of stock options	1,829	602
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	235	232
Net cash provided by financing activities	33,185	834
Net decrease in cash, cash equivalents, and restricted cash	(33,612)	(20,837)
Cash, cash equivalents, and restricted cash, beginning of period	100,474	119,212

Cash, cash equivalents, and restricted cash, end of period	$ 66,862	$ 98,375
Supplemental disclosure of cash and non-cash activities		
Capital expenditures incurred but not yet paid	$ —	$ 40

	Three Months Ended March 31,	
	2024	**2023**
Cash flow from operating activities		
Net (loss) income	$ (11,330)	$ 124,044
Adjustments to reconcile net (loss) income to net cash provided by operating activities:		
Stock-based compensation expense	3,573	2,558
Depreciation	1,196	1,075
Amortization of premiums and discounts on marketable securities	(1,931)	(17)
Loss on disposal of fixed assets	59	44
Changes in operating assets and liabilities:		
Accounts receivable	79,313	(25,000)
Related party collaboration receivable	721	(71)
Prepaid expenses and other current assets	(794)	1,411
Operating lease, right-of-use asset	1,057	469
Other non-current assets	(15)	—
Accounts payable	5,975	1,132
Accrued expenses	(9,305)	(122)
Operating lease liabilities	891	(683)
Deferred revenue	(10,643)	18,725
Net cash provided by operating activities	58,767	123,565
Cash flow from investing activities		
Purchases of property and equipment	(2,141)	(509)
Purchases of marketable securities	(203,852)	—
Proceeds from sales and maturities of marketable securities	109,928	15,000
Net cash (used in) provided by investing activities	(96,065)	14,491
Cash flow from financing activities		
Proceeds from the exercise of stock options	78	185
Proceeds from the issuance of common stock in connection with the underwritten public offering	93,473	—
Proceeds from the issuance of common stock in connection with the 2023 Novartis Stock Purchase Agreement	19,305	—
Proceeds from the issuance of common stock in connection with the 2023 Neurocrine Collaboration Agreement	—	31,121
Net cash provided by financing activities	112,856	31,306
Net increase in cash, cash equivalents, and restricted cash	75,558	169,362
Cash, cash equivalents, and restricted cash, beginning of period	70,395	100,474
Cash, cash equivalents, and restricted cash, end of period	$ 145,953	$ 269,836
Supplemental disclosure of cash and non-cash activities		
Capital expenditures incurred but not yet paid	$ —	$ 109
Operating lease right-of-use asset obtained in exchange for operating lease liability	$ 26,751	$ —

The accompanying notes are an integral part of these condensed consolidated financial statements.

VOYAGER THERAPEUTICS INC.

NOTES TO UNAUDITED CONDENSED CONSOLIDATED FINANCIAL STATEMENTS

1. Nature of business

Voyager Therapeutics, Inc. (the "Company") is a biotechnology company dedicated whose mission is to breaking through barriers in gene therapy leverage the power of human genetics to modify the course of and neurology. The Company focuses on leveraging its expertise in capsid discovery and neuropharmacology to address the delivery hurdles that have constrained the gene therapy and neurology disciplines, with the goal of either halting or slowing disease progression or reducing symptom severity, therefore providing clinically meaningful impact to patients. ultimately cure neurological diseases. The Company's gene therapy platforms enable it to engineer, optimize, manufacture pipeline includes programs for Alzheimer's disease; amyotrophic lateral sclerosis; Parkinson's disease, and deliver multiple other diseases of the central nervous system. Many of the Company's programs are derived from its TRACER™ adeno-associated virus ("AAV") based gene therapies that it believes have the potential to safely provide durable efficacy. The Company's team of experts in the fields of AAV gene therapy and neuroscience first identifies and selects diseases in capsid discovery platform, which the Company believes an AAV gene therapy or other biological therapy will answer a high unmet medical need, be supported by target validation, offer an efficient path has used to human proof of biology, present robust preclinical pharmacology, and offer strong commercial potential. The Company then engineers and optimizes an AAV vector or other biological therapy for activity in, efficacy in, or delivery to, the targeted tissue or cells.

The Company is identifying proprietary AAV generate novel capsids the outer viral protein shells that enclose genetic material that makes up the vector payload. The Company's team has developed a proprietary AAV capsid discovery platform called TRACERTM (Tropism Redirection of AAV by Cell Type-Specific Expression of RNA) that employs directed evolution to facilitate the selection of AAV capsids with enhanced tissue delivery characteristics, such as more effective delivery across the blood-brain barrier ("BBB"). 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 non-human primates. The Company believes that the capsids it discovers through its TRACER discovery platform ("TRACER Capsids") have the potential and identify associated receptors to significantly enhance the efficacy and safety of its single dose gene therapies, which the Company expects to be delivered potentially enable high brain penetration with systemic infusions, as compared with conventional capsids. The Company has leveraged the TRACER discovery platform to generate multiple families of TRACER Capsids with robust central nervous system ("CNS") tropism genetic medicines following intravenous delivery. The Company has presented data at scientific conferences demonstrating strong transduction to multiple areas within dosing. Some of the brain Company's programs are wholly-owned, and activity across multiple species. The Company has identified receptors for some of its TRACER Capsid families as well as a ligand for a particular receptor are advancing with licensees and is conducting experiments to evaluate the potential to leverage its receptors to shuttle non-viral genetic medicines across the BBB.

In addition to leveraging TRACER Capsids in potential licensing arrangements, the Company is advancing its own proprietary pipeline of drug candidates for neurological diseases, with a focus on Alzheimer's disease. The Company's wholly-owned prioritized pipeline programs include superoxide dismutase 1 collaborators including Alexion, AstraZeneca Rare Disease; Novartis Pharma AG, ("SOD1" Novartis") gene therapy for amyotrophic lateral sclerosis ("ALS") ; and an anti-tau antibody for Alzheimer's disease. The Company identified a lead development candidate in its anti-tau antibody program in the first quarter of 2023, initiated good laboratory practices toxicology studies in the third quarter of 2023, and expects to submit an investigational new drug application ("IND") to the U.S. Food and Drug Administration ("FDA") in the first half of 2024. The Company continues to evaluate the data from preclinical studies for its SOD1 program and expects to identify a

lead development candidate in 2023. The Company expects to submit an IND for its SOD1 program in mid-2025. The Company's pipeline also includes four early research initiatives to develop gene therapies for the treatment of Alzheimer's disease, Huntington's disease, and brain metastases from HER2+ metastatic breast cancer.

In addition to these wholly-owned programs, the Company is actively advancing two later preclinical-stage programs in collaboration with Neurocrine Biosciences, Inc. ("Neurocrine"): a glucocerebrosidase 1 ("GBA1") gene therapy program for Parkinson's disease and other GBA1-mediated diseases (the "GBA1 Program"), and a FXN gene therapy program for Friedreich's ataxia. The Company also maintains a robust early research pipeline of wholly-owned and collaborative gene therapy programs for neurological diseases. .

The Company has a history of incurring annual net operating losses. As of September 30, 2023 March 31, 2024, the Company had an accumulated deficit of $317.6 million $272.5 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, funding from fees, option exercise payments, and milestone payments, and cost reimbursements associated with its prior

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collaborations with Sanofi Genzyme Corporation ("Sanofi Genzyme") and AbbVie Biotechnology Ltd and AbbVie Ireland Unlimited Company, its ongoing collaborations with Neurocrine, its option and license agreement with Alexion, AstraZeneca Rare Disease ("Alexion") (successor-in-interest to former licensee Pfizer Inc. ("Pfizer")), and its option and license agreement with Novartis Pharma AG ("Novartis"), as well as public offerings and private placements of its equity securities. agreements.

As of September 30, 2023 March 31, 2024, the Company had cash, cash equivalents, and marketable securities of $252.9 million $400.5 million. The Based upon the Company's current operating plans, the Company is committed to maintaining a strong balance sheet expects that supports the advancement and growth of its platform and pipeline. The Company continues to assess its planned cash needs both during and in future periods. It expects its existing cash, cash equivalents, and marketable securities along with amounts expected to be received as reimbursement for development costs under the Neurocrine collaborations and interest income, at March 31, 2024 to be sufficient to meet the Company's planned operating expenses and capital expenditure requirements into mid-2025. 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 unaudited condensed consolidated financial statements of the Company have been prepared in accordance with accounting principles generally accepted in the United States ("GAAP") for interim financial reporting. Accordingly, they do not include all of the information and footnotes required by GAAP for complete financial statements. For

further information, refer to the consolidated financial statements and footnotes included in the Company's Annual Report on Form 10-K for the fiscal year ended December 31, 2022 December 31, 2023 as filed with the Securities and Exchange Commission ("SEC") on March 7, 2023 February 28, 2024. These interim condensed consolidated financial statements, in the opinion of management, reflect all normal recurring adjustments necessary for a fair presentation of the Company's financial position and results of operations for the periods presented. Any reference in these notes to applicable guidance is meant to refer to the authoritative United States generally accepted accounting principles as found in the Accounting Standards Codification ("ASC") and Accounting Standards Updates of the Financial Accounting Standards Board.

Principles of Consolidation

The unaudited interim consolidated financial statements include the accounts of the Company and its wholly-owned subsidiary as disclosed in Note 2, under the heading "Summary of Significant Accounting Policies and Basis of Presentation" within the "Notes to Consolidated Financial Statements" accompanying the Company's Annual Report on Form 10-K for the fiscal year ended December 31, 2022 December 31, 2023. Intercompany balances and transactions have been eliminated.

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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, incremental borrowing rate for leases, 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.

Summary of Significant Accounting Policies

There have been no changes in the Company's significant accounting policies as described in Note 2, "Summary of Significant Accounting Policies and Basis of Presentation" within the "Notes to Consolidated Financial Statements" accompanying the Company's Annual Report on Form 10-K for the year ended December 31, 2022 December 31, 2023.

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3. Fair value measurements

Assets and liabilities measured at fair value on a recurring basis as of September 30, 2023 March 31, 2024 and December 31, 2022 December 31, 2023 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)	Total	Quoted Prices in Active Markets for Identical Assets (Level 1)	Significant Other Observable Inputs (Level 2)	Significant Unobservable Inputs (Level 3)
September 30, 2023		*(in thousands)*						
March 31, 2024								
March 31, 2024								
March 31, 2024								
March 31, 2024								
March 31, 2024								
March 31, 2024								
March 31, 2024								
March 31, 2024								
March 31, 2024								
March 31, 2024								
March 31, 2024								
March 31, 2024								
March 31, 2024								
						(in thousands)		
Money market funds included in cash and cash equivalents	$ 58,098	$ 58,098	$ —	$ —	$ 49,444	$ 49,444	$ —	$ —
Marketable securities:								
U.S. Treasury notes	111,071	111,071	—	—	24,486	24,486	—	—
U.S. Government agency securities	34,232	34,232	—	—	138,191	138,191	—	—
Corporate bonds	27,780	—	27,780	—	88,983	—	88,983	—
Commercial paper	14,583	—	14,583	—	4,830	—	4,830	—
Total	$245,764	$ 203,401	$ 42,363	$ —				
December 31, 2022								
Total money market funds and marketable securities					$305,934	$ 212,121	$ 93,813	$ —

December 31, 2023								
Money market funds included in cash and cash equivalents	$ 91,724	$ 91,724	$ —	$ —	$ 65,589	$ 65,589	$ —	$ —
Marketable securities:								
U.S. Treasury notes	19,889	19,889	—	—	103,044	103,044	—	—
Total	$111,613	$ 111,613	$ —	$ —				
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	$ —

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 active markets or based on quoted market prices of similar instruments and other significant inputs derived from or corroborated by observable market data.

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4. Cash, cash equivalents, restricted cash, and available-for-sale marketable securities

Cash, cash equivalents, and marketable securities included the following at September 30, 2023 March 31, 2024 and December 31, 2022 December 31, 2023:

Amortized Cost	Unrealized Gains	Unrealized Losses	Fair Value	Amortized Cost	Unrealized Gains	Unrealized Losses	Fair Value
(in thousands)							

As of September 30, 2023								
						(in thousands)		
As of March 31, 2024								
Money market funds included in cash and cash equivalents	$ 58,098	$ —	$ —	$ 58,098	$ 49,444	$ —	$ —	$ 49,444
Marketable securities:								
U.S. Treasury notes	111,120	3	(52)	111,071	24,540	—	(54)	24,486
U.S. Government agency securities	34,273	—	(41)	34,232	138,285	7	(101)	138,191
Corporate bonds	27,811	—	(31)	27,780	89,207	11	(235)	88,983
Commercial paper	14,583	—	—	14,583	4,829	1	—	4,830
Total money market funds and marketable securities	$ 245,885	$ 3	$ (124)	$ 245,764	$306,305	$ 19	$ (390)	$305,934
As of December 31, 2022								
As of December 31, 2023								
Money market funds included in cash and cash equivalents	$ 91,724	—	—	$ 91,724	$ 65,589	—	—	$ 65,589
Marketable securities:								
U.S. Treasury notes	19,980	—	(91)	19,889	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	$ 111,704	$ —	$ (91)	$ 111,613	$227,583	$ 93	$ (13)	$227,663

All of the Company's The Company had $1.0 million in marketable securities as of September 30, 2023 March 31, 2024 with a contractual maturity of greater than one year. All other marketable securities 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 (loss) 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 condensed consolidated statement of operations. 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 $81.8 million $230.7 million and $19.9 million in $44.2 million marketable securities that were in an unrealized loss position as of September 30, 2023 March 31, 2024 and December 31, 2022 December 31, 2023, respectively. The unrealized losses at September 30, 2023 March 31, 2024 and December 31, 2022 December 31, 2023 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.

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The following table provides a reconciliation of cash, cash equivalents, and restricted cash within the condensed consolidated balance sheets that sum to the total of the same such amounts shown in the condensed consolidated statements of cash flows:

	As of September 30,	As of December 31,	As of March 31,	
	2023	2022	2024	2023
	(in thousands)			
				(in thousands)
Cash and cash equivalents	$ 65,269	$ 98,959	$143,078	$268,321
Restricted cash included in deposits and other non-current assets	1,593	1,515	2,875	1,515
Total cash, cash equivalents, and restricted cash	$ 66,862	$ 100,474	$145,953	$269,836

5. Accrued expenses

Accrued expenses as of September 30, 2023 March 31, 2024 and December 31, 2022 December 31, 2023 consist of the following:

	As of September 30, 2023	As of December 31, 2022	As of March 31, 2024	As of December 31, 2023
	(in thousands)			
				(in thousands)
Research and development costs			$2,914	$ 5,225
Employee compensation costs	$ 4,809	$ 4,559	2,248	6,614
Research and development costs	4,196	1,895		
Accrued goods and services			1,838	4,229
Professional services	755	726	595	755
Accrued goods and services	1,291	636		
Total	$ 11,051	$ 7,816	$7,595	$16,823

6. Lease obligation

Operating Leases

As of September 30, 2023 March 31, 2024, the Company has a lease for office and laboratory space at 64 Sidney Street in Cambridge, Massachusetts through November 30, 2026 and a lease for laboratory and office space at 75 Hayden Avenue in Lexington, Massachusetts through January 31, 2031.

In September 2021, the Company entered into an agreement with BioNTech US, Inc. (“BioNTech US”) to sublease part of the and a lease for additional office and laboratory space leased by the Company at 75 64 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 because 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, 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 three-month period ended June 30, 2022 through November 30, 2026.

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.

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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. space through January 31, 2031. The term Company received $1.8 million of leasehold improvement incentives associated with the First Amendment. The Company gained control of the First Amendment commences space on February 1, 2024 and recorded a $26.7 million right-of-use asset and a $26.7 million operating lease liability, accordingly, which reflect the date on which the landlord makes the space available for use by leasehold improvement incentive.

The Company's lease agreements require the Company and expires on January 31, 2031, unless sooner terminated to maintain a cash deposit or extended. As irrevocable letter of September 30, 2023, credit in the space is not yet available for use by the Company, and therefore, the lease has not yet commenced. The commencement date aggregate amount of $2.9 million payable to its landlords as security for the First Amendment is estimated to occur on or about February 1, 2024. performance of its obligations under the leases. These amounts are recorded as restricted cash and are included in deposits and other non-current assets in the accompanying condensed consolidated balance sheets.

During each of the three and nine months ended September 30, 2023, March 31, 2024 and 2023, the Company incurred lease expenses of $0.9 million $1.7 million and $2.7 million, respectively, for operating leases. During each of the three and nine months ended September 30, 2022, the Company incurred lease expenses of $1.0 million and $3.8 million $0.9 million, respectively, for operating leases. As of September 30, 2023 March 31, 2024, the weighted average remaining lease term was 5.3 5.5 years and the weighted average incremental borrowing rate used to determine the operating lease liability was 7.4% 6.9%.

The following table summarizes the operating sublease income generated under the Sublease Agreement for the three and nine months ended September 30, 2023 and 2022:

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2023	2022	2023	2022
	(in thousands)			
Operating sublease income	$ —	$ —	$ —	$ 1,380

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7. Other Commitments, contingencies and other liabilities

As of September 30, 2023 March 31, 2024 and December 31, 2022 December 31, 2023, other current and non-current liabilities consisted of the following:

	As of September 30, 2023	As of December 31, 2022	As of March 31, 2024	As of December 31, 2023
	(in thousands)		*(in thousands)*	
Other current liabilities				
Lease liability	3,106	2,832	5,940	3,200
Total other current liabilities	$ 3,106	$ 2,832	$ 5,940	$ 3,200
Other non-current liabilities				
Lease liability	$ 17,919	$ 20,294	$ 41,995	$ 17,093
Other	1,000	1,001	1,001	1,001
Total other non-current liabilities	$ 18,919	$ 21,295	$ 42,996	$ 18,094

8. Significant agreements

The Company's significant agreements are described in Note 9 of the December 31, 2022 consolidated financial statements included in its Annual Report on Form 10-K for the year ended December 31, 2022. During the nine months ended September 30, 2023 and 2022, there were no material changes to the Company's collaboration agreement with Neurocrine executed in March 2019 (the "2019 Neurocrine Collaboration Agreement") and the option and license agreement executed with Pfizer in October 2021 (the "Pfizer Agreement") other than the assignment of the Pfizer Agreement to Alexion. Accordingly, there were no changes to the Company's accounting treatment for these agreements through September 30, 2023.

The Company recorded revenue of $1.5 million and $1.1 million under the 2019 Neurocrine Collaboration Agreement during the three months ended September 30, 2023 and 2022, respectively. The Company recorded revenue of $5.2 million and $2.5 million under the 2019 Neurocrine Collaboration Agreement during the nine months ended

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September 30, 2023 and 2022, respectively. The Company did not recognize any collaboration revenue related to the Alexion Agreement during the three or nine months ended September 30, 2023 or 2022.

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 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 workplans, 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

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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, in February 2023 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. 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

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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, 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, 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 Topic 606 ("ASC 606") and that modification accounting was not required given that the 2023 Neurocrine Collaboration Agreement did not represent a legally enforceable change in 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 Program.

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 that these amounts do not require a constraint. As of September 30, 2023, the estimate of the expected reimbursement was $13.3 million of costs incurred based on expectations as of such date. The additional consideration to be paid to the Company upon reaching certain milestones is excluded from the transaction price at inception due to the uncertainty of the payment and the uncertainty of achieving the development and regulatory milestones.

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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 all of the 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 September 30, 2023:

Performance Obligation	Amount
	(in thousands)
Variable Consideration	
GBA1 Program	$ 6,189
2023 Discovery Program 1	3,417
2023 Discovery Program 2	2,354
2023 Discovery Program 3	1,292
Total	$ 13,252

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 three months ended March 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 three months ended September 30, 2023, the Company recognized $2.0 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 three months ended September 30, 2023, the Company recognized $1.1 million of revenue associated with the fixed transaction price allocated to the three 2023 Discovery Programs, and with research and development services performed during the period and the corresponding cost reimbursement receivable for the three 2023 Discovery Programs. During the nine months ended September 30, 2023, the Company recognized $4.2 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 nine months ended September 30, 2023, the Company recognized $1.8 million of revenue associated with the fixed transaction price allocated to the three 2023 Discovery Programs, and with research and development services performed during the period and the corresponding cost reimbursement receivable for the three 2023 Discovery Programs. As of September 30, 2023, there was $72.8 million of deferred revenue related to the 2023 Neurocrine Collaboration Agreement, of which $37.7 million was classified as current and $35.1 million was classified as non-current in the accompanying balance sheets based on the period the services are expected to be delivered.

The following table presents changes in the balances of the Company's related party collaboration receivables and contract liabilities under the 2023 Neurocrine Collaboration Agreement during the nine months ended September 30, 2023:

	Balance at December 31, 2022	Additions	Deductions	Balance at September 30, 2023
		(*in thousands*)		
Related party collaboration receivable	$ -	$ 4,364	$ (2,445)	$ 1,919
Contract liabilities:				
Deferred revenue	$ -	$ 74,420	$ (1,643)	$ 72,777

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. In conjunction with the recognition of collaboration revenue during the nine months ended September 30, 2023, approximately $0.2 million of costs to obtain the 2023 Neurocrine Collaboration Agreement were expensed.

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 with 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 central nervous system ("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.

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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

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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 three and nine months ended September 30, 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 three and nine months ended September 30, 2023.

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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 “Novartis Agreement”). Pursuant to the Novartis Agreement, the Company has 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 has 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.

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 Novartis 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, 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 applicable 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.

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Under the terms of the 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 novel capsids generated from the Company's TRACER Capsid discovery platform for use in gene therapy programs against two undisclosed Initial Novartis Targets. With Novartis' option exercise on two Initial Novartis Targets, the Company received a $25.0 million option exercise payment in April 2023, and is 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 the Company's internal 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, the Company 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 Novartis Agreement prior to the expiration of the applicable Novartis License Option. As a result, the non-exclusive research license that the Company granted to Novartis in connection with this Initial Novartis Target has terminated, the Novartis Research Term for this Initial Novartis Target has expired, and the Company is 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 the Company.

Under the terms of the 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 Novartis Agreement and in the course of the parties' activities under the 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' exercise of Novartis License Options, 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 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 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 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 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 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 Novartis Agreement was a contract with a customer under ASC 606. The Company assessed the promised goods and services and determined that the 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

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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 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 three months ended March 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 of $25.0 million. This amount was received by the Company during the second quarter of 2023.

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 include 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

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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 three months ended March 31, 2023, the Company recorded a $1.0 million fee to research and development expense per the terms of the Touchlight License Agreement in conjunction with Novartis' exercise of its Novartis License Options to license novel capsids generated from the Company's TRACER Capsid discovery platform for use in gene therapy programs against two undisclosed Initial Novartis Targets. This amount was paid to Touchlight during the second quarter of 2023.

Other Licensing Agreements

On June 28, 2023, the Company and Sangamo Therapeutics, Inc. ("Sangamo") entered into a definitive license agreement for a potential treatment of prion disease. Using its proprietary epigenetic regulation platform, Sangamo has developed zinc finger transcriptional regulators which it believes 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 the Company'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. The Company has evaluated this license agreement under ASC 606 and determined that this agreement is not material to the financial statements, and all variable consideration is fully constrained.

Other Agreements

During the year ended December 31, 2016,

In 2016, the Company entered into a research and development funding arrangement with a non-profit organization that provides up to $4.0 million in funding to the Company upon the achievement of clinical and development milestones. The agreement provides that the Company repay amounts received under certain circumstances including termination of the agreement, and to pay an amount up to 2.6 times the funding received upon successful development and commercialization of any products developed. During the year ended December 31, 2017, In 2017, the Company earned a milestone payment of $1.0 million. The Company evaluated the arrangement and concluded that it represents a research and development financing arrangement as it is probable that the Company will repay amounts received under the arrangement. As a result,

the $1.0 million for the year ended December 31, 2017 is recorded as a non-current liability in the condensed consolidated balance sheet.

Litigation

The Company was not a party to any material legal matters or claims as of September 30, 2023 March 31, 2024, or December 31, 2022 December 31, 2023. The Company did not have contingency reserves established for any litigation liabilities as of September 30, 2023 March 31, 2024, or December 31, 2022 December 31, 2023.

8. Significant agreements

The Company's significant agreements are described in Note 9 of the December 31, 2023 consolidated financial statements included in its Annual Report on Form 10-K for the year ended December 31, 2023. During the three months ended March 31, 2024, there were no material changes to the Company's collaboration agreements or option and license agreements and no new collaboration or license agreements. The Company recorded collaboration revenue of $19.5 million and $150.5 million during the three months ended March 31, 2024 and 2023, respectively.

2023 Neurocrine Collaboration Agreement

In the three months ended March 31, 2024, the Company revised its estimate of research services expected to be performed under the collaboration and license agreement with Neurocrine entered into in January 2023 (the "2023 Neurocrine Collaboration Agreement"). The change in estimate resulted in additional revenue recognized of approximately $7.3 million in the three months ended March 31, 2024.

2023 Novartis Stock Purchase Agreement

Under the stock purchase agreement entered into in December 2023 (the "2023 Novartis Stock Purchase Agreement"), Novartis purchased 2,145,002 shares of common stock of the Company (the "Novartis Shares") for an

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aggregate purchase price of approximately $20.0 million.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 was allocated to the development and commercialization licenses granted to Novartis for two programs pursuant to the license and collaboration agreement with Novartis entered into in December 2023 and was recognized as collaboration revenue during the first quarter of 2024, upon the issuance of the Novartis Shares under the 2023 Novartis Stock Purchase Agreement.

2019 Neurocrine Collaboration Agreement

In February 2024, the Company announced that the joint steering committee with Neurocine selected a lead development candidate for the gene therapy program for Friedreich's ataxia (the "FA Program") under the collaboration and license agreement with Neurocrine entered into in January 2019 (the "2019 Neurocrine Collaboration Agreement"), which

triggered a $5.0 million milestone payment to the Company that was received in the first quarter of 2024. The Company included the $5.0 million that had previously been constrained in the transaction price allocated to the FA performance obligation in the three months ended March 31, 2024, accordingly, which resulted in a cumulative catch-up adjustment to collaboration revenue of $4.4 million.

Related Party Collaboration Receivable

The following table presents changes in the balances of the Company's related party collaboration receivable and contract liabilities for the 2023 Neurocrine Collaboration Agreement and the 2019 Neurocrine Collaboration Agreement during the three months ended March 31, 2024:

	Balance at December 31, 2023	Additions	Deductions	Balance at March 31, 2024
		(in thousands)		
Related party collaboration receivables	$ 3,341	$ 7,340	$ (8,061)	$ 2,620
Contract liabilities:				
Deferred revenue	$ 75,240	$ 586	$ (11,230)	$ 64,596

The change in the related party collaboration receivable balance for the three months ended March 31, 2024 is primarily driven by amounts owed to the Company for research and development services provided, offset by amounts collected during the period, for the 2023 and 2019 Neurocrine Collaboration Agreements. Deferred revenue activity for the period includes the recording of $0.6 million of deferred revenue during the first quarter of 2024 related to the fixed transaction price allocation increase for the FA Program, offset by $11.2 million of collaboration revenue recognized on the proportional performance model during the period for the 2023 and 2019 Neurocrine Collaboration Agreements, 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.

9. Stock-based compensation

Stock-Based Compensation Expense

Total compensation cost recognized for all stock-based compensation awards in the condensed consolidated statements of operations and comprehensive (loss) income was as follows:

	Three Months Ended September 30,		Nine Months Ended September 30,		Three Months Ended March 31,	
	2023	2022	2023	2022	2024	2023
	(in thousands)					
					(in thousands)	
Research and development	$ 919	$ 588	$ 2,472	$2,170	$1,280	$ 863
General and administrative	1,959	1,601	5,636	4,828	2,293	1,695
Total stock-based compensation expense	$ 2,878	$ 2,189	$ 8,108	$6,998	$3,573	$2,558

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Stock-based compensation expense by type of award included within the condensed consolidated statements of operations and comprehensive (loss) income was as follows:

	Three Months Ended September 30,		Nine Months Ended September 30,		Three Months Ended March 31,	
	2023	2022	2023	2022	2024	2023
	(in thousands)					
						(in thousands)
Stock options	$ 2,021	$ 1,357	$ 5,587	$4,452	$2,408	$1,663
Restricted stock awards and units	774	748	2,339	2,382	1,090	841
Employee stock purchase plan awards	83	84	182	164	75	54
Total stock-based compensation expense	$ 2,878	$ 2,189	$ 8,108	$6,998	$3,573	$2,558

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 nine three months ended September 30, 2023 March 31, 2024 was as follows:

	Units	Weighted Average Grant Date Fair Value Per Unit	Units	Weighted Average Grant Date Fair Value Per Unit
Unvested restricted stock units as of December 31, 2022	1,112,563	$ 5.27		
Unvested restricted stock units as of December 31, 2023			1,370,897	$ 6.65
Granted	725,550	$ 7.52	696,908	$ 7.63
Vested	(459,745)	$ 5.68	(324,520)	$ 5.75
Forfeited	(65,641)	$ 4.65	(23,947)	$ 6.42
Unvested restricted stock units as of September 30, 2023	1,312,727	$ 6.40		
Unvested restricted stock units as of March 31, 2024			1,719,338	$ 7.22

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. Restricted stock units granted by the Company typically vest in equal amounts, annually over three years. All of the restricted stock units granted in the nine three months ended September 30, 2023 March 31, 2024 vest in equal amounts, annually over three years. The stock-based compensation expense related to restricted stock units and awards was $0.8 million $1.1 million and $2.3 million $0.8 million for the three and nine months ended September 30, 2023, respectively. The stock-based compensation expense related to restricted stock units was $0.7 million March 31, 2024 and $2.4 million for the three and nine months ended September 30, 2022, 2023, respectively.

As of September 30, 2023 March 31, 2024, the Company had unrecognized stock-based compensation expense related to its unvested restricted stock units of $6.0 million $10.9 million, which is expected to be recognized over the remaining average vesting period of 2.3 2.4 years.

Stock Options

The following is a summary of stock option activity for the nine three months ended September 30, 2023 March 31, 2024:

	Shares	Weighted Average Exercise Price	Remaining Contractual Life *(in years)*	Aggregate Intrinsic Value *(in thousands)*	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	7.9	$ 6,095				
Outstanding at December 31, 2023					7,425,444	$ 8.52		
Granted	2,250,400	$ 8.64			1,747,626	$ 7.68		
Exercised	(377,593)	$ 5.02			(32,500)	$ 4.91		
Cancelled or forfeited	(326,785)	$ 9.60			(230,974)	$ 12.28		
Outstanding at September 30, 2023	7,745,593	$ 8.42	7.9	$ 9,348				
Exercisable at September 30, 2023	3,527,536	$ 9.66	6.7	$ 4,355				
Outstanding at March 31, 2024					8,909,596	$ 8.27	7.8	$ 17,875
Exercisable at March 31, 2024					3,880,357	$ 9.04	6.2	$ 8,728

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As of September 30, 2023 March 31, 2024, the Company had unrecognized stock-based compensation expense related to its unvested stock options of $19.1 million $23.2 million which is expected to be recognized over the remaining weighted-average vesting period of 2.9 3.0 years.

10. Net (loss) income per share

The following table sets forth the outstanding potentially dilutive securities that have been excluded in the calculation of diluted net (loss) income per share because to include them would be anti-dilutive:

	Three Months Ended September 30,		Nine Months Ended September 30		As of March 31,	
	2023	2022	2023	2022	2024	2023
Unvested restricted common stock awards	22,500	64,608	22,500	64,608	22,500	45,000
Unvested restricted common stock units	1,312,727	523,627	714,164	941,275	1,719,338	1,336,159
Outstanding stock options	7,745,593	5,433,867	6,695,548	6,079,071	8,909,596	7,360,745
Total	9,080,820	6,022,102	7,432,212	7,084,954	10,651,434	8,741,904

Basic net (loss) income and diluted weighted-average shares outstanding are as follows for the three and nine months ended September 30, 2023 March 31, 2024 and 2022: 2023:

	Three Months Ended September 30		Nine Months Ended September 30		Three Months Ended March	
	2023	2022	2023	2022	2024	2023
Numerator:						
Net (loss) income *(in thousands)*	$ (25,901)	$ 17,624	$ 75,935	$ (22,782)	$ (11,330)	$ 124,(
Denominator for basic net (loss) income per share:						
Weighted average shares outstanding-basic	43,864,838	38,507,542	40,962,116	38,292,497	57,117,046	40,632,(
Denominator for diluted net (loss) income per share:						
Weighted average shares outstanding-basic	43,864,838	38,507,542	40,962,116	38,292,497	57,117,046	40,632,(
Common stock options and restricted stock units	—	1,062,852	1,648,608	—	—	1,529,2
Weighted average shares outstanding-diluted	43,864,838	39,570,394	42,610,724	38,292,497	57,117,046	42,161,3
Net (loss) income per share, basic:					$ (0.20)	$ 3
Net (loss) income per share, diluted:					$ (0.20)	$ 2

The pre-funded warrants issued in connection with the underwritten public offering discussed in Note 11 are included in basic and diluted weighted average shares outstanding for the three months ended March 31, 2024.

11. Underwritten public offering

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 Underwriters 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 and expired on February 2, 2024.

On January 9, 2024, the Company issued 7,777,778 shares of common stock and 3,333,333 Pre-Funded Warrants for net proceeds of approximately $93.5 million after deducting underwriting discounts and commissions and

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offering expenses pursuant to the underwritten public offering. The Pre-Funded Warrants met the equity classification guidance and therefore are classified as stockholders' equity.

12. Related-party transactions

During the nine three months ended September 30, 2023 March 31, 2024, the Company received scientific advisory board and other 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 three and nine months ended September 30, 2023, March 31, 2024 and 2023, was $157,500 $150,000 and $541,300, $199,800, respectively. The total amount of fees paid to Dr. Sah for services provided during the three and nine months ended September 30, 2022 was $93,600 and $185,925, respectively. During the second quarter of 2023, the Company and Dr. Sah agreed to a fee of $50,000 per month for advisory services from Dr. Sah. This agreement became effective in June 2023.

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 nine months ended September 30, 2022.

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Under each of the Company's collaboration agreements with Neurocrine, the Company and Neurocrine have agreed to conduct research, development, and commercialization activities for certain of the Company's AAV gene therapy product candidates. Amounts due from Neurocrine are reflected as related party collaboration receivables. As of September 30, 2023 March 31, 2024, the Company had approximately $1.3 million $1.2 million and $1.4 million in related party collaboration receivables associated with the 2019 Neurocrine Collaboration Agreement. As of September 30, 2023, Agreement and 2023 Neurocrine Collaboration Agreement, respectively.

13. Subsequent Events

In April 2024, the Company had approximately $1.9 million in related party collaboration receivables associated announced that the joint steering committee with Neurocrine selected a development candidate for the glucocerebrosidase 1 gene therapy program for Parkinson's disease and other GBA1-mediated diseases under the 2023 Neurocrine Collaboration Agreement. Agreement (the "GBA1 program"). The joint steering committee selection of a development candidate for the GBA1 Program triggered a $3.0 million milestone payment to the Company. The Company expects to receive the $3.0 million during the second quarter of 2024.

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ITEM 2. MANAGEMENT'S DISCUSSION AND ANALYSIS OF FINANCIAL CONDITION AND RESULTS OF OPERATIONS

The following discussion and analysis of our financial condition and results of operations should be read in conjunction with our unaudited condensed consolidated financial statements and related notes appearing elsewhere in this Quarterly Report on Form 10-Q and the audited financial information and the notes thereto included in our Annual Report on Form 10-K for the year ended December 31, 2022 December 31, 2023, which was filed with the Securities and Exchange Commission, or the SEC, on March 7, 2023 February 28, 2024.

Our actual results and timing of certain events may differ materially from the results discussed, projected, anticipated, or indicated in any forward-looking statements. We caution you that forward-looking statements are not guarantees of future performance and that our actual results of operations, financial condition and liquidity, and the development of the industry in which we operate may differ materially from the forward-looking statements contained in this Quarterly Report on Form 10-Q. In addition, even if our results of operations, financial condition and liquidity, and the development of the industry in which we operate are consistent with the forward-looking statements contained in this Quarterly Report on Form 10-Q, they may not be predictive of results or developments in future periods.

The following information and any forward-looking statements should be considered in light of factors discussed in Part I, Item 1A,"Risk Factors" of our Annual Report on Form 10-K for the year ended December 31, 2022 December 31, 2023, and, if applicable, those included under Part II, Item 1A of our Quarterly Reports on Form 10-Q., 10-Q, that could cause actual future results or events to differ materially from the forward-looking statements that we make. Additional risk factors may be identified from time to time in our future filings with the SEC.

These forward-looking statements are made under the safe harbor provisions of Section 27A of the Securities Act of 1933, as amended, or the Securities Act, and Section 21E of the Securities Exchange Act of 1934, as amended, or the Exchange Act. These statements are neither promises nor guarantees. We caution readers not to place undue reliance on any forward-looking statements made by us, which speak only as of the date they are made. We disclaim any obligation, except as specifically required by law and the rules of the SEC, to publicly update or revise any such statements to reflect any change in our expectations or in events, conditions or circumstances on which any such statements may be based, or that may affect the likelihood that actual results will differ from those set forth in the forward-looking statements.

Overview

We are a biotechnology company dedicated whose mission is to breaking through barriers in gene therapy leverage the power of human genetics to modify the course of and neurology. 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, or CNS. 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; and Neurocrine Biosciences, Inc., or Neurocrine.

We focus on leveraging our expertise in capsid discovery and neuropharmacology to address the delivery hurdles that have constrained the gene therapy 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. Our gene therapy platforms enable us to engineer, optimize, manufacture and deliver our adeno-associated virus, or AAV, based gene therapies that we believe have the potential to safely provide durable efficacy. Our team of experts in the fields of AAV gene therapy and neuroscience first identifies and selects diseases in which we believe an AAV gene therapy or other biological therapy will answer a high unmet medical need, be supported by target validation, offer an efficient path to human proof of biology, present robust preclinical pharmacology, and offer strong commercial potential. We then engineer and optimize an AAV vector or other biological therapy for activity in, efficacy in, or delivery to, the targeted tissue or cells.

We are identifying proprietary AAV capsids, the outer viral protein shells that enclose genetic material that makes up the vector payload. Our team has developed a proprietary AAV capsid discovery platform called TRACER™ (Tropism Redirection of AAV by Cell Type-Specific Expression of RNA) that employs directed evolution to facilitate

the selection of AAV capsids with enhanced tissue delivery characteristics, such as more effective delivery across the blood-brain barrier, or BBB. 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 non-human primates. We believe that capsids we discover through our TRACER discovery platform, which we refer to as TRACER Capsids, have the potential to significantly enhance the efficacy and safety of our single dose gene therapies, 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 central nervous system, or 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 species. We have identified receptors for some of our TRACER Capsid families as well as a ligand 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.

In addition to leveraging TRACER Capsids in potential licensing arrangements, we are advancing our own proprietary pipeline of drug candidates for neurological diseases, with a focus on Alzheimer's disease, or AD. Our wholly-owned prioritized pipeline programs include an anti-tau antibody for AD; a superoxide dismutase 1, or SOD1, silencing gene therapy for amyotrophic lateral sclerosis, or ALS, ALS; and an anti-tau antibody a tau silencing gene therapy for AD. We have identified a lead development candidate for our anti-tau antibody program in the first quarter of 2023, initiated good laboratory practices, or GLP, toxicology studies in the third quarter of 2023, and which we expect refer to submit as VY-TAU01. We submitted an investigational new drug, application, or IND, application to the U.S. Food and Drug Administration, or the FDA, for this program VY-TAU01 in March 2024 and we have obtained clearance of the IND. We expect to dose the first subject in a planned Phase 1a single ascending dose, or SAD, trial of VY-TAU01 in healthy volunteers in the first half of 2024. coming weeks. We continue to evaluate the data from preclinical studies for our SOD1 program and also expect to identify initiate a

Phase 1b multiple ascending dose, or MAD, trial of VY-TAU01 in patients with early AD in 2025, which has the potential to generate initial 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 2023. We the fourth quarter of 2023, which we refer to as VY9323, and we expect to submit the IND application for our SOD1 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 four an early research initiatives initiative to develop a gene therapies therapy for the treatment of AD, Huntington's disease, 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 brain metastases from HER2+ metastatic breast cancer. In addition to these wholly-owned programs, 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 in collaboration with Neurocrine: programs: a glucocerebrosidase 1, or GBA1, gene

therapy program for Parkinson's disease and other GBA1-mediated diseases, or the GBA1 Program, and a frataxin, or FXN, gene therapy program for Friedreich's ataxia.

AAV Gene Therapy

Gene therapy is an approach whereby gene expression is directly altered in patients to address the underlying cause or predominant manifestations of disease. We believe that the targeted nature of gene therapy may enable powerful treatment options and provide these patients with meaningful and durable benefits.

While AAV gene therapy can potentially be harnessed for multiple treatment methods, we are currently focused on gene replacement, gene knockdown and vectorized antibody approaches. Gene replacement is intended to restore the expression of a protein that is not expressed, expressed at abnormally low levels or functionally mutated with loss of function. Gene knockdown, or gene silencing, is intended to reduce the expression of a pathologically mutated RNA or protein that has detrimental effects. Vectorizing an antibody for delivery using AAV has the ability to increase exposure of large antibodies in brain parenchyma and interstitial fluid that otherwise show minimal penetration across the BBB when administered passively. Our gene therapy approach uses AAV vectors which we believe are ideal vectors for gene therapy for several reasons:

- ***Broad Applicability.*** AAV is able to transduce, or transfer a therapeutic gene, into numerous cell types including target cells in the CNS, cardiac, and other tissues.
- ***Safety.*** We do not believe AAV is known to cause any disease in humans.
- ***Does Not Readily Integrate.*** AAV does not readily integrate into the genome of the target cell, an attribute which we believe reduces the potential for oncogenesis, or the induction of cancer.
- ***Scalability.*** AAV is able to be manufactured at commercial quality and scale.

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We believe that neurological diseases are well-suited for treatment with AAV gene therapy for the following reasons:

- ***Validated Targets.*** Many neurological, cardiac, and other diseases are caused by well-defined mutations in genes and these genes represent genetically validated drug targets for AAV gene therapy.
- ***Targeted Delivery.*** We believe our TRACER Capsids may allow for significantly enhanced gene therapy delivery to specific types of cells and tissues at lower doses.
- ***Durable Expression.*** Long-term gene expression may be achievable in the CNS and other tissues following one-time dosing and transfer of the therapeutic gene with an AAV vector. Because repeated or continual dosing with direct injection of drugs into the CNS and other tissues is complex, a one-time AAV gene therapy has significant advantages.

The Voyager Gene Therapy Platform

We have built a gene therapy platform that we believe positions us to be the leading company at the intersection of AAV gene therapy and neurological diseases. Our team of experts in the field of AAV gene therapy first identifies and selects diseases that are well-suited for treatment using AAV gene therapy. We then engineer and optimize AAV vectors, identifying a capsid for delivery of a payload, comprising a therapeutic gene, which we refer to as a transgene, and a promoter to drive expression of the transgene, to the targeted tissue or cells. Finally, we leverage established routes of administration and

advances in dosing techniques to optimize delivery of our AAV vectors to target cells that are critical to the disease of interest. We believe that optimizing each of these parameters is a key factor for overall program success. We expect that our current and future pipeline programs will make use of technological advances generated with our gene therapy platform.

Disease Selection

Following an internal review process, we have prioritized pipeline programs for our development. This review evaluated the opportunity presented by each prioritized program 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.

Vector Engineering and Optimization

The key components of an AAV vector include: (a) the capsid; (b) the 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.

TRACER Capsid Discovery

Our scientists have developed TRACER, a proprietary AAV capsid discovery platform to facilitate the selection of TRACER Capsids for particular therapeutic applications based on BBB-crossing and cell-specific transduction properties in multiple species, including non-human primates, or NHPs. We believe these TRACER Capsids may allow for significantly enhanced gene delivery to specific types of cells in the brain at lower doses and, potentially, with fewer safety and tolerability issues than first-generation therapies. These TRACER Capsids are now in advanced stages of characterization for deployment in our gene therapy development programs. We continue to perform screening campaigns with our TRACER discovery platform to identify additional proprietary AAV9- and AAV5-derived TRACER Capsids and to refine previously-identified TRACER Capsids to target or de-target multiple tissue and cell types. At the American Society of Gene & Cell Therapy 26th Annual Meeting in May 2023, or the ASGCT 2023

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Meeting, we presented data demonstrating greater than 50% cell transduction in multiple areas of the brain at a dose of $2x10^{12}$ vector genomes per kilogram following intravenous administration of our VCAP-102 TRACER Capsid in marmosets.

We are actively engaged in discussions to make TRACER Capsids available to third parties for use in their drug development programs through potential option and license and other arrangements. We believe there is significant opportunity for option and license transactions related to our TRACER Capsids. To maximize the potential of our TRACER Capsids for both our own programs and option and license transactions, we have retained to date, and expect to retain in the future, all rights associated with such TRACER Capsids other than the rights specific to their use in combination with the optionee's or licensee's transgenes or collaborators' programs.

Collaboration Agreements

2019 Neurocrine Collaboration

In January 2019, we entered into a collaboration with Neurocrine, or the 2019 Neurocrine Collaboration Agreement, for the research, development and commercialization of certain of our AAV gene therapy products, or the 2019 Collaboration 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 program for the treatment of Friedreich's ataxia, or the FA Program, including the development of the VY-FXN01 product candidate, and two undisclosed programs, or the 2019 Discovery Programs. In August 2021, the collaboration was terminated with respect Program. Pursuant to the VY-AADC Program. Under the FA Program, we and Neurocrine are currently developing a gene therapy for the treatment of Friedreich's ataxia, 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. Development of the two targets approved by the joint steering committee under the 2019 Discovery Programs is ongoing.

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 VY-AADC Program of up to $170.0 million, which such agreements, we are 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. We may be entitled to receive aggregate commercial milestone payments of up to $275.0 million, subject to an aggregate cap on commercial milestone payments across all 2019 Neurocrine Programs of $1.1 billion.

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 VY-AADC Program, from the mid-teens to thirty and the low-teens to twenty, respectively, which we are 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 (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.

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2023 Neurocrine Collaboration

On January 8, 2023, we entered into a second collaboration agreement, or the 2023 Neurocrine Collaboration Agreement, working with Neurocrine for the research, development, manufacture and commercialization of gene therapy products directed to the gene that encodes glucosylceramidase beta 1, or GBA1, for the treatment of Parkinson's disease and other diseases associated with GBA1, or the GBA1 Program, and three new on five early-stage programs focused on for 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.

Under the terms of the 2023 Neurocrine Collaboration Agreement, in February 2023 Neurocrine paid us an upfront payment of approximately $136.0 million and approximately $39.0 million for the purchase of 4,395,588 shares of common stock at a price of $8.88 per share. The 2023 Collaboration Agreement targets. We have also provides for aggregate development milestone payments from Neurocrine for gene therapy products arising under the 2023 Neurocrine Programs, or the 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.

The 2023 Neurocrine Collaboration Agreement became effective on February 21, 2023. On February 23, 2023, we received the upfront payment, and the shares of our commons stock were issued and sold to Neurocrine pursuant to the applicable stock purchase agreement.

License Agreements

In October 2021, we entered into an option agreements with licensees including Novartis and Alexion to license agreement with Pfizer, or the Pfizer Agreement, pursuant to which we granted Pfizer provide options to receive an exclusive license, or the Pfizer License Options, licenses to certain 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. Capsids. In connection with the exercise of the Pfizer License Option for a rare neurological disease, December 2023, 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.

Under the terms of the Alexion Agreement, Pfizer has paid us an upfront payment of $30 million and a payment of $10 million in connection with the exercise of the Pfizer License Option, which we also refer to as the Alexion License Option, for a rare neurological disease during the fourth quarter of 2022. We are also eligible to receive specified development, regulatory, and commercialization milestone payments of up to an aggregate of $115 million for the first Pfizer Licensed CNS Product, which we also refer to as an Alexion Licensed CNS Product, to achieve the applicable 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 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

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limits. For a further description of the Alexion Agreement, refer to Note 8, *Significant agreements*, to our unaudited condensed consolidated financial statements included elsewhere in this Quarterly Report on Form 10-Q under the caption "—Alexion Option and License Agreement (Formerly Pfizer Option and License Agreement)."

In March 2022, we entered into an option and license agreement, or the Novartis Agreement, with our collaborative partner Novartis. Pursuant to the Novartis Agreement, we have 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 certain adeno-associated virus gene therapy candidates comprised of a Novartis Licensed Capsid and a payload directed to such target. Effective as of March 1, 2023, Novartis exercised its Novartis License Options to license novel capsids generated from our TRACER Capsid discovery platform for use in gene therapy programs against two undisclosed targets. Novartis elected not to license a capsid for a third target under the Novartis Agreement prior to the expiration of the applicable Novartis License Option. All capsid rights with respect to that target have reverted to us. For a further description of the Novartis Agreement, refer to Note 8, *Significant agreements*, to our unaudited condensed consolidated financial statements included elsewhere in this Quarterly Report on Form 10-Q under the caption "—Novartis Option and License Agreement."

In November 2022, we and Touchlight IP Limited, or Touchlight, entered into a license and collaboration agreement or with Novartis to provide Novartis certain rights regarding the Touchlight License Agreement, development of potential gene therapy product candidates for the treatment of spinal muscular atrophy and to authorize historical use by us 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 in February 2024, and we and Neurocrine expect the FA Program to advance into first-in-human clinical trials in 2025. The joint steering committee's selection of a certain DNA preparation process, or development candidate for the Subject DNA Preparation Process, FA Program triggered a $5.0 million milestone payment to us, which we received in March 2024. The joint steering committee with Neurocrine also selected a development candidate for the GBA1 Program in April 2024, and we and Neurocrine expect to authorize the prospective exploitation of TRACER Capsids created file an IND application with the use FDA for the GBA1 Program in 2025. The joint steering committee's selection of a development candidate for the GBA1 Program triggered a $3.0 million milestone payment to us, which we expect to receive during the second quarter of 2024.

All of the Subject DNA Preparation Process. 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 CNS tropism in multiple species, including non-human primates, or NHPs.

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 therapies therapy and other genetic medicines medicine

programs for the treatment of neurological diseases which we believe diseases. We have prioritized pipeline programs for our development based on the following criteria: high unmet medical need. 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, or gene silencing or vectorized antibody approach, and antibodies that will use a passive administration or vectorized delivery approach. Our goal is to address the underlying causes or the predominant manifestations

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Table of specific diseases by significantly increasing or decreasing expression of the relevant proteins in targeted tissues. Contents

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

Graphic

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Wholly-Owned Programs

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

Disease Overview

We are developing proprietary antibodies that selectively target and reduce the spread of pathological tau for the treatment of tauopathies, and our lead indication AD is AD. The spread of tau pathology closely correlates with a progressive neurodegenerative disease progression and cognitive decline in AD, which affects approximately 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 a growing health care burden to society. Recently, anti-amyloid antibodies have been approved for treatment of AD, and there is substantial remaining unmet medical need. 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 A reduction of toxic tau aggregates may slow disease progression and cognitive decline in these diseases. We are exploring passive administration of selected VY-TAU01 as our lead humanized anti-tau antibody. Our anti-tau antibodies have differentiated properties including improved targeting of specific regions of tau protein that could offer an improved profile compared antibody candidate to first-generation approaches. advance against AD. We believe that our antibody targeting the C-terminus VY-TAU01 is highly differentiated from other approaches. Further, we believe anti-tau antibodies based on the epitope, or the part of a foreign protein or antigen that following is capable of generating an immune response, it targets: VY-TAU01 targets an epitope which is located in the clearance C-terminal, rather than the N-terminal, mid-domain, or microtubule binding region of an IND application, clinical assessments utilizing positron emission tomography (PET) imaging of human the tau together with measuring plasma and cerebrospinal fluid biomarkers, have the potential to enable an efficient and accelerated demonstration of human proof-of-biology. 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, As previously reported, our C-terminal targeting anti-tau antibody blocked the seeding/propagation of filamentous tau and demonstrated substantial reduction of induced tau pathology. pathology in a mouse model. In March 2023, we presented new 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.

Program Status

In January 2023, March 2024, we selected a lead humanized anti-tau antibody candidate to advance against AD. The lead candidate, VY-TAU01, targets presented data at the C-terminal domain. AD/PD 2024 Conference demonstrating VY-TAU01 was selected for well-tolerated, and its affinity, selectivity, and biophysical characteristics. In April 2023, we received pre-IND written feedback from the FDA for VY-TAU01. Process development and manufacturing at a contracted manufacturer have been initiated, and we initiated a GLP toxicology study serum pharmacokinetic profile was as expected in the third quarter of 2023 to enable an IND submission in the first half of 2024.

Early Research Programs for the Treatment of AD

During the first quarter of 2023, we announced an early research initiative investigating a gene therapy targeting tau for the treatment of AD. The program combines an siRNA tau knockdown payload with an intravenously delivered TRACER Capsid.

In August 2023, we announced a separate 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. NHP study.

Program Status

In January 2023, we selected VY-TAU01 as our lead humanized anti-tau antibody candidate to advance against AD. We submitted an IND application for VY-TAU01 to the FDA in March 2024, and we have obtained clearance of the IND. We expect to dose the first subject in a planned Phase 1a SAD trial in healthy volunteers in the coming weeks. A Phase 1b MAD trial in subjects with early AD is expected to be initiated in 2025. The MAD trial has the potential to generate initial data for slowing the spread of pathological tau via tau PET imaging in 2026.

SOD1 Silencing Gene Silencing Therapy Program for the Treatment of ALS (VY9323)

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 three two to five years of diagnosis and impacts symptom onset. The disease is estimated to affect approximately 800 patients 20,000 people in the United States, 1,000 patients States. Multiple genes have been implicated in ALS; mutations in the European Union, and 500 patients SOD1 gene are estimated to occur in Japan. 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 small interfering RNA, or siRNA construct via intravenous administration of an AAV gene therapy may enable broad CNS knockdown of SOD1. This SOD1, which 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 could 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. We continue to evaluate the data from preclinical studies for this program and expect to identify a In December 2023, we selected VY9323 as our lead development candidate in 2023. for our SOD1 program. We expect plan to submit an IND application to the FDA in mid-2025 for VY9323 and to initiate a Phase 1 clinical trial of VY9323 in subjects with SOD1 ALS for the program as soon as possible thereafter. We expect to evaluate the safety and biological activity of VY9323 in this Phase 1 trial.

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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. A 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 presented 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 on 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 mid-2025. 2026.

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

In January August 2023, we announced the launch of an updated early research initiative investigating a gene therapy targeting anti-amyloid for the treatment of Huntington's disease. AD. The updated gene therapy program which leverages the latest insights in disease biology, combines an intravenous TRACER Capsid with vectorized siRNAs to enable specific knockdown of mHTT and MSH3.Early data on the selection and vectorization of siRNAs targeting mHTT were presented at the 18th Annual Huntington's Disease Therapeutics Conference held in Dubrovnik, Croatia, in April 2023.

Our wholly-owned early research programs also include a program exploring a vectorized anti-amyloid antibody against HER2 for the treatment of brain metastases from HER2+ metastatic breast cancer. Pre-clinical data has demonstrated that our vectorized antibody against HER2 inhibits proliferation and promote antibody-dependent cell cytotoxicity, a process that recruits natural killer cells, macrophages and/or brain-resident innate immune cells called microglia to eliminate tumor cells. 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 4,000 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

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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 we and

Neurocrine expect to advance the FA Program into first-in-human clinical trials in 2025. The selection of a lead development candidate triggered a $5.0 million milestone payment to us, which we received in March 2024.

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

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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, impacting 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

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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 a non-human primate 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. GBA1, or the GBA1 Program. The GBA1 Program is currently in preclinical development. We In April 2024, the joint steering committee with Neurocrine selected a development candidate for the GBA1 Program 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 expect to file an IND application with the FDA for the GBA1 Program in 2025. Selection of the development candidate for this program, triggered a $3.0 million milestone payment, which we plan expect to complete IND enabling studies to evaluate its safety and efficacy. receive in the second quarter of 2024.

Friedreich's Ataxia Program: VY-FXN01 (2019 Neurocrine Collaboration) HD Program (2023 Novartis Collaboration Agreement)

Disease Overview

Friedreich's ataxia

Huntington's disease is a debilitating fatal, inherited 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 that results in the United States. While one treatment for Friedreich's ataxia has recently been approved progressive decline of motor and cognitive functions and a range of behavioral and psychiatric disturbances. Huntington's disease is caused 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 huntingtin, or HTT, gene. Huntington's disease is an autosomal recessive dominant disorder, meaning which means that a person must obtain a defective copy an individual is at risk of inheriting the disease if only one parent is affected. While the exact function of the FXN HTT gene from both parents in order 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, or the 2023 Novartis Collaboration Agreement Effective Date, 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 condition. One healthy copy 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 FXN Novartis HD Program products and product candidates.

Collaboration Programs and Licensing Agreements

2023 Novartis Collaboration Agreement

On the 2023 Novartis Collaboration Agreement Effective Date, as described above 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 or 50% of normal frataxin protein levels, is sufficient therapy products and

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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 prevent the disease phenotype. We therefore believe that restoring FXN protein levels to at least 50% of normal levels by develop AAV gene therapy might lead products and product candidates under the Novartis HD Program, in each case, leveraging TRACER Capsids and other intellectual property controlled by us.

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. For a successful therapy. further description of the 2023 Novartis Collaboration Agreement, refer to Note 9, *Significant Agreements*, to our consolidated financial statements included in our Annual Report on Form 10-K for the year ended December 31, 2023 under the caption “2023 Novartis Collaboration Agreement.”

*Our Treatment Approach****2023 Novartis Stock Purchase Agreement***

We are seeking 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 Novartis 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. 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.

2022 Novartis Option and License 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 an and commercialize AAV gene therapy approach candidates comprised of Novartis Licensed Capsids and payloads directed to such targets, or the Novartis Payloads.

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 programs targeting specified genes, or the 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 products containing the corresponding Novartis Payload, or the Novartis Licensed Products, incorporating the Novartis Licensed Capsids.

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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 other targets, or the 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 believe will deliver granted to Novartis in connection with this Initial Novartis Target has terminated, the 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. For a functional version further description of the FXN gene 2022 Novartis Option and License Agreement, refer to Note 9, *Significant Agreements*, to our consolidated financial statements included in our Annual Report on Form 10-K for the sensory pathways through intravenous injection. We think this approach has year ended December 31, 2023 under the potential to improve balance, ability to walk, sensory capability, coordination, strength caption "2022 Novartis Option and functional capacity License Agreement."

2023 Neurocrine Collaboration Agreement

In January 2023, we entered into a collaboration agreement, or the 2023 Neurocrine Collaboration Agreement, with Neurocrine for the research, development, manufacture and commercialization of Friedreich's ataxia patients. Most Friedreich's ataxia patients produce low levels certain 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 our AAV gene therapy will trigger a harmful immune response. products. Under the 2023 Neurocrine Collaboration Agreement, we agreed to collaborate on the conduct of four collaboration programs, which we refer to collectively as the 2023 Neurocrine Programs: 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.

*Preclinical Studies*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 Neurocrine Collaboration Agreement provides for aggregate development milestone payments from Neurocrine to us for the research, development, manufacture, and commercialization of gene therapy products, or the 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.

We initially conducted preclinical studies 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 non-human primates and achieved high FXN expression levels within outside the target sensory ganglia, or clusters of neurons, along United States, range from (a) for the spinal region following intrathecal injection. More recently, we conducted preclinical studies in non-human primates with intravenous injection and achieved target FXN expression levels within sensory ganglia GBA1 Program, the low double-digits to twenty and the heart. The levels 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 FXN expression observed a 2023 Collaboration Product in such country and terminate upon 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 latest of (x) the cerebellar dentate nucleus, another area expiration, invalidation or the abandonment of the CNS that is often affected last patent covering the composition of the 2023 Collaboration Product or its approved method of use in Friedreich's ataxia, such country, (y) ten years from the first commercial sale of the 2023 Collaboration Product in such country and that is often considered difficult (z) the expiration of regulatory exclusivity in such country, or the 2023 Royalty Term. Royalty payments may be reduced by up to target therapeutically. 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.

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The 2023 Neurocrine Collaboration Agreement became effective on February 21, 2023. On February 23, 2023, we received the upfront payment, and the shares of our common stock were issued and sold to Neurocrine pursuant to the applicable stock purchase agreement. For a further description of the 2023 Neurocrine Collaboration Agreement, refer to Note 9, *Significant Agreements*, to our consolidated financial statements included in our Annual Report on Form 10-K for the year ended December 31, 2023 under the caption "2023 Neurocrine Collaboration Agreement."

Our

2019 Neurocrine Collaboration

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, Status and two other undisclosed programs, which we refer to as the 2019 Discovery Programs.

As part Under the terms of the 2019 Neurocrine Collaboration Agreement, we are developing VY-FXN01 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 the treatment research, development, manufacture, and commercialization of Friedreich's ataxia. VY-FXN01 is currently gene therapy products, or the 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 preclinical development. We 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 patent 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 are 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. For a further description of the process of identifying a lead candidate that will be 2019 Neurocrine Collaboration Agreement, refer to Note 9, *Significant Agreements*, to our consolidated financial statements included in our Annual Report on Form 10-K for the year ended December 31, 2023 under the caption "2019 Neurocrine Collaboration Agreement."

Other License Agreements

Alexion License Agreement

In October 2021, we entered into an option and license agreement, or the Pfizer Agreement, with Pfizer Inc., or Pfizer, pursuant to which we granted Pfizer options to receive an exclusive license, or the Pfizer License Options, to certain TRACER Capsids to develop and commercialize certain AAV gene therapy candidates comprised of a capsid promoter, and

FXN transgene 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

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exclusive, worldwide license, with the right to sublicense, under certain of our intellectual property, the rights to develop and are evaluating 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.

Effective upon the closing of the transaction on September 20, 2023, Alexion, AstraZeneca Rare Disease, or 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.

Under the terms of the Alexion Agreement, we have received an upfront payment of $30 million and a payment of $10 million in connection with the exercise of the Pfizer License Option, which we also refer to as the Alexion License Option, for a rare neurological disease during the fourth quarter of 2022. We are also eligible to receive specified development, regulatory, and commercialization milestone payments of up to an aggregate of $115 million for the first Pfizer Licensed CNS Product, which we also refer to as an Alexion Licensed CNS Product, to achieve the applicable 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 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. For a further description of the Alexion Agreement, refer to Note 9, *Significant Agreements*, to our consolidated financial statements included in our Annual Report on Form 10-K for the year ended December 31, 2023 under the caption "Alexion Option and License Agreement (Formerly Pfizer Option and License Agreement)."

Touchlight IP Limited License Agreement

In November 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 program. We historical use of the Subject DNA Preparation Process. Additionally, we are completing AAV capsid biodistribution experiments obligated to confirm capsid serotypes pay low single-digit royalties to Touchlight on future

payments we receive in connection with licensing of certain TRACER Capsids that effectively transduce disease target tissues in non-human primates following intravenous injection. If we and Neurocrine successfully identify a development lead candidate for this program, we plan to complete IND enabling studies to evaluate its safety and efficacy. were created with the historical use of the Subject DNA Preparation Process, excluding the licensing of or collaboration on any of our therapeutic programs.

Accumulated Deficit 2024 Underwritten Public Offering

We In January 2024, we issued and sold 7,777,778 shares of our common stock and, in lieu of common stock to certain investors, pre-funded warrants to purchase 3,333,333 shares of common stock in a public offering, or the 2024 Public Offering, at a public offering price of $9.00 per share of common stock and $8.999 per pre-funded warrant. The 2024 Public Offering resulted in net proceeds to the Company of approximately $93.5 million after deducting underwriting discounts and commissions and offering expenses.

Each pre-funded warrant has an exercise price of $0.001 per share and is exercisable for one share of common stock from the date of issuance until the pre-funded warrant is exercised in full. Under the terms of the pre-funded warrants, we may not effect the exercise of any such warrant, and a holder will not be entitled to exercise any portion of any such warrant, that, upon giving effect to or immediately prior to ,would cause: (1) the aggregate number of shares of our common stock beneficially owned by such holder (together with its affiliates) to exceed 9.99% of the number of shares of our common stock outstanding immediately after giving effect to the exercise; or (2) the combined voting power of our securities beneficially owned by such holder (together with its affiliates) to exceed 9.99% of the combined

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voting power of all of our securities outstanding immediately after giving effect to the exercise, as such percentage ownership is determined in accordance with the terms of the pre-funded warrants. However, any holder of a pre-funded warrant may increase or decrease such percentage to any other percentage not in excess of 19.99% provided that any such increase will not be effective until the 61st day after notice from the holder is delivered to us.

Accumulated Deficit; Expenses

Despite reporting $132.3 million in net income for the year ended December 31, 2023, we have a history of incurring significant losses. As of September 30, 2023 March 31, 2024, we had an accumulated deficit of $317.6 million $272.5 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 GLP toxicology IND application-enabling studies and clinical trials in connection with our tau anti-tau antibody program and our SOD1 ALS gene therapy program;
- continue investing in our proprietary antibody program, gene therapy platform to optimize capsid engineering and payload development, manufacturing, dosing, and delivery techniques by continuing to develop our proprietary antibodies and vectorized antibody platform; platforms and programs, and other research and development initiatives;
- increase our investment in and support for TRACER, our TRACER proprietary discovery platform to facilitate the selection of AAV capsids and expand our investment to discover TRACER Capsids with broad tropism in CNS and other tissues with cell-specific transduction properties for particular therapeutic applications;

- increase our investment in the identification of receptors for our TRACER Capsids and related initiatives to leverage these receptors for further novel capsid discovery and the development of modalities for receptor-mediated non-viral delivery of non-viral genetic medicines; 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 the 2019 Neurocrine Collaboration Agreement and our GBA1 gene therapy program Program pursuant to our the 2023 Neurocrine Collaboration Agreement;

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- continue Agreement, and the Novartis HD Program pursuant to meet obligations set forth by our existing partners and any new partnerships we may enter into; 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 and commercial 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 and our operations as a public company; efforts;
- increase continue our product liability and 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.

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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 three months ended September 30, 2023 March 31, 2024, we recognized $3.1 million $11.5 million of collaboration revenue from the 2023 Neurocrine Collaboration Agreement, and $1.5 million of collaboration revenue from the 2019 Neurocrine Collaboration Agreement. For the nine months ended September 30, 2023, we recognized $79.0 million of collaboration revenue from the Novartis Agreement, $75.5 million of collaboration revenue from the 2023 Neurocrine Collaboration Agreement, $5.2 million $6.5 million of collaboration revenue from the 2019 Neurocrine Collaboration Agreement, $0.8 million of collaboration revenue in connection with the 2023 Novartis Collaboration Agreement, and $0.3 million $0.7 million of other collaboration revenue. revenue in connection with the premium on the issuance of the Novartis Shares to Novartis pursuant to the 2023 Novartis Stock Purchase Agreement.

For additional information about our revenue recognition policy related to collaborations and a description of the key terms of the 2023 2019 Neurocrine Collaboration Agreement and the 2023 Novartis Collaboration Agreement, refer to Note 8, *Significant agreements*, 9 of our condensed the December 31, 2023 consolidated financial statements included in this Quarterly our Annual Report on Form 10-Q. 10-K for the year ended December 31, 2023.

For the foreseeable future, we expect substantially all of our revenue will be generated from the 2019 our current strategic collaborations and out-licensing arrangements with Neurocrine, Collaboration Agreement Novartis, and the 2023 Neurocrine Collaboration Agreement, the Alexion, Agreement, the Novartis 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. sales in the future.

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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 programs, proprietary antibody program and gene therapy platform, proprietary antibodies, and vectorized antibody platform 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 agreements.

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 nine three months ended September 30, 2023 March 31, 2024, our research and development expenses have increased as compared to the amounts recorded in the same period in the prior year. As our research and development programs progress and as we identify product candidates and initiate preclinical studies and clinical trials, including our planned SAD clinical trial to evaluate VY-TAU01, we expect research and development costs to continue

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to increase. However, at 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 nine three months ended September 30, 2023 March 31, 2024, our general and administrative expenses have increased decreased as compared to the amount recorded in the same period in prior year. As our development programs progress and we

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identify product candidates and initiate preclinical studies and clinical trials, we will continue to expect general and administrative expenses to increase to support these additional research and development activities.

Other Income Net

Other income net for the nine months ended September 30, 2023, consists primarily of interest income on our marketable securities.

Critical Accounting Policies and Estimates

We believe that several accounting policies are important to understanding our historical and future performance. We refer to these policies as critical because these specific areas generally require us to make judgments and estimates about matters that are uncertain at the time we make the estimate. There were no changes to our critical accounting policies during the three months ended March 31, 2024, as compared to those identified in our Annual Report on Form 10-K for the fiscal year ended December 31, 2023. It is important that the discussion of our operating results that follow be read in conjunction with the critical accounting policies disclosed in Item 7 "***Critical Accounting Policies and Estimates***

We believe that several accounting policies are important to understanding our historical and future performance. We refer to these policies as critical because these specific areas generally require us to make judgments and estimates about matters that are uncertain at the time we make the estimate. There were no changes to our critical accounting policies during the nine months ended September 30, 2023, as compared to those identified in our Annual Report on Form 10-K for the fiscal year ended December 31, 2022. It is important that the discussion of our operating results that follow be read in conjunction with the critical accounting policies disclosed" in our Annual Report on Form 10-K, as filed with the SEC on March 7, 2023 February 28, 2024.

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Results of Operations

Comparison of the three months ended September 30, 2023 March 31, 2024 and 2022 2023

The following table summarizes our results of operations for the three months ended September 30, 2023 March 31, 2024 and 2022, 2023, together with the changes in those items in dollars:

	Three Months Ended September 30,			Three Months Ended March 31,		
	2023	2022	Change	2024	2023	Change
	(in thousands)			*(in thousands)*		
Collaboration revenue	$ 4,614	$ 41,086	$ (36,472)	$ 19,516	$150,480	$(130,964)
Operating expenses:						

Research and development	25,863	19,337	6,526	27,092	18,568	8,524
General and administrative	8,258	7,307	951	8,607	9,028	(421)
Total operating expenses	34,121	26,644	7,477	35,699	27,596	8,103
Other income, net:						
Other income:						
Interest income	3,429	545	2,884	4,867	1,864	3,003
Other income	—	2,637	(2,637)			
Total other income, net	3,429	3,182	247			
Net (loss) income before income taxes	$ (26,078)	$ 17,624	$ (43,702)			
Total other income				4,867	1,864	3,003
(Loss) income before income taxes				(11,316)	124,748	(136,064)
Income tax provision				14	704	(690)
Net (loss) income				$(11,330)	$124,044	$(135,374)

Collaboration Revenue

Collaboration revenue was $4.6 million and $41.1 million for the three months ended September 30, 2023 and 2022, respectively. The decrease in collaboration revenue was largely a result of $40.0 million of revenue recognized during the three months ended September 30, 2022 in connection with Pfizer's decision to exercise the first material right for the Pfizer License Option, along with the expiration of the second material right associated with the Pfizer License Option. During the three months ended September 30, 2023, we recognized $3.1 million of revenue associated with the 2023 Neurocrine Collaboration Agreement and $1.5 million of revenue associated with the 2019 Neurocrine Collaboration Agreement.

Research and Development Expense

Research and development expenses increased by $6.5 million from $19.3 million for the three months ended September 30, 2022, to $25.9 million for the three months ended September 30, 2023. The following table summarizes

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our research and development expenses for the three months ended September 30, 2023 and 2022, together with the changes in those items in dollars:

	Three Months Ended September 30,		
	2023	2022	Change
	(in thousands)		
Employee and consultant	$ 11,264	$ 7,804	$ 3,459
External research and development	10,663	7,787	2,876
Facilities and other	1,836	1,846	(10)
Professional fees	2,100	1,900	201
Total research and development expenses	$ 25,863	$ 19,337	$ 6,526

The increase in research and development expenses for the three months ended September 30, 2023 was primarily attributable to the following:

- approximately $3.5 million for increased employee and consultant related costs associated with higher headcount in research and development functions, including targeted development team hires to support our advancing pipeline as compared to the three months ended September 30, 2022;
- approximately $2.9 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, along with increased Neurocrine program support during the third quarter of 2023; and
- approximately $0.2 million for increased professional fees primarily related to the increased program-related spend.

General and Administrative Expense

General and administrative expense increased by $1.0 million from $7.3 million for the three months ended September 30, 2022, to $8.3 million for the three months ended September 30, 2023. The increase in general and administrative expense was primarily attributable to the following:

- approximately $1.6 million of increased compensation costs and stock-based compensation associated with higher headcount in general and administrative functions as compared to the three months ended September 30, 2022;
- partially offset by approximately $0.4 million for decreased facility and other costs primarily related to decreased depreciation expense.
- partially offset by approximately $0.2 million for decreased legal and patent expenses.

Other Income, net

Other income, net of approximately $3.4 million was recognized during the three months ended September 30, 2023, as compared to $3.2 million during the three months ended September 30, 2022. Interest income was $3.4 million for the three months ended September 30, 2023, as compared to $0.5 million for the three months ended September 30, 2022. The increase was due to higher cash equivalents and marketable securities balances with increased interest rates during the three months ended September 30, 2023, as compared to the three months ended September 30, 2022. Other income of approximately $2.6 million was recognized during the three months ended September 30, 2022, primarily related to an employee retention tax credit under the Coronavirus Aid, Relief, and Economic Security Act, or the CARES Act.

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Comparison of the nine months ended September 30, 2023 and 2022

The following table summarizes our results of operations for the nine months ended September 30, 2023 and 2022, together with the changes in those items in dollars:

	Nine Months Ended September 30,		
	2023	2022	Change
	(in thousands)		

Collaboration revenue	$ 159,947	$ 42,457	$ 117,490
Operating expenses:			
Research and development	66,416	46,213	20,203
General and administrative	25,580	22,518	3,062
Total operating expenses	91,996	68,731	23,265
Other income, net:			
Interest income	8,567	816	7,751
Other income	3	2,676	(2,673)
Total other income, net	8,570	3,492	5,078
Net income (loss) before income taxes	$ 76,521	$ (22,782)	$ 99,303

Collaboration Revenue

Collaboration revenue was $159.9 million $19.5 million and $42.5 million $150.5 million for the nine three months ended September 30, 2023 March 31, 2024, and 2022, 2023, respectively. The increase in During the first quarter of 2024, we recognized collaboration revenue was the result of $79.0 million in revenue recognized during the nine months ended September 30, 2023, in connection with Novartis' decision to exercise two Novartis License Options, along with the expiration of a third Novartis License Option. In addition, during following agreements:

- $11.5 million with the 2023 Neurocrine Collaboration Agreement;
- $6.5 million with the 2019 Neurocrine Collaboration Agreement;
- $0.8 million with the 2023 Novartis Collaboration Agreement; and
- $0.7 million with the premium on the issuance of the Novartis Shares to Novartis pursuant to the 2023 Novartis Stock Purchase Agreement.

During the nine three months ended September 30, 2023 March 31, 2023, we recognized $75.5 million of collaboration revenue associated in connection with the 2023 Neurocrine Collaboration Agreement, $5.2 million of revenue associated with the 2019 Neurocrine Collaboration Agreement, and $0.3 million of other collaboration revenue. During the nine months ended September 30, 2022, collaboration revenue was primarily related to Pfizer's decision to exercise the first material right for 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 $20.2 million from $46.2 million for the nine months ended September 30, 2022, to $66.4 million for the nine months ended September 30, 2023. The following table summarizes our research and development expenses for the nine months ended September 30, 2023 and 2022, together with the changes in those items in dollars: agreements:

	Nine Months Ended September 30,		
	2023	2022	Change
		(in thousands)	
Employee and consultant	$ 31,280	$ 21,370	$ 9,910
External research and development	24,051	12,382	11,669
Facilities and other	5,039	6,465	(1,426)
Professional fees	6,046	5,996	50
Total research and development expenses	$ 66,416	$ 46,213	$ 20,203

- $79.0 million with Novartis' decision to exercise two Novartis License Options, along with the expiration of a third Novartis License Option;
- $69.5 million with the 2023 Neurocrine Collaboration Agreement related to the delivery of the development and commercialization license for the GBA1 Program; and
- $2.0 million in reimbursement of research and development services from the 2019 Neurocrine Collaboration Agreement.

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Research and Development Expense

Research and development expense increased by $8.5 million from $18.6 million for the three months ended March 31, 2023 to $27.1 million for the three months ended March 31, 2024. The increase in research and development expenses for the nine months ended September 30, 2023 expense was primarily attributable to the following:

- approximately $9.9 million $2.8 million for increased employee and consultant related costs associated with higher headcount in research and development functions, including targeted development team hires to support our advancing pipeline as compared to the same period in the prior year; and three months ended March 31, 2023;
- approximately $11.7 million $2.7 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 the initiation of spend on the Novartis HD Program during the 2023 period, first quarter of 2024; and the fee due to Touchlight in conjunction with Novartis' exercise of two Novartis License Options;
- partially offset by approximately $1.4 million $2.4 million for decreased increased facility and other costs primarily related to the termination addition of the first amendment to our existing lease for office laboratory and laboratory office space at 75 Sidney Street during the second quarter Hayden Avenue in Lexington, Massachusetts, which we took occupancy of 2022. on February 1, 2024.

General and Administrative Expense

General and administrative expense increased decreased by $3.1 million $0.4 million from $22.5 million $9.0 million for the nine three months ended September 30, 2022 March 31, 2023, to $25.6 million $8.6 million for the nine three months ended September 30, 2023 March 31, 2024. The increase decrease in general and administrative expense was primarily attributable to approximately $3.1 million of increased compensation costs and stock-based compensation decreased legal fees due to the legal fees associated with higher headcount in general and administrative functions as compared to the same period execution of the 2023 Neurocrine Collaboration Agreement in the prior year. first quarter of 2023.

Other Income net

Other income net of increased approximately $8.6 million $3.0 million. Approximately $4.9 million was recognized during the three months ended September 30, 2023 March 31, 2024, as compared to $3.5 million $1.9 million during the three months ended September 30, 2022 March 31, 2023. Interest Other income was $8.6 million for during both the

nine three months ended September 30, 2023 March 31, 2024, as compared to $0.8 million for the nine months ended September 30, 2022. and 2023 reflects interest income on marketable securities balances. The increase was is due to higher cash equivalents and marketable securities balances with increased interest rates on increased balances of marketable securities during the nine three months ended September 30, 2023 March 31, 2024, as compared to the nine three months ended September 30, 2022 March 31, 2023. Other income of approximately $2.7 million was recognized during the nine months ended September 30, 2022, primarily related to an employee retention tax credit under the CARES Act.

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 and pre-funded warrants to acquire our common stock, and strategic collaborations and option and license arrangements, including our 2019 Neurocrine Collaboration Agreement strategic collaborations and 2023 Neurocrine Collaboration Agreement, our ongoing option and license arrangements agreements with Alexion Neurocrine, Novartis, and Alexion.

During the three months ended March 31, 2024, the 2024 Public Offering resulted in net proceeds to the Company of approximately $93.5 million after deducting underwriting discounts and commissions and offering expenses.

We and Novartis under entered into the Alexion 2023 Novartis Stock Purchase Agreement, on December 28, 2023, for the sale and the Novartis Agreement, respectively, and with our prior collaboration agreements.

As issuance of September 30, 2023, we had cash, cash equivalents, and marketable securities of $252.9 million. We are committed to maintaining a strong balance sheet that supports the advancement and growth 2,145,002 shares of our platform common stock 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 pipeline. We continue conditions of the 2023 Novartis Stock Purchase Agreement, we issued and sold these shares to assess our planned cash needs both during and in future periods. We expect our cash, cash equivalents, and marketable securities, along with amounts expected to be received as reimbursement for development costs under the Neurocrine collaborations and interest income, to be sufficient to meet our planned operating expenses and capital expenditure requirements into mid-2025. Novartis on January 3, 2024.

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Cash Flows

The following table provides information regarding our cash flows for the nine three months ended September 30, 2023 March 31, 2024 and 2022: 2023:

	Nine Months Ended September 30,	
	2023	2022
	(in thousands)	
Net cash provided (used in) by:		

Operating activities	$ 101,679	$ (265)
Investing activities	(168,476)	(21,406)
Financing activities	33,185	834
Net decrease in cash, cash equivalents, and restricted cash	$ (33,612)	$ (20,837)

	Three Months Ended March 31,	
	2024	2023
	(in thousands)	
Net cash provided by (used in):		
Operating activities	$ 58,767	$ 123,565
Investing activities	(96,065)	14,491
Financing activities	112,856	31,306
Net increase in cash, cash equivalents, and restricted cash	$ 75,558	$ 169,362

Net Cash Provided by (Used in) Operating Activities

Net cash provided by operating activities was $101.7 million $58.8 million during the nine three months ended September 30, 2023 March 31, 2024, compared to $0.3 million of net $123.6 million during the three months ended March 31, 2023. Net cash used in provided by operating activities during the nine three months ended September 30, 2022. The increase March 31, 2024 was primarily due to comprised of a decrease in accounts receivable of $79.3 million from the receipt of the $80.0 million upfront payment under the 2023 Novartis Collaboration Agreement during the first quarter of 2024 offset by our net loss of $11.3 million. Net cash provided by operating activities during the first quarter of 2023 was primarily comprised of our net income for the nine months ended September 30, 2023 of $75.9 million as compared to our net loss for the nine months ended September 30, 2022 of $22.8 million $124.0 million.

Net Cash Used in (Used in) Provided by Investing Activities

Net cash used in investing activities was $168.5 million $96.1 million during the nine three months ended September 30, 2023 March 31, 2024, compared to $21.4 million $14.5 million of net cash provided by investing activities during the nine three months ended September 30, 2022 March 31, 2023. The change net cash used in investing activities for the three months ended March 31, 2024, was primarily due to increased purchases the purchase of $203.9 million in marketable securities offset by proceeds of $109.9 million from the sales and maturities of marketable securities during securities. The net cash provided by investing activities for the nine three months ended September 30, 2023 as compared March 31, 2023 was primarily due to the nine months ended September 30, 2022. $15.0 million in proceeds from sales and maturities of marketable securities.

Net Cash Provided by Financing Activities

Net cash provided by financing activities was $33.2 million $112.9 million during the nine three months ended September 30, 2023 March 31, 2024, primarily comprised of $93.5 million in net proceeds from the issuance of common stock and Pre-Funded Warrants in connection with the 2024 Public Offering and $19.3 million in proceeds from the issuance of common stock in connection with the 2023 Novartis Stock Purchase Agreement. Net cash provided by financing activities was $31.3 million during the three months ended March 31, 2023, driven by proceeds from the issuance of common stock in connection with the 2023 Neurocrine Collaboration Agreement. Net cash provided by financing activities during the nine months ended September 30, 2022 was primarily attributable to the exercise of stock options.

Funding Requirements

Our expenses increased during the nine three months ended September 30, 2023 March 31, 2024, increased as compared with the nine three months ended September 30, 2022 March 31, 2023, as we progressed our research and

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 our planned Phase 1a SAD clinical trial to evaluate VY-TAU01 in 2024, and as we continue to enter into or conduct activities perform our obligations 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 for our wholly-owned programs and to the extent that such sales, marketing and distribution are not the responsibility of potential collaborators. collaborators, as applicable. 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 employees will increase due to inflationary market conditions existing in

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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 March 31, 2024, we had cash, cash equivalents, and marketable securities of $400.5 million. Based upon our current operating plans, we expect that our existing cash, cash equivalents, and marketable securities at September 30, 2023 March 31, 2024, along with amounts expected to be received as reimbursement for development costs

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under our collaboration and license agreements with Neurocrine will enable us and Novartis, certain near-term milestones, and interest income, to be sufficient to meet our planned operating expenses and capital expenditure requirements into mid-2025. 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; candidates,including our planned Phase 1a SAD clinical trial to evaluate VY-TAU01;
- 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 we may be committed to within or outside in connection with 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

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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

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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 6 to our unaudited condensed consolidated financial statements included elsewhere in this Quarterly Report on Form 10-Q.

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 SEC rules.

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ITEM 3. QUANTITATIVE AND QUALITATIVE DISCLOSURES ABOUT MARKET RISK

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

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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 a material effect on our business, financial condition, or results of operations during the nine three months ended September 30, 2023 March 31, 2024.

ITEM 4. CONTROLS AND PROCEDURES

Management's Evaluation of Disclosure Controls and Procedures

We maintain "disclosure controls and procedures," as defined in Rules 13a-15(e) 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 SEC's 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 September 30, 2023 March 31, 2024. 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 September 30, 2023 March 31, 2024, 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.

Changes in Internal Control over Financial Reporting

During the nine three months ended September 30, 2023 March 31, 2024, there have been no changes in our internal control over financial reporting, as such term is defined in Rules 13a-15(f) and 15d-15(f) under the Exchange Act, that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

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PART II. OTHER INFORMATION

ITEM 1. 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, during the three months ended September 30, 2023 as of March 31, 2024, 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.

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ITEM 1A. RISK FACTORS

We are subject to a number of risks that could adversely affect our business, results of operations financial condition and future prospects including those identified in our Annual Report on Form 10-K for the year ended December 31, 2022 December 31, 2023, filed with the SEC on March 7, 2023, or the 2022 10-K Risk Factors. We note that, 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, Inc., or Pfizer. Effective upon the closing of the transaction on September 20, 2023, Alexion acquired all of Pfizer's rights under the option and license agreement we entered into with Pfizer on October 1, 2021, or the Pfizer Agreement, and became the successor-in-interest to Pfizer thereunder. The 10-K Risk Factors referring to Pfizer in the context of its ongoing rights and obligations under the Pfizer Agreement should be read to refer instead to Alexion as Pfizer's successor-in-interest thereunder on and after September 20, 2023 February 28, 2024. There have been no other material changes to the 2022 10-K Risk Factors.

ITEM 2. UNREGISTERED SALES OF EQUITY SECURITIES AND USE OF PROCEEDS

On July 10, 2023 Pursuant to that certain stock purchase agreement, dated as of December 28, 2023, by and between us and Novartis Pharma AG, or Novartis , we issued completed a private placement of 2,145,002 shares of common stock to two executives non-statutory stock options to purchase Novartis at a price of $9.324 per share, for an aggregate of 167,000 shares of our common stock at an exercise purchase price of $10.18 per share. approximately $20.0 million. We issued the shares to Novartis, effective January 3, 2024, in a private placement in reliance on the exemption from registration under Section 4(a)(2) of the Securities Act of 1933, as amended, or the Securities Act, for a transaction by an issuer not involving any public offering within the meaning of Section 4(a)(2) and/or under Rule 506 of Regulation D promulgated under the Securities Act and corresponding provisions of state securities or "blue sky" laws.

On August 7, 2023 March 25, 2024, we issued to an executive a non-statutory stock option to purchase an aggregate of 122,000 shares of our common stock at an exercise price of $8.68 per share. On September 5, 2023, we issued to two executives non-statutory stock options to purchase an aggregate of 90,000 210,000 shares of our common stock at an exercise price of $9.26 per share. On September 6, 2023, we issued to an executive a non-statutory stock The option to purchase an aggregate of 44,000 shares of our common stock at an exercise price of $9.39 per share. The options were was granted outside of our 2015 Stock Option and Incentive Plan in each case, as an inducement material to such executive's the recipient'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 under the Securities Act to register the shares of our common stock underlying the stock options option prior to the time at which the shares underlying the options option become exercisable.

ITEM 5. OTHER INFORMATION

Director and Officer Trading Arrangements

A portion of the compensation of our directors and officers (as defined in Rule 16a-1(f) under the Securities Exchange Act of 1934, as amended (the "Exchange Act")) is in the form of equity awards and, from time to time, directors and officers engage in open-market transactions with respect to the securities acquired pursuant to such equity awards or our other securities, including to satisfy tax withholding obligations when equity awards vest or are exercised, and for diversification or other personal reasons.

Transactions in our securities by directors and officers are required to be made in accordance with our insider trading policy, which requires that the transactions be in accordance with applicable U.S. federal securities laws that prohibit trading while in possession of material nonpublic information. Rule 10b5-1 under the Exchange Act provides an affirmative defense that enables directors and officers to prearrange transactions in our securities in a manner that avoids concerns about initiating transactions while in possession of material nonpublic information.

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The following table describes, for the quarterly period covered by this report, each trading arrangement for the sale or purchase of our securities adopted or terminated by our directors and officers that is either (1) a contract, instruction or written plan intended to satisfy the affirmative defense conditions of Rule 10b5-1(c) (a "Rule 10b5-1 trading arrangement") or (2) a "non-Rule 10b5-1 trading arrangement" (as defined in Item 408(c) of Regulation S-K):

Name (Title)	Action Taken (Date of Action)	Type of Trading Arrangement	Nature of Trading Arrangement	Duration of Trading Arrangement	Aggregate Number of Securities
Nancy Vitale (Director)	Adoption (March 19, 2024)	Rule 10b5-1 trading arrangement for exercises of options and sales of shares	Sale	Until August 29, 2025, or such earlier date upon which all transactions are completed or expire without execution	Up to 89,000 shares
Toby Ferguson, M.D., Ph.D. (Chief Medical Officer)	Adoption (March 22, 2024)	Durable Rule 10b5-1 trading arrangement for sell-to-cover transactions related to restricted stock units ("RSUs") granted on or after April 1, 2024	Sale	Until final settlement of any covered RSU	Indeterminable (1)

(1) The number of shares subject to covered RSUs that will be sold to satisfy applicable tax withholding obligations upon vesting is unknown as the number will vary based on the extent to which vesting conditions are satisfied, the market price of our common stock at the time of settlement, and the potential future grant of additional RSUs subject to this arrangement. This trading arrangement, which applies to RSUs whether vesting based on the passage of time or the achievement of performance goals, provides for the automatic sale of shares that would otherwise be issuable on each settlement date of a covered RSU in an amount sufficient to satisfy the applicable withholding obligation, with the proceeds of the sale delivered to us in satisfaction of the applicable withholding obligation.

ITEM 6. EXHIBITS

The exhibits filed or furnished as part of this Quarterly Report are set forth on the Exhibit Index, which is incorporated herein by reference.

INDEX TO EXHIBITS

		Incorporated by Reference to:				
Exhibit No.	Description	Form or Schedule	Exhibit No.	Filing Date with SEC	SEC File Number	Filed Herewith
4.1	Form of Pre-Funded Warrant	8-K	4.1	01/08/2024	001-37625	
10.1	Amendment No. 4 to Consulting Agreement by and between the Registrant and Dinah Sah, Ph.D., dated as of February 1, 2024.					X
10.2	Employment Agreement, by and between the Registrant and Toby Ferguson, M.D., Ph.D., dated as of February 29, 2024.	8-K	10.1	03/13/2024	001-37625	

10.3	Transition, Separation and Release of Claims Agreement, by and between the Registrant and Peter P. Pfreundschuh, dated April 1, 2024.	8-K	10.1	04/02/2024	001-37625	
10.4	Consulting Agreement by and between the Registrant and Peter P. Pfreundschuh, dated as of May 6, 2024					X

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INDEX TO EXHIBITS

		Incorporated by Reference to:				
Exhibit No.	**Description**	**Form or Schedule**	**Exhibit No.**	**Filing Date with SEC**	**SEC File Number**	**Filed Herewith**
10.1	First Amendment to Lease Agreement, by and between Voyager Therapeutics, Inc. and LS 75 Hayden, LLC, dated August 11, 2023.	8-K	10.1	08/16/2023	001-37625	
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+	Certification 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
101.INS	Inline XBRL Instance Document – the instance document does not appear in the Interactive Data File because 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 Linkbase Document.	X
101.DEF	Inline XBRL Taxonomy Extension Definition Linkbase Document.	X
101.LAB	Inline XBRL Taxonomy Extension Label Linkbase Document.	X
101.PRE	Inline XBRL Taxonomy Extension Presentation Linkbase 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	
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+	Certification 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
101.INS	Inline XBRL Instance Document – the instance document does not appear in the Interactive Data File because 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 Linkbase Document.	X
101.DEF	Inline XBRL Taxonomy Extension Definition Linkbase Document.	X
101.LAB	Inline XBRL Taxonomy Extension Label Linkbase Document.	X

101.PRE	Inline XBRL Taxonomy Extension Presentation Linkbase 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	

* 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 Quarterly Report on Form 10-Q 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.

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SIGNATURES

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned, thereunto duly authorized.

Date: November 6, 2023 May 13, 2024

VOYAGER THERAPEUTICS, INC.

By: */s/ Alfred Sandrock, M.D., Ph.D.*
Alfred Sandrock, M.D., Ph.D.
Chief Executive Officer, President, and Director
(Principal Executive Officer)

By: */s/ Peter P. Pfreundschuh Robin Swartz*
Peter P. Pfreundschuh Robin Swartz
Chief Financial Operating Officer
(Principal Financial and Accounting Officer)

Exhibit 10.1

AMENDMENT NO. 4 TO

CONSULTING AGREEMENT

This Amendment No. 4 to Consulting Agreement (this "Amendment") effective as of February 1, 2024 ("Amendment Effective Date") is entered into by and between (i) **Voyager Therapeutics, Inc.**, a Delaware corporation with an office located at 75 Hayden Avenue, Lexington, MA 02421 ("Voyager") and (ii) **Dinah Sah, Ph.D.**, an individual residing at [**] ("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, (ii) Amendment No. 2 effective as of June 27, 2022 and (iii) Amendment No. 3 effective as of May 1, 2023 (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 February 28, 2025, 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:

I. **Equity Grant:** Subject to approval by Voyager's Board of Directors (the "Board") or the Compensation Committee of the Board (the "Compensation Committee"), and as consideration for Consultant providing the Services, Voyager shall grant to Consultant, effective as of February 2, 2024 (the "Sah Option Grant Date"), an option to purchase **15,000** shares (the "Sah Option") of common stock of Voyager ("Common Stock"), subject to the execution and delivery of a stock option agreement in substantially the form approved and adopted by the Board or the Compensation Committee, as the case may be, such Sah Option to (i) vest and become exercisable as to all of the shares underlying the Sah Option on the one-year anniversary of the Sah Option Grant Date, (ii) have an exercise price per share equal to the closing sale price (for the primary trading session) of the Common Stock on the Nasdaq Global Select Market on the Sah Option Grant Date, and (iii) be granted pursuant to and in accordance with the Company's 2015 Stock Option and Incentive Plan.

II. **Fees**: During the Term, Voyager will pay Consultant fees for Services as follows:

A. For the period May 1, 2023 to February 28, 2025, **$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.

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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 February 28, 2025."

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 May 1, 2023, 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. 4 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 10.4
Execution Version

CONSULTING AGREEMENT

THIS AGREEMENT (together with the attached Services Form, the "**Agreement**"), is entered into as of May 6, 2024 (the "**Effective Date**"), by and between Peter P. Pfreundschuh, an individual (the "**Consultant**"), and **Voyager Therapeutics, Inc.**, a Delaware corporation located at 75 Hayden Avenue, Lexington, MA 02421 (hereinafter "**Voyager**").

WHEREAS, Voyager desires to retain the consulting and advisory services of Consultant with respect to certain activities as described in this Agreement, and Consultant is willing to so act.

NOW, THEREFORE, Consultant and Voyager agree as follows:

1. Description of Services. Voyager hereby retains Consultant as a consultant to Voyager and Consultant hereby agrees to use Consultant's best efforts to provide advice and assistance to Voyager in the area of Consultant's expertise from time to time as requested by Voyager (the "**Services**"). In particular, the Services shall include any specific activities described in the attached **Services Form** attached hereto as Exhibit A, as well as a reasonable amount of additional advisory services to Voyager's personnel or designees by telephonic means, or in the form of reports and summaries, and such additional activities agreed to by the parties from time to time, subject to the terms and limitations set forth on the Services Form. Any changes to the Services (and any related compensation adjustments) must be agreed to in writing between Consultant and Voyager prior to implementation of the changes.

2. Term & Termination. The term of this Agreement shall be from the Effective Date through June 28, 2024, subject to Voyager's right to extend the term until July 31, 2024 upon written notice to Consultant, unless earlier terminated in accordance with this Agreement or extended by mutual written agreement (the "**Term**"). This Agreement may be terminated prior to its expiration in the following manner: (i) by Voyager at any time immediately upon written notice to Consultant if Consultant has materially breached this Agreement, the Transition, Separation and Release of Claims Agreement between Consultant and Voyager to which this Consulting Agreement is attached as Exhibit C (the "**Separation Agreement**"), or the Restrictive Covenants Agreement referenced in the Separation Agreement; (ii) by Consultant at any time immediately upon written notice if Voyager has materially breached this Agreement or the Separation Agreement; (iii) at any time upon the mutual written consent of both parties; (iv) by Voyager at any time without cause upon not less than thirty (30) days' prior written notice to Consultant, or by Consultant at any time without cause upon not less than thirty (30) days' prior written notice to Voyager; or (v) automatically upon (x) Consultant's failure to timely sign the Additional Release (as defined in the Separation Agreement), (y) Consultant's revocation of the Additional Release, or (z) the death, physical incapacitation or mental incompetence of Consultant. Any expiration or termination of this Agreement shall be without prejudice to any obligation of either party that has accrued prior to the effective date of expiration or termination. Upon expiration or termination of this Agreement, neither Consultant nor Voyager will have any further obligations under this Agreement, except that (a) Consultant will terminate all Services in progress in an orderly manner as soon as practicable and in accordance with a schedule agreed to by Voyager, unless Voyager specifies in the notice of termination that Services in progress should be completed; (b) Consultant will deliver to Voyager all Work Product (defined below) made through expiration or termination; (c) Voyager will pay Consultant any monies due and

owing Consultant, up to the time of termination or expiration, for Services properly performed and all authorized expenses actually incurred; (d) Consultant will immediately return to Voyager all Voyager Property (defined below) and other Confidential Information (defined below) and copies thereof provided to Consultant under this Agreement; and (e) the terms, conditions and obligations under Sections 2 and 4 through 14 will survive expiration or termination of this Agreement.

3. Payment of Fees and Expenses. Voyager will pay Consultant for fees, expenses and pass-through costs in accordance with the Services Form, including reasonable and necessary travel, lodging and meals in connection with the Services, subject to Voyager's travel policy. Unless otherwise agreed in an Services Form, the following shall apply:

(a) At the end of any month in which Consultant performs Services pursuant to this Agreement, Consultant shall submit to Voyager an invoice (containing an itemized statement of the Services performed, including the number of hours worked) for the fees payable to Consultant for such Services in accordance with the Services Form;

(b) Consultant will invoice Voyager monthly for any pre-approved expenses and pass-through costs relating to the Services;

(c) Invoices will reference the applicable purchase order number provided by Voyager, and are to be sent directly to Accounts Payable, Voyager Therapeutics, Inc., 75 Hayden Avenue, Lexington, MA 02421 or submitted via e-mail to: ap@vygr.com; and

(d) Voyager shall pay all undisputed amounts invoiced in accordance with the terms of this Section 3 by the date that is thirty (30) days following receipt of the invoice by Voyager.

Upon execution of this Agreement, Consultant shall submit a W-9/W-8BEN/W-8ECI (as applicable) to Voyager's Accounts Payable department at the address above. Invoices will not be paid without Voyager's receipt of Consultant's W-9/W-8BEN/W-8ECI information.

4. Compliance with Laws. Consultant represents and warrants that Consultant will render Services in compliance with all applicable laws, rules and regulations, including but not limited to the U.S. Food, Drug and Cosmetic Act, as amended from time to time, and the highest professional standards. Further, Consultant represents and warrants that he has not been, and is not under consideration to be (a) debarred from providing services pursuant to Section 306 of the United States Federal Food Drug and Cosmetic Act, 21 U.S.C. § 335a; (b) excluded, debarred or suspended from, or otherwise ineligible to participate in, any federal or state health care program or federal procurement or non-procurement programs (as that term is defined in 42 U.S.C. §1320a-7b(f)); (c) disqualified by any government or regulatory agencies from performing specific services, and is not subject to a pending disqualification proceeding; or (d) convicted of a criminal offense related to the provision of health care items or services, or under investigation or subject to any such action that is pending.

5. Compliance with Obligations to Third Parties. Consultant represents and warrants to Voyager that the terms of this Agreement and Consultant's performance of Services do not and will not conflict with any of Consultant's obligations to any third parties. Consultant represents that Consultant has not brought and will not bring with Consultant to Voyager or use in the performance of Services any equipment, funds, space, personnel, facilities, confidential information, trade secrets or other

resources of any third party which are not generally available to the public, unless Consultant has obtained written authorization for their possession and use, nor will Consultant take any other action that would result in a third party, including without limitation, an employer of Consultant, asserting ownership of, or other rights in, any Work Product, unless agreed upon in writing in advance by Voyager. To the extent Consultant is subject to any policy of his employer that requires approval of agreements governing external consulting services, Consultant represents that such approval has been given and covenants that such approval will be obtained prior to entering into any amendment to this Agreement requiring such approval. Consultant will notify Voyager immediately of any breach of this Section 5.

6. Work Product. Consultant will promptly and fully disclose in confidence to Voyager all inventions, discoveries, improvements, ideas, concepts, designs, processes, formulations, products, computer programs, works of authorship, databases, mask works, trade secrets, know-how, information, data, documentation, reports, research, creations and other products arising from or made in the performance of (solely or jointly with others) the Services (whether or not patentable or subject to copyright or trade secret protection) (collectively, the "**Work Product**"). Consultant assigns and agrees to assign to Voyager all rights in the United States and throughout the world to Work Product. Consultant will keep and maintain adequate and current written records of all Work Product, and such records will be available to and remain the sole property of Voyager at all times. For purposes of the copyright laws of the United States, Work Product will constitute "works made for hire," except to the extent such Work Product cannot by law be "works made for hire". Consultant represents and warrants that Consultant has and will have the right to transfer and assign to Voyager ownership of all Work Product. Consultant will execute all documents, and take any and all actions needed, all without further consideration, in order to confirm Voyager's rights as outlined above. In the event that Consultant should fail or refuse to execute such documents within a reasonable time, Consultant appoints Voyager as attorney to execute and deliver any such documents on Consultant's behalf.

7. Confidentiality & Non-Use. During the Term and thereafter, except as otherwise permitted as set forth below, Consultant agrees to (a) hold the Confidential Information in confidence; (b) exercise reasonable precautions to physically protect the integrity and confidentiality of the Confidential Information; (c) not disclose any Confidential Information to any third party without the prior written consent of Voyager; (d) not use the Confidential Information for any purpose except as may be necessary in the ordinary course of performing Services without the prior written consent of Voyager; (e) treat Confidential Information with no less than a reasonable degree of care; and (f) reproduce Confidential Information solely to the extent necessary to provide the Services, with all such reproductions being considered Confidential Information.

Voyager's "**Confidential Information**" means (i) all Work Product; (ii) all information contained in or comprised of Voyager Property (defined in Section 8); and (iii) all confidential and proprietary data, trade secrets, business plans, and other information of a confidential or proprietary nature in written, electronic or other media, belonging to Voyager or its subsidiaries or third parties with whom Voyager may have business dealings, disclosed or otherwise made available to Consultant by Voyager or on behalf of Voyager in connection with this Agreement and/or Consultant's services hereunder. Consultant's obligations of non-disclosure and non-use under this Agreement will not apply to any portion of Confidential Information that Consultant establishes by competent proof: (a)

was in the public domain at the time of disclosure through no wrongful act on the part of Consultant; (b) after disclosure, becomes part of the public domain by publication or otherwise, except by a wrongful act on the part of Consultant; (c) becomes known to Consultant on a non-confidential basis through disclosure by sources other than Voyager having the legal right to disclose such Confidential Information; or (d) is independently developed by Consultant without reference to or reliance upon Confidential Information.

Nothing in this Agreement prohibits Consultant from communicating with or voluntarily providing information he believes indicates possible or actual violations of the law to local, state or federal government agencies (including but not limited to the Securities & Exchange Commission), any legislative body, law enforcement, or self-regulatory organizations. Consultant is not required to notify Voyager of any such communications. Further, Consultant is hereby advised as follows pursuant to the Defend Trade Secrets Act: “An individual shall not be held criminally or civilly liable under any Federal or State trade secret law for the disclosure of a trade secret that (A) is made (i) in confidence to a Federal, State, or local government official, either directly or indirectly, or to an attorney; and (ii) solely for the purpose of reporting or investigating a suspected violation of law; or (B) is made in a complaint or other document filed in a lawsuit or other proceeding, if such filing is made under seal. An individual who files a lawsuit for retaliation by an employer for reporting a suspected violation of law may disclose the trade secret to the attorney of the individual and use the trade secret information in the court proceeding, if the individual (A) files any document containing the trade secret under seal; and (B) does not disclose the trade secret, except pursuant to court order.”

8. Voyager Property. All documents, data, records, apparatus, equipment and other physical property furnished or made available by or on behalf of Voyager to Consultant in connection with this Agreement (“**Voyager Property**”) shall be and remain the sole property of Voyager and shall be returned promptly to Voyager if requested. In any event, Consultant shall return and deliver all Voyager Property, including any copies thereof, upon termination or expiration of this Agreement, irrespective of the reason for such termination. Consultant will use Voyager Property only as necessary to perform the Services and will not transfer or make available to any third party the Voyager Property without the express prior written consent of Voyager. Consultant recognizes that Voyager’s facilities are private and Consultant will abide by Voyager’s security requirements and conditions for access and usage and agrees that only those subjects, areas and programs designated by Voyager as necessary to fulfill Voyager’s requirements will be accessed and/or perused Consultant. In no event will any Confidential Information, programs or other information be copied or removed without Voyager’s express written approval.

9. Publication; Publicity. Work Product may not be published or referred to, in whole or in part, by Consultant without the prior express written consent of Voyager. Consultant shall not use the name, logo, trade name, service mark, or trademark, or any simulation, abbreviation, or adaptation of same, or the name of Voyager or its subsidiaries for publicity, promotion, or similar non-regulatory uses without Voyager’s prior written consent.

10. Independent Contractor Relationship. Nothing contained in this Agreement shall be deemed to constitute Consultant an employee of Voyager, it being the intent of the parties to establish an independent contractor relationship, nor shall Consultant have authority to bind Voyager in any manner whatsoever by reason of this Agreement. Consultant shall at all times while on Voyager

premises observe all security and safety policies of Voyager. Consultant is excluded from participating in any fringe benefit plans or programs as a result of the performance of the Services, without regard to Consultant's independent contractor status, including, but not limited to, health, sickness, accident or dental coverage, life insurance, disability benefits, accidental death and dismemberment coverage, unemployment insurance coverage, workers' compensation coverage, 401(k) benefit(s), and any other benefits provided by Voyager to its employees. Consultant agrees, as an independent contractor, that Consultant is not entitled to unemployment benefits in the event this Agreement terminates, or workers' compensation benefits in the event that Consultant is injured in any manner or becomes ill while performing the Services under this Agreement. Because Consultant is an independent contractor, Voyager will not make any withholdings, deductions, or contributions (e.g., social security, unemployment insurance, disability insurance) from Consultant's fees, and will report Consultant's fees and other payments to Consultant on a 1099 form. Consultant shall bear sole responsibility for paying and reporting its own applicable federal and state income taxes, social security taxes, unemployment insurance, workers' compensation, and health or disability insurance, retirement benefits, and other welfare or pension benefits, if any, and shall indemnify and hold Voyager harmless from and against any liability with respect thereto.

11. Notices. All notices required or permitted under this Agreement must be in writing. Any notice given under this Agreement shall be deemed delivered when delivered by hand, by certified mail, by air courier or via facsimile to Voyager at its address set forth above (or at such other address as it may provide to Consultant in writing from time to time) and to Consultant at such address as Consultant may provide to Voyager in writing from time to time. Notices will be effective upon receipt or at a later date stated in the notice.

12. Assignment. The rights and obligations of the parties hereunder shall inure to the benefit of, and shall be binding upon their respective successors and assigns. This Agreement may not be assigned by Consultant, and Consultant's obligations under this Agreement may not be subcontracted or delegated by Consultant, without the prior written consent of Voyager. For clarity, this Agreement may be assigned by Voyager with prompt notice of such assignment to Consultant.

13. Specific Enforcement. Consultant acknowledges that Voyager will have no adequate remedy at law in the event Consultant breaches the terms of Sections 4 through 9. In addition to any other rights it may have, Voyager shall have the right to obtain in any court of competent jurisdiction injunctive or other relief to restrain any breach or threatened breach of this Agreement.

14. Prior Agreements; Governing Law; Severability; Amendment. This Agreement embodies the entire understanding between the parties with respect to the subject matter of this Agreement and supersedes any prior or contemporaneous agreements with respect to the subject matter of this Agreement; provided, however, for the avoidance of doubt, that Consultant's obligations pursuant to Sections 6, 7 and 8 hereunder are in addition to any and all similar ongoing obligations that Consultant has to Voyager pursuant to the Separation Agreement and/or the Restrictive Covenants Agreement referenced therein. This Agreement shall be governed by and construed in accordance with the laws of the Commonwealth of Massachusetts, without regard to any choice of law principle that would dictate the application of the law of another jurisdiction, and Consultant submits to the jurisdiction and agrees to the proper venue of all state and federal courts located within the Commonwealth of Massachusetts. Each provision in this Agreement is independent and severable

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from the others, and no provision will be rendered unenforceable because any other provision is found by a proper authority to be invalid or unenforceable in whole or in part. If any provision of this Agreement is found by such an authority to be invalid or unenforceable in whole or in part, such provision shall be changed and interpreted so as to best accomplish the objectives of such unenforceable or invalid provision and the intent of the parties, within the limits of applicable law. This Agreement may be executed in one or more counterparts, each of which will be deemed an original, and all of which together will be deemed to be one and the same instrument. A facsimile or electronic copy of this Agreement, including the signature pages, will be deemed an original. This Agreement may not be amended, and its terms may not be waived, except pursuant to a written amendment or waiver signed by both parties.

15. Insurance. Consultant as its election shall maintain such insurance as shall be reasonably necessary to insure itself against any claim or claims for damages arising out of the Services or this Agreement. Consultant shall, if such insurance has been obtained, provide evidence of such coverage to Voyager upon request.

16. Certain Other Conflicts of Interest; Trading in Voyager Securities.

(a) Consultant represents that, except as disclosed in writing to Voyager, Consultant: (i) does not own directly or indirectly five percent (5%) or more of the stock or other equity securities of any entity which is a present or prospective competitor, customer or supplier of Voyager; (ii) is not aware of any legal proceedings pending or threatened against Consultant, or any reasonable basis for such proceedings, which (1) would conflict with Consultant's obligations hereunder or question the validity of this Agreement; or (2) may materially or adversely affect the business or prospects of Voyager; and (iii) is not aware of any fact concerning Consultant (either professionally or personally) which may materially or adversely affect the business or prospects of Voyager.

(b) 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 may gain access to information in connection with the provision of Services that could potentially subject Consultant to insider trading liability (as defined under the US federal securities laws and regulations adopted by the United States Securities and Exchange Commission) in connection with trading in Voyager securities. Consultant shall comply with all relevant laws respecting any trading in Voyager securities.

[Remainder of page intentionally left blank]

IN WITNESS WHEREOF, the parties hereto have entered into this Agreement as of the Effective Date.

CONSULTANT	VOYAGER THERAPEUTICS, INC.
By: */s/ Peter P. Pfreundschuh*	By: */s/ Michelle Quinn Smith*
Name: Peter P. Pfreundschuh	Name: Michelle Quinn Smith
Tax ID#: [**]	Title: Chief Human Resources Officer

[Signature Page to Consulting Agreement]

EXHIBIT A

SERVICES FORM

Consulting Agreement Between Voyager Therapeutics, Inc. (“Voyager”) and

Peter P. Pfreundschuh (“Consultant”) Dated May 6, 2024

1. Services:

Consultant will provide the following Services to Voyager:

Consultant, who immediately prior to the Effective Date was the Chief Financial Officer of Voyager, will provide transitional services associated with Consultant's transfer of his former duties and responsibilities to persons at Voyager assuming such duties and responsibilities.

Consultant will provide Services on a schedule and at a location or locations mutually agreed between Consultant and Voyager's Chief Operating Officer, including being available for telephone and/or written consultations.

The intention of Voyager and of Consultant is that Consultant will generally not provide Services to Voyager in excess of 10 hours per month, and Consultant shall request and receive written approval from Voyager's Chief Operating Officer in advance of performing Services in excess of 10 hours per month.

2. Compensation:

Fees: During the Term, Voyager will pay Consultant consulting fees in the amount of five hundred dollars ($500.00) per hour of Services performed, with such fees to be paid to Consultant in accordance with Section 3 of the Agreement.

Expenses: Voyager will reimburse Consultant for any pre-approved expenses actually incurred by Consultant in connection with the provision of Services. Requests for reimbursement will be in a form reasonably acceptable to Voyager, will include supporting documentation and will accompany Consultant's invoices.

3. Period of Performance:

Services are anticipated to commence on the Effective Date and be completed no later than June 28, 2024, subject to Voyager's right to extend the Term until July 31, 2024 upon written notice to Consultant.

Exhibit 31.1

Certification

I, Alfred Sandrock, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q for the period ended September 30, 2023 March 31, 2024 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

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.

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: November 6, 2023 May 13, 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, Robin Swartz, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q for the period ended September 30, 2023 March 31, 2024 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: November 6, 2023 May 13, 2024 /s/ *Peter P. Pfreundschuh Robin Swartz*

Peter P. Pfreundschuh Robin Swartz
Chief Financial Operating 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 Quarterly Report on Form 10-Q of Voyager Therapeutics, Inc. (the "Company") for the period ended September 30, 2023 March 31, 2024, 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 his or her knowledge:

1) the Report which this statement accompanies 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: November 6, 2023 May 13, 2024

/s/ Alfred Sandrock, M.D., Ph.D.
Alfred Sandrock, M.D., Ph.D.
Chief Executive Officer, President, and Director
(Principal Executive Officer)

Date: November 6, 2023 May 13, 2024

/s/ Peter P. Pfreundschuh Robin Swartz
Peter P. Pfreundschuh Robin Swartz
Chief Financial Operating Officer
(Principal Financial and Accounting Officer)

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