UNITED STATES
SECURITIES AND EXCHANGE COMMISSION

WASHINGTON, DC 20549

FORM 10-K

(Mark One)

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

For the fiscal year ended December 31 , 2023

or

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

For the transition period from _____ to _____

Commission File Number: 001-41477

Biohaven Ltd.

(Exact Name of Registrant as Specified in its Charter)

British Virgin Islands	**Not applicable**
(State or other jurisdiction of incorporation or organization)	(I.R.S. Employer Identification No.)
c/o Biohaven Pharmaceuticals, Inc. **215 Church Street , New Haven , Connecticut**	**06510**
(Address of principal executive offices)	(Zip Code)

(203) 404-0410

(Registrant's telephone number, including area code)

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

Title of each class	Trading Symbol	Name of each exchange on which registered
Common Shares, without par value	BHVN	New York Stock Exchange

Securities registered pursuant to Section 12(g) of the Act: **None**

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

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

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 ý No o

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 ý No o

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	☐	Small reporting company	☐
		Emerging growth company	☐

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

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

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

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

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

The aggregate market value of the registrant's common shares held by non-affiliates of the registrant as of the last business day of the registrant's most recently completed second fiscal quarter, June 30, 2023, based on the last reported sale price of the registrant's common shares on the New York Stock Exchange on June 30, 2023 of $23.92, was $ 986.7 million. The calculation does not reflect a determination that certain persons are affiliates of the registrant for any other purpose. As of February 26, 2024, there were 81,579,914 common shares, no par value per share, outstanding.

Documents Incorporated by Reference

Portions of the registrant's definitive Proxy Statement to be filed pursuant to Regulation 14A under the Securities Exchange Act of 1934 for its 2024 Annual Meeting of Shareholders are incorporated by reference into Part III of this Annual Report on Form 10-K.

Unless the context requires otherwise, references in this report to “Biohaven,” the “Company,” “we,” “our” or “us” refer to Biohaven Ltd. and its subsidiaries.

Cautionary Note Regarding Forward-Looking Statements

This Annual Report on Form 10-K, or this report, contains forward-looking statements that involve risks and uncertainties. We make such forward-looking statements pursuant to the safe harbor provisions of the Private Securities Litigation Reform Act of 1995 and other federal securities laws. All statements other than statements of historical facts contained in this report, including statements regarding our future results of operations and financial position, strategy and plans, and our expectations for future operations, are forward-looking statements. In some cases, you can identify forward-looking statements by terminology such as “believe,” “may,” “will,” “estimate,” “continue,” “anticipate,” “design,” “intend,” “expect,” “could,” “plan,” “potential,” “predict,” “seek,” “should,” “would”, or the negative of these terms or other comparable terminology. Forward-looking statements are not guarantees of performance and are based on certain assumptions, discuss future expectations, describe plans and strategies or state other forward-looking information.

These forward-looking statements include, but are not limited to, statements about:

- our ongoing and planned clinical trials, including discovery and proof of concept trials, the status of our ongoing clinical trials, commencement dates for new clinical trials, and the timing of clinical trial results;
- our plans to pursue research and development of other products;
- anticipated future milestones, contingent and royalty payments and lease payments (and, in each case, their expected impact on liquidity);
- our commercialization, marketing and manufacturing capabilities and strategy; and
- our estimates regarding future revenues, expenses and needs for additional financing.

Important factors that could cause actual results to differ materially from those reflected in such forward-looking statements and that should be considered in evaluating our outlook include, but are not limited to, the following:

- the risk of litigation and/or regulatory actions related to the Separation or our business;
- future business combinations or disposals;
- our ability to enter into additional collaborations with third parties;
- the timing of and our ability to obtain and maintain regulatory approvals for our product candidates;
- our intellectual property position;
- the rate and degree of market acceptance of our products or product candidates, and our estimates regarding the potential market opportunity for our product candidates;
- our competitive position, including our competitors and competing products (including biosimilars);
- the impact of economic conditions, including increases in interest rates and inflation, on the costs of raw materials, wages, manufacturing and clinical trials and on borrowing costs;
- the timing and anticipated amounts of future tax payments and benefits (including the potential recognition of unrecognized tax benefits), as well as timing of conclusion of tax audits; and
- other factors identified elsewhere in this Annual Report on Form 10-K and our other filings with the SEC.

Any forward-looking statements in this report reflect our current views with respect to future events and with respect to our future financial performance and involve known and unknown risks, uncertainties and other 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 these forward-looking statements. Factors that may cause actual results to differ materially from current expectations include, among other things, those described under Part I, Item 1A. Risk Factors, Part II, Item 7. Management’s Discussion and Analysis of Financial Condition and Results of Operations, and elsewhere in this report. Given these uncertainties, you should not place undue reliance on any forward-looking statements. Except as required by law, we assume no obligation to update or revise any forward-looking statements for any reason, even if new information becomes available in the future.

This report contains estimates, projections and other information concerning our industry, the general business environment, and the markets for certain diseases, including estimates regarding the potential size of those markets and the estimated incidence and prevalence of certain medical conditions. Information that is based on estimates, forecasts, projections, market research or similar methodologies is inherently subject to uncertainties and actual events, circumstances or numbers, including actual disease prevalence rates and market size, may differ materially from the information reflected in this report. Unless otherwise expressly stated, we obtained this industry, business information, market data, prevalence information and other data from reports, research surveys, studies and similar data prepared by market research firms and other third parties, industry, medical and general publications, government data, and similar sources, in some cases applying our own assumptions and analyses that may, in the future, not prove to have been accurate.

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PART I

Item 1. Business

Overview

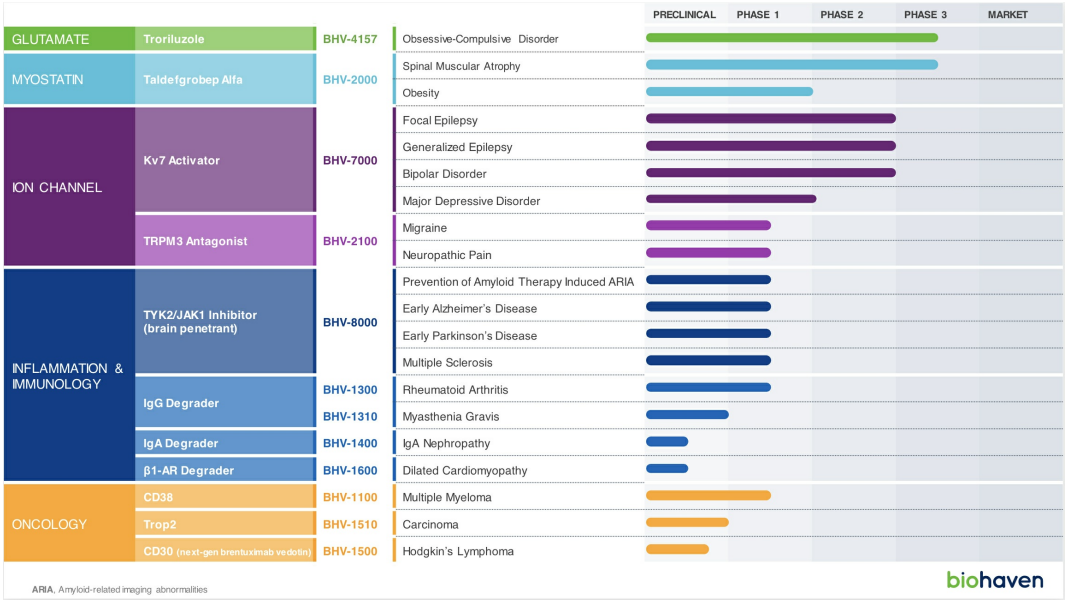
Biohaven is a biopharmaceutical company focused on the discovery, development, and commercialization of life-changing treatments in key therapeutic areas, including immunology, neuroscience, and oncology. We are advancing our innovative portfolio of therapeutics, leveraging our proven drug development experience and multiple proprietary drug development platforms. Our extensive clinical and preclinical programs include Kv7 ion channel modulation for epilepsy and mood disorders; extracellular protein degradation for immunological diseases; Transient Receptor Potential Melastatin 3 ("TRPM3") antagonism for migraine and neuropathic pain; Tyrosine Kinase 2/Janus Kinase 1 ("TYK2/JAK1") inhibition for neuroinflammatory disorders; glutamate modulation for obsessive-compulsive disorder (“OCD”) and spinocerebellar ataxia ("SCA"); myostatin inhibition for neuromuscular and metabolic diseases, including spinal muscular atrophy ("SMA") and obesity; and antibody recruiting, bispecific molecules and antibody drug conjugates ("ADCs") for cancer.

Separation from Biohaven Pharmaceutical Holding Company Ltd.

On October 3, 2022, Biohaven Pharmaceutical Holding Company Ltd. (the “Former Parent”) completed the distribution (the “Distribution”) to holders of its common shares of all of the outstanding common shares of Biohaven Ltd. and the spin-off of Biohaven Ltd. from the Former Parent (the “Spin-Off”) described in Biohaven’s Information Statement attached as Exhibit 99.1 to Biohaven’s Registration Statement on Form 10, as amended (Reg. No. 001-41477). Collectively, we refer to the Distribution and Spin-Off throughout this Annual Report on Form 10-K as the "Separation." As a result of the Separation, Biohaven Ltd. became an independent, publicly traded company as of October 3, 2022, and commenced regular way trading under the symbol “BHVN”’ on the New York Stock Exchange (the "NYSE") on October 4, 2022. Where we describe historical business activities in this report, we do so as if the Former Parent’s activities related to such assets and liabilities had been performed by the Company.

Product Candidates

The following table summarizes our programs for our product candidates. We hold the worldwide rights to substantially all of our product candidates.



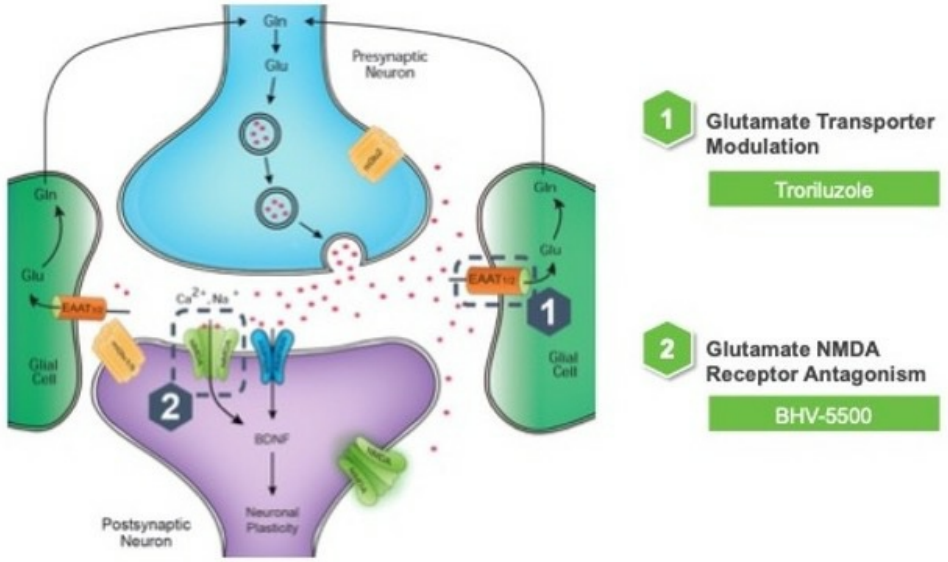
Glutamate Modulation Platform

The most advanced product candidate from our glutamate receptor antagonist platform is troriluzole (previously referred to as trigriluzole and BHV-4157), which is currently in two Phase 3 trials in OCD and, for which the Company

submitted a new drug application (“NDA”) in Spinocerebellar Ataxia Type 3 (“SCA3”) to the U.S. FDA and marketing authorisation application ("MAA") to the European Medicines Agency ("EMA"). Troriluzole is also being evaluated by the Global Coalition for Adaptive Research ("GCAR") as part of Glioblastoma Adaptive Global Innovative Learning Environment - NCT03970447 ("GBM AGILE"), a revolutionary patient-centered, adaptive platform trial for registration that tests multiple therapies for patients with newly-diagnosed and recurrent glioblastoma ("GBM"). Other product candidates include BHV-5500, which is an antagonist of the glutamate N-methyl-D-aspartate (“NMDA”) receptor and its oral prodrug BHV-5000.

Glutamate is an important neurotransmitter present in over 90% of all brain synapses. Glutamate plays an essential role in normal brain functioning and its levels must be tightly regulated. Abnormalities in glutamate levels can disrupt nerve health and communication, and in extreme cases may lead to nerve cell death. Nerve cell dysfunction and death leads to devastating diseases, including ataxia, amyotrophic lateral sclerosis (“ALS”) and other neurodegenerative disorders. Glutamate clearance is necessary for proper synaptic activation and to prevent neuronal damage from excessive activation of glutamate receptors. Excitatory amino-acid transporters (“EAATs”) help regulate glutamate clearance, and are responsible for most of the glutamate uptake within the brain.

The mechanism of action of our glutamate platform is depicted below. Glutamate must be tightly regulated once released from a pre-synaptic neuron. It acts as a signaling neurotransmitter to stimulate the post-synaptic neuron via glutamate receptors (e.g., NMDA, alpha-amino-3-hydroxy-5-methyl-4-isoxazole-propionic acid (“AMPA”) or Kainate receptors). Glial cells surrounding the synaptic junction are predominantly responsible for clearing glutamate through transporters, specifically the EAATs. There are five distinct types of glutamate transporters. The figure below depicts the areas of modulation that are affected by our product candidates.



Adapted from *Glutamate abnormalities in obsessive compulsive disorder: Neurobiology, pathophysiology, and treatment,* C. Pittenger,

M. Bloch, and K. Williams

(1) As depicted in the glial cell to the right in the figure, troriluzole increases the activity and expression of the EAATs to increase the clearance of glutamate released from the pre-synaptic neuron. Troriluzole also inhibits presynaptic ion channels that may inhibit the release of glutamate from presynaptic neurons.

(2) As depicted in the postsynaptic neuron to the bottom of the figure, BHV-5500 blocks glutamate signaling that is mediated by post-synaptic NMDA receptors. Modulating glutamate also has the potential to be neuroprotective and increase the release of neurotrophic factors, including brain derived neurotrophic factor (“BDNF”) which are endogenous molecules that help to support the survival of existing neurons, and encourage the growth and differentiation of new neurons and synapses.

Glutamate Transporter Modulation

Abnormal glutamate release or dysfunction of glutamate clearance can cause overstimulation of glutamate receptors which can lead to a dangerous neural injury called excitotoxicity, which has been associated with a wide range of neurodegenerative diseases. The FDA has approved anti-excitotoxicity drugs that act on the glutamatergic system by blocking NMDA receptors, such as memantine (“Namenda”) for Alzheimer’s disease, lamotrigine (“Lamictal”) for epilepsy and bipolar disorder and riluzole (“Rilutek”) for ALS. Although these drugs show the therapeutic potential of glutamate receptor antagonists and other glutamate modulators in the treatment of a range of neurological diseases, these approved drugs have serious side effects and other drawbacks that we have attempted to solve with our development of troriluzole.

Troriluzole

Troriluzole is a new chemical entity (“NCE”) and tripeptide prodrug of the active metabolite, riluzole. Based on its mechanism of action, preclinical data and clinical studies, troriluzole has potential for therapeutic benefit in a range of neurological and neuropsychiatric illnesses. Initial development has focused on its use in treating SCA, an orphan neurological indication that currently has no approved drug therapies and for which the active metabolite, riluzole, has demonstrated preliminary efficacy in two prior randomized controlled trials conducted by third parties.

Ristori et al. reported a randomized, double-blind, placebo-controlled trial of 40 patients presenting with cerebellar ataxias of diverse etiologies, including SCA. Subjects were randomized to receive 8 weeks treatment with either placebo or riluzole (50 mg Riluzole tablets, twice daily). Statistically significant improvement in the riluzole treated group was demonstrated on the International Cooperative Ataxia Rating Scale (“ICARS”). The number of patients with a 5-point ICARS drop was higher in the riluzole group than in the placebo group after 4 weeks (9/19 vs 1/19; odds ratio [“OR”] =16.2; 95% confidence interval [“CI”] 1.8–147.1) and 8 weeks (13/19 vs 1/19; OR = 39.0; 95% CI 4.2– 364.2). The mean change in the riluzole group ICARS after treatment revealed a decrease ($p < 0.001$) in the total score (-7.05 [4.96] vs 0.16 [2.65]).

Romano et al. described results of a second randomized, placebo-controlled trial in subjects diagnosed with a hereditary ataxia (including SCAs) randomized to receive 12 months of treatment with either placebo or riluzole (50 mg, twice daily). 60 patients were randomized. Statistically significant improvement in the riluzole treated group was demonstrated on the Scale for the Assessment of Ataxia (“SARA”). The proportion with decreased SARA score was 14 (50%) of 28 patients in the riluzole group versus three (11%) of 27 in the placebo group (OR 8.00, 95% CI 1.95– 32.83; p=0.002).

We acquired troriluzole from ALS Biopharma, LLC (“ALS Biopharma”) and Fox Chase Chemical Diversity Center, Inc. (“FCCDC”), along with an estate of over 300 prodrugs. A prodrug is a compound that, after administration, is metabolized in the body into an active drug. Troriluzole is actively transported by virtue of recognition of its tripeptide moiety by the PepT1 transporter in the gut and is responsible for the increased bioavailability of the drug. Once inside the body, the prodrug, troriluzole is cleaved by enzymes in the blood to the parent, riluzole. To mitigate the limitations of riluzole, several classes of prodrugs were designed, synthesized, and evaluated in multiple in vitro stability assays that predict in vivo drug levels. Troriluzole is a third generation of prodrug development and the product of six years of intensive chemistry efforts.

Riluzole is currently only indicated for ALS and has a number of non-desirable attributes that have limited its clinical use. Key limitations of riluzole include poor oral bioavailability, difficulty swallowing due to tablet formulation, food reducing efficacy, liver toxicity, pharmacokinetic variability, and oral numbness.

The prodrug design and selected administration pathway that was pursued with troriluzole is intended to address all of these limitations of riluzole. In addition, a prodrug can be engineered to enhance absorption and protect from diminished absorption when taken with meals. The troriluzole preclinical development strategy was based on optimizing in vivo and in vitro features, such as stability in gastrointestinal and stomach fluids; stability in liver microsomes; limiting off-target effects (particularly liver effects); metabolic cleavage in the plasma to release the active moiety; and enhanced gastrointestinal absorption properties. In in vivo studies in rodents, the intended benefits of this optimization program were observed, including delayed peak concentrations and greater exposure.

After six years of chemistry development and preclinical testing, the resulting lead prodrug from the chemistry program was troriluzole. Troriluzole is chemically comprised of riluzole linked via an amide bond to a tripeptide that is a substrate for PepT1 and which contributes to its improved bioavailability. The tripeptide moiety is cleaved by plasma aminopeptidases. We believe that the estate of compounds we acquired, combined with our internally developed intellectual property, will provide a significant protection for our innovations. The safety and tolerability of troriluzole has now been demonstrated in approximately 2000 subjects in early- and late-stage clinical studies.

Our Clinical Program for Troriluzole

Phase 1 Studies with Troriluzole

In July 2016, we began a Phase 1 randomized, double-blind, placebo-controlled study to evaluate the safety, tolerability and pharmacokinetics (“PK”) of single and multiple ascending doses of troriluzole in normal healthy volunteers. 58 healthy volunteers were dosed with troriluzole and 20 were dosed with placebo. Both single and multiple doses up to 200 mg were well tolerated without evidence of novel, clinically significant safety signals or lab abnormalities. There was no apparent dose response regarding the frequency or severity of adverse events (“AEs”). In the blinded group, including subjects treated with both placebo and troriluzole, the most common AEs were headache (five subjects, two with moderate severity and three with mild severity) and constipation (two subjects). No pattern of AEs or lab abnormalities were apparent to provide specific cautions or to suggest cautions beyond what is appropriate for the active metabolite, riluzole. Commencing in December 2017, an additional single and multiple dose study was conducted to assess the safety, tolerability and PK of a 280 mg dose in 10 healthy young and elderly volunteers (eight active; two placebo). The results supported adequate safety and tolerability and yielded mean exposures comparable to what would be expected from a 200 mg dose, a dose that has been safely used in clinic populations and associated with efficacy in a range of disorders in randomized controlled trials (Huntington Study Group Neurology 2003; Lacomblez Neurology 1996). In addition, a bioequivalence study was conducted to bridge a commercial formulation with a Phase 2/3 formulation in 32 healthy volunteers. The commercial formulation was well-tolerated and provided bioequivalent exposure with the Phase 2/3 formulation.

Troriluzole for SCA

Spinocerebellar Ataxias ("SCAs") are a group of ultra-rare, dominantly inherited neurodegenerative disorders predominantly characterized by atrophy of the cerebellum, brainstem, and spinal cord. The disease course of SCA is one of relentless progression over years and inevitably leads to clinical deterioration of motor function, gait imbalance with frequent falling, severe speech impairment, swallowing difficulties, and premature death. SCAs are thought to be pathogenetically related but disease course and brain region involvement are known to vary between the different genotypes. SCA3, also known as Machado-Joseph disease, is the most common genotype, with a prevalence of up to 6,000 in North America and 4,600 in the European Union (“EU”) and Japan, and accounts for approximately 30% to 50% of SCAs worldwide. Currently, there are no approved symptomatic or neuroprotective treatments for SCA.

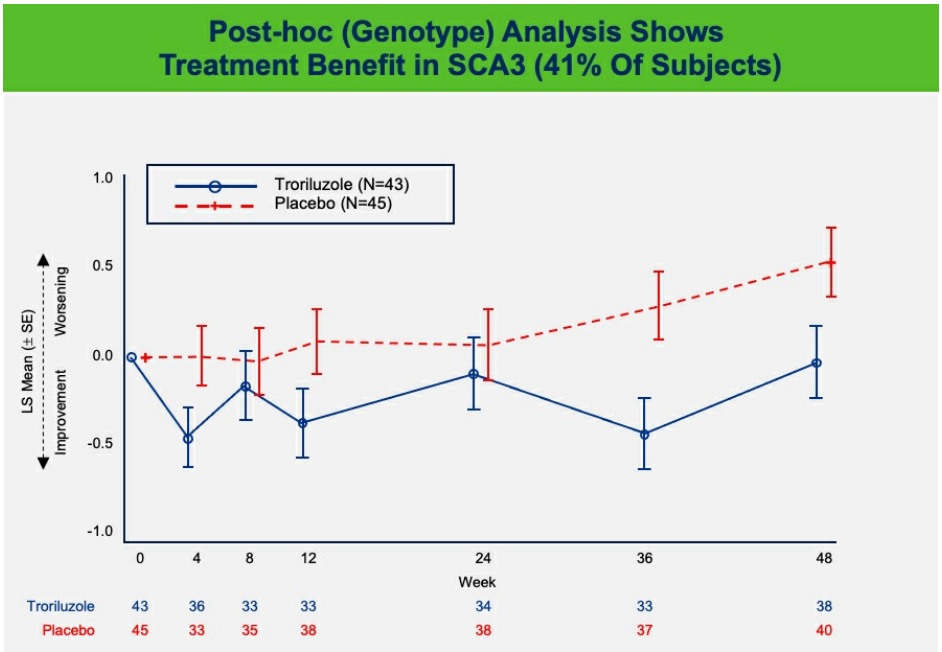
Based on the results of our Phase 1 trial with troriluzole and two third-party academic trials (Ristori et al 2020, published in Neurology in 2010 and Romano et al 2015, published in The Lancet in 2015) that have shown preliminary efficacy of riluzole in cerebellar ataxias, we advanced troriluzole into development for SCA. Initially, we conducted a Phase 2b/3, randomized, double-blind, placebo-controlled, parallel-group study to assess the safety and efficacy of troriluzole over 8 weeks in subjects with SCA (Study BHV4157-201). In October 2017, we announced that troriluzole at a dose of 140 mg once daily (“QD”) did not differentiate from placebo on the primary endpoint of the mean change from baseline on the SARA total score after 8 weeks of treatment. After eight weeks of treatment, troriluzole treated subjects (n = 64) demonstrated an improvement of –0.81 points [95% CI: –1.4 to –0.2] on the SARA versus –1.05 points [95% CI: –1.6 to –0.4] improvement in placebo-treated (n = 68), p-value = 0.52. In this trial, we observed a favorable safety and tolerability profile of troriluzole, with no drug-related serious adverse events (“SAEs”) and low discontinuation rates due to AEs. During open-label treatment over the open-label extension phase, however, troriluzole did show slowing of disease progression in troriluzole-treated subjects in contrast to the measurable decline expected as compared to a matched cohort of untreated patients from the US natural history study (Clinical Research Consortium for Spinocerebellar Ataxias (“CRC-SCA”)).

Based on our learnings from the proof of concept Study BHV4157-201, including analyses from the open-label extension phase, we advanced troriluzole into a pivotal Phase 3, randomized, double-blind, placebo-controlled, parallel-group study to assess the safety and efficacy of troriluzole over 48 weeks in subjects with SCA (Study BHV4157-206). Randomization was stratified by genotype (consisting of these three groupings: SCA1 and SCA2; SCA3; and SCA6, SCA7, SCA8, and SCA10) in order to ensure balance within each of these subgroups. We enriched this trial with specific SCA genotypes, extended the treatment period of this trial to 48 weeks, implemented the use of a modified SARA scale (“f-SARA”), and increased the dose of troriluzole to 200 mg QD. Notably, the f-SARA is a novel, 16-point scale developed in collaboration with FDA and key opinion leaders as the primary outcome measure for this trial; the scale was designed to limit subjectivity of the scale and focus on functional aspects of the disease so that significant changes would reflect a clinically meaningful change in function.

In May 2022, the Company announced top-line results from the Phase 3 clinical trial evaluating the efficacy and safety of its investigational therapy, troriluzole, in adult patients with SCA. The primary endpoint, change from baseline to week 48 on the f-SARA, did not reach statistical significance in the overall SCA population as there was less than expected disease progression in the placebo arm over the course of the study. In the overall study population (n = 213), the troriluzole and placebo groups each had mean baseline scores of 4.9 on the f-SARA and the two groups showed minimal change at the 48-week endpoint with f-SARA scores of 5.1 and 5.2, respectively (p=0.76). Preliminary post hoc analysis of

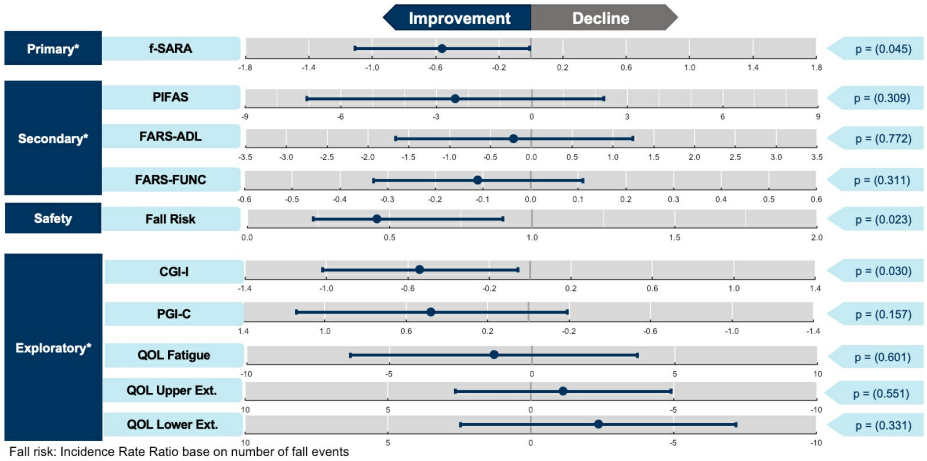
efficacy measures by genotype suggested a treatment effect in patients with the SCA3 genotype. A risk reduction in falls was also observed in the SCA3 population, as well as across all SCA genotypes. Troriluzole was well tolerated with an adverse event profile similar to placebo.

In May 2023, the Company presented further analysis of Study BHV4157-206 (summarized in the figure below) by prespecified genotype strata that revealed consistent treatment effects of troriluzole in SCA3, the most common genotype worldwide, which represented 41% of study participants. In SCA3 subjects, troriluzole 200mg QD demonstrated benefit on the f-SARA compared with placebo at 48 weeks (LS mean treatment difference = -0.56; 95% CI = -1.11, -0.01; p = 0.0450). This genotype analysis was post hoc as the All SCA study population (SCA1, SCA2, SCA3, SCA6, SCA7, SCA8, SCA 10) was the mITT population for the primary analysis. Results for the SCA3 group are based on a model with no covariates with fixed effects for treatment, visit, and visit-by-treatment interaction; p-values are descriptive.



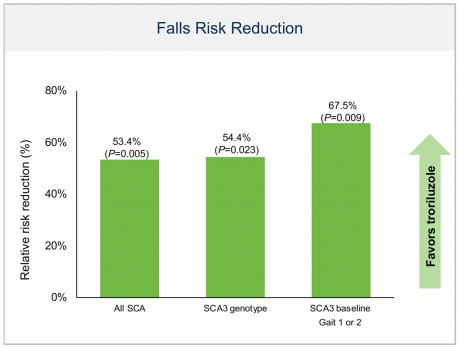
In addition to the beneficial effects observed on the f-SARA, the forest plot below demonstrates a consistent treatment benefit of troriluzole in SCA3 genotype subjects across multiple prespecified primary, secondary, and

exploratory study outcome measures. The SCA3 genotype analysis of the clinician- and patient-rated endpoints in the table represent all of the prespecified endpoints in the study protocol.



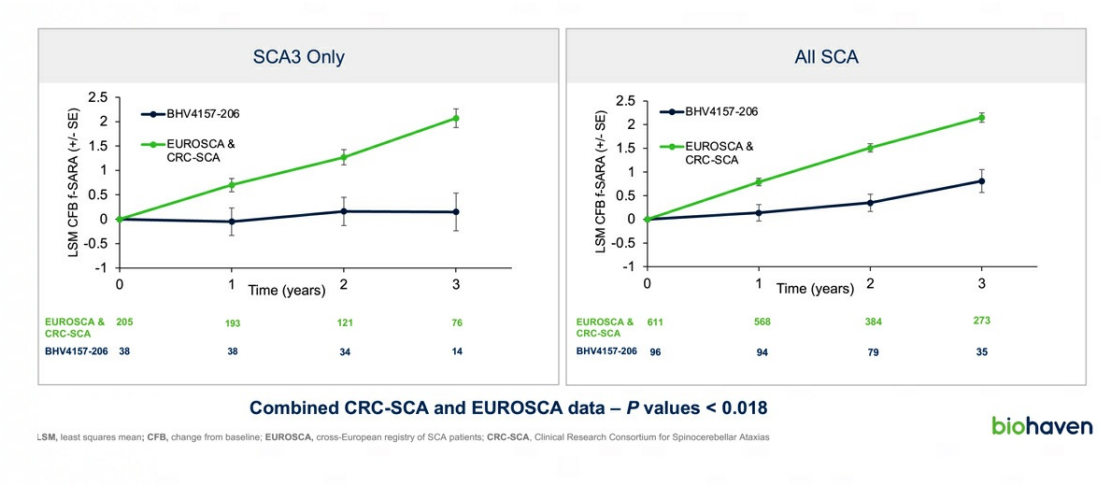
Fall risk: Incidence Rate Ratio base on number of fall events

As shown in the figure below, safety data from Study BHV4157-206 showed that troriluzole-treated subjects showed a substantial risk reduction in falls in the All SCA and SCA3 genotype study populations. Treatment with troriluzole for 48 weeks reduced the risk of fall events by approximately 53% in subjects in the overall (All SCA) population (p = 0.005), by approximately 54% in subjects in the SCA3 population (p = 0.023), and by approximately 68% in subjects with SCA3 who were ambulatory (i.e., baseline Gait 1 or 2) (p = 0.009). This analysis, demonstrating that subjects who were more ambulatory with less severe disease at baseline were more likely to show a benefit from troriluzole, is consistent with the early treatment paradigm for other neurodegenerative diseases.

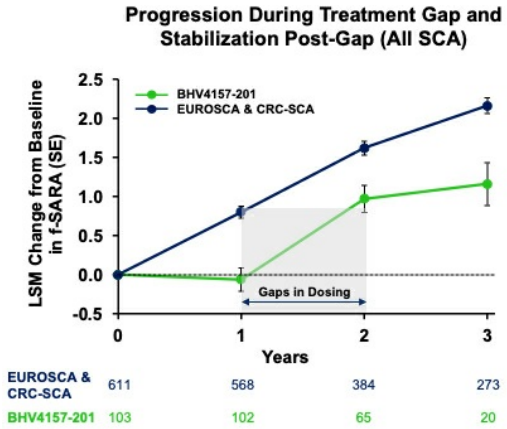


Two independent natural history cohorts for SCA, one in the US (CRC-SCA) and one in Europe (EUROSCA), have characterized disease progression in this genetically defined neurodegenerative disorder. A Matching Adjusted Indirect Comparison (“MAIC”) was performed to match natural history subjects to subjects in BHV4157-206. As demonstrated in the figure below, at years 1, 2, and 3 change from baseline in f-SARA scores was significantly better among troriluzole patients vs the matched natural history referent (combined CRC-SCA and cross-European registry of SCA patients

("EUROSCA") data - all p values ~0.0004). These results were consistent for both SCA3 only and All SCA analyses. Validation metrics from f-SARA confirm that these changes are clinically relevant and meaningful to patients.



As seen in the figure below, an additional comparative study was conducted using OLE data from up to 3 years with subjects from BHV4157-201. Using a matched control pooled from the same two independent natural history studies (US and European), troriluzole-treated All SCA subjects showed clinically relevant benefit compared to expected progression from the external control at 1 year, 2 years, and 3 years. Of note, there was an administrative gap in treatment in BHV4157-201 between the end of the 48-week OLE and the resumption of treatment at the start of the 96-week period. This gap was observed to result in a decline in f-SARA scores. However, after resumption of treatment patients re-stabilized (did not progress) resulting in the estimation of compelling treatment effects at years 2 and 3.



Given these findings and the debilitating nature of SCA, in May 2023 we announced that we submitted a New Drug Application ("NDA") to the FDA for troriluzole for the treatment of SCA3. In July 2023, the FDA informed us that it would not review the recently submitted NDA application for troriluzole given that the study's primary endpoint was not met and thus, would not permit a substantive review. In followup to the regulatory decision on the NDA application, we held followup meetings with the FDA regarding the SCA data. We continue to have constructive dialogue with the FDA regarding our SCA development program and potential future data analyses to address regulatory concerns in the previously issued refuse-to-file decision on it's NDA application for SCA3. We will provide further updates on the SCA development program as warranted by any continued positive progress from the outcome of future regulatory interactions

on this topic. In October 2023, the European Medicines Agency ("EMA") informed us that our Marketing Authorization Application ("MAA") for troriluzole (Dazluma) in the treatment of SCA has been validated and is now under review by EMA's Committee for Medicinal Products for Human Use ("CHMP"). We remain committed to working closely with the health authorities to bring troriluzole to people with SCA3, given no therapy is currently approved for this ultra-rare genetic disorder.

Troriluzole for OCD

OCD is a chronic neuropsychiatric disorder characterized by symptoms of obsessions (intrusive thoughts) and compulsions (repetitive behaviors) that can interfere with patients' functional abilities. According to the National Institute of Mental Health, the 12-month prevalence of OCD is 1% of the U.S. adult population, and approximately half of these cases are characterized as severe. First-line treatment for OCD includes cognitive behavioral therapy, selective serotonin reuptake inhibitors ("SSRIs") and adjunctive use of atypical antipsychotics. Nonetheless, up to 60% of patients have an inadequate response to conventional intervention strategies and some seek invasive neurosurgical procedures to ameliorate symptoms.

We are currently developing troriluzole as a potential treatment option for patients suffering from OCD. Despite the significant public health burden, no novel mechanisms of action have been approved by the FDA for OCD in over two decades. The rationale for use of troriluzole in OCD is supported by clinical data with its active metabolite, riluzole, in populations with OCD in open-label and placebo-controlled clinical trials as well as in preclinical, genetic and neuroimaging studies implicating the glutamatergic hyperactivity in the pathogenesis of OCD.

In multiple case studies, the use of riluzole in patients with refractory OCD has commonly been associated with meaningful improvement of symptoms. A small-scale randomized controlled trial in adults with OCD conducted by a third party showed favorable trends for the use of riluzole in an outpatient setting. Another randomized controlled third-party study demonstrated statistically significant therapeutic effects with the adjunctive use of riluzole as compared to adjunctive placebo in 50 adults with refractory OCD. These clinical effects are consistent with findings such as genetic associations of glutamate transporter genes with OCD and increased glutamate concentrations in brain and cerebrospinal fluid of patients with OCD. Taken together, we believed there was a clear rationale for advancement of troriluzole, a prodrug of riluzole, into a Phase 2 proof-of-concept trial in OCD.

We commenced a Phase 2/3 double-blind, randomized controlled trial on the use of troriluzole in adults with OCD in late 2017. Results from the Phase 2/3 trial were announced in June 2020. Troriluzole 200 mg administered once daily as adjunctive therapy in OCD patients with inadequate response to standard of care treatment showed consistent numerical improvement over placebo on the Yale-Brown Obsessive Compulsive Scale ("Y-BOCS") at all study timepoints (weeks 4 to 12) but did not meet the primary endpoint at week 12. Troriluzole treated subjects (n = 111) had a mean Y-BOCS improvement of -3.4 points from baseline versus -2.9 for placebo-treated (n = 115) subjects [difference -0.5 and p-value = 0.451] at week 4, -5.1 points (n = 96) versus -3.6 for placebo-treated (n = 108) subjects [difference -1.5 and p-value = 0.041] at week 8, and -5.9 points (n = 99) versus -4.9 for placebo-treated (n = 102) subjects [difference -1.0 and p-value = 0.220] at week 12. Troriluzole's safety profile was generally consistent with past clinical trial experience with its active metabolite, riluzole. Treatment emergent adverse events ("TEAEs") were mostly reported to be mild in intensity. TEAEs that occurred in at least 5% of patients in the troriluzole group, and more frequently in the troriluzole group than in the placebo group, were headache, dizziness, fatigue, somnolence, nausea and nasopharyngitis.

Given the strong signal in the Phase 2/3 proof of concept study and after receiving feedback from the FDA in an End of Phase 2 meeting, in December 2020, we initiated enrollment in a Phase 3 program. The Phase 3 program will have an estimated total enrollment of up to 700 participants in each trial with a primary endpoint of change from baseline on the Y-BOCS total score at week 4, 8 and 10. The two Phase 3 randomized, double-blind, placebo-controlled trials that make-up our Phase 3 program for OCD are currently ongoing.

In January 2024, we announced plans to conduct a pre-planned interim analysis ("IA") to evaluate efficacy in the first of our two Phase 3 studies in OCD. The IA is planned to be conducted by an independent Data Monitoring Committee, after 350 subjects have had the opportunity to complete the study (representing approximately 70% of subjects in the primary analysis population reach primary endpoint). The IA is powered to detect a clinically meaningful difference between troriluzole and placebo on the Y-BOCS primary outcome that is based on the effect size observed in the Phase 2 proof of concept ("POC") study. The potential results of the IA include (1) a highly statistically significant evidence of efficacy leading to stopping the study or (2) continuation of the study, with possible sample size re-estimation. Last Patient Last Visit and database lock for the IA are anticipated in the first quarter of 2024, with expected IA topline results in the second quarter of 2024.

Troriluzole for Glioblastoma

Preclinical and small-scale pilot studies are underway to explore troriluzole's use in the treatment of a pipeline of other indications such as some cancers whose spread is thought mediated by glutamate transmission, such as melanoma and GBM.

In collaboration with Johns Hopkins University, we explored the potential applicability of troriluzole for GBM. The oncology collaboration with Johns Hopkins was based upon the mechanistic rationale that some tumors over express glutamate receptors, the central role that glutamate may have in cancer metabolism and the effect of glutamate on the tumor microenvironment.

In December 2021, GCAR selected troriluzole for evaluation in GBM AGILE. GBM AGILE is a revolutionary patient-centered, adaptive platform trial for registration that tests multiple therapies for patients with newly-diagnosed and recurrent GBM, the most fatal form of brain cancer. Troriluzole will be evaluated in all patient subgroups of the trial which include newly-diagnosed methylated O6-methylguanine DNA methyltransferase ("MGMT"), newly-diagnosed unmethylated MGMT, and recurrent GBM. Troriluzole was selected for inclusion in GBM AGILE based on compelling evidence showing deregulation of glutamate in GBM. The therapeutic potential of troriluzole in GBM and other oncology indications is supported by several recent clinical and translational research studies conducted with troriluzole and its active moiety. For example, Medikonda et al. showed a survival benefit with troriluzole, alone and in combination with anti-programmed cell death protein-1 ("PD-1") immunotherapy, utilizing a frequently used murine brain tumor model. C57BL/6J mice were intracranially implanted with luciferase-tagged GL261 glioma cells. Mice were randomly assigned to the control, anti-PD-1, troriluzole or combination anti-PD-1 plus troriluzole treatment arms, and median overall survival was assessed. The troriluzole treatment arm demonstrated improved survival compared with the control arm (median survival of 36% vs. 0%; $p < 0.0001$), as did the combination anti-PD-1 plus troriluzole treatment arm (overall survival of 80% vs. 0; $p = 0.0007$).

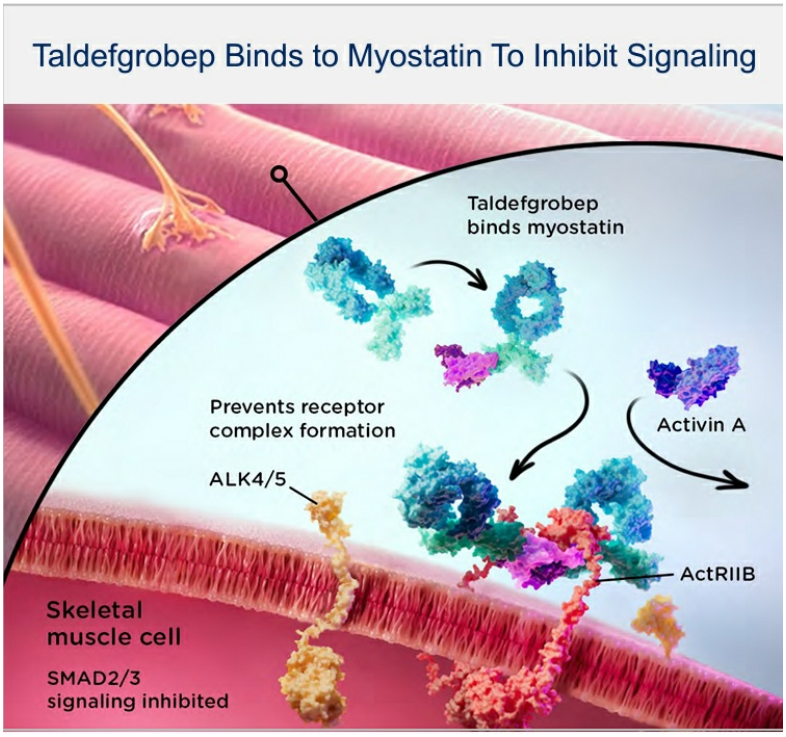
In July 2022, the Company and GCAR announced that enrollment has commenced in GBM AGILE for the evaluation of troriluzole. GBM AGILE is a multi-arm, platform trial. The evaluation of each therapy in GBM AGILE proceeds in 2 possible stages. A therapy's Stage 1 is an adaptively randomized screening stage for evaluating the therapy within patient signatures compared against a common control. A therapy in Stage 1 will stop accruing patients if it reaches its maximal sample size, drops for futility, or evinces inadequate safety. If a therapy reaches an efficacy threshold for graduation from Stage 1, it will move into Stage 2 within one of the prospectively defined signatures. The maximum sample size in Stage 1 is 150 patients. For a therapy graduating to Stage 2 there is a fixed randomization, expansion cohort. The maximum sample size in Stage 2 is 50 experimental patients in the graduating signature. The primary analysis of a regimen's effect on overall survival uses all patients in both its stages and all control patients in the trial in the graduating signature, suitably adjusted for any possible time trends. Enrollment in the study is ongoing.

Myostatin Platform

Taldefgrobep Alfa (BHV-2000)

In February 2022, we announced a worldwide license agreement with BMS for the development and commercialization rights to taldefgrobep alfa (also known as BMS-986089), a novel Phase 3 asset. Myostatin, a negative regulator of muscle growth, is a key member of the Transforming Growth Factor ("TGF-β") superfamily. Taldefgrobep's novelty in a field of myostatin inhibitors is based on the mechanism where it binds to myostatin to both lower overall free myostatin levels, but also to function as a receptor antagonist to block myostatin signaling in skeletal muscles. Recently generated data has shown that the taldefgrobep alfa-myostatin complex is stable, and blocks myostatin signaling to both myostatin and to a lesser extent to activin A potentially for a protracted period after cessation of dosing. Blocking myostatin activity and signaling has shown to improve muscle function and strength in a number of disease models for neuromuscular wasting along with physical and metabolic changes important to individuals living with overweight and obesity. Blocking activin contributes to decreased adipose tissue and improved glucose homeostasis. Clinical studies have confirmed that taldefgrobep improved lean body mass directly through increase on contractile muscle and loss of adipose tissue as demonstrated in both normal healthy volunteers and in patients with Duchenne muscular dystrophy ("DMD"). The mechanism of increasing overall muscle size and reducing adipose tissue volume provide development opportunities in both neuromuscular disease and individuals living with overweight and obesity.

The advanced taldefgrobep alfa anti-myostatin development program offers extensive human safety data, especially in the pediatric population.



Previous Clinical Trials

Taldefgrobep was previously studied by Bristol Meyers Squibb ("BMS") and Roche in 4 completed clinical studies in healthy volunteers and subjects with DMD. An estimated 360 subjects received taldefgrobep; 179 healthy subjects and 181 subjects with DMD.

Completed clinical pharmacology studies in healthy adult subjects included:

- CN001001 was a randomized, placebo-controlled, Phase 1 study designed to evaluate the safety, tolerability, PK, and PD of single and multiple ascending subcutaneous doses of taldefgrobep (vial) in healthy adults.
- CN001023 was a randomized, open-label, single dose, parallel-group, Phase 1 study designed to compare the bioavailability of subcutaneous injections in the arm, thigh, and abdomen and to evaluate the safety, tolerability, PK, and immunogenicity of taldefgrobep (pre-filled syringe) in healthy adults.

Completed clinical studies in subjects with DMD included:

- CN001006 was a multi-site, randomized, placebo-controlled, double-blind, dose-ranging, Phase 1b/2 study to evaluate the safety, tolerability, and PK of taldefgrobep in ambulatory boys with DMD aged ≥ 5 to < 11 years.
- CN001016 was a randomized, double-blind, placebo-controlled, Phase 2/3 study to assess the efficacy, safety, and tolerability of 2 doses of taldefgrobep in ambulatory boys with DMD aged ≥ 6 to < 12 years.

All studies supported the safety and tolerability of taldefgrobep with fixed doses from 35 mg up to 180 mg administered weekly, subcutaneously ("SC"). The pharmacokinetics and safety data from the Phase 1 studies supported the continued development with doses of 35 mg and 50 mg administered weekly SC.

In the Phase 3 Clinical Study, CN001016, a futility analysis based on this primary endpoint, conducted after approximately 30% of subjects had completed 48 weeks of study drug treatment, did not show any statistically significant

treatment differences. A summary of the development and outcome from these studies are published (Neurol Ther. 2024 Feb;13(1):183-219).

There are significant differences between a neuromuscular (DMD) and neurodegenerative disease (SMA). The differences in residual muscle in SMA along with adjunctive survival of motor neuron ("SMN") upregulation therapy support the continued development of taldefgrobep alfa in SMA.

Spinal Muscular Atrophy

SMA is a rare genetic neurodegenerative disorder characterized by the loss of motor neurons, atrophy of the voluntary muscles of the limbs and trunk and progressive muscle weakness that is often fatal and typically diagnosed in young children. The underlying pathology of SMA is caused by insufficient production of the survival of motor neuron ("SMN") protein, essential for the survival of motor neurons, and is encoded by two genes, SMN1 and SMN2. In the U.S., SMA affects approximately 1 in 11,000 births, and about 1 in every 50 Americans is a genetic carrier. Newborn screening is now available in 48 U.S. states and covers over 94% of all births.

Our Clinical Trial for Taldefgrobep Alfa in SMA

In September 2023, we completed enrollment in a Phase 3 clinical trial assessing the efficacy and safety of taldefgrobep alfa in SMA. The Phase 3 placebo-controlled, double-blind trial is designed to evaluate the efficacy and safety of taldefgrobep as an adjunctive therapy for participants who are already taking a stable dose of nusinersen or risdiplam or have a history of treatment with onasemnogene abeparvovec-xioi, compared to placebo. The primary outcome measures of the study will be efficacy of taldefgrobep alfa compared to placebo in the change in the 32 item Motor Function Measure ("MFM-32") total score from baseline to Week 48. Scores range from 0-3 on each item, with higher scores indicating higher functioning. The study is neither restricted nor limited to patients based on ambulatory status or classification of SMA and is designed to randomize approximately 180 patients in this randomized, double-blind, placebo-controlled global trial. We expect to report topline data from our Phase 3 study in the second half of 2024.

In February 2023, we received Fast Track designation from the FDA for taldefgrobep alfa for the treatment of SMA. Fast Track designation enables important new drugs to reach patients earlier by facilitating more frequent communications with the FDA and expeditious review of a drug which treats a serious condition and fills an unmet medical need. In December 2022, we received orphan drug designation from the FDA for taldefgrobep in the treatment of SMA and from the European Commission in July 2023.

Taldefgrobep Alfa's Role in Spinal Muscular Atrophy

In the past three years, significant advancements were made to address the underlying cause of disease in SMA with the up-regulation of SMN1 and SMN2 expression which positions taldefgrobep as a potential combination therapy to enhance muscle performance. Data from both an SMA animal model study that shows advantages of combination SMN therapy with taldefgrobep and the extensive clinical data in DMD support the advancement of taldefgrobep into a SMA Phase 3 study. Other indications in muscle wasting diseases may be a fast follow-on for taldefgrobep along with other life-cycle opportunities.

Metabolic Disorders

Obesity is a disease of excess and/or abnormal deposits of adipose tissue and a current global public health crisis. By 2030, it is expected that nearly one billion people will be living with obesity, including 50% of the adult and 25% of the adolescent US population. The primary driver of obesity-related morbidity and mortality is metabolically active visceral adipose tissue and associated deposits of adipose tissue in and around organs such as the heart, liver, kidneys, and muscle.

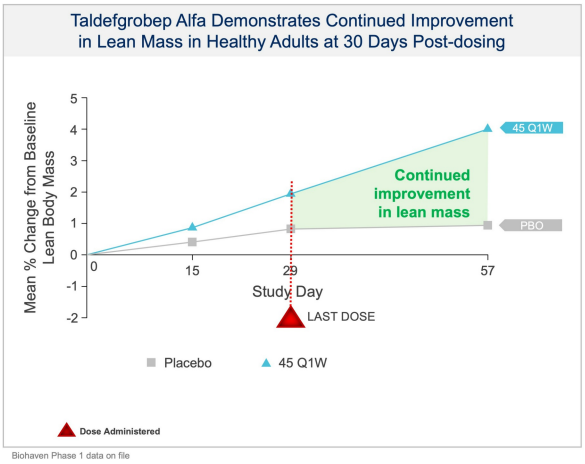
Taldefgrobep Alfa's Role in the Management of Overweight and Obesity

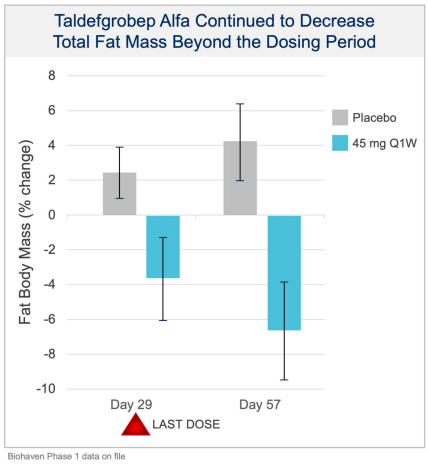
Preclinical and clinical data have demonstrated the potential for anti-myostatin therapies to produce physical and metabolic changes that are highly relevant to individuals living with overweight and obesity, including reducing total body fat and visceral adiposity, and improving insulin sensitivity and bone mineral density, while increasing lean muscle mass. Taldefgrobep's novel mode of action and unique impact on body composition suggest it could be used as monotherapy or in combination with other anti-obesity medications.

In October 2023, we announced preclinical data demonstrating the ability of taldefgrobep to significantly reduce fat mass while increasing lean mass in an obese mouse model. In a mouse model of diet-induced obesity, untreated mice exhibited an increase in fat mass of 31%, while the mice treated with taldefgrobep demonstrated increases in lean mass of 25% from baseline ($p \leq .0.001$) and lost 11% of their baseline fat ($p \leq .0.001$) compared to vehicle (placebo) treated mice. Insulin and leptin levels were consistently lower in mice treated with taldefgrobep compared to the untreated mice. There was no difference in food intake over time across the taldefgrobep and untreated mice, counter to what has been observed with incretin mimetics (e.g., semaglutide) which are consistently associated with a reduction in energy intake.

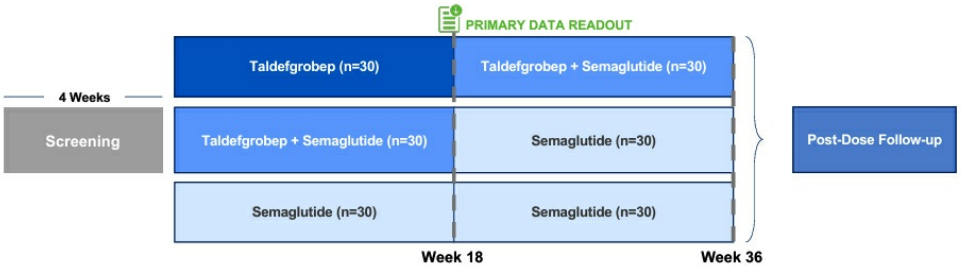
Our Clinical Trial for Taldefgrobep Alfa in Obesity

In a Phase 1 MAD study conducted by BMS in normal healthy volunteers, the cohort using the projected therapeutic 45mg dose of taldefgrobep demonstrated a continued improvement in lean mass and a reduction in fat mass during the 29 day dosing period that continued to increase through the observation period at 4 weeks post dosing (see figure below).





We plan to initiate a Phase 2 clinical trial of taldefgrobep in the management of metabolic disease in the second quarter of 2024. The study will evaluate the ability of taldefgrobep to maintain lean mass muscle as an adjunctive to standard of care GLP-1 therapy in adults living with overweight and obesity (see figure below for anticipated trial design).



Ion Channel Platform

Kv7

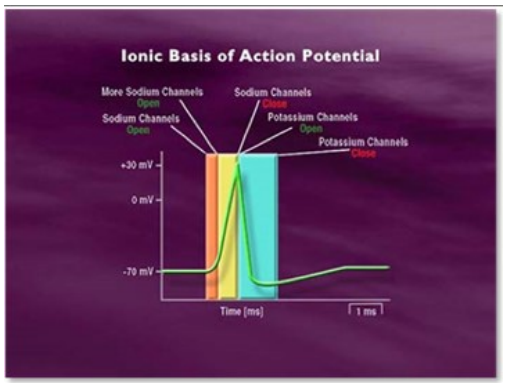
Kv7 Platform Acquisition

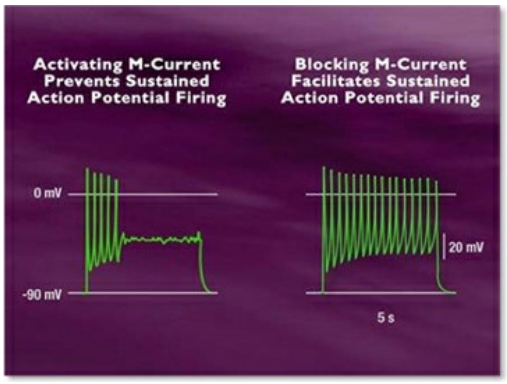
In February 2022, we announced that we entered into a definitive agreement with Channel Biosciences, LLC, a subsidiary of Knopp Biosciences, LLC, to acquire a drug discovery platform targeting Kv7 ion channels, adding the latest advances in ion channel modulation to our growing neuroscience portfolio.

Kv7's Role in Epilepsy, Mood Disorders, and Other Central Nervous System Disorders

Because of their fundamental role in health and their aberrant role in disease, ion channels in cell membranes represent a broad and important class of drug targets. Sodium channels and potassium channels form the ionic basis of the action potential in electrically charged cells throughout the body (see figures below). The Kv7 protein in particular forms a channel that exquisitely regulates the flow of charged potassium ions (K+) across cell membranes, repolarizing nerve cells and resetting them for normal action potential firing. Kv7 channels include a family of channel subtypes, designated as Kv7.1 through Kv7.5, and they are formed by tetramers of identical or compatible subunits. Some of these channel subtypes localize in nerve cells (neurons) while others can be found in cardiac muscle, smooth muscle, and other tissue types.

The Kv7 subunits, Kv7.2 and Kv7.3, are widely expressed in the brain, notably in the cortex and hippocampus, and together they form Kv7.2/7.3 heteromeric channels that produce the M-current (I_{KM}), a critical regulator of neuronal excitability (see figures below). Kv7.2/7.3 channels normally perform a natural "braking" function by regulating the electrical excitability and hyperexcitability of brain cells. Dysfunction of these channels, due to genetic mutations or other factors, increases seizure risk, while augmenting the 'open' activity of these channels has been demonstrated to reduce neuronal hyperexcitability and seizure frequency in electrophysiology laboratories, in animal models, and, most importantly, in patients.





White, Role of Potassium Channel Ions in Epilepsy, Medscape.org

We are synthesizing novel Kv7.2/7.3 activators that improve on the selectivity, potency, and other characteristics of ezogabine (Potiga in the U.S. and Trobalt (retigabine) in Europe), a drug approved in 2011 for the treatment of refractory epilepsy and voluntarily withdrawn from the market in 2017 because of poor tolerability and structure-related toxicities that limited its clinical use, and ezogabine-like compounds, while averting its negative attributes, including off-target activity at a different brain ion channel, gamma-aminobutyric acid ("GABA") A receptor ("$GABA_A$-R").

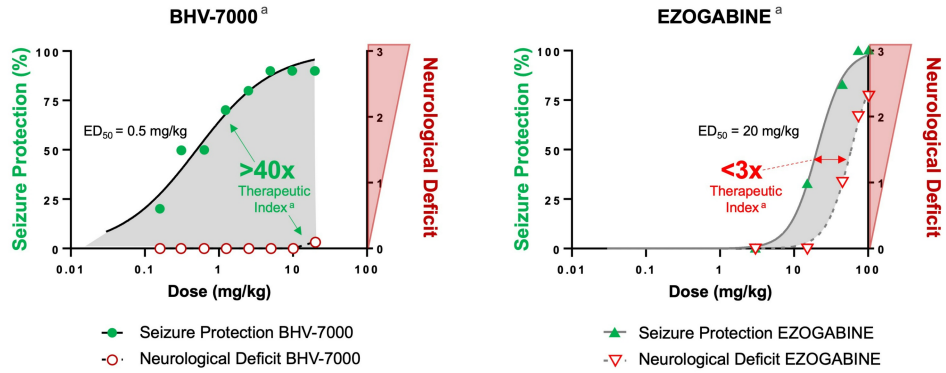
Using a structure-based approach, supplemented by in silico modeling, we have identified structural features of our molecules critical to Kv7 activation. We have applied these analyses to the generation of proprietary chemical leads structurally distinct from known Kv7 activators, including ezogabine and flupirtine, the only other approved Kv7 modulator, approved in Europe for the treatment of acute pain. Our team has synthesized a large library of Kv7-activating molecules and are advancing them according to stringent criteria requiring improvements over ezogabine, including chemical stability, synthetic tractability, the avoidance of structural motifs associated with the generation of reactive metabolites and other unwanted, off-target activity, including $GABA_A$-R activation.

Epilepsy, Major Depressive Disorder ("MDD"), and Bipolar Disorder are the initial indications we are targeting with activators from our Kv7 platform. In addition to their role in treating epilepsy, anti-seizure medications ("ASMs") are often

used in the management of bipolar disorder and Kv7 activators have recently shown great promise in the the treatment of MDD. While the use of ASMs is often accompanied by dose-limiting side effects, our Kv7 activators are specifically designed to target subtypes of Kv7 potassium channels without engagement of $GABA_A$ receptors. The lack of $GABA_A$-R activity potentially gives activators from our Kv7 platform a wide therapeutic window and is expected to result in an improved side effect profile, limiting the somnolence and fatigue often seen in patients receiving ASMs.

BHV-7000

BHV-7000 (formerly known as KB-3061), the lead asset from the Kv7 platform, is a potent Kv7.2/7.3 ion channel activator from a novel, bicyclic imidazole class that is structurally-distinct from other Kv7 modulators (e.g., ezogabine and related aniline compounds) with significant in vivo anticonvulsant activity and a wide therapeutic index. In the most widely used and positively-predictive preclinical model of epilepsy, the maximal electroshock ("MES") model, data for BHV-7000 and ezogabine were collected in independent experiments (see figures below), measuring the activity of both compounds in preventing seizures (ED50) and recording the neurologic deficit five minutes prior to the MES test to calculate the tolerability index ("TI"). The neurologic deficit is a behavioral index ranging from normal activity (score of 0) to a loss of righting reflex (score of 3). As shown below, BHV-7000 was demonstrated to have an ED50 = 0.5 mg/kg with almost no impact on behavior producing a TI > 40x. In a separate rotarod experiment of preclinical tolerability, BHV-7000 had no impact on rat motor behavior up to 30 mg/kg, the highest dose tested. In contrast, ezogabine was 40x less potent (ED50 = 20 mg/kg) in the MES model with a narrow TI < 3x. The narrow preclinical TI for ezogabine is consistent with the clinical experience with the drug where side effects such as somnolence and dizziness limited its use at doses that prevented seizures in patients.



a. Biohaven data on file (2022)

Phase 1 Clinical Development

In the second quarter of 2022, our Clinical Trial Application for BHV-7000 was approved by Health Canada, and we subsequently began phase 1 clinical development. First-in-human single ascending dose ("SAD") and multiple ascending dose ("MAD") studies have now been completed. In the SAD and MAD cohorts, 77 subjects received BHV-7000 (N=58) or placebo (N= 19). Thirty-nine SAD subjects were randomized to BHV-7000 or placebo and thirty-eight MAD subjects were

randomized to BHV-7000 or placebo. The rates of adverse events ("AEs") by MedDRA System Organ Class across the pooled SAD and MAD cohorts among subjects treated with BHV-7000 and placebo are presented in the tables below.

Table 1: Treatment-emergent adverse events ("TEAEs") Occurring in ≥ 2 Subjects Receiving BHV-7000 in SAD Cohorts

	BHV-7000							
AE, n (%)	4 mg n = 6	10 mg n = 6	25 mg (Fasted) n = 6	25 mg (Fed) n = 6	50 mg n = 6	100 mg n = 5	BHV-7000 Overall n = 29	Placebo n = 10
Headache	0	1 (16.7)	1 (16.7)	0	1 (16.7)	0	3 (10.3)	0
Abdominal discomfort	0	1 (16.7)	0	1 (16.7)	0	0	2 (6.9)	0

All AEs reported in the SAD cohorts were mild in severity.

Table 2: TEAEs Occurring in ≥ 2 Subjects Receiving BHV-7000 in MAD Cohorts

	BHV-7000						
AE, n (%)	10 mg n = 5	25 mg n = 6	40 mg n = 6	80 mg[a] n = 6	120 mg[a] n = 6	BHV-7000 Overall[b] n = 29	Placebo[b] n = 9
Headache	0	0	3 (50.0)	1 (16.7)	2 (33.3)	6 (20.7)	3 (33.3)
Back pain	1 (20.0)	0	2 (33.3)	2 (33.3)	1 (16.7)	6 (20.7)	0
Constipation	0	0	1 (16.7)	1 (16.7)	1 (16.7)	3 (10.3)	3 (33.3)
Dizziness	0	0	0	2 (33.3)	1 (16.7)	3 (10.3)	2 (22.2)
Abdominal pain	0	0	0	2 (33.3)	0	2 (6.9)	1 (11.1)
Fatigue	0	0	0	1 (16.7)	1 (16.7)	2 (6.9)	2 (22.2)

All AEs reported in the MAD cohorts were mild in severity, except 1 case of back pain (moderate severity, 40 mg) and 1 case of dizziness (moderate severity, 80 mg).

[a] Data are included from a separate study evaluating higher MAD doses.

[b] Data are pooled across studies.

In 2023, we initiated a Phase 1 open-label electroencephalogram ("EEG") study designed to evaluate the effects of BHV-7000 on EEG parameters in healthy adults. The study's objective was to demonstrate BHV-7000 target engagement in the cerebral cortex and to help refine dose selection for Phase 3 trials. Study measures included continuous EEG monitoring, time locked pharmacokinetic ("PK") sampling, and changes in EEG spectral power post dose.

The Phase 1 EEG study was designed to evaluate qualitative changes from baseline in EEG spectral power after administration of single doses of BHV-7000 (10, 25, or 50 mg) to 11 healthy male and female adult volunteers. EEG spectral power is a measure derived from quantitative analysis of EEG signals that assesses the amount of rhythmic activity in different frequency bands, including delta [1-3.5 Hz], theta [3.5-7.5 Hz], alpha [7.5-13 Hz], beta [13-30 Hz], and gamma [30-100 Hz]. Changes in spectral power have been used to evaluate the risk, onset and progression of seizures, assess cognitive and behavioral impairments, and characterize the effects of anti-seizure medications ("ASMs"); and, they may also have utility in refining dose selection in clinical trials of ASMs. Spectral analysis was performed by Epilog (Ghent, Belgium), a global leader in EEG analytics.

Interim data from the Phase 1 EEG study were presented in December 2023 at the American Epilepsy Society meeting in Orlando, Florida. BHV-7000 was well-tolerated at all doses studied, without the typical CNS AEs associated with other ASMs, such as somnolence or cognitive/mood disturbances, and EEG data showed dose-dependent increases in brain spectral power in healthy subjects (Figure 1 below). Unlike prior reports where EEG effects of a Kv7.2/7.3 activator showed the greatest power increase in the delta frequency band (Biondi et al. 2022), the highest spectral power increases with BHV-7000 were seen in alpha, beta, and gamma frequency bands (Figure 2 below). While changes in spectral power were observed across all frequency bands with BHV-7000, the minimal impact on slower frequencies (i.e., delta) is consistent with the low incidence of CNS adverse events, in particular somnolence, seen in the BHV-7000 Phase 1

SAD/MAD studies. EEG delta activity is associated with somnolence, an undesirable CNS adverse event often seen with other ASMs.

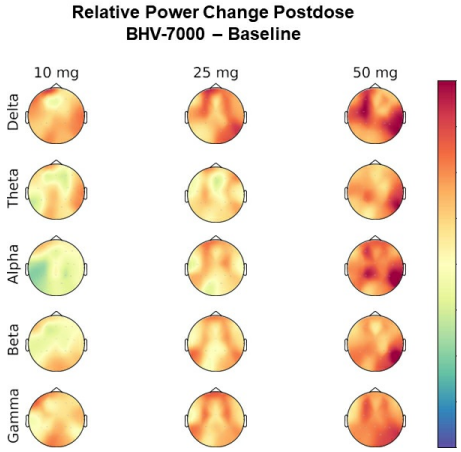


Figure 1: BHV-7000 shows dose-dependent increases in spectral power over all brain regions.

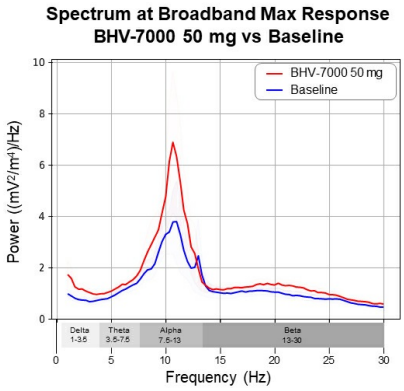


Figure 2: BHV-7000 effect on spectral power in resting - state EEGs. Greatest impact observed on alpha frequency with minimal impact on delta and theta frequencies.

The preliminary EEG study results confirm the CNS activity of BHV-7000 at projected therapeutic concentrations, dose-dependent and time-dependent changes in EEG spectral power, and are consistent with the quantitative EEG effects observed with other ASMs approved for the treatment of epilepsy.

Based on the results from the EEG study and the safety profile in SAD/MAD trials, along with PK data from a new once-daily extended-release (“ER”) formulation, Biohaven plans on exploring three oral doses of BHV-7000 (once daily 25 mg ER, once daily 50 mg ER, and once daily 75 mg ER) in the Phase 2/3 clinical trials in epilepsy and mood disorders. This

dosing approach with a Kv7 activator will allow for assessment of distinct target concentrations over a wide range, above and below projected efficacious EC50 drug concentrations (Figure 3), not previously feasible with drugs in this class.

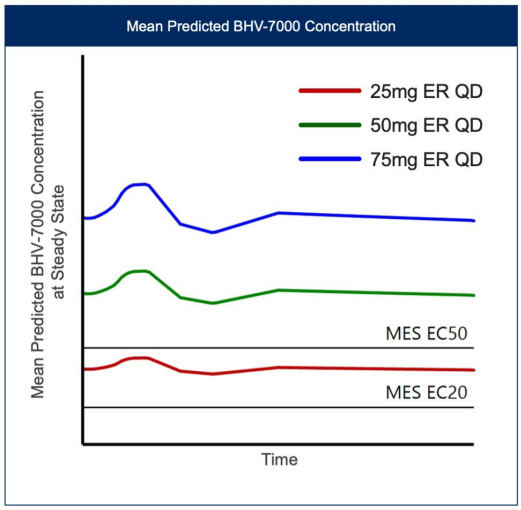


Figure 3: Predicted PK profile of BHV-7000 ER, mean predicted concentration vs. time profiles for 25 mg ER, 50 mg ER and 75 mg ER once-daily dosing at steady state relative to EC20 and EC50 (EC values from preclinical MES models).

Epilepsy

Epilepsy affects approximately 3.5 million Americans, or more than 1.2% of adults and 0.6% of children in the U.S., and more than 50 million patients worldwide, according to the World Health Organization ("WHO"). It is the fourth most common neurological disorder, and many patients struggle to achieve freedom from seizures, with more than one third of patients requiring two or more medications to manage their epilepsy. While the use of anti-seizure medications is often accompanied by dose-limiting side effects, our clinical candidate BHV-7000 is specifically designed to target subtypes of Kv7 potassium channels without engagement of $GABA_A$ receptors. The lack of $GABA_A$-R activity potentially gives BHV-7000 a wide therapeutic window and is expected to result in an improved side effect profile, limiting the somnolence and fatigue often seen in patients receiving anti-seizure medications. This preclinical profile is supported by safety data from our Phase 1 SAD/MAD trial of BHV-7000 in healthy volunteers, which showed a favorable CNS tolerability profile. We aim to bring this potassium channel modulator as a potential solution to patients with epilepsy who remain uncontrolled on their current regimens.

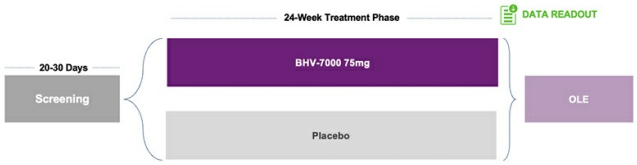
In January 2024, we completed our End-of-Phase 2 meeting with the FDA to advance to Phase 3 trials and announced that more than 110 global clinical sites have been selected in the first of two focal epilepsy trials, with enrollment commencing in the first quarter of 2024. The two pivotal studies evaluating the efficacy of BHV-7000 in refractory focal epilepsy are planned as randomized, double-blind, placebo-controlled, 8- and 12-week trials with a primary endpoint of change from baseline in 28-day average seizure frequency in adults and adolescents with focal epilepsy. One of the focal epilepsy studies will evaluate 25 mg and 50 mg doses of BHV-7000 and the second study will evaluate 50 mg and 75 mg doses of BHV-7000 (see figure below).

Focal Design

DESIGN	Randomized, double-blind, placebo-controlled trial
POPULATION	Subjects 18–75 years old with intractable focal epilepsy
SAMPLE SIZE	390 subjects (randomized 1:1:1)
TREATMENT	BHV-7000 (75/50 mg) and (50/25 mg) vs. placebo
TREATMENT DURATION	12- or 8-week treatment phase
ENDPOINTS	Change in seizure frequency, 50% seizure reduction, seizure freedom, safety

In addition to the focal epilepsy program, we anticipate initiating a Phase 2/3 study of BHV-7000 in idiopathic generalized epilepsy ("IGE") in the second quarter of 2024. The pivotal study evaluating the efficacy of BHV-7000 with IGE is planned as a randomized, double-blind, placebo-controlled 24-week time-to-event trial with a primary endpoint of time to second generalized seizure in adults and adolescents with IGE (see figure below).

Generalized Design



DESIGN	Randomized, double-blind, placebo-controlled trial
POPULATION	Subjects 18–75 years old with idiopathic generalized epilepsy with intractable generalized tonic-clonic seizures
SAMPLE SIZE	242 subjects (randomized 1:1:), study ends with the 127th seizure event
TREATMENT	BHV-7000 75 mg vs. placebo
TREATMENT DURATION	Up to 24-week double-blind phase, subject will transition to open label extension
ENDPOINTS	Time to event (2nd day with generalize tonic-clonic seizure)

Mood disorders

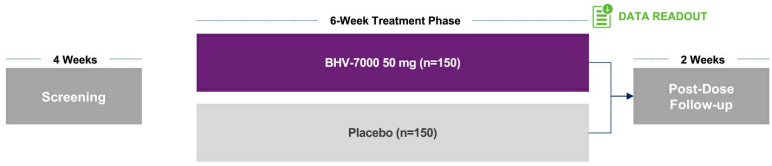
Approximately 1 in 5 adults in the U.S. are living with neuropsychiatric illnesses that are associated with inadequate treatment, poor quality of life, disability, and considerable direct and indirect costs. There is significant unmet need for

novel and effective therapeutic options that are not limited by long latency periods to clinical effects, low response rates, and significant risks and side effects. Increasing evidence from animal models and clinical trials now suggests that Kv7.2/7.3 targeting drugs offer the potential to treat a spectrum of these neuropsychiatric diseases including, but not limited to, mood disorders such as major depressive disorder, bipolar disorder and anxiety.

Major Depressive Disorder

Major depressive disorder is a leading cause of morbidity. Prevalence estimates for MDD episodes in the U.S. are 7.1%; accordingly, there is an urgent need for more effective treatments. Within the current armamentarium of treatments nearly one third of patients fail to respond. Kv7 activation has emerged as a novel strategy to treat and prevent depressive episodes and holds significant potential as a novel treatment for patients with MDD. The therapeutic role for Kv7 activation in MDD is supported by a broad range of epigenetic, mechanistic, preclinical, and clinical evidence. Multiple depression models have demonstrated the antidepressant efficacy of enhancing potassium channel activity. As preclinical studies demonstrate upregulation of Kv7 channels in mice subject to chronic stress, increasing potassium channel function either through genetic over expression or via potassium channel activation (via ezogabine) reverses depressive and anhedonic behaviors in mice across multiple studies. Recent randomized, controlled clinical trials of Kv7 activators demonstrated efficacy in MDD. Together, these data, coupled with the favorable clinical safety and tolerability profile exhibited by BHV 7000 to date, provide a compelling rationale for the evaluation of Kv7 activation with BHV-7000 in MDD.

We plan to initiate a Phase 2 clinical trial with BHV-7000 for the treatment of MDD in the first half of 2024. We anticipate the study will be a 6 week, randomized, double-blind, placebo-controlled trial in approximately 300 subjects, with a primary endpoint of measurement on the Montgomery-Asberg Depression Rating Scale ("MADRS"). See below for expected trial design detail.



DESIGN	Randomized, double-blind, placebo-controlled trial
POPULATION	Subjects with Major Depressive Disorder (MDD) with at least one prior depressive episode who are currently experiencing a depressive episode with anhedonia (HAM-D ≥ 20, SHAPS ≥ 20)
SAMPLE SIZE	300 subjects (randomized 1:1)
TREATMENT	BHV-7000 vs. placebo
TREATMENT DURATION	6 weeks
ENDPOINTS	MADRS (primary), SHAPS, CGI-S, Q-LES-Q-SF, safety and tolerability

HAM-D: Hamilton Depression Rating Scale; **SHAPS**: Snaith-Hamilton Pleasure Scale; **MADRS**: Montgomery–Åsberg Depression Rating Scale; **CGI-S**: Clinical Global Impression, Severity; **Q-LES-Q-SF**: Quality of Life, Enjoyment, and Satisfaction Questionnaire-Short Form

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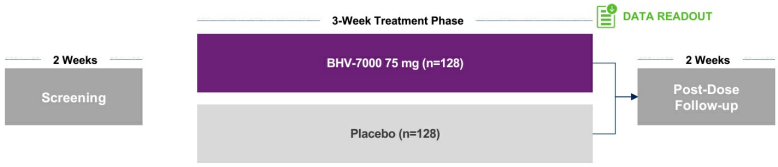
Bipolar disorder

Bipolar disorder affects approximately 7 to 11 million Americans, with an estimated 4.4% of U.S. adults having bipolar disorder over their lifetime. Bipolar disorder is associated with significant morbidity, decreased quality of life and economic burden. Treatment guidelines recommend patients receive life-long treatment for bipolar disorder. However, medication adherence is typically very low in this population, due in large part to undesirable side effects that are poorly tolerated by patients. The mainstays of treatment include mood stabilizing agents that are also used as anti-seizure medicines (i.e., valproic acid, lamotrigine, and topiramate). Preclinical and pilot clinical data similarly suggest a potential therapeutic role for Kv7 activation in bipolar disorder, which is expected to result in an improved side effect profile compared to other anti-seizure medications.

We are advancing BHV-7000 as a potential treatment for patients with bipolar disorder and intend to start a Phase 2/3 clinical trial targeting this indication in the first half of 2024. We expect the study to be a 3 week, randomized, double-

blind, placebo-controlled trial in approximately 256 subjects, with a primary endpoint of measurement on the Young Mania Rating Scale ("YMRS"). See below for expected trial design detail.



DESIGN	Randomized, double-blind, placebo-controlled trial
POPULATION	Subjects with Bipolar Disorder I with at least one prior mood episode who are currently experiencing a manic episode (YMRS ≥ 20)
SAMPLE SIZE	256 subjects (randomized 1:1)
TREATMENT	BHV-7000 vs. placebo
TREATMENT DURATION	3 weeks
ENDPOINTS	YMRS (primary), CGI-S, safety and tolerability

YMRS: Young Mania Rating Scale; **CGI-S**: Clinical Global Impression, Severity

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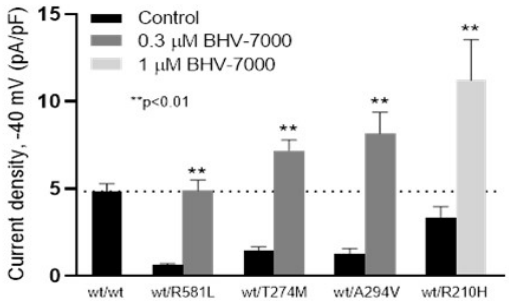
Developmental Epileptic Encephalopathies

KCNQ2 developmental epileptic encephalopathy ("KCNQ2-DEE") is a rare pediatric epileptic encephalopathy first described in 2012 resulting from dominant-negative mutations in the KCNQ2 gene. Epileptic encephalopathies ("EE") comprise a group of epilepsy syndromes in which onset of recurrent and medically refractory seizures are associated with cognitive and broader developmental delay or regression. Although only recently described, heterozygous de novo variants in KCNQ2 are a highly validated cause of early onset epileptic encephalopathy, and KCNQ2-DEE has emerged as a well-defined clinical entity with a characteristic neonatal presentation, including hypotonia, treatment-resistant tonic seizures, a profoundly abnormal EEG, and most often with moderate-to-profound global developmental delay. KCNQ2-DEE is thus both a seizure disorder and a developmental disorder caused by pathogenic KCNQ2 mutations.

Identification of genetic etiologies has created the opportunity to treat not just the symptoms of KCNQ2-DEE, including seizures, but also the underlying causes, including attenuating or reversing the effects of the disease-causing variants. In addition to its activity in the MES model, we explored the ability of BHV-7000 to reverse the reduced current density associated with KCNQ2-DEE and support its use as potential treatment for the disease). To determine the effects of BHV-7000 on the function of Kv7.2 and Kv7.2/7.3 channels poisoned by dominant-negative KCNQ2 mutations, four highly recurrent human missense variants known to cause KCNQ2-DEE were evaluated in an in vitro model measuring current density in cells expressing the pathologic proteins.

The figure below shows the effects of BHV-7000 on current density of wt/wt Kv7.2 channels and those formed by 1:1 coexpression of wt KCNQ2 genes with four disease-causing KCNQ2 variants (T274M, A294V, R581L, R210H). In the control condition, all pathogenic variants produced a marked reduction in current density to below wt/wt levels. BHV-7000 at either 0.3µM or 1.0 µM restored current density in all mutated channels to or beyond wt control current density (**<0.01).



BHV-7000 has been granted Rare Pediatric Disease Designation by the U.S. Food and Drug Administration ("FDA") for the treatment of KCNQ2-DEE.

Neuropathic Pain

Neuropathic pain, as defined by the International Association for the Study of Pain, is pain caused by a lesion or disease of the somatosensory nervous system and includes a collection of heterogeneous conditions that are often chronic and debilitating and for which long term therapy is difficult. In the United States, over 30 million adults are estimated to be living with neuropathic pain. Pharmacological treatments for neuropathic pain vary according to patient needs, although recommendations such as the WHO analgesic ladder, United States Centers for Disease Control (“CDC”), and FDA guidelines are in use. Initial or first line treatment for neuropathic pain includes non-opioid analgesics, in particular, antidepressants, anticonvulsants, steroids, and anxiolytics. Second line treatment of persistent, severe pain may require escalation to opiates, often less potent ones at first, followed by more potent opiates for intense refractory pain.

Thus, an urgent need exists for effective, non-addictive pain therapies. Flupirtine, a non-selective Kv7 activator, was previously approved in several European countries and indicated for the treatment of pain. However, the European Medicines Agency recommended withdrawal of its marketing authorization in 2018 because of the risk of serious liver injury. Selective Kv7 potassium channel activators represent a new approach in the development of non-opioid therapeutic options for neuropathic pain. In addition to leveraging the reduced abuse and addiction risk potential of potassium channel activators, our Kv7 potassium channel platform addresses the complexities of channel subtype physiology through targeted pharmacology to overcome the limitations inherent in nonselective Kv7 activators and is intended to deliver a well-tolerated, highly effective, non-opioid treatment for neuropathic pain.

Our Kv7 program research was supported in part with funding from the National Institutes of Health (“NIH”) to advance the development of novel Kv7 non-opioid therapies for the treatment of chronic pain. The NIH funding is by the NIH Helping to End Addiction Long-term Initiative (“NIH HEAL Initiative”), which aims to improve treatments for chronic pain, curb the rates of opioid use disorder and overdose, and achieve long-term recovery from opioid addiction. The goal of our Kv7 program is to discover a small-molecule activator of the Kv7.2/7.3 voltage-gated potassium channel to treat neuropathic pain. Similar to our epilepsy program, we are targeting compounds with these characteristics:

- Biased for Kv7.2/3 activation vs. Kv7.4 activation to minimize potential adverse smooth muscle effects
- Selective against $GABA_A$ receptors to minimize potential tolerability issues
- Selective against Kv7.1/KCNE1 (IKs) and hERG (IKr) to minimize cardiac side-effects
- Potent and effective across animal models of neuropathic pain

Axonal excitability and neurotransmitter release are altered in neuropathic pain due to sodium channel plasticity, increased voltage-gated calcium channels in the spinal cord, and diminished potassium channel activity in dorsal root ganglion (“DRG”) neurons. These changes in ion channel number, distribution, and function are common to many neuropathic pain subtypes. The functional density of Kv7.2/3 channels is a key variable governing sensory DRG control of intrinsic excitability. Rose et. al demonstrated downregulation of Kv7 potassium channel mRNA in an experimental nerve injury model and further showed alleviation of neuropathic hyperalgesia with administration of flupirtine.

Using human induced pluripotent stem cell (“iPSC”)-derived DRG sensory neurons, we have assessed the physiological activity of these neurons by modulating Kv7 channels across three electrophysiologic parameters: resting membrane potential (Vm), rheobase = the current required to stimulate an action potential (“AP”), and the number of APs elicited by a suprathreshold stimulus (3x rheobase). We are currently evaluating the activity of various compounds from our proprietary series of selective Kv7.2/7.3 activators in multiple preclinical models of neuropathic pain. The Company recently initiated a sponsored research agreement with Yale to evaluate the activity of BHV-7000 in an iPSC model of inherited erythromelalgia, a severe rare genetic neuropathy.

Migraine

We are currently exploring BHV-7000 as a potential treatment for migraine. Kv7.2/7.3 openers have shown significant activity in cortical spreading depression models of migraine.

TRPM3 Ion Channel Antagonists

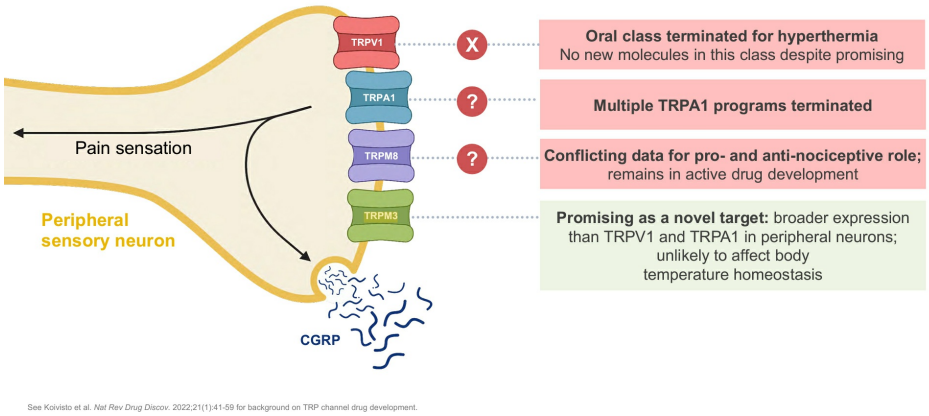
KU Leuven Agreement

In January 2022, we entered into an agreement with KU Leuven ("the KU Leuven Agreement") to develop and commercialize TRPM3 antagonists to address the growing proportion of people worldwide living with chronic pain disorders. The TRPM3 antagonist platform was discovered at the Centre for Drug Design and Discovery and the Laboratory of Ion Channel Research at KU Leuven. Under the KU Leuven Agreement, we receive exclusive global rights to develop, manufacture and commercialize KU Leuven's portfolio of small-molecule TRPM3 antagonists. The portfolio includes the lead candidate, BHV-2100, which we are evaluating in several preclinical pain models and advancing towards the clinic in 2023. We are continuing to support further basic and translational research on the role of TRPM3 in pain and other disorders through our collaboration with professors in Transient Receptor Potential ("TRP") biology at KU Leuven.

Efforts to target TRP Ion Channels for pain

Since the Nobel Prize-winning discovery of the capsaicin receptor TRPV1 in 1997, members of the TRP cation channel family have been elusive drug targets for the treatment of pain. Initially, there was much excitement and investment in TRPV1 antagonists due to promising preclinical efficacy and some evidence of clinical pain reduction. However, trials of most TRPV1 antagonists were terminated after the class consistently caused clinically-significant hyperthermia in study participants. Several companies then made efforts to progress antagonists of TRPA1, the receptor for mustard oil. Though Glenmark's GRC 17536 showed encouraging results in a subset of diabetic peripheral neuropathic pain subjects in a Phase 2a study, it suffers from poor physiochemical properties and pharmacokinetics like many other TRPA1 antagonists.

TRPM3 is a novel target in the TRP family. Like TRPV1 and TRPA1, preclinical data and human genetic validation support TRPM3's role in neuropathic pain and migraine. Unlike TRPV1 antagonists, TRPM3 antagonists are unlikely to possess significant thermal liabilities, and unlike TRPA1 antagonists, Biohaven's TRPM3 antagonists have desirable physiochemical properties and good pharmacokinetic profiles. The figure below illustrates TRPM3 as a differentiated target for the treatment of pain in the TRP family.

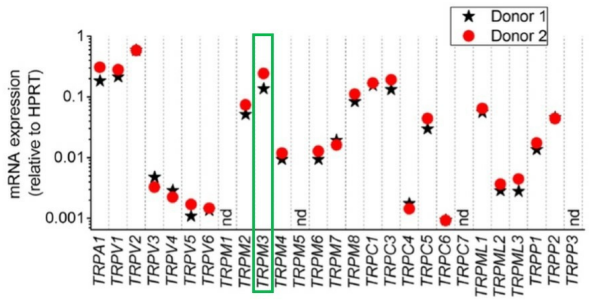


Adapted from *Efforts to target TRP channels for pain*, Kovivisto et al. 2022

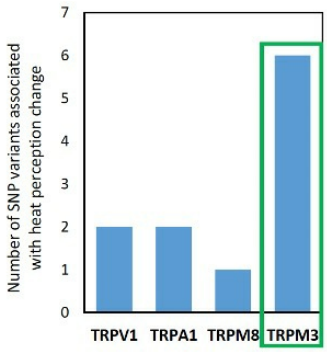
About TRPM3

TRPM3 is a novel druggable target in the TRP cation channel family. TRPM3 is functionally expressed in the human dorsal root ganglion, and several single nucleotide polymorphisms ("SNPs") in TRPM3 are associated with altered pain sensation in response to ultraviolet B ("UVB") (see figure below). Additionally, people with TRPM3 gain-of-function mutations experience altered pain sensation (de Sainte Agathe 2020, Dyment 2019, Van Hoeymissen 2020). Knocking out or antagonizing TRPM3 in animal models attenuates the development of various pain states, including those associated with nerve injury, chemotherapy, and diabetic peripheral neuropathy, further indicating that TRPM3 is a promising target

for neuropathic pain. Lastly, preclinical evidence suggests that antagonizing TRPM3 may avoid the on-target body temperature effects and TRPV1 antagonist-induced malignant hyperthermia.



Vangeel et al, 2020



Lotsch et al, 2020

BHV-2100

BHV-2100 is an orally-bioavailable small molecule antagonist of TRPM3. TRPM3 is expressed in the relevant human tissue types for neuropathic pain, and both preclinical models and human genetics implicate TRPM3 in pain signaling.

Our Phase 1 Study with BHV-2100

We have an ongoing Phase 1 study of BHV-2100 in Canada. The Phase 1 study is a randomized, double-blind, placebo-controlled, SAD/MAD study in healthy subjects to evaluate the safety, tolerability, pharmacokinetics, and pharmacodynamics of BHV-2100. Single ascending dose cohorts are ongoing (including subjects randomized to placebo) with ascending dose levels. Each dose cohort will be initiated with sentinel dosing. Dose levels for subsequent cohorts are determined based on available PK, PD, safety and tolerability from previous cohort(s). Up to approximately 88 subjects are planned to be evaluated.

Our Development of BHV-2100 for the Treatment of Migraines

Nearly 40 million people in the U.S. suffer from migraine and the World Health Organization classifies migraine as one of the 10 most disabling medical illnesses. Migraine is characterized by debilitating attacks lasting four to 72 hours with multiple symptoms, including pulsating headaches of moderate to severe pain intensity that can be associated with nausea or vomiting, and/or sensitivity to sound (phonophobia) and sensitivity to light (photophobia). There is a significant unmet need for new treatments as more than 90 percent of migraine sufferers are unable to work or function normally during an attack. We expect to initiate a BHV-2100 Phase 2 study in acute migraine in the second half of 2024. We are evaluating and have not yet finalized clinical trial design, including trial size, and primary and secondary endpoints for the anticipated clinical trial.

Our Development of BHV-2100 for the Treatment of Neuropathic Pain

BHV-2100 is also being developed as a potential non-opioid treatment for neuropathic pain. We are evaluating the ability of BHV-2100 to reduce pain behaviors across several preclinical models of neuropathic pain, including chemotherapy induced neuropathy, diabetic neuropathy, and nerve injury.The Company expects to conduct a proof of concept study for neuropathic pain in the second half of 2024.

Additional research on TRPM3-mediated disorders

Under the KU Leuven Agreement, Biohaven is supporting further basic and translational research at KU Leuven on the role of TRPM3 in pain and other disorders. In addition to BHV-2100, we are optimizing other lead compounds for TRPM3-mediated disorders of the peripheral and central nervous systems.

Inflammation and Immunology Platform

TYK2/JAK1

Agreement with Hangzhou Highlightll Pharmaceutical Co. Ltd.

In March 2023, we entered into an exclusive, worldwide (excluding People's Republic of China and its territories and possessions) license agreement with Hangzhou Highlightll Pharmaceutical Co. Ltd. ("Highlightll") (the "Highlightll Agreement"), pursuant to which we obtained the right to research, develop, manufacture and commercialize Highlightll's brain penetrant dual TYK2/JAK1 inhibitor program.

BHV-8000

Dysregulation of the immune system has been implicated in several neurodegenerative and neuroinflammatory disorders including Parkinson's Disease, Multiple Sclerosis, Alzheimer's Disease, Amyotrophic Lateral Sclerosis and Autoimmune Encephalitis. Over-active immune cells and microglia driving chronic neuroinflammation results in release of cytokines with activation of leukocytes and is thought to contribute to neuronal injury, death, gliosis, and demyelination. The tyrosine kinase 2 ("TYK2") and Janus kinase 1 ("JAK1") signal transduction pathways mediate highly complementary immune and inflammatory signaling events. Targeted, small-molecule therapies that inhibit TYK2 or JAK kinases have separately demonstrated robust efficacy in autoimmune, dermatologic and gastrointestinal disorders. TYK2 is a validated immune target as evidenced by a recent peripheral program that gained FDA approval, and there are multiple additional peripheral non-CNS programs in clinical development. Brain penetrant inhibitors of TYK2/JAK1 have the potential to bring this validated immune target to brain disorders.

There are currently no brain penetrant, selective, dual TYK2/JAK1 inhibitors approved for brain disorders. In May 2023, we began dosing with BHV-8000 (previously TLL-041), in a phase 1 study in normal healthy volunteers. The planned phase 1 study is a randomized, double-blind, placebo-controlled, sequential parallel group, single ascending dose / multiple ascending dose study in healthy subjects to evaluate the safety, tolerability, pharmacokinetics and pharmacodynamics of BHV-8000 following oral administration. In this study, single ascending dose cohorts are planned and ongoing (including subjects randomized to placebo) with up to 6 dose levels. Each dose cohort will be initiated with sentinel dosing, e.g., one active and placebo patient will be dosed simultaneously. Doses for subsequent cohorts are determined based on available PK, PD, safety and tolerability from previous cohort(s). Up to 40 subjects are planned to be evaluated with approximately 30 subjects randomized to receive active drug and approximately 10 subjects randomized to receive placebo in a double-blind fashion.

In July 2023, we reported that we had successfully dosed three dose cohorts with single ascending doses of BHV-8000 in the ongoing Phase 1 study. Based on the preliminary data that are available, BHV-8000 achieved projected therapeutic concentrations and was well tolerated with only mild adverse events reported.

We anticipate beginning clinical trials with BHV-8000 in the second half of 2024 targeting neuroinflammatory conditions, potentially including Parkinson's disease, Amyloid-Related Imaging Abnormalities ("ARIA") in Alzheimer's disease, Alzheimer's disease, and multiple sclerosis. We are evaluating and have not yet finalized clinical trial designs, including trial size, and primary and secondary endpoints for these anticipated clinical trials.

MoDE Degraders

Bispecific Molecular Degraders of Extracellular Proteins

Molecular Degraders of Extracellular Proteins ("MoDEs") are bispecific molecules that target pathologic circulating proteins and direct them to the liver (or other organ systems) for degradation by the endosomal/lysosomal pathway. Our MoDE platform is being explored for use in a wide range of therapeutic areas, including indications in immune-mediated diseases, cancer and other diseases. We are planning for MoDEs to be administered as intravenous or subcutaneous formulations. We expect to initiate a total of 4 INDs for the degrader program in 2024.

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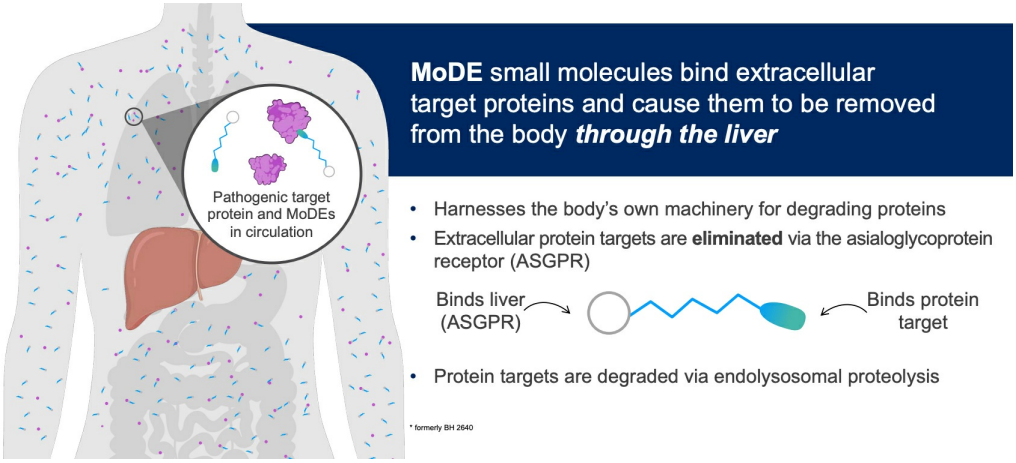


Antibody-based Galactose-deficient IgA ("Gd-IgA") MoDEs

IgA nephropathy ("IgAN") is the most common primary glomerulonephritis that can progress to renal failure and is characterized by immunoglobulin deposits in the renal mesangium comprised exclusively of the IgA1 subclass. Patients with IgAN have increased serum levels of IgA1 with a hinge region containing truncated galactose-deficient O-linked saccharides ("Gd-IgA") and can present with a range of symptoms, from hematuria or proteinuria to severe hypertension owing to renal damage. The clinical progression varies, with 30–40% of patients reaching end-stage renal disease 20–30 years after the first clinical presentation. Patients are managed by therapeutic immunosuppression, with the aim of controlling blood pressure and maintaining renal function.

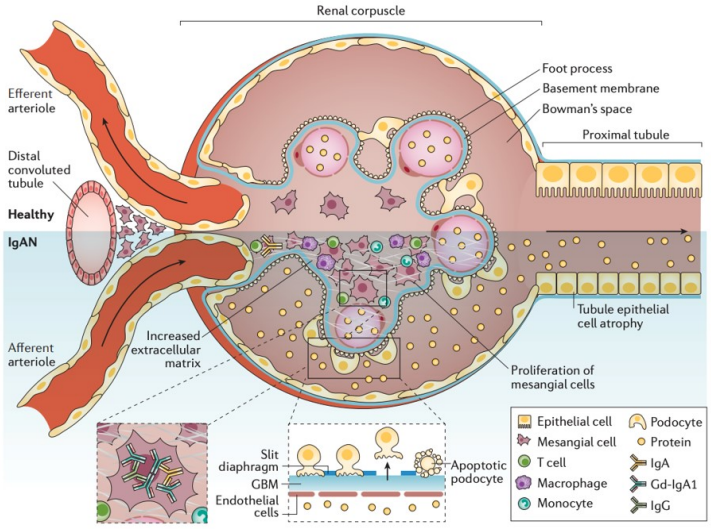


Figure 1 | **The glomerulus in IgA nephropathy.** In a normal glomerulus, normal filtration of plasma occurs and intact podocytes prevent the loss of proteins. In IgA nephropathy (IgAN), deposition (or possibly *in situ* formation) of pathogenetic polymeric IgA1 immune complexes in the glomerular mesangium induces proliferation of mesangial cells and increases the synthesis of extracellular matrix. Humoral mediators attract infiltrating macrophages, monocytes and T cells. Humoral mediators also downregulate the expression of podocyte proteins, leading to apoptosis and protein loss. GBM, glomerular basement membrane; Gd-IgA1, galactose-deficient IgA1.

Lai, *Nat Rev Dis Primers* (2016)

We are leveraging our MoDE platform to develop novel bispecific molecules for the treatment of IgA nephropathy (“IgAN”) that remove potentially disease-causing Gd-IgA and associated immune complexes in patients and thereby prevent harmful kidney deposits. We have taken a published rodent format IgG antibody that recognizes Gd-IgA and converted it into a partially-humanized antibody, then subsequently completed the molecule liver-targeted degrader MoDE. This compound potently binds Gd-IgA and causes its endocytosis in human liver cells through its ASGPR binding motif. Nonclinical development work is ongoing to progress this as a potential IgAN treatment.

BHV-1400

BHV-1400 is a MoDE which is being developed to target Gd-IgA for the treatment of IgA Nephropathy. Specific removal of pathogenic Gd-IgA and associated circulating immune complexes with preservation of normal IgA potentially permits disease remission without incurring an infection risk. We shared preliminary data demonstrating the chimeric antibody-ASGPR ligand conjugate specifically mediated endocytosis of Gd-IgA, as opposed to normal IgA, in an endocytosis assay with HepG2 cells, and that MoDE degraders successfully internalize and degrade these immune-complexes. We expect to initiate Phase 1 studies of BHV-1400 in the second half of 2024. We are evaluating and have not yet finalized potential clinical trial designs, including size and primary and secondary endpoints.

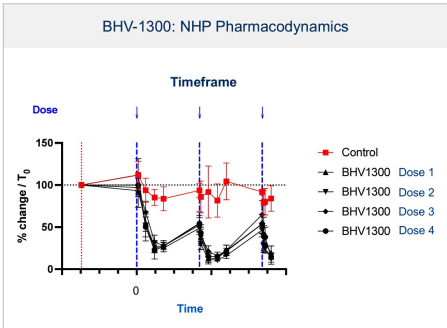
Therapeutic pan-IgG depletion

Analogous to the depletion of pan-IgA or dg-IgA with molecular degraders, hepatic asialoglycoprotein receptor ("ASGPR") ligand degraders are able to recognize potentially pathogenic isoforms of immunoglobulin ("IgG") and represent a novel, competitive platform with differentiated and improved profile relative to FcRN inhibitors. These molecules are specifically designed to spare the IgG3 subclass, to promote better host defense. FcRN inhibitors such as efgartigimod (Vyvgart) and nipocalimab also deplete IgG, but are not selective and deplete all IgG subclasses. Specifically, high circulating levels of antibodies (monoclonal or polyclonal gammopathy) drive conditions such as myasthenia gravis, rheumatoid arthritis, systemic lupus erythematosus, pemphigus vulgaris and many other diseases. It is hypothesized that rapid and sustained lowering of pathogenic antibody titers in blood will significantly reduce disease symptoms. As this has been shown with FcRN inhibitors for myasthenia gravis (Vyvgart), therapeutic pan-IgG depletion using Biohaven’s proprietary MoDE platform technology is expected to have significant potential benefit for multiple diseases including but not limited to the conditions outlined above. Drug candidates utilizing this technology are in nonclinical development, approaching IND.

BHV-1300

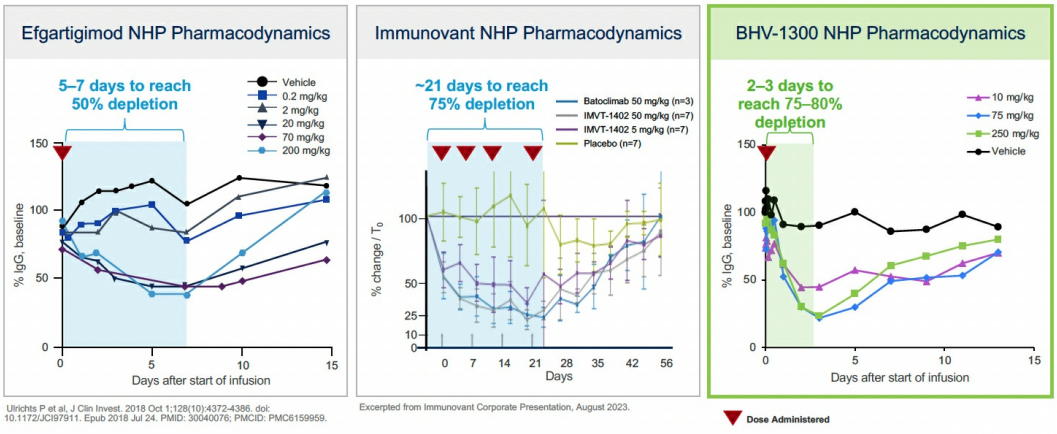
BHV-1300 is an IgG1, IgG2 and IgG4 bispecific degrader which we are initially developing for the treatment of rheumatoid arthritis ("RA"). RA is a chronic autoimmune disease estimated to affect 1 to 2% of the global population. RA primarily affects the joints, causing pain, swelling, stiffness, and loss of function.

We evaluated the effect of single and multiple doses of BHV-1300 in cynomolgus monkeys. In September 2023, we reported data from confirmatory studies that showed a 75-80% reduction of IgG levels two days after a single dose and over 90% of IgG lowering after three doses.

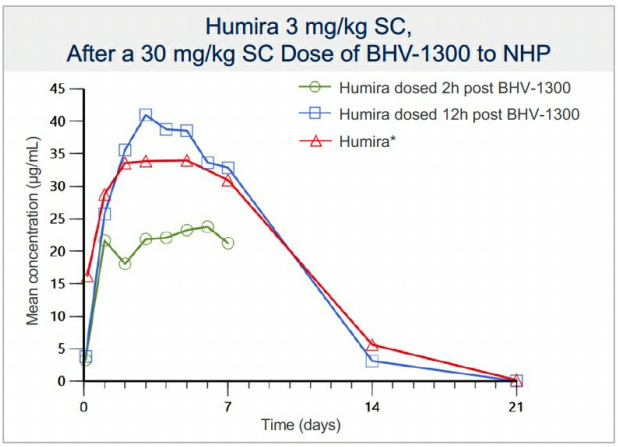


Maximal lowering across FcRn inhibitors is 60-80% within approximately 7 to 21 days after initiation of single or multiple doses, respectively, in cynomolgus. In contrast, a single dose of BHV-1300 lowers IgG by approximately 75 to 80% after approximately 2 days, and after three rapid doses to greater than 90% lowering. The length of significant exposure to BHV-1300 is approximately one day within the dosage interval compared to continuous exposure required of the FcRn inhibitors. Mechanism related liabilities of FcRn inhibitors seen in animals and man, including

hypoalbuminemia and hypercholeresterolema, are not expected and do not occur with BHV-1300 in cynomolgus. See figures below comparing the speed and depth of lowering to FcRn inhibitors.



In January 2024, we reported preclinical pharmacodynamic single dose data with BHV-1300 which demonstrated the Biohaven IgG degrader technology allows for co-administration with Fc-containing biologics. The PK of Humira® was unaltered after being dosed 12 hours after BHV-1300 administration (see figure below).



* Adapted from BLA 761154, IND 116471, Study no. r-fkb327-01

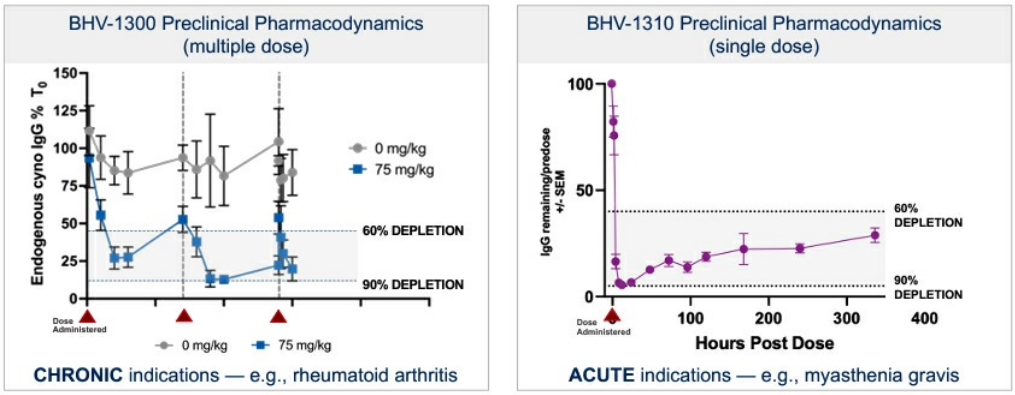
The Phase 1 SAD study examining BHV-1300 in healthy subjects was initiated in the first quarter of 2024 and we expect preliminary results late in the first quarter or early in the second quarter of 2024. The FDA indicated that the MAD assessment of BHV-1300 should be performed in a relevant patient population. Upon completion of the SAD study, we are planning the MAD portion of the study in a relevant patient population with the possibility of benefit from BHV-1300. The Phase 1 study is a randomized, open-label, placebo-controlled, SAD study in healthy subjects to evaluate the safety, tolerability, pharmacokinetics, and pharmacodynamics of BHV-1300. Each dose cohort will be initiated with sentinel dosing. Dose levels for subsequent cohorts will be determined based on available PK, PD, safety and tolerability from previous cohort(s). Up to approximately 32 subjects are planned to be evaluated.

Phase 2 studies of BHV-1300 are anticipated to initiate in 2024. We are evaluating and have not yet finalized potential clinical trial designs, including size and primary and secondary endpoints.

BHV-1310

BHV-1310 is a next generation bispecific IgG degrader with the same specificity as BHV-1300 for IgG1, IgG2 and IgG4 which is initially being developed for the treatment of generalized myasthenia gravis ("gMG") and potentially other acute conditions or conditions with acute exacerbations or flares. MG is a chronic autoimmune disorder of the musculoskeletal system that is estimated to affect approximately 36,000 to 60,000 people in the United States. Patients with gMG develop antibodies that attack critical signaling receptor proteins at the junction between nerve and muscle cells, inhibiting communication between nerves and muscle and resulting in weakness of the skeletal muscles. GMG affects the voluntary muscles of the body, especially those that control the eyes, mouth, throat and limbs.

In January 2024, we demonstrated optimization of degrader technology with BHV-1310 which allows for deeper reductions in IgG after single dose (see figure below). The deep and rapid reductions observed suggest that BHV-1310 could have potential application in acute settings. We expect to initiate Phase 1 studies of BHV-1310 in the second half of 2024. We are evaluating and have not yet finalized potential clinical trial designs, including size and primary and secondary endpoints.



BHV-1600

BHV-1600 is a selective MoDE designed to remove circulating agonistic antibodies of all isotypes and subclasses directed against myocardial beta-1 adrenergic receptor ("β-1 AR") through hepatic ASGPR binding and hepatocellular degradation. This molecule was created using a peptide that mimics the antigenic epitope common to most patients with autoantibodies directed to β-1 AR. This peptide is sufficiently similar to the native sequence such that circulating antibodies are efficiently trapped and subsequently removed by hepatic endocytosis through the ASGPR receptor. As these agonistic antibodies will be markedly depleted, the rapid cessation of inappropriate agonism of the myocardial β-1 AR receptors should result in rapid reversal of symptoms, as precedented with plasmapheresis.

We are developing BHV-1600 for the treatment of dilated cardiomyopathy. Dilated cardiomyopathy is a condition where the cardiac muscle contracts less effectively, the chambers of the heart are enlarged and thinning of cardiac walls results. This can lead to cardiac valvular incompetency, arrhythmias, thrombosis, and heart failure. We expect to initiate Phase 1 studies of BHV-1600 in the second half of 2024. We are evaluating and have not yet finalized potential clinical trial designs, including size and primary and secondary endpoints.

Oncology Platform

CD-38

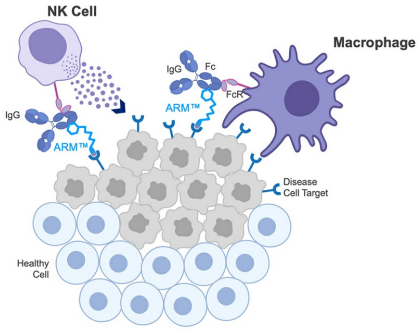
BHV-1100

Antibody Recruiting Molecules

Antibody Recruiting Molecules (“ARMs”) are bispecific molecules that recruit endogenous antibodies to target cancer, virally infected cells, and disease-causing microorganisms for immune-mediated clearance. These molecules are

engineered as modular components that are readily interchangeable, giving the platform tremendous flexibility for a variety of indications and therapy areas.

By recruiting antibodies to coat the disease cell target, ARMs mark it for removal by the body's innate antibody-mediated immune mechanisms (antibody dependent cellular cytotoxicity and antibody dependent cellular phagocytosis).



Platform advantages

Similar to biologics, ARMs directly engage patients' immune system to destroy disease cells by connecting target disease cells with components of the immune system. However, unlike biologics, ARMs are smaller in size than an antibody potentially allowing for enhanced tumor penetration and biodistribution, and may offer manufacturing advantages including enhanced shelf stability.

ARM™ NK Combination Therapy

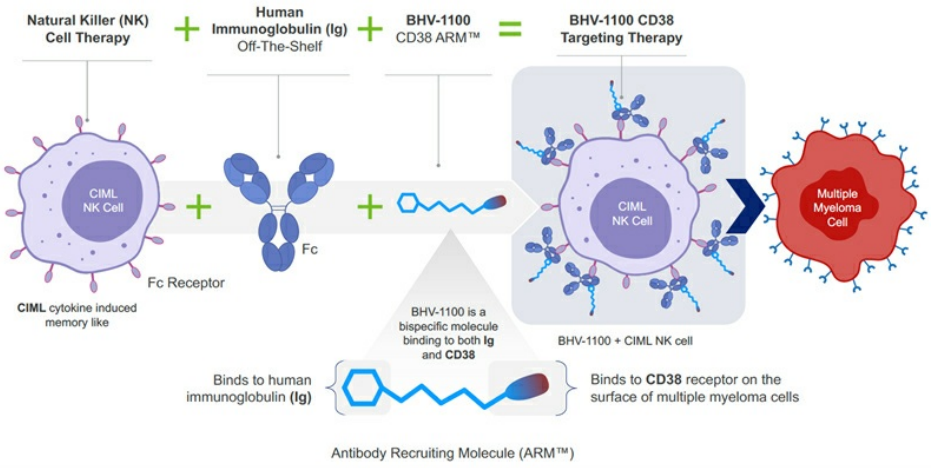
ARMs provide target specificity to Natural Killer ("NK") cell therapies without needing to design chimeric antigen receptors ("CARs") or other methods of genetic manipulation. NK cells are a type of immune effector cell that can recognize and destroy non-self targets and certain diseased cells. NK cells do not target specific protein epitopes like T cells of the adaptive immune system. Our ARMs are being used to provide antigen target specificity to NK cell therapies (both allogeneic and autologous) with the goal of enhancing efficacy and safety. ARM NK combination therapy directs NK cells to a disease target of interest.

Our Clinical Trial for BHV-1100 in Newly Diagnosed Multiple Myeloma Patients

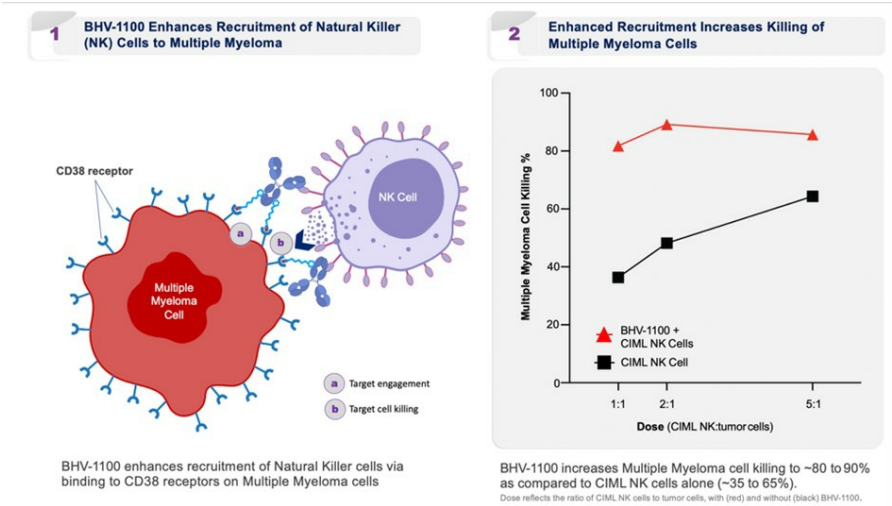
We have initiated dosing in a Phase 1a/1b trial in recovering, post-myeloablative multiple myeloma patients. Our ARM, BHV-1100, in combination with autologous cytokine induced memory-like ("CIML") NK cells and immune globulin ("Ig"), is expected to target and kill multiple myeloma cells expressing the cell surface protein CD38. The trial is supported by compelling preclinical data showing that BHV-1100 enhanced recruitment of autologous CIML NK cells increases killing of multiple myeloma cells.

This open-label single center Phase 1a/1b study assesses the safety and tolerability as well as exploratory efficacy endpoints in newly diagnosed multiple myeloma patients who have tested positive for minimal residual disease ("MRD+") in first remission prior to autologous stem cell transplant ("ASCT"). We expect to enroll 30 newly diagnosed multiple myeloma patients. The primary outcome measures are dose limiting toxicities following combination product administration (time frame: 100 days post-combination product administration) and incidence and severity of side effects related to the combination product (time frame: 90 to 100 days post-combination product administration).

BHV-1100 Binds CD38 and Ig to Create a Targeting Therapy to Kill Multiple Myeloma Cells



BHV-1100 Enhances Recruitment of NK Cells and Increases Killing of Multiple Myeloma Cells



Antibody Drug Conjugates

We are using the MATE conjugation technology to generate site-specific antibody drug conjugates (“ADC”s) from native IgG1 proteins and have shown show superior stability in comparison with those using current industry-standard cysteine maleimide and click conjugation methodologies. Our expectation is that the enhanced in vivo stability and expected superior physicochemical properties of these ADCs will lead to increased therapeutic indices in patients (more cytotoxic payload reaching cancer cells and less reaching normal tissues, as has been shown in vitro, in mice and cynomolgus). Several site-specific ADCs, including BHV-1500, using the well validated valine-citrulline monomethyl auristatin E ("vcMMAE") payload linker system have been prepared and are undergoing biological testing in comparison with industry standard maleimide conjugated ADCs.

BHV-1510

In January 2024, we acquired BHV-1510 through our acquisition of Pyramid Biosciences, Inc. ("Pyramid"). BHV-1510 is a next-generation Trophoblast Cell Surface Antigen 2 ("TROP-2") directed ADC employing an optimized next-generation construct with novel linker-payload and enzymatic, site-specific conjugation, targeting TROP2-expressing carcinomas. Carcinoma refers to a malignant neoplasm of epithelial origin. Carcinomas account for 80 to 90 percent of all cancer cases and several examples have been successfully treated with ADCs. Abundant TROP-2 expression has been described for many carcinoma subtypes.

In preclinical TROP-2 expressing tumor models, BHV-1510 has shown improved antitumor activity versus other TROP-2 directed ADCs, in addition to improved plasma stability, more potent in vitro cytotoxicity, superior bystander effect, and greater immunogenic cell death with the novel TopoIx payload. Improved and differentiated safety has been seen in cynomolgus monkey GLP toxicology studies, suggesting a wife therapeutic index. BHV-1510 has similar favorable characteristics to our proprietary MATE conjugation technology, which should allow highly stable site-specific conjugation, resulting in a favorable PK, toxicity and manufacturability profile.

The IND for BHV-1510 was approved by the FDA in January 2024. The First-in Human trial evaluating BHV-1510 in patients with advanced solid tumors is expected to start in the second quarter of 2024. This trial consists of two parts; Phase 1 dose escalation and Phase 2 dose expansion, in patients with advanced incurable cancer that have progressed on or are intolerant to standard therapy. The primary objective of Phase 1 is safety, to identify a recommended dose for expansion ("RDE") or maximum tolerated dose. Phase 1 dose escalation will be implemented based on a Bayesian optimal interval design, with the lowest dose initiated as a single patient cohort. Patients are expected to be dosed in escalating cohorts, with dosing regimens administered intravenously every three weeks. The Phase 2 dose expansion part of the study will consist of non-randomized efficacy finding expansion cohorts, defined by specific tumor types that will be treated at the RDE to estimate the anti-tumor activity of BHV-1510. Up to approximately 170 subjects are planned to be evaluated.

BHV-1500

BHV-1500 is a next-generation CD30-directed ADC employing a Biohaven proprietary site-specific conjugation (MATE reagent), targeting CD30-expressing tumors such as Hodgkin's and other lymphoma and the MMAE payload. Hodgkin's lymphoma, also referred to as Hodgkin's Disease, is a malignant neoplasm of mesothelial origin. Approximately 9,000 new Hodgkin's disease cases are diagnosed each year. Hodgkin's disease and other CD30-expressing lymphoma are characterized by the uncontrolled growth of malignant lymphocytes or lymphoblasts. Adcetris has demonstrated effectiveness in the treatment of Hodgkin's Lymphoma.

In preclinical CD30 expressing murine tumor models, BHV-1500 has shown improved antitumor activity versus Adcetris (brentuximab vedotin), and substantially improved safety, plasma stability and pharmacokinetics in monkeys. We expect to submit an IND for BHV-1500 in 2024.

Clinical-Stage Milestones

Our clinical-stage milestones include the following:

Competition

The biotechnology and pharmaceutical industries are characterized by rapidly advancing technologies, intense competition and a strong emphasis on proprietary drugs. While we believe that our knowledge, experience and scientific resources provide us with competitive advantages, we face potential competition from many different sources, including major pharmaceutical, specialty pharmaceutical and biotechnology companies, academic institutions and governmental agencies and public and private research institutions. Any product candidates that we successfully develop and commercialize will compete with existing therapies and new therapies that may become available in the future.

The key competitive factors affecting the success of all of our product candidates, if approved, are likely to be their safety, efficacy, convenience, price, the level of generic competition and the availability of coverage and reimbursement from government and other third-party payors.

Many of the companies against which we are competing, or against which we may compete in the future, have significantly greater financial resources and expertise in research and development, manufacturing, preclinical testing, conducting clinical trials, obtaining regulatory approvals and marketing approved drugs than we do. Mergers and acquisitions in the pharmaceutical and biotechnology industries may result in even more resources being concentrated among a smaller number of our competitors. Smaller or early-stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies. These competitors also compete with us in recruiting and retaining qualified scientific and management personnel and establishing clinical trial sites and patient registration for clinical trials, as well as in acquiring technologies complementary to, or necessary for, our programs.

Manufacturing

We have an experienced chemistry and manufacturing leadership team that manages our relationships with third party manufacturers. We currently rely, and expect to continue to rely, on third parties for the development and manufacturing of our product candidates for preclinical and clinical testing, as well as for commercial manufacturing of our products if our product candidates receive marketing approval.

We expect to continue to develop product candidates that can be produced cost-effectively at contract manufacturing facilities.

Commercialization

We intend to develop and, if approved by the FDA, commercialize our product candidates in the United States, and we may enter into distribution or licensing arrangements for commercialization rights for other regions. With respect to our product candidates, we currently intend to build a neurological specialty sales force to manage commercialization for these product candidates, potentially in combination with a larger

pharmaceutical partner, to maximize patient coverage in the United States and to support global expansion.

Members of our management team and board of directors have deep experience leading neuroscience research and have been involved in the development and commercialization of drugs such as Abilify, Opdivo and, most recently, Nurtec ODT and Zavzpret.

Our Chief Executive Officer, Vlad Coric, M.D. was the Chief Executive Officer of the Former Parent from 2015 through the Separation, leading the Former Parent's development and successful commercial launch of Nurtec ODT (rimegepant) in the U.S., which received FDA approval for the acute and preventative treatment of migraine in February 2020 and May 2021, respectively. Under Dr. Coric's leadership, the Former Parent entered into several strategic arrangements, including its Collaboration and License agreement with Pfizer, Inc. for the development of rimegepant and zavegepant outside of the United States.

Intellectual Property

We own or license patents in the U.S. and foreign countries that protect our products, their methods of use and manufacture, as well as other innovations relating to the advancement of our science to help bring new therapies to patients. We also develop brand names and trademarks for our products to differentiate them in the marketplace. We consider the overall protection of our patents, trademarks, licenses and other intellectual property rights to be of material value and act to protect these rights from infringement. We also rely on trade secrets to protect aspects of our business that are not amenable to, or that we do not consider appropriate for, patent protection. Our success will depend significantly on our ability to obtain and maintain patent and other proprietary protection for commercially important technology, inventions and know-how related to our business, defend and enforce our patents, preserve the confidentiality of our trade secrets and operate without infringing the valid and enforceable patents and other proprietary rights of third parties. We also rely on know-how, continuing technological innovation and in-licensing opportunities to develop, strengthen and maintain the proprietary position of our products and development programs.

In the biopharmaceutical industry, a substantial portion of an innovative product's commercial value is usually realized during the period in which the product has market exclusivity. A product's market exclusivity is generally determined by two forms of intellectual property: patent rights held by the innovator company and any regulatory forms of exclusivity to which the innovative drug is entitled.

Patents are a key determinant of market exclusivity for most pharmaceuticals. Patents provide the innovator with the right to exclude others from practicing an invention related to the medicine. Patents may cover, among other things, the active ingredient(s), various uses of a drug product, discovery tools, pharmaceutical formulations, drug delivery mechanisms and processes for (or intermediates useful in) the manufacture of products. Protection for individual products extends for varying periods in accordance with the expiration dates of patents in the various countries. The protection afforded, which may also vary from country to country, depends upon the type of patent, its scope of coverage and the availability of meaningful legal remedies in the country.

Market exclusivity can also be influenced by regulatory data protection ("RDP"). Many developed countries provide certain non-patent incentives for the development of medicines. For example, in the U.S., the EU, United Kingdom, Japan, and certain other countries, RDP intellectual property rights are offered to: (i) provide a time period of data protection during which a generic company is not allowed to rely on the innovator's data in seeking approval; (ii) restore patent term lost during drug development and approval; and (iii) provide incentives for research on medicines for rare diseases, or orphan drugs, and on medicines useful in treating pediatric patients. These incentives can extend the market exclusivity period on a product beyond the patent term.

Patents and Patent Applications

We have many U.S. and foreign patents and patent applications in our portfolio related to the composition of matter, methods of use, methods of manufacture or formulations of our product candidates which have been filed in major markets throughout the world, including the U.S., Europe, the United Kingdom, Japan, Korea, China, Hong Kong and Australia.

Kv7

In April 2022, we acquired Channel Biosciences, LLC. This acquisition included Channel's Kv7 channel targeting platform and related patents and patent applications. The patents and patent applications are directed to the composition of matter of compounds that are activators of Kv7.2/Kv7.3 and their use in treating diseases such as epilepsy. U.S. Patent 10,851,067 (the "'067 Patent"), issued December 1, 2020, specifically claims BHV-7000 and will expire in March 2039, not including possible patent term extensions. Ex-U.S. counterparts to the '067 patent have been granted in Australia and Mexico, and patent applications to the '067 patent are pending in Brazil, Canada, China, European Union, United Kingdom, Hong Kong, Israel, India, Japan, Republic of Korea, New Zealand, Singapore and South Africa. The ex-U.S. patents, and patent applications, if granted, will expire in March 2039, not including possible patent term extensions in countries where such extensions are available. In addition, U.S. Patent 9,481,653 (the "'653 patent"), issued November 1, 2016, claims a class of compounds including BHV-7000 and will expire in September 2035, not including possible patent term extensions. Ex-US counterparts to the '653 patent are granted in Belgium, Switzerland, Germany,

Denmark, Spain, Finland, France, United Kingdom, Ireland, Iceland, Italy, Netherlands, Norway and Sweden. The ex-U.S. patents will expire in September 2035, not including possible patent term extensions in countries where such extensions are available. In addition, PCT/US23/10295, PCT/US23/63111, PCT/US23/63113 and PCT/US23/63115 directed to various Kv7 activator chemotypes were filed in January and February 2023. These applications are pending in the United States Receiving Office of the Patent Cooperation Treaty and all countries were designated for filing. The patent applications, if granted, will expire in January/February 2043, not including possible patent term extensions in countries where such extensions are available. Other patent application directed to the combination treatment with a Kv7 activator and N-methyl-D-aspartate ("NMDA") receptor antagonist (PCT/US23/73125), a Kv7 activator and glutamate modulator (PCT/US23/73504), and a Kv7 activator and TDP-43 binder (PCT/US23/73491) were filed in August and September 2023. These applications are pending in the United States Receiving Office of the Patent Cooperation Treaty and all countries were designated for filing. The patent applications, if granted, will expire in August/September 2043, not including possible patent term extensions in countries where such extensions are available.

Troriluzole

We have a portfolio of patents and patent applications in the U.S. and foreign countries directed to prodrugs of riluzole, including, among others, U.S. Patent 10,485,791, issued November 26, 2019, which is directed to troriluzole and other prodrugs of riluzole. This patent expires in February 2036, not including possible patent term extensions. Ex-US counterparts to the ‘791 patent have been granted in Albania, Armenia, Austria, Australia, Azerbaijan, Belgium, Brazil, Bulgaria, Belarus, Canada, Switzerland, China, Cyprus, Czechia, Germany, Denmark, Estonia, Spain, Finland, France, United Kingdom, Greece, Hong Kong, Croatia, Hungary, India, Ireland, Israel, Italy, Japan, Kyrgyzstan, Kazakhstan, Lithuania, Luxembourg, Latvia, Monaco, North Macedonia, Macao, Malta, Mexico, Netherlands, Norway, Philippines, Poland, Portugal, Republic of Korea, Romania, Serbia, Russia, Sweden, Slovenia, Slovakia, Tajikistan, Turkmenistan, Turkey and South Africa, and a patent application is pending in Singapore. The ex-US patents and patent applications will expire in February 2036, not including possible patent term extensions in countries where such extensions are available. In addition, the use of these compounds for treating OCD, ALS, SCA, depression, Alzheimer’s Disease and other diseases are described and claimed in these patents and patent applications. These patent applications are subject to an agreement with ALS Biopharma and FCCDC. In addition, we have filed patent applications relating to drug product formulations containing troriluzole and methods of using the formulations to treat various diseases, including, for example, the use of troriluzole with immunotherapies to treat cancer, including among others U.S. Patent 11,400,155, issued August 2, 2022, which expires in May 2037, not including possible patent term extensions. Ex-US counterparts to the ‘155 patent have been granted in Albania, Austria, Australia, Belgium, Bulgaria, Switzerland, China, Cyprus, Czechia, Germany, Denmark, Estonia, Spain, Finland, France, United Kingdom, Greece, Hong Kong, Croatia, Hungary, Israel, Ireland, Italy, Japan, Republic of Korea, Lithuania, Luxembourg, Latvia, Mexico, Monaco, North Macedonia, Malta, Netherlands, Norway, Poland, Portugal, Romania, Serbia, Sweden, Slovenia, Slovakia and South Africa and patent applications are pending in Brazil, Canada, India, Philippines and Singapore. The ex-US patents and patent applications will expire in May 2037, not including possible patent term extensions in countries where such extensions are available. In addition, a patent application directed to the use of troriluzole for treating various SCA genotypes was filed in November 2018 (PCT/US18/60232) and national applications are pending in major jurisdictions with patents granted in Japan and Mexico. Another patent application directed to the treatment of SCA3 (PCT/US23/67326) was filed in May 2023 and is pending in the United States Receiving Office of the Patent Cooperation Treaty and all countries were designated for filing. These patents, if granted, will expire in 2038 through 2043, not including possible patent term extensions in countries where such extensions are available. Also, a patent application directed to the use of troriluzole for treating OCD was filed in June 2021 (PCT/US21/38789) and national applications are pending in major jurisdictions. These patents, if granted, will expire in 2041, not including possible patent term extensions in countries where such extensions are available. Another patent application directed to the treatment of glioblastoma (PCT/US23/69038) was filed June 26, 2023 and is pending in the United States Receiving Office of the Patent Cooperation Treaty and all PCT countries were designated for filing.

MoDEs Platform, ARMs, MATEs

In January 2021, we entered into a worldwide, exclusive license agreement with Yale University for the development and commercialization of a novel Molecular Degrader of Extracellular Protein (MoDEs) platform. The platform pertains to the clearance of disease-causing protein and other biomolecules by targeting them for lysosomal degradation using multi-functional molecules. The platform is differentiated from existing approaches in that it does not rely on ubiquitin ligases, and it allows for a broad range of targets to be degraded. The patent portfolio is directed to the composition of matter of bifunctional degraders and their use in degrading circulating proteins and treating diseases. U.S. Serial No. 17/046221, filed October 8, 2020, relates to bifunctional small molecules to target selective degradation of circulating proteins. Ex-US counterparts to the '221 application have been filed in China, European Union and Hong Kong and, if granted, will expire in April 2039, not including possible patent term extensions in countries where such

extensions are available. U.S. Serial No. 17/768166, filed April 11, 2022, relates to bifunctional compounds as degraders of autoantibodies. Ex-US counterparts to the '166 application have been filed in the United Arab Emirates, Australia, Brazil, Canada, China, European Union, Israel, Japan, Republic of Korea, Mexico, Philippines, Saudi Arabia, Singapore, and South Africa and, if granted, will expire in October 2040, not including possible patent term extensions in countries where such extensions are available. U.S. Serial No. 17/046192, filed October 8, 2020 and issued as U.S. Patent No. 11,767,301 on September 26, 2023, relates to bifunctional molecules to degrade circulating proteins. Ex-US counterparts to the '192 application have been filed in the European Union and Hong Kong, and, if granted, will expire in April 2039, not including possible patent term extensions in countries where such extensions are available. U.S. Serial No. 17/768145, filed April 11, 2022 relates to engineered antibodies as molecular degraders through cellular receptors. Ex-US counterparts to the '145 application have been filed in the United Arab Emirates, Australia, Brazil, Canada, China, countries of the Eurasian Patent Organization, European Union, Israel, India, Japan, Republic of Korea, Mexico, New Zealand, Philippines, Saudi Arabia, South Africa and Singapore and, if granted, will expire in October 2040, not including possible patent term extensions in countries where such extensions are available. PCT/US2022/017319, which relates to targeted bifunctional degraders, was filed February 22, 2022, and national applications are pending in the United States and major jurisdictions worldwide. The patent applications, if granted, will expire in February 2042, not including possible patent term extensions in countries where such extensions are available. PCT/US2022/019658, which relates to bifunctional degraders of galactose deficient immunoglobulins, was filed March 10, 2022, and is pending in the United States and major jurisdictions worldwide. The patent applications, if granted, will expire in March 2042, not including possible patent term extensions in countries where such extensions are available. PCT/US2022/075527 and PCT/US2022/075535, filed August 26, 2022, which relate to difunctional degraders of pathogenic anti-β1ECII (the second extracellular loop of the β1 adrenergic receptor) autoantibodies, are pending in the United States Receiving Office of the Patent Cooperation Treaty and all countries were designated for filing.

We also acquired Kleo Pharmaceuticals, Inc. in January 2021. This acquisition included Kleo's proprietary technology platforms which are modular in design and enable rapid generation of novel immunotherapies that can be optimized against specified biological targets and combined with existing cell- or antibody-based therapies. These include ARMs and MATEs, which complement the MoDEs technology licensed from Yale. U.S. Serial No. 17/769924, filed April 18, 2022, relates to directed conjugation technologies. Ex-US counterparts to the '924 application have been filed in the United Arab Emirates, Australia, Brazil, Canada, China, countries of the Eurasian Patent Organization, European Union, Israel, India, Japan, Republic of Korea, Mexico, New Zealand, Philippines, Saudi Arabia, South Africa and Singapore and, if granted, will expire in November 2040, not including possible patent term extensions in countries where such extensions are available. U.S. Serial No. 17/912563, filed September 19, 2022, relates to technologies for treating COVID infections. Ex-US counterparts to the '563 application have been filed in the United Arab Emirates, Australia, Brazil, Canada, China, countries of the Eurasian Patent Organization, European Union, Israel, India, Japan, Republic of Korea, Mexico, New Zealand, Philippines, Saudi Arabia, South Africa and Singapore and, if granted, will expire in March 2041, not including possible patent term extensions in countries where such extensions are available. PCT/US2022/015390, which relates to technologies for preventing or treating infections, was filed February 6, 2022 and national applications are pending in the United States and major jurisdictions worldwide. The patent applications, if granted, will expire in February 2042, not including possible patent term extensions in countries where such extensions are available. PCT/US2022/029533, which relates to compositions including conjugated therapy enhancers, was filed May 17, 2022 and national applications are pending in the United States and major jurisdictions worldwide. The patent applications, if granted, will expire in May 2042, not including possible patent term extensions in countries where such extensions are available. PCT/US2022/029535, which relates to agents for directed conjugation techniques and conjugated products, was filed May 17, 2022 and national applications are pending in the United States and major jurisdictions worldwide. The patent applications, if granted, will expire in May 2042, not including possible patent term extensions in countries where such extensions are available. PCT/US2022/030070, which relates to antibody drug conjugates using MATE technology for delivering cytotoxic agents, was filed May 19, 2022 and national applications are pending in the United States and major jurisdictions worldwide. The patent applications, if granted, will expire in May 2042, not including possible patent term extensions in countries where such extensions are available.

TRPM3

In January 2022, we entered into an exclusive global license and research agreement to develop and commercialize TRPM3 antagonists to address the growing proportion of people worldwide living with chronic pain disorders. The TRPM3 antagonist platform was discovered at the Centre for Drug Design and Discovery ("CD3") and the Laboratory of Ion Channel Research ("LICR") at Katholieke Universiteit Leuven (KU Leuven). PCT/EP2021/082853, which relates to aryl derivatives for treating TRPM3 mediated disorders, was filed November 24, 2021 and national applications are pending in the United Arab Emirates, Australia, Brazil, Canada, China, countries of the Eurasian Patent Organization, European Union, Israel, India, Japan,

Republic of Korea, Mexico, New Zealand, Philippines, Saudi Arabia, South Africa, Singapore, Taiwan and the U.S. The patent applications, if granted, will expire in November 2041, not including possible patent term extensions in countries where such extensions are available. PCT/EP2021/082865, which relates to heterocycle derivatives for treating TRPM3 mediated disorders, was filed November 24, 2021 and national applications are pending in the United Arab Emirates, Australia, Brazil, Canada, China, Eurasia, European Union, Israel, Japan, Republic of Korea, Mexico, New Zealand, Philippines, Saudi Arabia, Singapore, Taiwan, South Africa and the U.S. The patent applications, if granted, will expire in November 2041, not including possible patent term extensions in countries where such extensions are available. U.S. Patent 9,194,863, issued November 24, 2015, which relates to screening methods for analgesic agents, has also been granted in Belgium, Switzerland, Germany, Denmark, Spain, Finland, France, United Kingdom, Ireland, Italy, Netherlands and Sweden. The patents, will expire in May 2032, not including possible patent term extensions in countries where such extensions are available. In addition, seven PCT applications directed to various TRPM3 antagonist chemotypes were filed in May 2023 (PCT/EP2023/063992, PCT/EP2023/063994, PCT/EP2023/063996, PCT/EP2023/063997, PCT/US2023/067443, PCT/US2023/067446, PCT/US2023/067448). The patent applications, if granted, will expire in May 2043, not including possible patent term extensions in countries where such extensions are available.

Myostatin

In December 2021, we entered into a worldwide license agreement with Bristol Myers Squibb for the global development and commercialization rights to taldefgrobep alfa (BHV-2000), a novel, Phase 3-ready anti-myostatin adnectin. Myostatin is a natural protein that limits skeletal muscle growth, an important process in healthy muscular development.

U.S. Patent 8,853,154 issued October 7, 2014, U.S. Patent 8,933,199, issued January 13, 2015, U.S. Patent 8,993,265, issued March 31, 2015, U.S. Patent 9,493,546, issued November 15, 2016, U.S. Patent 9,662,373, issued May 30, 2017, U.S. Patent 10,245,302, issued April 2, 2019, U.S. Patent 10,406,212, issued September 10, 2019, and U.S. Patent 11,813,315, issued November 14, 2023, are directed to fibronectin based scaffold domain proteins that bind to myostatin. The U.S. patents expire in September 2033, not including possible patent term extensions. Ex-US counterparts to the U.S. patents have been granted in Argentina, Austria, Australia, Belgium, Bulgaria, Brazil, Canada, Switzerland, Chile, China, Colombia, Czechia, Germany, Denmark, Algeria, Egypt, Spain, Finland, France, United Kingdom, Greece, Hong Kong, Croatia, Hungary, Indonesia, Ireland, Israel, India, Italy, Japan, Republic of Korea, Lithuania, Morocco, Macao, Mexico, Malaysia, Netherlands, Norway, New Zealand, Peru, Philippines, Poland, Portugal, Romania, Serbia, Russia, Sweden, Singapore, Slovenia, Slovakia, Thailand, Tunisia, Turkey, Taiwan, Uruguay, Venezuela, Vietnam and South Africa. The ex-US patents will expire in September 2033, not including possible patent term extensions in countries where such extensions are available. U.S. Serial No. 16/607688, filed May 3, 2018, relates to stable formulations fibronectin based scaffold domain proteins that bind to myostatin. Ex-US counterparts to the '688 application have been granted in Japan and are pending in Australia, Canada, China, European Union, Hong Kong, Israel, Republic of Korea, Mexico, Singapore and Taiwan. The ex-US patents and patent applications, if granted, will expire in May 2038, not including possible patent term extensions in countries where such extensions are available.

TYK2 / JAK1

In March 2023, we entered into an exclusive, worldwide (excluding People's Republic of China and its territories and possessions) license agreement with Highlightll pursuant to which we obtained the right to research, develop, manufacture and commercialize Highlightll's brain penetrant dual TYK2/JAK1 inhibitor program

U.S. Patent RE49834 (the "'834 Patent"), having an issue date of February 13, 2024, is directed to BHV-8000 and will expire in September 2037, not including possible patent term extensions. Ex-U.S. counterparts to the '834 patent have been granted in Australia, European Union, Israel, Japan, Mexico, and New Zealand, and patent applications to the '834 patent are pending in Brazil, Canada, Eurasia, India, and Republic of Korea. The ex-U.S. patents and patent applications, if granted, will expire in September 2037, not including possible patent term extensions in countries where such extensions are available. In addition, PCT/CN2022/113807, filed August 22, 2022, which relate to methods of treating CNS disorders with dual TYK2/JAK1 inhibitors, is pending in the CNIPA Receiving Office of the Patent Cooperation Treaty and all countries were designated for filing. The patent applications, if granted, will expire in August 2042, not including possible patent term extensions in countries where such extensions are available.

Licensing and Other Agreements

In addition to our independent efforts to develop and market products, we enter into agreements such as licensing agreements, option-to-license agreements and strategic collaborations. The licensing and other agreements typically include, among other terms and conditions, non-refundable upfront license fees, option fees and option exercise payments, milestone payments and royalties. See Note 11, "License Agreements," to the Consolidated Financial Statements included in this report for additional information regarding our licenses and other agreements.

Government Regulation

In the United States, the FDA regulates drugs under the Federal Food, Drug and Cosmetic Act

("FDCA") and its implementing regulations. The process of obtaining regulatory approvals and the subsequent compliance with appropriate federal, state, local and foreign statutes and regulations requires the expenditure of substantial time and financial resources. Failure to comply with the applicable U.S. requirements at any time during the product development process, approval process or after approval may subject an applicant and/or sponsor to a variety of administrative or judicial sanctions, including imposition of a clinical hold, refusal by the FDA to approve applications, withdrawal of an approval, import/export delays, issuance of warning letters and other types of enforcement letters, product recalls, product seizures, total or partial suspension of production or distribution, injunctions, fines, refusals of government contracts, restitution, disgorgement of profits, or civil or criminal investigations and penalties brought by the FDA and the Department of Justice or other governmental entities.

The clinical testing, manufacturing, labeling, storage, distribution, record keeping, advertising, promotion, import, export and marketing, among other things, of our product candidates are governed by extensive regulation by governmental authorities in the United States and other countries. The FDA, under the FDCA, regulates pharmaceutical products in the United States. The steps required before a drug may be approved for marketing in the United States generally include:

- nonclinical laboratory tests and animal tests conducted under Good Laboratory Practices ("GLP");
- the submission to the FDA of an IND application for human clinical testing, which must become effective before human clinical trials commence;
- approval by an independent institutional review board ("IRB"), representing each clinical site before each clinical trial may be initiated;
- adequate and well-controlled human clinical trials to establish the safety and efficacy of the product for each indication and conducted in accordance with Good Clinical Practices ("GCP");
- the preparation and submission to the FDA of an NDA;
- FDA acceptance, review and approval of the NDA, which might include an Advisory Committee review;
- satisfactory completion of an FDA inspection of the manufacturing facilities at which the product, or components thereof, are made to assess compliance with current Good Manufacturing Practices ("cGMPs").

The testing and approval process requires substantial time, effort and financial resources, and the receipt and timing of any approval is uncertain. The FDA may suspend clinical trials at any time on various grounds, including a finding that the subjects or patients are being exposed to an unacceptable health risk.

Nonclinical and Human Clinical Trials in Support of an NDA

Nonclinical studies include laboratory evaluations of the product candidate, as well as in vitro and animal studies to gather information on the safety and efficacy of the product candidate. The conduct of nonclinical trials is subject to federal regulations and requirements including GLP regulations. The results of the nonclinical studies, together with manufacturing information and analytical data, among other things, are submitted to the FDA as part of the IND, which must become effective before clinical trials may be commenced. The IND will become effective automatically 30 days after receipt by the FDA, unless the FDA raises concerns or questions about the conduct of the trials as outlined in the IND prior to that time. In this case, the IND sponsor and the FDA must resolve any outstanding concerns before clinical trials can proceed. The FDA may nevertheless initiate a clinical hold after the 30 days if, for example, a deficiency is found in the IND application.

Clinical trials involve the administration of the product candidate to human subjects under the supervision of qualified investigators in accordance with GCP requirements. Each clinical trial must be reviewed and approved by an IRB at each of the sites at which the trial will be conducted. The IRB will consider, among other things, ethical factors, the safety of human subjects and the possible liability of the institution.

Clinical trials are typically conducted in three sequential phases prior to approval, but the phases may overlap or be combined. These phases generally include the following:

Phase 1. Phase 1 clinical trials represent the initial introduction of a product candidate into human subjects, frequently healthy volunteers. In Phase 1, the product candidate is usually tested for safety, including adverse effects, dosage tolerance, absorption, distribution, metabolism, excretion and pharmacodynamics.

Phase 2. Phase 2 clinical trials usually involve studies in a limited patient population with a specific disease or condition to (1) evaluate the efficacy of the product candidate for specific indications, (2) determine dosage tolerance and optimal dosage and (3) identify possible adverse effects and safety risks.

Phase 3. If a product candidate is found to be potentially effective and to have an acceptable safety profile in Phase 2 clinical trials, the clinical trial program will be expanded to Phase 3 clinical trials to further demonstrate clinical efficacy, optimal dosage and safety within an expanded patient population at geographically dispersed clinical trial sites. These clinical studies are intended to establish the overall risk/benefit ratio of the product and provide an adequate basis for product approval and labeling.

Phase 4. Clinical trials may be conducted after approval to gain additional experience from the treatment of patients in the intended therapeutic indication and to document a clinical benefit in the case of drugs approved under accelerated approval regulations, or when otherwise requested by the FDA in the form of post-market requirements or commitments. Failure to promptly conduct any required Phase 4 clinical trials could result in enforcement action or withdrawal of approval.

A *Phase 2/3* trial design, which we have used in our troriluzole development program, is often used in the development of pharmaceutical and biological products. The trial includes Phase 2 elements, such as an early interim analysis of safety or activity, and Phase 3 elements, such as larger patient populations with less restrictive enrollment criteria. An early interim analysis of clinical or physiologic activity and/or safety allows the study to be stopped, changed or continued before a large number of patients have been enrolled, while still allowing all data from enrolled patients to count in the analysis used to support approval.

Submission and Review of an NDA

The results of nonclinical studies and clinical trials, together with detailed information on the product's manufacture, composition, quality, controls and proposed labeling, among other things, are submitted to the FDA in the form of an NDA, requesting approval to market the product. The application must be accompanied by a significant user fee payment, which typically increases annually, although waivers may be granted in limited cases. The FDA has substantial discretion in the approval process and may refuse to accept an application if they determine that the data are insufficient for approval and require additional nonclinical, clinical or other studies.

Once an NDA has been accepted for filing, which occurs, if at all, 60 days after submission, the FDA sets a user fee goal date that informs the applicant of the specific date by which the FDA intends to complete its review. A standard review typically takes 10 months from the date that the application is accepted for filing by the FDA and a priority review typically takes 6 months from the date that the application is accepted by the FDA for filing. The review process can be extended by FDA requests for additional information or clarification. The FDA reviews NDAs to determine, among other things, whether the proposed product is safe and effective for its intended use, and whether the product is being manufactured in accordance with cGMPs to assure and preserve the product's identity, strength, quality and purity. Before approving an NDA, the FDA typically will inspect the facilities at which the product is manufactured and will not approve the product unless the manufacturing facilities comply with cGMPs. Additionally, the FDA will typically inspect one or more clinical trial sites, as well as the Sponsor of the NDA, for compliance with GCP and integrity of the data supporting safety and efficacy.

During the approval process, the FDA also will determine whether a risk evaluation and mitigation strategy ("REMS") is necessary to assure the safe use of the product post approval. If the FDA concludes a REMS is needed, the sponsor of the application must submit a proposed REMS, and the FDA will not approve the application without an approved REMS, if required. A REMS can substantially increase the costs of obtaining approval. The FDA could also require a special warning, known as a boxed warning, to be included in the product label in order to highlight a particular safety risk. The FDA may also convene an advisory committee of external experts to provide input on certain review issues relating to risk, benefit and interpretation of clinical trial data. The FDA may delay approval of an NDA if applicable regulatory criteria are not satisfied and/or the FDA requires additional testing or information. The FDA may require post-marketing testing and surveillance to monitor safety or efficacy of a product.

On the basis of the FDA's evaluation of the NDA and accompanying information, including the results of the inspection of the manufacturing facilities, the FDA will issue either an approval of the NDA or a Complete Response Letter ("CRL"), detailing the deficiencies in the submission and the additional testing or information required for reconsideration of the application. The deficiencies identified may be minor, for example, requiring labeling changes, or major, for example, requiring additional clinical studies. If a CRL is issued, the applicant may either resubmit the NDA, addressing all of the deficiencies identified in the letter, withdraw the application, or request a hearing. Even with submission of this additional information, the FDA may ultimately decide that the application does not satisfy the regulatory criteria for approval.

Post-Approval Requirements

Approved drugs that are manufactured or distributed in the United States pursuant to FDA approvals are subject to pervasive and continuing regulation by the FDA, including, among other things, requirements relating to recordkeeping, periodic reporting, product sampling and distribution, advertising and promotion and reporting of adverse experiences with the product. After approval, most changes to the approved product, such as adding new indications or other labeling claims and some manufacturing and supplier changes are subject to prior FDA review and approval. There also are continuing, annual program user fee requirements for marketed products.

The FDA may impose a number of post-approval requirements as a condition of approval of an NDA. For example, the FDA may require post-marketing testing, including Phase 4 clinical trials, and surveillance programs to further assess and monitor the product's safety and effectiveness after commercialization. The FDA may also require a REMS, which could involve requirements for, among other things, medication

guides, special trainings for prescribers and dispensers, patient registries, and elements to assure safe use.

In addition, entities involved in the manufacture and distribution of approved drugs are required to register their establishments with the FDA and state agencies, and are subject to periodic unannounced inspections by the FDA and these state agencies for compliance with cGMP requirements. The FDA has promulgated specific requirements for drug cGMPs. Changes to the manufacturing process are strictly regulated and often require prior FDA approval before being implemented. FDA regulations also require investigation and correction of any deviations from cGMP requirements and impose reporting and documentation requirements upon the sponsor and any third-party manufacturers that the sponsor may decide to use. Accordingly, manufacturers must continue to expend time, money, and effort in the area of production and quality control to maintain cGMP compliance.

Once an approval is granted, the FDA may issue enforcement letters or withdraw the approval if compliance with regulatory requirements and standards is not maintained or if problems occur after the product reaches the market. Corrective action could delay product distribution and require significant time and financial expenditures. Later discovery of previously unknown problems with a product, including AEs of unanticipated severity or frequency, or with manufacturing processes, or failure to comply with regulatory requirements, may result in revisions to the approved labeling to add new safety information; imposition of post-market studies or clinical trials to assess new safety risks; or imposition of distribution or other restrictions under a REMS program. Other potential consequences include, among other things:

- restrictions on the marketing or manufacturing of the product, suspension of the approval, complete withdrawal of the product from the market or product recalls;
- fines, warning letters or holds on post-approval clinical trials;
- refusal of the FDA to approve applications or supplements to approved applications, or suspension or revocation of product approvals;
- product seizure or detention, or refusal to permit the import or export of products; or
- injunctions or the imposition of civil or criminal penalties.

The FDA strictly regulates marketing, labeling, advertising and promotion of products that are placed on the market. Drugs may be promoted only for the approved indications and in accordance with the provisions of the approved label. The FDA and other agencies actively enforce the laws and regulations prohibiting the promotion of off-label uses, and a company that is found to have improperly promoted off-label uses may be subject to significant liability, including investigation by federal and state authorities.

Section 505(b)(2) NDAs

As an alternative path to FDA approval for modifications to formulations or uses of drugs previously approved by the FDA, an applicant may submit an NDA under Section 505(b)(2) of the FDCA. Section 505(b)(2) was enacted as part of the Hatch-Waxman Amendments. A Section 505(b)(2) NDA is an application that contains full reports of investigations of safety and effectiveness, but where at least some of the information required for approval comes from studies not conducted by, or for, the applicant and for which the applicant has not obtained a right of reference or use from the company by or for whom the investigations were conducted. This type of application permits reliance for such approvals on literature or on an FDA finding of safety, effectiveness or both for an approved drug product. As such, under Section 505(b)(2), the FDA may rely, for approval of an NDA, on data not developed by the applicant. The FDA may also require companies to perform additional studies or measurements, including clinical trials, to support the change from the approved branded reference drug. The FDA may then approve the new product candidate for the new indication sought by the 505(b)(2) applicant.

Our clinical program for troriluzole for the treatment of SCA and the treatment of OCD is based on a regulatory pathway under section 505(b)(2) of the FDCA that allows reference to data on riluzole for the purpose of safety assessments.

Product Exclusivity - United States

In the United States, biopharmaceutical products are protected by patents with varying terms depending on the type of patent and the filing date. A significant portion of a product's patent life, however, is lost during the time it takes an innovative company to develop and obtain regulatory approval of a new drug. As compensation at least in part for the lost patent term due to regulatory review periods, the innovator may, depending on a number of factors, apply to the government to restore lost patent term by extending the expiration date of one patent up to a maximum term of five years, provided that the extension cannot cause the patent to be in effect for more than 14 years from the date of drug approval. A company seeking to market an innovative pharmaceutical in the U.S. must submit a complete set of safety and efficacy data to the FDA. If the innovative pharmaceutical is a chemical product, the company files an NDA. If the medicine is a biological product, a Biologic License Application ("BLA") is filed. The type of application filed affects regulatory data protection ("RDP") exclusivity rights.

Small Molecule Products

A competitor seeking to launch a generic substitute of small molecule drug in the U.S. must file an Abbreviated New Drug Application ("ANDA") with the

FDA. In the ANDA, the generic manufacturer needs to demonstrate only "bioequivalence" between the generic substitute and the approved NDA drug. The ANDA relies upon the safety and efficacy data previously filed by the innovator in its NDA. An innovator company is required to list certain of its patents covering the medicine with the FDA in what is commonly known as the FDA's Orange Book. The FDA cannot approve an ANDA until after the innovator's listed patents expire unless there is a successful patent challenge. However, after the innovator has marketed its product for four years, a generic manufacturer may file an ANDA and allege that one or more of the patents listed in the Orange Book under an innovator's NDA is either invalid or not infringed (a Paragraph IV certification). The innovator then must decide whether to file a patent infringement suit against the generic manufacturer. From time to time, ANDAs, including Paragraph IV certifications, could be filed with respect to certain of our products.

In addition to patent protection, certain innovative pharmaceutical products can receive periods of regulatory exclusivity. An NDA that is designated as an orphan drug can receive seven years of exclusivity for the orphan indication. During this time period, neither NDAs nor ANDAs for the same drug product can be approved for the same orphan use. A company may also earn six months of additional exclusivity for a drug where specific clinical studies are conducted at the written request of the FDA to study the use of the medicine to treat pediatric patients, and submission to the FDA is made prior to the loss of basic exclusivity. Medicines approved under an NDA can also receive several types of RDP. An innovative chemical pharmaceutical product is entitled to five years of RDP in the U.S., during which the FDA cannot approve generic substitutes. If an innovator's patent is challenged, as described above, a generic manufacturer may file its ANDA after the fourth year of the five-year RDP period. A pharmaceutical drug product that contains an active ingredient that has been previously approved in an NDA, but is approved in a new formulation, but not for the drug itself, or for a new indication on the basis of new clinical studies, may receive three years of RDP for that formulation or indication.

Biologic products

The ACA, which includes a subtitle called the Biologics Price Competition and Innovation Act of 2009, created an approval pathway for biosimilar versions of innovative biological products that did not previously exist. Prior to that time, innovative biologics had essentially unlimited regulatory exclusivity. Under the new regulatory mechanism, the FDA can approve products that are similar to (but not generic copies of) innovative biologics on the basis of less extensive data than is required by a full BLA. After an innovator has marketed its product for four years, any manufacturer may file an application for approval of a "biosimilar" version of the innovator product. However, although an application for approval of a biosimilar version may be filed four years after approval of the innovator product, qualified innovative biological products will receive 12 years of regulatory exclusivity, meaning that the FDA may not approve a biosimilar version until 12 years after the innovative biological product was first approved by the FDA. The law also provides a mechanism for innovators to enforce the patents that protect innovative biological products and for biosimilar applicants to challenge the patents. Such patent litigation may begin as early as four years after the innovative biological product is first approved by the FDA.

In the U.S., the increased likelihood of generic and biosimilar challenges to innovators' intellectual property has increased the risk of loss of innovators' market exclusivity. First, generic companies have increasingly sought to challenge innovators' basic patents covering major pharmaceutical products. Second, statutory and regulatory provisions in the U.S. limit the ability of an innovator company to prevent generic and biosimilar drugs from being approved and launched while patent litigation is ongoing. As a result of all of these developments, it is not possible to predict the length of market exclusivity for a particular product with certainty based solely on the expiration of the relevant patent(s) or the current forms of regulatory exclusivity.

Foreign Regulation

In order to market any product outside of the United States, we would need to comply with numerous and varying regulatory requirements of other countries and jurisdictions regarding quality, safety and efficacy and governing, among other things, clinical trials, marketing authorization, commercial sales and distribution of our products. Although many of the issues discussed above with respect to the United States apply similarly in the context of the European Union and other geographies, the approval process varies between countries and jurisdictions and can involve additional product testing and additional administrative review periods. The time required to obtain approval in other countries and jurisdictions might differ from and be longer than that required to obtain FDA approval. Regulatory approval in one country or jurisdiction does not ensure regulatory approval in another, but a failure or delay in obtaining regulatory approval in one country or jurisdiction may negatively impact the regulatory process in others.

European Union

A typical route used by innovator companies to obtain marketing authorization of pharmaceutical products in the EU is through the "centralized procedure." A company seeking to market an innovative pharmaceutical product through the centralized procedure must file a complete set of safety data and efficacy data as part of a MAA with the EMA. After the EMA evaluates the MAA, it provides a recommendation to the European Commission ("EC") and the EC then approves or denies the MAA. Regulatory approval via the centralized procedure results in a marketing authorization for the innovative pharmaceutical product

in each EU member state. It is also possible for new chemical products to obtain marketing authorization in the EU through a “mutual recognition procedure,” in which an application is made to a single member state, and if the member state approves the pharmaceutical product under a national procedure, then the applicant may submit that approval to the mutual recognition procedure of some or all other member states. After obtaining marketing authorization approval, a company must obtain pricing and reimbursement for the pharmaceutical product, which is typically subject to member state law. In certain EU countries, this process can take place simultaneously while the product is marketed but in other EU countries, this process must be completed before the company can market the new product. The pricing and reimbursement procedure can take months and sometimes years to complete. Throughout the EU, all products for which marketing authorizations have been filed after October/November 2005 are subject to an “8+2+1” regime. Eight years after the innovator has received its first community authorization for a medicinal product, a generic company may file a MAA for that product with the health authorities. If the MAA is approved, the generic company may not commercialize the product until after either 10 or 11 years have elapsed from the initial marketing authorization granted to the innovator. The possible extension to 11 years is available if the innovator, during the first eight years of the marketing authorization, obtains an additional indication that is of significant clinical benefit in comparison with existing treatments. For products that were filed prior to October/November 2005, there is a 10-year period of data protection under the centralized procedures and a period of either six or 10 years under the mutual recognition procedure (depending on the member state). In contrast to the U.S., patents in the EU are not listed with regulatory authorities. Generic versions of pharmaceutical products can be approved after data protection expires, regardless of whether the innovator holds patents covering its drug. Thus, it is possible that an innovator may be seeking to enforce its patents against a generic competitor that is already marketing its product. Also, the European patent system has an opposition procedure in which generic manufacturers may challenge the validity of patents covering innovator products within nine months of grant. In general, EU law treats chemically-synthesized drugs and biologically-derived drugs the same with respect to intellectual property and data protection. In addition to the relevant legislation and annexes related to biologic medicinal products, the EMA has issued guidelines that outline the additional information to be provided for biosimilar products, also known as generic biologics, in order to review an application for marketing approval.

Japan

In Japan, medicines of new chemical entities are generally afforded eight years of data exclusivity for approved indications and dosage. Patents on pharmaceutical products are enforceable. Generic copies can receive regulatory approval after data exclusivity and patent expirations. As in the U.S., patents in Japan may be extended to compensate for the patent term lost during the regulatory review process. In general, Japanese law treats chemically-synthesized and biologically-derived drugs the same with respect to intellectual property and market exclusivity.

China

To obtain marketing authorization of pharmaceutical products in China, an NDA must be submitted to the National Medical Products Administration ("NMPA") once safety and efficacy has been established in Chinese patients. For imported drugs, this means issuance of an import license. The applicant must submit evidence of foreign approval (certificate of pharmaceutical product), unless it is an innovative drug that has never been approved anywhere in the world.

In China, medicines of new chemical entities are generally afforded six years of data exclusivity for approved indications and dosage. Generic copies can receive regulatory approval after data exclusivity and patent expirations.

South Korea

To obtain marketing authorization of pharmaceutical products in South Korea, a marketing application must be submitted to the Ministry of Food and Drug Safety ("MFDS"). The application must contain data in South Korean patients, information regarding safety and efficacy, quality, a good manufacturing practice certificate, and a certificate of pharmaceutical product in an approved country to show that the drug being imported is being sold in the approved country in accordance with the with the relevant rules and regulations in that country.

In South Korea, medicines of new chemical entities are generally afforded six years of data exclusivity for first approved indications and dosage. Generic copies can receive regulatory approval after data exclusivity and patent expirations.

Rest of the World

In countries outside of the U.S., the EU, Japan, China and South Korea, there is a wide variety of legal systems with respect to intellectual property and market exclusivity of pharmaceuticals. Most other developed countries utilize systems similar to either the U.S. or the EU. Among developing countries, some have adopted patent laws and/or regulatory exclusivity laws, while others have not. Some developing countries have formally adopted laws in order to comply with World Trade Organization ("WTO") commitments, but have not taken steps to implement these laws in a meaningful way. Enforcement of WTO actions is a long process between governments, and there is no assurance of the outcome.

Coverage, Reimbursement and Pricing

Challenges exist that pertain to the coverage and reimbursement status of any products for which regulatory approval is sought. In the United States and

foreign markets, sales of any products that receive regulatory approval for commercial sale will depend, in part, on the availability of coverage and the adequacy of reimbursement from third-party payors. Third-party payors include government authorities, such as Medicare and Medicaid, and private entities, such as managed care organizations, private health insurers and other organizations. The process for determining whether a third-party payor will provide coverage for a product may be separate from the process for setting the reimbursement rate that the payor will pay for the product. The latter is often informed by entities such as the Institute for Clinical and Economic Review ("ICER") which provides a reimbursement rate based on a multifactorial value assessment. Third-party payors may limit coverage to specific products on an approved list, or formulary, which might not include all of the FDA-approved products for a particular indication. Typically, patients must "step through," or fail less expensive therapies such as generics in order to be prescribed a branded therapy. Moreover, a third-party payor's decision to provide coverage for a product does not imply that an adequate reimbursement rate will be approved. For example, the payor's reimbursement payment rate may not be adequate or may require patient co-payments that patients find unacceptably high. Additionally, coverage and reimbursement for products can differ significantly from one payor system to the next. Private payor systems set reimbursement policy in accordance with their particular model. For example, some payor systems mandate value based pricing wherein a particular price point is premised upon achieving a particular goal. These can include an improvement in patients' clinical course (therapeutic effectiveness), or reductions in drug and health care utilization and cost. Thus a third-party payor's decision to cover a particular product does not ensure that other payors will also provide the same level of coverage for the product, or will provide coverage at an adequate reimbursement rate. Adequate third-party reimbursement may not be available to enable us to maintain price levels sufficient to realize an appropriate return on our investment in product development.

Third-party payors require evidence of value that are supplemental to the regulatory mandates of safety and efficacy in order to support a particular price. To obtain coverage and reimbursement for any product that might be approved for sale, there is often a need to conduct expensive pharmacoeconomic studies to demonstrate the medical necessity (based on evidence of disease burden and unmet need) and cost-effectiveness of the therapy. As mentioned, the ICER evidence review mandates such information. These studies will be in addition to the studies required to obtain regulatory approvals. If third-party payors do not consider a product to be cost-effective compared to other available therapies, they may not cover the product after approval as a benefit under their plans or, they may deem a subpopulation of eligible patients based on greater unmet need as eligible for reimbursement. Thus, obtaining and maintaining reimbursement status can be time-consuming and costly. However, drug developers accept these requirements as a condition of reimbursement, analogous to their acceptance of the level of evidence needed to obtain regulatory approval.

The U.S. and foreign governments regularly consider reform measures that affect health care coverage and costs. For example, the U.S. and particularly state legislatures have implemented cost containment programs that include price controls, restrictions on reimbursement and "first use" of generic products prior to access to branded prescriptions. The Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act ("collectively, the ACA") contains provisions such as increased rebates for products sold to Medicaid programs, extension of Medicaid rebates to Medicaid managed care plans, mandatory discounts for certain Medicare Part D beneficiaries and annual fees based on pharmaceutical companies' share of sales to federal health care programs. The Centers for Medicare and Medicaid Services ("CMS") may develop new payment and delivery models, such as bundled payment models. For example, the U.S. Department of Health and Human Services ("HHS") moved 41% of Medicare fee-for-service payments to alternative payment models ("APMs") tied to the quality or value of services by the end of 2018. HHS had set a goal of moving 50% of such Medicare payments into these alternative payment models by the end of 2018, but in 2019, this performance goal was discontinued and replaced it with a new developmental goal to increase the percentage of Medicare health care dollars tied to APMs incorporating downside risk, with a target of 40% for fiscal year 2021. These constitute significant challenges, which are analogous to the regulatory hurdles in many aspects and drug developers acknowledge these challenges as the path to providing safe and effective therapies to the patients that require them.

European Union Coverage Reimbursement and Pricing

In the European Union, pricing and reimbursement requirements can vary widely from country to country. Some countries link market authorization to reimbursement decisions. Others may require the completion of additional studies that assess the cost-effectiveness or comparative effectiveness of novel approved drugs relative to standard of care. These are compiled as health technology assessments ("HTAs"), that constitute a requisite for reimbursement or pricing approval. For example, the European Union provides options for its member states to restrict the range of drug products for which their national health insurance systems provide reimbursement and to control the prices of medicinal products for human use. European Union member states may approve a specific price for a drug product or may instead adopt a system of access restrictions that typically target sub populations with high unmet need.

Healthcare Laws and Regulations

Physicians, other healthcare providers, and third-party payors will play a primary role in the recommendation and prescription of any product candidates for which we obtain marketing approval. Future arrangements with healthcare professionals, principal investigators, consultants, customers and third-party payors are and will be subject to various federal, state and foreign fraud and abuse laws and other healthcare laws and regulations. These laws and regulations may impact, among other things, healthcare professionals who participate in our clinical research programs, and our proposed sales, marketing, distribution, and education programs. The U.S. federal and state healthcare laws and regulations that may affect our ability to operate include, without limitation, the following:

- The federal Anti-Kickback Statute, which prohibits persons from, among other things, knowingly and willfully soliciting, receiving, offering or paying remuneration, directly or indirectly, in cash or in kind, to induce or reward either the referral of an individual for, or the purchase, order or recommendation of, any good or service, for which payment may be made under federally funded healthcare programs, such as Medicare and Medicaid. The term "remuneration" has been broadly interpreted to include anything of value;

- The federal civil and criminal false claims laws, including, without limitation, the federal civil monetary penalties law and the civil False Claims Act (which can be enforced by private citizens through qui tam actions), prohibit individuals or entities from, among other things, knowingly presenting, or causing to be presented, false or fraudulent claims for payment of federal funds, and knowingly making, or causing to be made, a false record or statement material to a false or fraudulent claim to avoid, decrease or conceal an obligation to pay money to the federal government;

- The federal Health Insurance Portability and Accountability Act of 1996 ("HIPAA") which imposes criminal liability for executing or attempting to execute a scheme to defraud any healthcare benefit program and creates federal criminal laws that prohibit knowingly and willfully falsifying, concealing or covering up a material fact or making any materially false statement in connection with the delivery of or payment for healthcare benefits, items or services;

- HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act ("HITECH") enacted as part of the American Recovery and Reinvestment Act of 2009 and its implementing regulations, which imposes certain obligations, including mandatory contractual terms, on entities subject to the law, such as healthcare providers, health plans, and healthcare clearinghouses and their respective business associates to safeguard the privacy, security and transmission of individually identifiable health information from any unauthorized use or disclosures;

- The federal transparency requirements under the Physician Payments Sunshine Act, created under the ACA, which requires certain manufacturers of drugs, devices, biologics and medical supplies reimbursed under Medicare, Medicaid, and other programs such as CHIP to report to HHS information related to payments and other transfers of value provided to physicians and teaching hospitals and physician ownership and investment interests; and

- Analogous state laws and regulations, such as state anti-kickback and false claims laws, that impose similar restrictions and may apply to items or services reimbursed by non-governmental third-party payors, including private insurers; state laws that require pharmaceutical companies to implement compliance programs, comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government, or to track and report gifts, compensation and other remuneration provided to physicians and other health care providers; and state health information privacy and data breach notification laws, which govern the collection, use, disclosure, and protection of health-related and other personal information, many of which differ from each other in significant ways and some of which are not pre-empted by HIPAA, thus complicating compliance efforts.

We will be required to spend substantial time and money to ensure that our business arrangements with third parties comply with applicable healthcare laws and regulations. Healthcare reform legislation has strengthened these federal and state healthcare laws. For example, the ACA amended the intent requirement of the federal Anti-Kickback Statute and criminal healthcare fraud statutes to clarify that liability under these statutes does not require a person or entity to have actual knowledge of the statutes or a specific intent to violate them. Moreover, the ACA provides that the government may assert that a claim that includes items or services resulting from a violation of the federal Anti-Kickback Statute constitutes a false or fraudulent claim for purposes of the civil False Claims Act. Because of the breadth of these laws and the narrowness of the statutory exceptions and safe harbors available, it is possible that some of our business activities could be subject to challenge under one or more of such laws.

Violations of these laws can subject us to criminal, civil and administrative sanctions including monetary penalties, damages, fines, disgorgement, individual imprisonment, and exclusion from participation in government funded healthcare programs, such as Medicare and Medicaid, additional reporting requirements and oversight if we become subject to a corporate integrity agreement or similar agreement to resolve allegations of non-compliance with these laws, and reputational harm, we may be required to curtail or restructure our operations. Moreover, we expect that

there will continue to be federal and state laws and regulations, proposed and implemented, that could impact our future operations and business.

Healthcare Reform

The legislative landscape in the United States continues to evolve. There have been a number of legislative and regulatory changes to the healthcare system that could affect our future results of operations. In particular, there have been and continue to be a number of initiatives at the United States federal and state levels that seek to reduce healthcare costs. In March 2010, the ACA was enacted, which includes measures that have significantly changed health care financing by both governmental and private insurers. Since its enactment, there have been judicial, executive and Congressional challenges to certain aspects of the ACA. On June 17, 2021, the U.S. Supreme Court dismissed the most recent judicial challenge to the ACA brought by several states, without specifically ruling on the ACA's constitutionality.

The provisions of the ACA of importance to the pharmaceutical and biotechnology industry are, among others, the following:

- an annual, non-deductible fee on any entity that manufactures or imports certain branded prescription drugs and biologic agents, which is apportioned among these entities according to their market share in certain government healthcare programs;
- a Medicare Part D coverage gap discount program, in which manufacturers must now agree to offer 70% point-of-sale discounts off negotiated prices of applicable brand drugs to eligible beneficiaries during their coverage gap period, as a condition for the manufacturer's outpatient drugs to be covered under Medicare Part D;
- requirements to report certain financial arrangements with physicians and certain others, including reporting "transfers of value" made or distributed to prescribers and other healthcare providers and reporting investment interests;
- an increase in the statutory minimum rebates a manufacturer must pay under the Medicaid Drug Rebate Program to 23.1% and 13.0% of the average manufacturer price for branded and generic drugs, respectively;
- a methodology by which rebates owed by manufacturers under the Medicaid Drug Rebate Program are calculated for drugs that are inhaled, infused, instilled, implanted or injected;
- extension of a manufacturer's Medicaid rebate liability to covered drugs dispensed to individuals who are enrolled in Medicaid managed care organizations;
- expansion of eligibility criteria for Medicaid programs by, among other things, allowing states to offer Medicaid coverage to certain individuals with income at or below 133% of the federal poverty level, thereby potentially increasing a manufacturer's Medicaid rebate liability;
- expansion of the entities eligible for discounts under the Public Health Service pharmaceutical pricing program;
- a new Patient-Centered Outcomes Research Institute to oversee, identify priorities in, and conduct comparative clinical effectiveness research, along with funding for such research; and
- establishment of the Center for Medicare Innovation at the Centers for Medicare and Medicaid Services ("CMS"), to test innovative payment and service delivery models to lower Medicare and Medicaid spending, potentially including prescription drug spending.

Some of the provisions of the ACA have yet to be implemented, and there have been judicial and Congressional challenges to certain aspects of the ACA. In January of 2021, an Executive Order entitled "Executive Order on Strengthening Medicaid and the Affordable Care Act" repealed two previous Executive Orders delaying the implementation of certain provisions of the ACA. Concurrently, Congress has considered legislation that amend all or part of the ACA.

In addition, other federal health reform measures have been proposed and adopted in the United States since the ACA was enacted. These changes include aggregate reductions to Medicare payments to providers of up to 2% per fiscal year pursuant to the Budget Control Act of 2011 (known as Medicare sequestration) and subsequent extensions, which began in 2013 and will remain in effect through 2030 (with the exception of a temporary suspension from May 1, 2020 through March 31, 2022, with a subsequent one quarter phase-in of 1%) unless additional Congressional action is taken. Further, the American Taxpayer Relief Act of 2012 reduced Medicare payments to several providers and increased the statute of limitations period for the government to recover overpayments from providers from three to five years. The Medicare Access and CHIP Reauthorization Act of 2015 also introduced a quality payment program under which certain individual Medicare providers will be subject to certain incentives or penalties based on new program quality standards.

Further, there have been several Congressional inquiries and proposed federal and state legislation designed to, among other things, bring more transparency to product pricing, review the relationship between pricing and manufacturer patient programs, and reform government program reimbursement methodologies for products. At the federal level, the costs of prescription pharmaceuticals in the United States has also been the subject of considerable discussion. The previous administration released a "Blueprint" to lower drug prices and reduce out-of-pocket costs of drugs. HHS solicited feedback on some

of these measures and, concurrently, implemented others under its existing authority. President Biden continues to push for reforms that would address the high cost of drugs. In response to an Executive Order from President Biden, the Secretary of HHS issued a comprehensive plan for addressing high drug prices that describes a number of legislative approaches and identifies administrative tools to address the high cost of drugs. Democrats included drug pricing reform provisions reflecting elements of the plan in a broader spending package in late 2021—such as capping Medicare Part D patients' out-of-pocket costs, establishing penalties for drug prices that increase faster than inflation in Medicare, and authorizing the federal government to negotiate prices on certain select, high-cost drugs under Medicare Parts B and D. While a number of these and other proposed measures would require authorization through additional legislation to become effective, Congress has indicated that it will continue to seek new legislative and/or administrative measures to control drug costs. In August 2022, Congress passed the Inflation Reduction Act of 2022, which included, among other things, a provision allowing Medicare to negotiate drug prices directly with pharmaceutical manufacturers.

At the state level, legislatures are increasingly aggressive in passing legislation and implementing regulations designed to control pharmaceutical and biological product pricing, including price or patient reimbursement constraints, discounts, restrictions on certain product access and marketing cost disclosure and transparency measures, and, in some cases, designed to encourage importation from other countries and bulk purchasing. In addition, regional healthcare authorities and individual hospitals are increasingly using bidding procedures to determine what pharmaceutical products and which suppliers will be included in their prescription drug and other healthcare programs. These measures could reduce the ultimate demand for our products, once approved, or put pressure on our product pricing.

The Foreign Corrupt Practices Act

The Foreign Corrupt Practices Act (the "FCPA") prohibits any U.S. individual or business from paying, offering, or authorizing payment or offering of anything of value, directly or indirectly, to any foreign official, political party or candidate for the purpose of influencing any act or decision of the foreign entity in order to assist the individual or business in obtaining or retaining business. The FCPA also obligates companies whose securities are listed in the United States to comply with accounting provisions requiring the company to maintain books and records that accurately and fairly reflect all transactions of the corporation, including international subsidiaries, and to devise and maintain an adequate system of internal accounting controls for international operations. Activities that violate the FCPA, even if they occur wholly outside the United States, can result in criminal and civil fines, imprisonment, disgorgement, oversight, and debarment from government contracts.

Environmental, Social, Governance, and Human Capital

Governance and Leadership

Our commitment to integrating sustainability across our organization begins with our Board of Directors. The Nominating and Governance Committee of the Board has oversight of strategy and risk management related to Environmental, Social and Governance ("ESG"). Applying NYSE's listing standards for independence, six of our eight directors are independent.

At the management level, we have implemented a cross-functional Sustainability Working Group, which meets on a regular basis and reports to the Board of Directors periodically. We also maintain a Chief Talent & Sustainability Officer position who works closely with the working group and coordinate efforts related to the advancement of ESG capabilities across the organization.

Business Ethics

We are committed to creating an environment where we are able to excel in our business while maintaining the highest standards of conduct and ethics. Our Code of Business Conduct and Ethics (the "Code of Conduct") will reflect the business practices and principles of behavior that support this commitment, including our policies on bribery, corruption, conflicts of interest and our whistleblower program. We expect every director, officer, and employee to read, understand, and comply with the Code of Conduct and its application to the performance of his or her business responsibilities.

We encourage employees to come to us with observations and complaints, ensuring we understand the severity and frequency of an event in order to escalate and assess accordingly. Our Chief Compliance Officer strives to ensure accountability, objectivity, and compliance with our Code of Conduct. If a complaint is financial in nature, the Audit Committee Chair is notified concurrently, which triggers an investigation, action, and report. All incidents are reported up to the Board of Directors on a quarterly basis.

Environmental Commitment

We are committed to protecting the environment and attempt to mitigate any negative impact of our operations. We monitor resource use, improve efficiency, and at the same time reduce our emissions and waste.

Externally, we strive to reduce the overall impact of our product on the environment by taking steps to enhance the sustainability of our manufacturing processes for our drug substances.

In collaboration with our contract research organization partners, we apply various green chemistry methodologies to our commercial and development

pipeline. We have especially focused on using biocatalysis, a technology that makes use of enzymes instead of chemicals to accomplish specific chemical reactions used to construct organic small molecules such as Active Pharmaceutical Ingredients.

We have also initiated work in removing hazardous organic solvents from certain reactions and replacing them with water. This green technology relies on the use of micelles to enable such reactions to occur in water where they would normally not occur due in part to the very poor solubility of most organic compounds in water. These greener processes not only create less waste, but the waste that is produced is much less hazardous, therefore reducing the environmental impact of the manufacturing process.

Social Responsibility

For third-party vendor selection and oversight, we have adopted standard operating procedures that apply to employees and subcontractors who on our behalf, oversee and conduct research regulated by the FDA. We retain ultimate authority and responsibility for the conduct of regulated research, manufacturing, and testing and we must ensure that contracted services are conducted in accordance with Good Practice Guidelines and all applicable regulations.

Human Capital Management

We foster and encourage a workplace environment that holds possibilities for everyone, with a commitment to respect and acceptance without biases.

Development and continuous feedback are priorities for our organization, which was comprised of 239 employees as of December 31, 2023. We believe each person is critical to our success and we invest in our people by supporting continuous training programs and courses. We encourage each employee to engage with their manager in developmental discussions designed to focus on feedback rather than a rating.

An important part of our talent recruitment is our robust paid internship program for high school, college and graduate-level students. This program offers opportunities to students in the community and develops a roadmap for ‘entry-level’ candidates. We evaluate the success of our recruitment program through metrics such as time to hire, offer acceptance rate, turnover rate and business results.

We strive to provide an inclusive workplace to foster growth and innovation. Our efforts to achieve gender diversity in the workforce are evidenced by a workforce that is approximately 57% female and includes a robust group of female leaders in the scientific and associated fields.

Biohaven engages in forward-thinking people policies to allow for our employees to thrive in our workforce. Regular attendance at an office is only required of our lab professionals, allowing over 50% of our workforce to work remotely full-time. Our vacation policy is unlimited and is aimed at giving employees the ability to achieve work/life balance in a way that is bespoke to their circumstances.

Information about Segments

We currently operate in a single business segment developing a portfolio of treatments in therapeutic areas including neuroscience, immunology and oncology. See additional information in our financial statements contained in Part II, Item 8 of this Annual Report.

Corporate Information

We are a business company limited by shares organized under the laws of the British Virgin Islands. Our registered office is located at P.O. Box 173, Road Town, Tortola, British Virgin Islands and our telephone number is +1 (284) 852-3000. Our U.S. subsidiary's office is located at 215 Church Street, New Haven, Connecticut 06510 and telephone number is (203) 404-0410. Our website address is www.biohaven.com. The information contained on our website is not incorporated by reference into this Annual Report, and you should not consider any information contained on, or that can be accessed through, our website as part of this Annual Report or in making an investment decision regarding our common shares. On September 16, 2022, the Company changed its name from “Biohaven Research Ltd.” to “Biohaven Ltd.”

Available Information

Our internet website address is www.biohaven.com. In addition to the information about us and our subsidiaries contained in this Annual Report, information about us can be found on our website. Our website and information included in or linked to our website are not part of this Annual Report.

Our annual reports on Form 10-K, quarterly reports on Form 10-Q, current reports on Form 8-K and amendments to those reports filed or furnished pursuant to Section 13(a) or 15(d) of the Securities Exchange Act of 1934, as amended, are available free of charge through our website as soon as reasonably practicable after they are electronically filed with or furnished to the Securities and Exchange Commission ("SEC"). A copy of these reports is also available at the SEC's website (www.sec.gov).

Item 1A. Risk Factors

In connection with any investment decision with respect to our securities, you should carefully consider the risks described below, as well as general economic and business risks and the other information in this Annual Report on Form 10-K and in our other filings with the SEC. The occurrence of any of the events or circumstances described below or other adverse events could have a material adverse effect on our business, results of operations and financial condition and could cause the trading price of our common shares to decline. Additional risks or uncertainties not presently known to us or that we currently deem immaterial may also harm our business.

SUMMARY

An investment in our common shares is subject to a number of risks, including risks related to our product candidates, risks related to our business and risks related to our common shares. The following list of risk factors is not exhaustive. Please read the information in the section captioned “Risk Factors” for a more thorough description of these and other risks.

Risks Related to Our Financial Position and Need for Additional Capital

- We have a limited operating history, have incurred significant operating losses since inception and anticipate that we will continue to incur substantial operating losses for the foreseeable future.
- Deterioration in general economic conditions in the United States and globally, including the effect of prolonged periods of inflation on our suppliers, third-party service providers and potential partners, may have a negative impact on our business and results of operations.
- An inability to raise capital when needed or on terms favorable to us could force us to curtail our planned operations and growth strategy.
- Credit risk with respect to our investments or the financial institutions at which we deposit funds could adversely affect us.

Risks Related to the Development of Our Product Candidates

- We depend entirely on the success of a limited number of product candidates.
- Clinical trials are very expensive, time consuming and difficult to design and implement, involve uncertain outcomes and may not be predictive of results of future trials.
- Regulatory approval processes in the U.S. and foreign jurisdictions are lengthy, time consuming and unpredictable.
- Our product candidates may fail to demonstrate safety and efficacy in clinical trials, or may cause serious adverse or unacceptable side effects.
- We may become exposed to costly and damaging liability claims, which may not be covered by insurance.

Risks Related to Commercialization of Our Product Candidates

- We have never commercialized a product candidate and may lack the necessary expertise, personnel and resources to successfully commercialize any product candidate that may receive regulatory approval.
- We operate in a highly competitive and rapidly changing industry.
- Failure to obtain or maintain adequate coverage and reimbursement for our approved product candidates could limit our ability to market those products and decrease our ability to generate revenue.
- Our product candidates, if approved, will be subject to ongoing regulatory oversight.
- Our approved product candidates may fail to achieve the degree of market acceptance by physicians, patients, third-party payors and others in the medical community necessary for commercial success.

Risks Related to Our Dependence on Third Parties

- We rely on third parties to conduct our preclinical studies and clinical trials and to supply, manufacture and distribute clinical drug supplies for our product candidates, which may expose our business to risks.
- We may not establish or maintain collaborations with third parties to develop or commercialize product candidates.

Risks Related to Regulatory Compliance

- Enacted and future legislation may increase the difficulty and cost for us to obtain marketing approval of and commercialize our product candidates and may affect the prices we may set.
- Our business operations and relationships with investigators, health care professionals, consultants, third-party payors and customers are subject to federal and state healthcare and other laws.
- We may not obtain or maintain orphan drug designation or exclusivity for our product candidates.

Risks Related to Our Intellectual Property

- We could lose market exclusivity earlier than expected.

- If we were unable to obtain licenses from third parties on commercially reasonable terms or lose our rights to licensed intellectual property, we may not be able to continue developing or commercializing our product candidates.
- Patent terms may not provide exclusivity for our product candidates for an adequate amount of time to realize sufficient commercial benefits.
- Third parties may seek to invalidate our patents.
- Third parties may initiate legal proceedings alleging that we are infringing their intellectual property rights, the outcome of which would be uncertain and could have a negative impact on the success of our business.

Risks Related to Our Business Operations, Employee Matters and Managing Growth

- Our future growth and ability to compete depend on, among other things, retaining key personnel and recruiting additional qualified personnel and on our ability to penetrate foreign markets.
- Laws and regulations governing our international operations may preclude us from developing, manufacturing and selling certain product candidates and products outside of the United States and require us to develop and implement costly compliance programs.
- We may encounter difficulties in managing our growth, which could disrupt our operations.
- Our employees, independent contractors, consultants, commercial collaborators, principal investigators, CROs and vendors may engage in improper activities.
- Our business and operations may be materially adversely affected in the event of computer system failures or security breaches.

Risks Related to Ownership of Our Common Shares

- Substantially all of our total outstanding shares may be sold freely into the market. This could cause the market price of our common shares to drop significantly, even if our business is doing well.
- Because we do not expect to pay dividends on our common shares in the foreseeable future, capital appreciation, if any, would be your sole source of gain.
- The trading price of our common shares may be volatile and may fluctuate.
- If we are or become a passive foreign investment company, there could be adverse U.S. federal income tax consequences to U.S. holders.

Risks Related to Our Financial Position and Need for Additional Capital

We have a limited operating history and have never generated any product revenues, which may make it difficult to evaluate the success of our business to date and to assess our future viability.

We were incorporated on May 2, 2022 as a direct, wholly-owned subsidiary of the Former Parent. Our operations to date have been largely focused on organizing and staffing, raising capital and in-licensing the rights to, and advancing the development of, our product candidates, including conducting preclinical studies and clinical trials. We have not yet demonstrated an ability to obtain marketing approvals for any product candidates, manufacture products on a commercial scale, or arrange for a third party to do so on our behalf, or conduct sales and marketing activities necessary for successful commercialization. Consequently, predictions about our future success or viability may not be as accurate as they could be if we had a longer operating history or a history of successfully developing and commercializing products.

We expect our financial condition and operating results to continue to fluctuate from quarter to quarter and year to year due to a variety of factors, many of which are beyond our control. We will need to eventually transition from a company with a research and development focus to a company capable of undertaking commercial activities. We may encounter unforeseen expenses, difficulties, complications and delays, and may not be successful in such a transition.

Deterioration in general economic conditions in the United States and globally, including the effect of prolonged periods of inflation on our suppliers, third-party service providers and potential partners, may have a negative impact on our business and results of operations.

Our business and results of operations could be adversely affected by changes in national or global economic conditions. These conditions include, but are not limited to, high levels of, and rising, inflation, high and rising interest rates, any volatility in the capital markets, energy availability and costs, the negative impacts from pandemics and public health crises (including any lingering or recurring adverse impacts from COVID-19), negative impacts resulting from the military conflict between Russia and the Ukraine, and the effects of governmental initiatives to manage economic conditions. Impacts of such conditions could be passed on to our business in the form of higher costs for labor and materials, higher investigator fees, possible reductions in pharmaceutical industry-wide spending on research and development and acquisitions and higher costs of capital.

We have incurred significant operating losses since our inception as a business of the Former Parent and anticipate that we will continue to incur substantial operating losses for the foreseeable future and may never achieve or maintain profitability.

Since our inception as a business of the Former Parent, we have incurred significant operating losses. Our net loss was $408.2 million, $570.3 million and $213.8 million for the years ended December 31, 2023, 2022 and 2021, respectively. We expect to continue to incur significant expenses and increasing operating losses for the foreseeable future. None of our product candidates has been approved for marketing in the United States, or in any other jurisdiction, and may never receive such approval. It could be several years, if ever, before we have a commercialized product that generates significant revenues. As a result, we are uncertain when or if we will achieve profitability and, if so, whether we will be able to sustain it. The net losses we incur have in the past and may in the future fluctuate significantly from quarter to quarter and year to year. Our expenses have increased, and we anticipate that our expenses will further increase substantially as we:

- initiate, continue, or complete planned or ongoing clinical trials of our current product candidates, including related support activities;
- continue to initiate and progress other supporting studies required for regulatory approval of our product candidates, including long-term safety studies, drug-drug interaction studies, preclinical toxicology and carcinogenicity studies;
- make required milestone and royalty payments under the license agreements by which we acquired some of the rights to our product candidates;
- initiate preclinical studies and clinical trials for any additional indications for our current product candidates and any future product candidates that we may pursue;
- continue to build our portfolio of product candidates through the acquisition or in-license of additional product candidates or technologies;
- continue to develop, maintain, expand and protect our intellectual property portfolio;
- pursue regulatory approvals for our current and future product candidates that successfully complete clinical trials;
- ultimately establish a sales, marketing and distribution infrastructure to commercialize any product candidate for which we may obtain marketing approval;
- hire additional clinical, medical, commercial, and development personnel; and
- incur additional legal, accounting and other expenses in operating as a public company.

To become and remain profitable, we must develop and eventually commercialize one or more product candidates with significant market potential. This will require us to be successful in a range of challenging activities, including completing clinical trials of our product candidates, developing commercial scale manufacturing processes, obtaining marketing approval, manufacturing, marketing and selling any current and future product candidates for which we may obtain marketing approval, and satisfying any post-marketing requirements. We are only in the preliminary stages of most of these activities and, in some cases, have not yet commenced certain of these activities.

We may never succeed in any or all of these activities and, even if we do, we may never generate sufficient revenue to achieve profitability.

Because of the numerous risks and uncertainties associated with product development, we are unable to accurately predict the timing or amount of expenses or when, or if, we will obtain marketing approval to commercialize any of our product candidates. If we are required by the FDA or other regulatory authorities such as the EMA to perform studies and trials in addition to those currently expected, or if there are any delays in the development, or in the completion of any planned or future preclinical studies or clinical trials of our current or future product candidates, our expenses could increase and profitability could be further delayed.

Even if we do achieve profitability, we may not be able to sustain or increase profitability on a quarterly or annual basis. Our failure to become and remain profitable would decrease the value of our company and could impair our ability to raise capital, maintain our research and development efforts, expand our business or continue our operations. A decline in the value of the Company also could cause you to lose all or part of your investment.

We will need substantial additional funding to pursue our business objectives. If we are unable to raise capital when needed or on terms favorable to us, we could be forced to curtail our planned operations and the pursuit of our growth strategy.

Identifying potential product candidates and conducting preclinical studies and clinical trials is a time-consuming, expensive and uncertain process that takes years to complete, and we may never generate the necessary data or results required to obtain regulatory approval and achieve product sales. We expect our expenses to increase in connection with our ongoing activities, particularly as we continue to develop our product candidates. Our expenses could increase beyond our current expectations if the FDA requires us to perform clinical trials and other studies in addition to those that we currently anticipate.

In addition, our product candidates, if approved, may not achieve commercial success. Our revenue, if any, will be derived from sales of products that we do not expect to be commercially available for a number of years, if at all. Additionally, if we obtain marketing approval for our product candidates, we expect to incur significant expenses related to manufacturing, marketing, sales and distribution and, with respect to certain of our product candidates, the payment of milestone and royalty fees. Furthermore, we expect to incur additional costs associated with operating as a public company.

As of December 31, 2023, we had cash, cash equivalents and marketable securities of $381.8 million, excluding restricted cash of $3.7 million. We expect that our existing cash, cash equivalents and marketable securities will be sufficient to fund our planned operating expenses, financial commitments and other cash requirements for at least 12 months from the date of filing of this report. This estimate is based on assumptions that may prove to be wrong, and we could use our available capital resources sooner than we expect. Changes may occur beyond our control that would cause us to consume our available capital before that time, including changes in and progress of our development activities and changes in regulation. Our future capital requirements will depend on many factors, including:

- the scope, progress, results and costs of our ongoing and planned preclinical studies and clinical trials for our product candidates;
- the timing and amount of milestone and royalty payments we are required to make under our license agreements;
- the extent to which we in-license or acquire other product candidates and technologies;
- the number and development requirements of other product candidates that we may pursue, and other indications for our current product candidates that we may pursue;
- the costs, timing and outcome of regulatory review of our product candidates;
- the costs and timing of future commercialization activities, including drug manufacturing, marketing, sales and distribution, for any of our product candidates for which we receive marketing approval;
- the revenue, if any, received from commercial sales of our product candidates for which we receive marketing approval;
- our ability to establish strategic collaborations for the development or commercialization of some of our product candidates; and
- the costs and timing of preparing, filing and prosecuting patent applications, maintaining and enforcing our intellectual property rights and defending any intellectual property-related claims brought by third parties against us.

We will require additional capital to complete our planned clinical development programs for our current product candidates to seek regulatory approval. If we receive regulatory approval for any of our product candidates, we expect to incur significant commercialization expenses related to product manufacturing, sales, marketing and distribution. Any additional capital-raising efforts may divert our management from their day-to-day activities, which may adversely affect our ability to develop and commercialize our current and future product candidates, if approved.

In addition, we cannot guarantee that future financing will be available on a timely basis, in sufficient amounts or on terms acceptable to us, if at all. Moreover, the terms of any financing may adversely affect the holdings or the rights of our shareholders and the issuance of additional securities by us, whether equity or debt, or the market perception that such issuances are likely to occur, could cause the market price of our common shares to decline. As a result, we may not be able to access the capital markets as frequently as comparable companies. If we are unable to obtain funding on a timely basis on acceptable terms, we may be required to significantly curtail, delay or discontinue one or more of our research or development programs or the commercialization of any product candidates, if approved, or be unable to expand our operations or otherwise capitalize on our business opportunities, as desired.

Raising additional capital may cause dilution to our shareholders, restrict our operations or require us to relinquish rights to our intellectual property or future revenue streams.

Until such time as we can generate substantial product revenue, if ever, we expect to finance our operations through a combination of equity offerings, debt financings and license and development agreements in connection with any future collaborations. We do not have any committed external source of funds. In the event we seek additional funds, we may raise additional capital through the sale of equity securities, including through our "at-the-market" equity program, or convertible debt securities. In such an event, our existing shareholders may experience substantial dilution, and the terms of these securities may include liquidation or other preferences that adversely affect the rights of the holders of our common shares. Debt financing, if available, could result in increased fixed payment obligations and may involve agreements that include restrictive covenants, such as limitations on our ability to incur additional debt, make capital expenditures, acquire, sell or license intellectual property rights or declare dividends, and other operating restrictions that could hurt our ability to conduct our business.

Further, if we raise additional capital through collaborations, strategic alliances, or marketing, distribution, licensing or funding arrangements with third parties, we may have to relinquish valuable rights to our intellectual property future revenue streams, research programs or product candidates, or grant licenses on terms that may not be favorable to us.

Credit risk with respect to our investments or the financial institutions at which we deposit funds could adversely affect us.

Financial instruments that expose us to concentrations of credit risk consist of cash, cash equivalents, and short-term debt securities. Market conditions and changing circumstances, many of which are beyond our control, could reduce the value of our investments or impair our ability to access our existing cash, cash equivalents or other investments. For example, rising interest rates could negatively impact the value of investments that are not held to maturity.

We maintain cash deposits that are in excess of the Federal Deposit Insurance Corporation (“FDIC”) insurance limit in FDIC-insured financial institutions. If any financial institution with which we have a banking relationship were to be placed into receivership or become insolvent in the future, we may be unable to access, temporarily or over a longer-term, or we may lose, a portion of our funds on deposit with that institution. For example, on March 10, 2023 and March 12, 2023, Silicon Valley Bank and Signature Bank, respectively, were placed into receivership with the FDIC, which resulted in all funds held at those banks being temporarily inaccessible by their customers.

While we have no relationship with the financial institutions above, any delay in our ability to access our cash, cash equivalents and investments, or the loss of some or all of such funds, could result in us not being able to pay our employees, vendors or others on a timely basis, or at all, and could hinder us from being able to enter into commercial arrangements that could be advantageous to us. Conversely, if any of our counterparties are impacted by any banking failures, that could impact their ability to transact with us. Any of the foregoing could adversely impact, possibly materially, our business and operations.

Risks Related to the Development of Our Product Candidates

Our current business depends entirely on the success of a limited number of product candidates, which are in clinical development. If we do not obtain or are delayed in obtaining regulatory approval for and successfully commercialize one or more of our product candidates, our business, financial condition and results of operations could be materially impacted and we may never become profitable.

Biotechnology product development is a highly speculative undertaking and involves a substantial degree of risk. We do not have any products that have received regulatory approval, and therefore we have never generated any revenue from product sales, and we may never be able to develop product candidates that receive regulatory approval or are successfully

commercialized after regulatory approval is received. Consequently, the revenue-generating potential of our business is unproven and uncertain. We expect that a substantial portion of our efforts and expenses over the next few years will be devoted to the development of our product candidates; specifically, completion of our Phase 3 clinical trials of troriluzole in OCD, execution of clinical trials for BHV-7000, including Phase 2/3 studies in epilepsy and bipolar disorder and a Phase 2 study in MDD, completion of a Phase 2/3 clinical trial of troriluzole in glioblastoma, execution of clinical trials for BHV-2000, including a Phase 3 clinical trial in SMA and a Phase 2 clinical trial in metabolic disorders, initiation of a Phase 1 clinical trial for BHV-1300 in immune mediated diseases, and initiation of a Phase 2/3 clinical trial for BHV-8000 in Parkinson's Disease. As a result, our business currently depends heavily on the successful development, regulatory approval and, if approved, commercialization of these product candidates. We cannot be certain that we will be able to submit a new drug application ("NDA"), biologics license application ("BLA") or comparable applications in other jurisdictions for any of our product candidates within the timeframes we expect, or that any NDA, BLA or similar application we submit will be accepted by the FDA or comparable foreign regulators for filing in a timely manner or at all. The research, testing, manufacturing, safety, efficacy, labeling, approval, sale, marketing and distribution of our product candidates are, and will remain, subject to comprehensive regulation by the FDA and similar foreign regulatory authorities. The success of our product candidates will depend on various factors, including:

- completing clinical trials that demonstrate our product candidates' efficacy and safety;
- receiving marketing approvals from applicable regulatory authorities;
- completing any post-marketing studies required by applicable regulatory authorities;
- establishing commercial manufacturing capabilities;
- launching commercial sales, marketing and distribution operations;
- the prevalence and severity of adverse events experienced with our product candidates;
- acceptance of our product candidates by patients, the medical community and third-party payors;
- a continued acceptable safety profile following approval;
- obtaining and maintaining healthcare coverage and adequate reimbursement for our product candidates;
- competing effectively with other therapies, including with respect to the sales and marketing of our product candidates, if approved; and
- qualifying for, maintaining, enforcing and defending our intellectual property rights and claims.

Many of these factors are beyond our control, including the time needed to adequately complete clinical testing, the regulatory submission process, potential threats to our intellectual property rights and changes in the competitive landscape. Our failure to achieve one or more of these factors in a timely manner or at all could materially harm our business, financial condition and results of operations.

Clinical trials are very expensive, time-consuming and difficult to design and implement and involve uncertain outcomes. Furthermore, results of earlier preclinical studies and clinical trials may not be predictive of results of future preclinical studies or clinical trials.

Clinical testing is expensive and can take many years to complete, and delay or failure can occur at any time during the clinical trial process.

For example, in September 2021, we reported negative topline results from our Phase 3 clinical trial evaluating verdiperstat compared to placebo for the treatment of participants with MSA. In September 2022, we reported negative topline results from the Phase 2/3 HEALEY ALS Platform trial evaluating verdiperstat compared to placebo for the treatment of participants with ALS. At this time, we have no plans to continue development of verdiperstat in ALS, and we are evaluating whether or not to pursue any additional clinical trials evaluating verdiperstat in other disease indications.

In addition, the results generated to date in preclinical studies or clinical trials for our product candidates do not ensure that later preclinical studies or clinical trials will demonstrate similar results. Further, we have limited clinical data for many of our product candidates. Product candidates in later stages of clinical trials may fail to show the desired safety and efficacy traits despite having progressed through preclinical and earlier stage clinical trials. In later-stage clinical trials, we will likely be subject to more rigorous statistical analyses than in completed earlier stage clinical trials.

In some instances, there can be significant variability in safety or efficacy results between different clinical trials of the same product candidate due to numerous factors, including changes in clinical trial procedures set forth in protocols, differences in the size and type of the patient populations, adherence to the dosing regimen, and the rate of dropout among clinical trial participants.

If we fail to produce positive results in our planned preclinical studies or clinical trials of any of our product candidates, the development timeline and regulatory approval and commercialization prospects for our product candidates, and, correspondingly, our business and financial prospects, would be materially adversely affected.

Interim topline and preliminary data from our clinical trials that we announce or publish from time to time may change as more patient data become available and are subject to audit and verification procedures that could result in material changes in the final data.

From time to time, we may publish interim topline or preliminary data from our clinical trials. Interim data from clinical trials that we may complete are subject to the risk that one or more of the clinical outcomes may materially change as patient enrollment continues and more patient data become available. Preliminary or topline data also remain subject to audit and verification procedures that may result in the final data being materially different from the preliminary data we previously published. As a result, interim and preliminary data should be viewed with caution until the final data are available. Adverse differences between preliminary or interim data and final data could significantly harm our reputation and business prospects.

We have limited experience in drug discovery and drug development.

Because we in-licensed some of our investigational agents from other companies, including BHV-2000 from BMS and BHV-5000, we were not involved in and had no control over the preclinical and clinical development of these product candidates prior to entering into these in-license agreements. In addition, we are relying on the other companies from which we licensed our investigational agents to have conducted such research and development in accordance with the applicable protocol, legal, regulatory and scientific standards, accurately reported the results of all clinical trials conducted prior to our acquisition of the applicable product candidate, and correctly collected and interpreted the data from these studies and trials. To the extent any of these has not occurred, our expected development time and costs may be increased, which could adversely affect our prospects for marketing approval of, and receiving any future revenue from, these product candidates.

Clinical trials may be delayed, suspended or terminated for many reasons, which will increase our expenses and delay the time it takes to develop our product candidates.

We may experience delays in our ongoing or future preclinical studies or clinical trials, and we do not know whether future preclinical studies or clinical trials need to be redesigned, enroll an adequate number of patients on time or begin or be completed on schedule, if at all. The commencement and completion of clinical trials for our clinical product candidates may be delayed, suspended or terminated as a result of many factors, including:

- the FDA or other regulators disagreeing as to the design, protocol or implementation of our clinical trials;
- the delay or refusal of regulators (including the FDA) or institutional review boards (“IRBs”) to authorize us to commence a clinical trial;
- regulators (including the FDA), IRBs, ethics committees of the institutions at which trials are being conducted or the data safety monitoring board for such trials requiring that we or our investigators suspend or terminate clinical research for various reasons, including noncompliance with regulatory requirements (including the FDA’s current Good Clinical Practice (“GCP”) regulations) or our clinical protocols, safety concerns, adverse side effects, or lack of adequate funding to continue the clinical trial, among others;
- changes in regulatory requirements, policies and guidelines;
- delays or failure to reach agreement on acceptable terms with prospective clinical research organization (“CROs”) and clinical trial sites, the terms of which can be subject to extensive negotiation and may vary significantly among different CROs and trial sites; delays in patient enrollment and variability in the number and types of patients available for clinical trials;
- the inability to enroll a sufficient number of patients in trials, particularly in orphan indications, to observe statistically significant treatment effects in the trial;
- having clinical sites deviate from the trial protocol or dropping out of a trial;
- negative or inconclusive results from ongoing preclinical studies or clinical trials, which may require us to conduct additional preclinical studies or clinical trials or to abandon projects that we expect to be promising;
- safety or tolerability concerns (including due to reports from testing of similar therapies) that could cause us to suspend or terminate a trial if we find that the participants are being exposed to unacceptable health risks;
- regulators or IRBs requiring that we or our investigators suspend or terminate clinical research for various reasons, including noncompliance with regulatory requirements or safety concerns, among others;
- lower than anticipated retention rates of patients and volunteers in clinical trials;
- our CROs or clinical trial sites failing to comply with regulatory requirements or meet their contractual obligations to us in a timely manner, or at all, deviating from the protocol or dropping out of a trial;
- delays relating to adding new clinical trial sites;
- difficulty in maintaining contact with patients after treatment, resulting in incomplete data;

- delays in establishing the appropriate dosage levels;
- the quality or stability of the product candidate falling below acceptable standards;
- the inability to produce or obtain sufficient quantities of the product candidate to commence or complete clinical trials; and
- exceeding budgeted costs due to difficulty in accurately predicting costs associated with clinical trials.

Any delays in completing our clinical trials will increase our costs, slow down our product candidate development and approval process and jeopardize our ability to commence product sales and generate revenue from product sales. Any of these occurrences may significantly harm our business, financial condition and prospects. In addition, many of the factors that cause, or lead to, a delay in the commencement or completion of clinical trials may also ultimately lead to the denial of regulatory approval of our product candidates.

The regulatory approval processes of the FDA and comparable foreign regulatory agencies are lengthy, time-consuming and unpredictable, and if we are ultimately unable to obtain regulatory approval for our product candidates, our business will be materially harmed.

Neither we nor any future collaborator is permitted to market any of our product candidates in the United States or abroad until we receive regulatory approval of an NDA or BLA from the FDA or approval from the EMA, NMPA or other applicable foreign regulatory agency. The time required to obtain approval by the FDA is unpredictable but typically takes many years following the commencement of clinical trials and depends upon numerous factors, including those beyond our control, such as the substantial discretion of the regulatory authorities. In addition, approval policies, regulations, or the type and amount of clinical data necessary to gain approval is generally uncertain, may change during the course of a product candidate's clinical development and may vary among jurisdictions.

Prior to obtaining approval to commercialize a product candidate in any jurisdiction, we must demonstrate to the satisfaction of the FDA, EMA, NMPA or any comparable foreign regulatory agency, that such product candidates are safe and effective for their intended uses. The FDA, EMA, NMPA or any comparable foreign regulatory agency can delay, limit or deny approval of our product candidates or require us to conduct additional preclinical or clinical testing or abandon a program for many reasons, including:

- the FDA, EMA, NMPA or the applicable foreign regulatory agency's disagreement with the number, design, conduct or implementation of our preclinical studies and clinical trials;
- negative or ambiguous results from our clinical trials or results that may not meet the level of statistical significance required by the FDA, EMA, NMPA or any comparable foreign regulatory agency for approval;
- serious and unexpected drug-related side effects experienced by participants in our clinical trials or by individuals using drugs similar to our product candidates;
- our inability to demonstrate to the satisfaction of the FDA, EMA, NMPA or the applicable foreign regulatory agency that our product candidates are safe and effective for their proposed indications, or that the clinical and other benefits of our product candidates outweigh any safety or other perceived risks;
- the FDA's, EMA's, NMPA's or the applicable foreign regulatory agency's disagreement with the interpretation of data from preclinical studies or clinical trials;
- actions by the CROs that we retain to conduct our preclinical studies and clinical trials, which are outside of our control and that materially adversely impact our preclinical studies and clinical trials;
- the FDA's, EMA's, NMPA's or the applicable foreign regulatory agency's disagreement regarding the formulation, labeling or the specifications of our product candidates;
- the FDA's, EMA's, NMPA's or the applicable foreign regulatory agency's failure to approve the manufacturing processes or facilities of third-party manufacturers with which we contract; and
- the potential for approval policies or regulations of the FDA, EMA, NMPA or the applicable foreign regulatory agencies to significantly change in a manner rendering our clinical data insufficient for approval.

For example, with respect to our randomized, controlled clinical trial of troriluzole for the treatment of SCA, we undertook discussions with the FDA regarding the acceptability of the primary endpoint and necessary secondary endpoints, including our proposal to use a modified SARA scale. In our first Phase 2/3 clinical trial, the FDA stated that while certain items measured by the SARA scale appeared capable of reflecting a clinically meaningful benefit for patients depending on how the scoring of those items is defined, the use of the SARA scale was not appropriate as a primary endpoint in the trial. Based on our post-hoc analyses of data from the open-label extension phase of the trial, we proposed modifications to the SARA scale that we believe may address some of these shortcomings. Based on feedback received from the FDA, we incorporated trial design modifications that include utilization of a modified SARA scale. However, notwithstanding the feedback that we have received from the FDA, there remains substantial risk that the FDA or any foreign regulatory agency may nevertheless conclude that

results obtained using the modified SARA scale would not be an adequate basis for approval.

In addition, in our Phase 3 clinical trial ("Study BHV4157-206") evaluating the efficacy and safety of troriluzole in adult patients with SCA, the primary endpoint, change from baseline to week 48 on the modified SARA scale, did not reach statistical significance in the overall SCA population as there was less than expected disease progression over the course of the study. Post-hoc analysis of efficacy measures by genotype suggests a treatment effect in patients with the SCA Type 3 ("SCA3") genotype. There is substantial risk that the FDA, EMA, NMPA or the applicable foreign regulatory agency may disagree with the interpretation of our data, and there can be no assurance that any such regulatory agency will find the data sufficient to support approval, or that we will not be required to conduct additional testing on the safety and efficacy of troriluzole.

In May 2023, we presented further analysis of Study BHV4157-206 by prespecified genotype strata that revealed consistent treatment effects of troriluzole in SCA3, which represented 41% of study participants. These results were further supported by consistent results across the range of secondary and exploratory endpoints assessed in the SCA3 subgroup. In July 2023, the FDA informed us that it would not review the recently submitted NDA application for troriluzole given that the study's primary endpoint was not met and thus, would not permit a substantive review. In October, 2023, the EMA informed us that our MAA for troriluzole (Dazluma) in the treatment of SCA has been validated and is now under review by EMA's CHMP.

We generally plan to seek regulatory approval to commercialize our product candidates in the United States, the EU and other key global markets, which requires compliance with numerous and varying regulatory requirements of such other countries regarding safety, efficacy, chemistry, manufacturing and controls, clinical trials, commercial sales, pricing and distribution of our product candidates. Even if we are successful in obtaining approval in one jurisdiction, we cannot ensure that we will obtain approval in any other jurisdiction. Failure to obtain approval in one jurisdiction may negatively impact our ability to obtain approval elsewhere. If we fail to obtain approval in any jurisdiction, the geographic market for our product candidates could be limited.

Moreover, even if we were to obtain approval to market any product candidate we develop, regulatory authorities may approve any of our product candidates for fewer or more limited indications than we request, may not approve the price we intend to charge for our products, may grant approval contingent on the performance of costly post-marketing clinical trials, or may approve a product candidate with a label that does not include the labeling claims necessary or desirable for the successful commercialization of that product candidate. Any of the foregoing scenarios could materially harm the commercial prospects for our product candidates.

Our product candidates may fail to demonstrate safety and efficacy in clinical trials, or may cause serious adverse or unacceptable side effects that could prevent or delay regulatory approval and commercialization, limit the commercial profile of an approved label, increase our costs, necessitate the abandonment or limitation of the development of some of our product candidates or result in significant negative consequences following marketing approval, if any.

Before obtaining regulatory approvals for the commercial sale of our product candidates, we must demonstrate through lengthy, complex and expensive preclinical testing and clinical trials that our product candidates are both safe and effective for use in each target indication, and failures can occur at any stage of testing. Clinical trials often fail to demonstrate efficacy or safety of the product candidate studied for the target indication.

For example, in September 2021 we reported negative topline results from a Phase 3 clinical trial to evaluate the efficacy and safety of verdiperstat in participants with MSA. Results of the trial showed that verdiperstat did not statistically differentiate from placebo on the prespecified primary efficacy measure, nor on the key secondary efficacy measures. In September 2022, we reported negative topline results from the Phase 2/3 HEALEY ALS Platform trial evaluating verdiperstat compared to placebo for the treatment of participants with ALS. At this time, we do not have plans to pursue any additional clinical trials evaluating verdiperstat in ALS, but we are evaluating its potential in other disease indications.

Moreover, undesirable side effects caused by our product candidates could cause us or regulatory authorities to interrupt, delay or halt clinical trials and could result in a more restrictive label, the limitation of commercial potential or the delay or denial of regulatory approval by the FDA or a foreign regulatory agency. Results of our clinical trials could reveal a high and unacceptable severity and prevalence of side effects or unexpected characteristics. Accordingly, we may need to abandon the development of certain product candidates or limit development to certain uses or sub-populations in which such side effects are less prevalent, less severe or more acceptable from a risk-benefit perspective. Many compounds that initially showed promise in preclinical or early-stage testing have later been found to cause side effects that restricted their use and prevented further development of the compound in the tested indication.

Occurrence of serious treatment-related side effects could impede subject recruitment and clinical trial enrollment or the ability of enrolled patients to complete the trial, delay the clinical trial, and prevent receipt of regulatory approval from the FDA and other regulators. They could also adversely affect physician or

patient acceptance of our product candidates or result in potential product liability claims. Any of these occurrences may harm our business, financial condition and prospects significantly.

If any of our product candidates receives marketing approval and we, or others, later discover that the drug is less effective than previously believed or causes undesirable side effects that were not previously identified, our ability to market the drug could be compromised.

Clinical trials of our product candidates by their nature are conducted in carefully defined subsets of patients who have agreed to enter into clinical trials. Consequently, it is possible that our clinical trials may indicate an apparent positive effect of a product candidate that is greater than the actual positive effect, if any, or alternatively fail to identify undesirable side effects.

We have monitored the subjects in our studies for certain safety concerns and we have not seen evidence of significant safety concerns in our clinical trials. However, if one or more of our product candidates receives regulatory approval, and we, or others, later discover that they are less effective than previously believed, or cause undesirable side effects that had not previously been identified, a number of potentially significant negative consequences could result, including:

- withdrawal or limitation by regulatory authorities of approvals of such product;
- seizure of the product by regulatory authorities;
- recall of the product;
- restrictions on the marketing of the product or the manufacturing process for any component thereof;
- requirement by regulatory authorities of additional warnings on the label, such as a “black box” warning or contraindication;
- requirement that we implement a REMS or similar program or create a medication guide outlining the risks of such side effects for distribution to patients;
- commitment to expensive additional safety studies prior to approval or post-marketing studies required by regulatory authorities of such product;
- the product may become less competitive;
- initiation of regulatory investigations and government enforcement actions;
- initiation of legal action against us to hold us liable for harm caused to patients; and
- harm to our reputation and resulting harm to physician or patient acceptance of our products.
- Any of these events could prevent us from achieving or maintaining market acceptance of the particular product candidate, if approved, and could significantly harm our business, financial condition, and results of operations.

We depend on enrollment of patients in our clinical trials for our product candidates. If we are unable to enroll patients in our clinical trials, our research and development efforts could be adversely affected.

Identifying and qualifying patients to participate in clinical trials of our product candidates is critical to our success. If we are unable to enroll a sufficient number of patients in our clinical trials, our timelines for recruiting patients, conducting clinical trials and obtaining regulatory approval of potential products may be delayed. These delays could result in increased costs, delays in advancing our product development, delays in testing the effectiveness of our technology or termination of our clinical trials altogether. We cannot predict how successful we will be at enrolling patients in future clinical trials. Patient enrollment is affected by other factors including:

- the eligibility criteria for the trial in question;
- the perceived risks and benefits of the product candidate in the trial;
- clinicians’ and patients’ perceptions as to the potential advantages of the product candidate being studied in relation to other available therapies, including any new drugs that may be approved for the indications we are investigating or drugs that may be used off-label for these indications;
- the size of the patient population required for analysis of the trial’s primary endpoints;
- competition for patients for competitive product candidates undergoing clinical trials;
- the efforts to facilitate timely enrollment in clinical trials;
- the design of the trial;
- the patient referral practices of physicians;
- the ability to monitor patients adequately during and after treatment;
- the risk that patients enrolled in clinical trials will drop out of the trials before completion;
- the ability to obtain and maintain patient consents;
- the number of patients with the indication being studied; and
- the proximity and availability of clinical trial sites for prospective patients.

We may expend our limited resources to pursue a particular product candidate or indication and fail to capitalize on product candidates or indications that may be more profitable or for which there is a greater likelihood of success.

We have limited financial and managerial resources. As a result, we may forego or delay pursuit of opportunities with other product candidates or for other indications that later prove to have greater commercial potential. Our resource allocation decisions may cause us to fail to capitalize on viable commercial products or profitable market opportunities. Our spending on current and future research and development programs and product candidates for specific indications may not yield any commercially viable products. If we do not accurately evaluate the commercial potential or target market for a particular product candidate, we may relinquish valuable rights to that product candidate through collaboration, licensing or other royalty arrangements in cases in which it would have been more advantageous for us to retain sole development and commercialization rights to such product candidate.

We may become exposed to costly and damaging liability claims, either when testing our product candidates in the clinic or at the commercial stage, and our product liability insurance may not cover all damages from such claims.

We are exposed to potential product liability and professional indemnity risks that are inherent in the research, development, manufacturing, marketing, and use of pharmaceutical products. The current and future use of product candidates by us in clinical trials, and the sale of any approved products in the future, may expose us to liability claims. These claims might be made by patients that use the product, healthcare providers, pharmaceutical companies, or others selling such products. In addition, we have agreed to indemnify the licensors of the intellectual property related to our product candidates against certain intellectual property infringement claims. Any claims against us, or with respect to which we are obligated to provide indemnification, regardless of their merit, could be difficult and costly to defend or settle, and could compromise the market acceptance of our product candidates or any prospects for commercialization of our product candidates, if approved.

Although the clinical trial process is designed to identify and assess potential side effects, it is always possible that a drug, even after regulatory approval, may exhibit unforeseen side effects. If any of our product candidates were to cause adverse side effects during clinical trials or after approval of the product candidate, we may be exposed to substantial liabilities. Physicians and patients may not comply with any warnings that identify known potential adverse effects or identify patients who should not use our product candidates.

Although we maintain product liability insurance coverage, such insurance may not be adequate to cover all our liabilities. We may need to increase our insurance coverage each time we commence a clinical trial and if we successfully commercialize any product candidate. As the expense of insurance coverage is increasing, we may not be able to maintain insurance coverage at a reasonable cost or in an amount adequate to satisfy any liability that may arise. If a successful product liability claim or series of claims is brought against us for uninsured liabilities or in excess of insured liabilities, our assets may not be sufficient to cover such claims and our business operations could be impaired.

If serious adverse events or other undesirable side effects are identified during the use of our product candidates in trials, it may adversely affect our development of such product candidates.

Undesirable side effects caused by our product candidates could cause us or regulatory authorities to interrupt, delay or halt nonclinical studies and clinical trials, or could make it more difficult for us to enroll patients in our clinical trials. If serious adverse events or other undesirable side effects or unexpected characteristics of our product candidates are observed in investigator-sponsored trials, further clinical development of such product candidate may be delayed or we may not be able to continue development of such product candidate at all, and the occurrence of these events could have a material adverse effect on our business. Undesirable side effects caused by our product candidates could also result in the delay or denial of regulatory approval by the FDA or other regulatory authorities or in a more restrictive label than we expect.

Risks Related to Commercialization of Our Product Candidates

We have never commercialized a product candidate and we may lack the necessary expertise, personnel and resources to successfully commercialize any of our products that receive regulatory approval on our own or together with suitable collaborators.

We have never commercialized a product candidate. Our operations to date have been limited to organizing and staffing our company, business planning, raising capital, acquiring the rights to our product candidates and undertaking preclinical studies and clinical trials of our product candidates. We currently have no sales force, marketing or distribution capabilities. To achieve commercial success of our product candidates, if any are approved, we will have to develop our own sales, marketing and supply capabilities or outsource these activities to a third party.

Factors that may affect our ability to commercialize our product candidates on our own include: recruiting and retaining adequate numbers of effective sales and marketing personnel, obtaining access to or persuading adequate numbers of physicians to prescribe our product candidates, and other

unforeseen costs associated with creating an independent sales and marketing organization. Developing a sales and marketing organization requires significant investment, is time-consuming and could delay the launch of our product candidates. We may not be able to build an effective sales and marketing organization in the United States, the EU or other key global markets. If we are unable to build our own distribution and marketing capabilities or to find suitable partners for the commercialization of our product candidates, we may have difficulties generating revenue from such product candidates or be able to achieve or sustain profitability.

To the extent that we enter into collaboration agreements with respect to marketing, sales or distribution of any approved product, our product revenue may be lower than if we directly marketed or sold such product. In addition, any revenue we receive will depend in whole or in part upon the efforts of these third-party collaborators, which may not be successful and are generally not within our control. If we are unable to enter into these arrangements on acceptable terms or at all, we may not be able to successfully commercialize any approved products.

We operate in a highly competitive and rapidly changing industry. Failure to compete successfully could adversely affect our business, financial condition and results of operations.

Biopharmaceutical product development is highly competitive and subject to rapid and significant technological advancements. Our success is highly dependent upon our ability to in-license, acquire, develop and obtain regulatory approval for new and innovative products on a cost-effective basis and to market them successfully. In doing so, we face and will continue to face intense competition from a variety of businesses, including large, fully integrated, well-established pharmaceutical companies who already possess a large share of the market, specialty pharmaceutical and biopharmaceutical companies, academic institutions, government agencies and other private and public research institutions in the United States, the EU and other jurisdictions.

With respect to troriluzole, which we are currently developing for the treatment of ataxias and other neurologic disorders, with SCA as our initial indication, there are currently no approved drug treatments for SCA in the United States. We are also developing troriluzole for the potential treatment of OCD and other indications. If we continue to pursue these indications, we would face substantial competition from companies that develop or sell products that treat OCD. With respect to BHV-5000, which we are developing for the treatment of neuropsychiatric conditions the market size and competition will depend on each indication.

Many of the companies which we are competing with or which we may compete with in the future have significantly greater financial resources and expertise in research and development, manufacturing, preclinical testing, conducting clinical trials, obtaining regulatory approvals and marketing approved drugs than we do. These third parties compete with us in recruiting and retaining qualified scientific and management personnel, establishing clinical trial sites and patient registration for clinical trials, as well as in acquiring technologies complementary to, or necessary for, our programs. Mergers and acquisitions in the biopharmaceutical industry could result in even more resources being concentrated among our competitors.

Competition may further increase as a result of advances in the commercial applicability of technologies and greater availability of capital for investment in the biopharmaceutical industry. Our competitors may succeed in developing, acquiring or licensing, on an exclusive basis, products that are more effective or less costly than any product candidates that we may develop.

Established biopharmaceutical companies may invest heavily to accelerate research and development of novel compounds or to in-license novel compounds that could make our product candidates less competitive. In addition, any new product that competes with an approved product must demonstrate compelling advantages in efficacy, convenience, tolerability and safety in order to overcome price competition and to be commercially successful. Accordingly, our competitors may succeed in obtaining patent protection, and in discovering, developing, receiving FDA approval for or commercializing drugs before we do, which would have an adverse impact on our business and results of operations.

The availability of our competitors' products could limit the demand and the price we are able to charge for any product candidates we commercialize, if any. The inability to compete with existing or subsequently introduced drugs would harm our business, financial condition and results of operations.

The successful commercialization of certain of our product candidates will depend in part on the extent to which governmental authorities and health insurers establish adequate coverage, reimbursement levels and pricing policies. Failure to obtain or maintain adequate coverage and reimbursement for our product candidates, if approved, could limit our ability to market those products and decrease our ability to generate revenue.

The availability and adequacy of coverage and reimbursement by governmental healthcare programs, such as Medicare and Medicaid, private health insurers and other third-party payors, are essential for most patients to be able to afford products such as our product candidates, if approved. Our ability to achieve acceptable levels of coverage and reimbursement for products by third-party payors will have an effect on our ability to successfully commercialize our product candidates, if approved, and attract additional collaboration partners to invest in the development of our product candidates. Assuming we obtain coverage

for a given product by a third-party payor, the resulting reimbursement payment rates may not be adequate or may require co-payments that patients find unacceptably high. We cannot be sure that coverage and adequate reimbursement in the United States, the EU or elsewhere will be available for any product that we may develop, and any reimbursement that becomes available may be decreased or eliminated in the future.

Third-party payors increasingly are challenging prices charged for pharmaceutical products and services, and many third-party payors may refuse to provide coverage and reimbursement for particular drugs when an equivalent generic drug or a less expensive therapy is available. Our competitors may offer their products and services on a less expensive basis to gain coverage and reimbursement from third-party payors. It is possible that a third-party payor may consider our product candidates as substitutable by less expensive therapies and only offer to reimburse patients for the less expensive product. Even if we show improved efficacy or improved convenience of administration with our product candidates, pricing of existing drugs may limit the amount we will be able to charge for our product candidates, once approved. These payors may deny or revoke the reimbursement status of a given product or establish prices for new or existing marketed products at levels that are too low to enable us to realize an appropriate return on our investment in product development.

There is significant uncertainty related to the insurance coverage and reimbursement of newly approved products. In the United States, third-party payors, including private and governmental payors, play an important role in determining the extent to which new drugs and biologics will be covered. The Medicare and Medicaid programs increasingly are used as models for how private payors and other governmental payors develop their coverage and reimbursement policies for drugs and biologics. Some third-party payors may require pre-approval of coverage for new or innovative devices or drug therapies before they will reimburse healthcare providers who use such devices or therapies.

Obtaining and maintaining reimbursement status is time-consuming and costly. No uniform policy for coverage and reimbursement for products exists among third-party payors in the United States. As a result, the coverage determination process is often a time-consuming and costly process that will require us to provide scientific and clinical support for the use of our products to each payor separately, with no assurance that coverage and adequate reimbursement will be applied consistently or obtained in the first instance. Furthermore, rules and regulations regarding reimbursement change frequently, in some cases at short notice, and we believe that changes in these rules and regulations are likely.

Moreover, increasing efforts by governmental and third-party payors in the United States and abroad to cap or reduce healthcare costs may cause such organizations to limit both coverage and the level of reimbursement for newly approved products and, as a result, they may not cover or provide adequate payment for our product candidates. We expect to experience pricing pressures in connection with the sale of any of our product candidates due to the trend toward managed healthcare, the increasing influence of health maintenance organizations, and additional legislative changes. The downward pressure on healthcare costs in general, particularly prescription drugs and surgical procedures and other treatments, has become very intense. As a result, increasingly high barriers are being erected to the entry of new products. The continuing efforts of the government, insurance companies, managed care organizations and other payors of healthcare services to contain or reduce costs of healthcare may adversely affect:

- the demand for any products for which we may obtain regulatory approval;
- our ability to set a price that we believe is fair for our products;
- our ability to obtain coverage and adequate reimbursement approval for a product;
- our ability to generate revenues and achieve or maintain profitability; and
- the level of taxes that we are required to pay.

Even if we obtain regulatory approval for our product candidates, they will remain subject to ongoing regulatory oversight.

Even if we obtain regulatory approval for any of our product candidates, they will be subject to extensive and ongoing regulatory requirements for manufacturing processes, labeling, packaging, distribution, adverse event reporting, storage, advertising, promoting, sampling and record-keeping. These requirements include submissions of safety and other post-marketing information and reports, registration, as well as continued compliance with current good manufacturing practices (“cGMP”) regulations and GCPs, for any clinical trials that we conduct post-approval, all of which may result in significant expense and limit our ability to commercialize such products. In addition, any regulatory approvals that we receive for our product candidates may also be subject to limitations on the approved indicated uses for which the product may be marketed or to the conditions of approval, or contain requirements for potentially costly post-marketing testing, including Phase 4 clinical trials, and surveillance to monitor the safety and efficacy of the product candidate. The FDA may also require a REMS as a condition of approval of our product candidates, which could include requirements for a medication guide, physician communication plans or additional elements to ensure safe use, such as restricted distribution methods, patient registries and other risk minimization tools.

We cannot predict the likelihood, nature or extent of government regulation that may arise from future legislation or administrative action, either in the United States or abroad. We may not be able to adapt to changes in existing requirements or the adoption of new requirements or policies. If there are changes in the application of legislation or regulatory policies, or if problems are discovered with a product or our manufacture of a product, or if we or one of our distributors, licensees or co-marketers fails to comply with regulatory requirements, the regulators could take various actions. These include:

- issuing warning or untitled letters;
- seeking an injunction or imposing civil or criminal penalties or monetary fines;
- suspension or imposition of restrictions on operations, including product manufacturing;
- seizure or detention of products, refusal to permit the import or export of products, or requesting that we initiate a product recall;
- suspension or withdrawal of our marketing authorizations;
- suspension of any ongoing clinical trials;
- refusal to approve pending applications or supplements to applications submitted by us;
- refusal to permit the import or export of products; or
- requiring us to conduct additional clinical trials, change our product labeling or submit additional applications for marketing authorization.

Moreover, the FDA strictly regulates the promotional claims that may be made about drug products. In particular, a product may not be promoted for uses that are not approved by the FDA as reflected in the product's approved labeling. The FDA and other agencies actively enforce the laws and regulations prohibiting the promotion of off-label uses, and a company that is found to have improperly promoted off-label uses may be subject to significant civil, criminal and administrative penalties.

If any of these events occurs, our ability to sell such product may be impaired, and we may incur substantial additional expenses to comply with regulatory requirements, which could adversely affect our business, financial condition and results of operations.

Even if any of our product candidates receives marketing approval, it may fail to achieve the degree of market acceptance by physicians, patients, third-party payors and others in the medical community necessary for commercial success.

Even if the FDA approves the marketing of any product candidates that we develop, physicians, patients, third-party payors or the medical community may not accept or use them. Efforts to educate the medical community and third-party payors on the benefits of our product candidates may require significant resources and may not be successful. Such efforts may require more resources than are typically required due to the complexity and uniqueness of our product candidates. If any of our product candidates do not achieve an adequate level of acceptance, we may not generate significant product revenue or any profits from operations. Because we expect sales of our product candidates, if approved, to generate substantially all of our product revenue for the foreseeable future, the failure of our product candidates to find market acceptance would harm our business and could require us to seek additional financing. The degree of market acceptance of our product candidates that are approved for commercial sale will depend on a variety of factors, including:

- the efficacy, cost, convenience and ease of administration, and other potential advantages compared to alternative treatments, including any similar generic treatments;
- effectiveness of sales and marketing efforts;
- the willingness of the target patient population to try new therapies and of physicians to prescribe these therapies;
- the strength of marketing and distribution support;
- the availability of third-party coverage and adequate reimbursement, and patients' willingness to pay out-of-pocket in the absence of third-party coverage or adequate reimbursement;
- the prevalence and severity of any side effects; and
- any restrictions on the use of our products, if approved, together with other medications.

In addition, the potential market opportunity for our product candidates is difficult to estimate precisely. Our estimates of the potential market opportunity are predicated on several key assumptions such as industry knowledge and publications, third-party research reports and other surveys. While we believe that our internal assumptions are reasonable, these assumptions may be inaccurate. If any of the assumptions proves to be inaccurate, then the actual market for our product candidates could be smaller than our estimates of the potential market opportunity, our revenue from product sales may be limited and we may be unable to achieve or maintain profitability.

If the FDA or comparable foreign regulatory authorities approve generic versions of any of our products that receive marketing approval, or such authorities do not grant our products sufficient periods of exclusivity before approving generic versions of our products, the sales of our products could be adversely affected.

Once an NDA is approved, the product covered thereby becomes a "reference listed drug" in the FDA's publication, "Approved Drug Products with Therapeutic Equivalence Evaluations," commonly known as the Orange Book. Manufacturers may seek approval of generic versions of reference listed drugs through submission of abbreviated new drug applications ("ANDAs") in the United States. In support of an ANDA, a generic manufacturer need not conduct clinical trials. Rather, the applicant generally must show that its product has the same active ingredient(s), dosage form, strength, route of administration and conditions of use or labeling as the reference listed drug and that the generic version is bioequivalent to the reference listed drug, meaning it is absorbed in the body at the same rate and to the same extent. Generic products may be significantly less costly to bring to market than the reference listed drug and companies that produce generic products are generally able to offer them at lower prices. Thus, following the introduction of a generic drug, a significant percentage of the sales of any branded product or reference listed drug is typically lost to the generic product.

The FDA may not approve an ANDA for a generic product until any applicable period of non-patent exclusivity for the reference listed drug has expired. The U.S. Federal Food, Drug, and Cosmetic Act (the "FDCA") provides a period of five years of non-patent exclusivity for a new drug containing a new chemical element ("NCE"). Specifically, in cases where such exclusivity has been granted, an ANDA may not be submitted to the FDA until the expiration of five years unless the submission is accompanied by a Paragraph IV certification that a patent covering the reference listed drug is either invalid or will not be infringed by the generic product, in which case the applicant may submit its application four years following approval of the reference listed drug.

While we believe that troriluzole, a prodrug of riluzole will be treated as an NCE under current FDA interpretations and, therefore, if approved, should be afforded five years of data exclusivity, the FDA may disagree with that conclusion and may approve generic products after a period that is less than five years. Manufacturers may seek to launch these generic products following the expiration of the applicable marketing exclusivity period, even if we still have patent protection for our product.

Competition that our products may face from generic versions of our products could materially and adversely impact our future revenue, profitability and cash flows and substantially limit our ability to obtain a return on the investments we have made in those product candidates.

Risks Related to Our Dependence on Third Parties

We rely on third parties to conduct our preclinical studies and clinical trials and if these third parties perform in an unsatisfactory manner, our business could be substantially harmed.

We have historically conducted, and we intend to continue to conduct our clinical trials using our own clinical resources, while also leveraging expertise and assistance from medical institutions, clinical investigators, contract laboratories and other third parties, such as contract research organizations as appropriate. We are reliant upon such third parties to assist us in conducting GCP-compliant clinical trials on our product candidates properly and on time, and may not currently have all of the necessary contractual relationships in place to do so. Once we have established contractual relationships with such third-parties, we will have only limited control over their actual performance of these activities.

We and our CROs and other vendors are required to comply with cGMP, GCP and good laboratory practices ("GLP"), which are regulations and guidelines enforced by the FDA, the Competent Authorities of the Member States of the EU and any comparable foreign regulatory authorities for all of our product candidates in preclinical and clinical development. Regulatory authorities enforce these regulations through periodic inspections of trial sponsors, principal investigators, clinical trial sites and other contractors. Although we rely on CROs to conduct any current or planned GLP-compliant preclinical studies and GCP-compliant clinical trials and have limited influence over their actual performance, we remain responsible for ensuring that each of our preclinical studies and clinical trials is conducted in accordance with its investigational plan and protocol and applicable laws and regulations, and our reliance on the CROs does not relieve us of our regulatory responsibilities. If we or any of our CROs or vendors fail to comply with applicable regulations, the data generated in our preclinical studies and clinical trials may be deemed unreliable and the FDA, the EMA or any comparable foreign regulatory agency may require us to perform additional preclinical studies and clinical trials before approving our marketing applications. We cannot assure you that upon inspection by a given regulatory agency, such regulatory agency will determine that all of our clinical trials comply with GCP regulations. In addition, our clinical trials must be conducted with products produced under cGMP requirements. Our failure to comply with these requirements may require us to repeat clinical trials, which would delay the regulatory approval process.

While we will have agreements governing their activities, we are not, and will not be able to control whether or not our CROs devote sufficient time and resources to our future preclinical and clinical

programs. These CROs may also have relationships with other commercial entities, including our competitors, for whom they may also be conducting clinical trials, or other drug development activities which could harm our business. If our CROs do not successfully carry out their contractual duties or obligations, or fail to meet expected deadlines, or if the quality or accuracy of the clinical data they obtain is compromised due to the failure to adhere to our clinical protocols or regulatory requirements or for any other reason, our clinical trials may be extended, delayed or terminated, the clinical data generated in our clinical trials may be deemed unreliable, and we may not be able to obtain regulatory approval for, or successfully commercialize any product candidate that we develop. As a result, our financial results and the commercial prospects for any product candidate that we develop would be harmed, our costs could increase, and our ability to generate revenue could be delayed.

If our relationships with these CROs terminate, we may not be able to enter into arrangements with alternative CROs or do so on commercially reasonable terms. Switching or adding additional CROs involves substantial cost and requires management time and focus, and could delay development and commercialization of our product candidates. In addition, there is a natural transition period when a new CRO commences work. As a result, delays occur, which can negatively impact our ability to meet our desired clinical development timelines. Though we intend to carefully manage our relationships with our CROs, there can be no assurance that we will not encounter challenges or delays in the future or that these delays or challenges will not have a negative impact on our business and financial condition.

We rely completely on third-party contractors to supply, manufacture and distribute clinical drug supplies for our product candidates, including certain sole-source suppliers and manufacturers; we intend to rely on third parties for commercial supply, manufacturing and distribution if any of our product candidates receive regulatory approval; and we expect to rely on third parties for supply, manufacturing and distribution of preclinical, clinical and commercial supplies of any future product candidates.

We do not currently have, nor do we plan to acquire, the internal infrastructure or capability to supply, manufacture or distribute preclinical, clinical or commercial quantities of drug substances or products.

Our ability to develop our product candidates depends and our ability to commercially supply our products will depend, in part, on our ability to successfully obtain the active pharmaceutical ingredients (“APIs”) and other substances and materials used in our product candidates from third parties and to have finished products manufactured by third parties in accordance with regulatory requirements and in sufficient quantities for preclinical and clinical testing and commercialization. If we fail to develop and maintain supply relationships with these third parties, we may be unable to continue to develop or commercialize our product candidates. In addition, our results of operations and cash flows could be adversely impacted by any inability to obtain favorable terms from our suppliers, including any acceleration of payment terms to our suppliers and/or the imposition of more restrictive credit terms and other contractual requirements.

While we have auditing rights with all our current manufacturing counterparties, we do not have direct control over the ability of our contract suppliers and manufacturers to maintain adequate capacity and capabilities to serve our needs, including quality control, quality assurance and qualified personnel. Although we are ultimately responsible for ensuring compliance with regulatory requirements such as cGMPs, we are dependent on our contract suppliers and manufacturers for day-to-day compliance with cGMPs for production of both APIs and finished products. Facilities used by our contract suppliers and manufacturers to produce the APIs and other substances and materials or finished products for commercial sale must pass inspection and be approved by the FDA and other relevant regulatory authorities. Our contract suppliers and manufacturers must comply with cGMP requirements enforced by the FDA through its facilities inspection program and review of submitted technical information. If our contract suppliers or manufacturers fail to achieve and maintain compliance with applicable laws and regulatory requirements, our business could be adversely affected in a number of ways, and cause, among other things:

- an inability to initiate or continue clinical trials of our product candidates under development;
- delay in submitting regulatory applications, or receiving regulatory approvals, for our product candidates;
- subjecting third-party manufacturing facilities or our own facilities to additional inspections by regulatory authorities;
- requirements to cease distribution or to recall batches of our product candidates;
- suspension of manufacturing of our product candidates;
- revocation of obtained approvals; and
- inability to meet commercial demands for our product candidates in the event of approval.

Further, if the safety of any product or product candidate or component is compromised due to a failure to adhere to applicable laws and regulatory requirements, or for other reasons, we may not be able to successfully commercialize or obtain regulatory approval for the affected product or product candidate, and we may be held liable for injuries sustained as a result. Any of these factors could cause a delay or

termination of preclinical studies, clinical trials or regulatory submissions or approvals of our product candidates, and could entail higher costs or result in our being unable to effectively commercialize our approved products on a timely basis, or at all.

We may rely on certain third parties as the sole source of the materials they supply or the finished products they manufacture. We may also have sole-source suppliers for one or more of our other product candidates. Some of the APIs and other substances and materials used in our product candidates are currently available only from one or a limited number of domestic or foreign suppliers and foreign manufacturers and certain of our finished product candidates are manufactured by one or a limited number of contract manufacturers.

In the event an existing supplier or manufacturer fails to supply or manufacture, as applicable, product on a timely basis or in the requested amount, fails to meet regulatory requirements or our specifications, becomes unavailable through business interruption or financial insolvency or loses its regulatory status as an approved source, or if we or our manufacturers are unable to renew current supply agreements when such agreements expire and we do not have a second supplier, we likely would incur added costs and delays in identifying or qualifying replacement suppliers, manufacturers and materials and there can be no assurance that replacements would be available to us on a timely basis, on acceptable terms or at all. In certain cases, we may be required to get regulatory approval to use alternative suppliers and manufacturers, and this process of approval could delay production of our products or development of product candidates indefinitely. We and our manufacturers do not currently maintain inventory of these APIs and other substances and materials. Any interruption in the supply of an API or other substance or material or in the manufacture of a finished product could have a material adverse effect on our business, financial condition, operating results and prospects.

In addition, these contract manufacturers are or may be engaged with other companies to supply and manufacture materials or products for such companies, which also exposes our suppliers and manufacturers to regulatory risks for the production of such materials and products. As a result, failure to meet the regulatory requirements for the production of those materials and products may also affect the regulatory clearance of a contract supplier's or manufacturer's facility. If the FDA or a comparable foreign regulatory agency does not approve these facilities for the supply or manufacture of our product candidates, or if it withdraws its approval in the future, we may need to find alternative supply or manufacturing facilities, which would negatively impact our ability to develop, obtain regulatory approval of or market our product candidates, if approved.

We expect to continue to depend on third-party contract suppliers and manufacturers for the foreseeable future, but supply and manufacturing arrangements do not guarantee that a contract supplier or manufacturer will provide services adequate for our needs. We and our contract suppliers and manufacturers may attempt to improve production processes, certain aspects of which are complex and unique, and we may encounter difficulties with new or existing processes. While we attempt to build in certain contractual obligations on such third-party suppliers and manufacturers, we may not be able to ensure that such third parties comply with these obligations. Depending on the extent of any difficulties encountered, we could experience an interruption in clinical or commercial supply, with the result that the development, regulatory approval or commercialization of our product candidates may be delayed or interrupted. In addition, third-party suppliers and manufacturers may have the ability to increase the price payable by us for the supply of the APIs and other substances and materials used in our product candidates, in some cases without our consent.

Additionally, any damages to or destruction of our third-party manufacturers' or suppliers' facilities or equipment may significantly impair our ability to have our product candidates manufactured on a timely basis. Furthermore, if a contract manufacturer or supplier becomes financially distressed or insolvent, or discontinues our relationship beyond the term of any existing agreement for any other reason, this could result in substantial management time and expense to identify, qualify and transfer processes to alternative manufacturers or suppliers, and could lead to an interruption in clinical or commercial supply.

In addition, the manufacturing facilities of certain of our suppliers are located outside of the United States. This may give rise to difficulties in importing our products or product candidates or their components into the United States or other countries as a result of, among other things, regulatory agency approval requirements or import inspections, incomplete or inaccurate import documentation or defective packaging.

We, or third-party manufacturers on whom we rely, may be unable to successfully scale-up manufacturing of our product candidates in sufficient quality and quantity, which would delay or prevent us from developing our product candidates and commercializing approved products, if any.

As we prepare for later-stage clinical trials and potential commercialization, we will need to take steps to increase the scale of production of our product candidates, which may include transferring production to new third-party suppliers or manufacturers. In order to conduct larger or late-stage scale clinical trials for our product candidates and supply sufficient commercial quantities of the resulting drug product and its components, if that product candidate is approved for sale, our contract manufacturers and suppliers will need to produce our product candidates in larger

quantities, more cost effectively and, in certain cases, at higher yields than they currently achieve. We, or our manufacturers, may be unable to successfully increase the manufacturing capacity for any of our product candidates in a timely or cost-effective manner, or at all. In addition, quality issues may arise during scale-up activities because of the inherent properties of a product candidate itself or of a product candidate in combination with other components added during the manufacturing and packaging process, or during shipping and storage of the APIs or the finished product. If we, or any of our manufacturers, are unable to successfully scale up the manufacture of our product candidates in sufficient quality and quantity, the development, testing, and clinical trials of that product candidate may be delayed or infeasible, and regulatory approval or commercial launch of any resulting product may be delayed or not obtained, which could significantly harm our business. If we are unable to obtain or maintain third-party manufacturing for commercial supply of our product candidates, or to do so on commercially reasonable terms, we may not be able to develop and commercialize our product candidates successfully.

We may in the future enter into collaborations with third parties to develop and commercialize our product candidates. If these collaborations are not successful, or if we are not able to establish or maintain these collaborations, our business could be harmed.

Our product development programs and the potential commercialization of our product candidates will require substantial additional capital to fund expenses. For some of our product candidates, we may decide to collaborate with pharmaceutical and biotechnology companies for the future development and potential commercialization of those product candidates. Furthermore, we may find that our programs require the use of proprietary rights held by third parties, and the growth of our business may depend in part on our ability to acquire, in-license or use these proprietary rights. Collaboration arrangements are complex and time-consuming to negotiate, document, implement and maintain. We may not be successful in our efforts to establish and implement collaborations or other alternative arrangements, and the terms of any collaborations or other arrangements that we may establish may not be favorable to us.

We face significant competition in seeking appropriate collaborators, and a number of more established companies may also be pursuing strategies to license or acquire third-party intellectual property rights that we may consider attractive. These established companies may have a competitive advantage over us due to their size, financial resources and greater clinical development and commercialization capabilities. In addition, companies that perceive us to be a competitor may be unwilling to assign or license rights to us. Whether we reach a definitive agreement for a collaboration will depend, among other things, upon our assessment of the collaborator's resources and expertise, the terms and conditions of the proposed collaboration and the proposed collaborator's evaluation of a number of factors. Those factors may include the design or results of clinical trials, the likelihood of approval by regulatory authorities, the potential market for the subject product candidate, the costs and complexities of manufacturing and delivering such product candidate to patients, competing or alternative products, the existence of uncertainty with respect to our ownership of technology, which can exist if there is a challenge to such ownership without regard to the merits of the challenge, and industry and market conditions generally. We may also be restricted under existing license agreements from entering into agreements on certain terms with potential collaborators. Even if we are able to obtain a license to intellectual property of interest, we may not be able to secure exclusive rights, in which case others could use the same rights and compete with us.

The success of our collaboration arrangements will depend heavily on the efforts and activities of our collaborators. Collaborations are subject to numerous risks, including for example, that the collaborators may not: adequately perform their obligations under the collaboration agreement; devote sufficient resources to the collaboration to ensure success; or agree with us on the strategy or tactical aspects of the collaboration.

If any such potential future collaborations do not result in the successful development and commercialization of product candidates, or if one of our future collaborators terminates its agreement with us, we may not receive any future research funding or milestone or royalty payments under the collaboration. If we do not receive the funding we expect under these agreements, the development of our product candidates could be delayed and we may need additional resources to develop our product candidates. In addition, if one of our future collaborators terminates its agreement with us, we may find it more difficult to attract new collaborators and the perception of us in the business and financial communities could be adversely affected. All of the risks relating to product development, regulatory approval and commercialization apply to the activities of our potential future collaborators.

Risks Related to Regulatory Compliance

We are required to comply with a wide variety of laws and regulations, and are subject to regulation by various federal, state and foreign agencies, and our failure to comply with existing and future regulatory requirements could adversely affect our results of operations and financial condition. Enacted and future legislation may increase the difficulty and cost for us to obtain marketing approval of and commercialize our product candidates and may affect the prices we may set.

Our operations are subject to a broad array of regulatory requirements globally. We are subject to federal, state, local, international and transnational laws and regulations, including the operating, quality and security standards of the FDA, the U.S. Department of

Health and Human Services (“HHS”), and other regulatory authorities such as the EMA, and in the future, any changes to such laws and regulations could adversely affect us. In particular, changes in the FDA’s regulation of drug discovery and development or manufacturing processes could adversely affect our results of operations and financial condition. We may be required to register for permits and/or licenses with the FDA, HHS, or other regulatory authorities such as the EMA, and there can be no assurance that we will be able to maintain or renew existing permits, licenses or other regulatory approvals or obtain, without significant delay, future permits, licenses or other approvals needed for the operation of our business. Any noncompliance by us with applicable laws and regulations or the failure to maintain, renew or obtain necessary permits and licenses could have an adverse effect on our results of operations and financial condition.

In the United States, the EU, and other foreign jurisdictions, there have been, and we expect there will continue to be, a number of legislative and regulatory changes and proposed changes to the healthcare system that could affect our future results of operations. In particular, there have been and continue to be a number of initiatives at the United States federal and state levels that seek to reduce healthcare costs and improve the quality of healthcare. For example, in March 2010, the Affordable Care Act, as amended by the Health Care and Education Reconciliation Act of 2010 (together, the "ACA") was enacted, which substantially changed the way healthcare is financed by both governmental and private insurers.

Moreover, payment methodologies may be subject to changes in healthcare legislation and regulatory initiatives. For example, CMS is developing new payment and delivery models, such as bundled payment models. HHS moved 30% of Medicare payments to alternative payment models tied to the quality or value of services in 2016. Additionally, HHS had set a goal of moving 50% of Medicare payments into these alternative payment models by the end of 2018, but in 2019, it discontinued this performance goal and replaced it with a new developmental goal to increase the percentage of Medicare health care dollars tied to APMs incorporating downside risk, with a target of 40% for fiscal year 2021. We expect that additional U.S. federal healthcare reform measures will be adopted in the future, any of which could limit the amounts that the U.S. federal government will pay for healthcare products and services, which could result in reduced demand for our product candidates or additional pricing pressures.

Further, there have been several recent U.S. congressional inquiries and proposed and enacted state and federal legislation designed to, among other things, bring more transparency to drug pricing, review the relationship between pricing and manufacturer patient programs, reduce the costs of drugs under Medicare and reform government program reimbursement methodologies for drug products. HHS has already started the process of soliciting feedback on some of these measures and, at the same time, is immediately implementing others under its existing authority. For example, in September 2018, CMS announced it will allow Medicare Advantage Plans the option to use step therapy for Part B drugs beginning January 1, 2019, and in May 2019, CMS finalized a new rule that would require direct-to-consumer television advertisements of prescription drugs and biological products, for which payment is available through or under Medicare or Medicaid, to include in the advertisement the Wholesale Acquisition Cost, or list price, of that drug or biological product. However, this rule was struck down by a federal court before it went into effect. Although some of these and other proposals will require authorization through additional legislation to become effective, members of Congress and the Biden Administration have stated that they will continue to seek new legislative and administrative measures to control drug costs. In response to an Executive Order from President Biden, the Secretary of HHS issued a comprehensive plan for addressing high drug prices that describes a number of legislative approaches and identifies administrative tools to address the high cost of drugs. In late 2021, Democrats included drug pricing reform provisions reflecting elements of the plan in a broader spending package—such as capping Medicare Part D patients’ out-of-pocket costs, establishing penalties for drug prices that increase faster than inflation in Medicare, and authorizing the federal government to negotiate prices on certain select, high-cost drugs under Medicare Parts B and D. At the state level, legislatures are increasingly passing legislation and implementing regulations designed to control pharmaceutical and biological product pricing, including price or patient reimbursement constraints, discounts, restrictions on certain product access and marketing cost disclosure and transparency measures, and, in some cases, designed to encourage importation from other countries and bulk purchasing. Legally mandated price controls on payment amounts by third-party payors or other restrictions could harm our business, results of operations, financial condition and prospects. In addition, regional healthcare authorities and individual hospitals are increasingly using bidding procedures to determine what pharmaceutical products and which suppliers will be included in their prescription drug and other healthcare programs. This could reduce the ultimate demand for our products or put pressure on our product pricing, which could negatively affect our business, results of operations, financial condition and prospects.

On May 30, 2018, the Trickett Wendler, Frank Mongiello, Jordan McLinn, and Matthew Bellina Right to Try Act of 2017 was signed into law. The law, among other things, provides a federal framework for patients to access certain investigational new drug products that have completed a Phase 1 clinical trial. Under certain circumstances, eligible patients can seek treatment without enrolling in clinical trials and without obtaining FDA approval under the FDA expanded access program.

In the EU, similar political, economic and regulatory developments may affect our ability to profitably commercialize any of our product candidates, if approved. In addition to continuing pressure on prices and cost containment measures, legislative developments at the EU or member state level may result in significant additional requirements or obstacles that may increase our operating costs. The delivery of healthcare in the EU, including the establishment and operation of health services and the pricing and reimbursement of medicines, is almost exclusively a matter for national, rather than EU, law and policy. National governments and health service providers have different priorities and approaches to the delivery of healthcare and the pricing and reimbursement of products in that context. In general, however, the healthcare budgetary constraints in most EU member states have resulted in restrictions on the pricing and reimbursement of medicines by relevant health service providers. Coupled with EU and national regulatory burdens on those wishing to develop and market products, this could prevent or delay marketing approval of our product candidates, restrict or regulate post-approval activities and affect our ability to commercialize any products for which we obtain marketing approval. In international markets, reimbursement and healthcare payment systems vary significantly by country, and many countries have instituted price ceilings on specific products and therapies.

We cannot predict the likelihood, nature or extent of government regulation that may arise from future legislation or administrative action, either in the United States or abroad. If we or our collaborators are slow or unable to adapt to changes in existing requirements or the adoption of new requirements or policies, or if we or our collaborators are not able to maintain regulatory compliance, our product candidates may lose any regulatory approval that may have been obtained and we may not achieve or sustain profitability, which would adversely affect our business.

Our business operations and current and future relationships with investigators, healthcare professionals, consultants, third-party payors and customers will be subject, directly or indirectly, to federal and state healthcare fraud and abuse laws, false claims laws, health information privacy and security laws, and other healthcare laws and regulations. If we are unable to comply, or have not fully complied, with such laws, we could face substantial penalties.

Although we do not currently have any products on the market, if we obtain FDA approval for our product candidates, and begin commercializing those products in the United States, our operations may be directly, or indirectly through our prescribers, customers and third-party payors, subject to various U.S. federal and state healthcare laws and regulations, including, without limitation, the U.S. federal Anti-Kickback Statute, the U.S. federal civil and criminal false claims laws and Physician Payments Sunshine Act and regulations.

Healthcare providers, physicians and others play a primary role in the recommendation and prescription of any products for which we obtain marketing approval. These laws may impact, among other things, our current business operations, including our clinical research activities proposed sales and marketing and education programs and constrain the business or financial arrangements and relationships with healthcare providers, physicians and other parties through which we market, sell and distribute our products for which we obtain marketing approval. In addition, we may be subject to patient data privacy and security regulation by both the U.S. federal government and the states in which we conduct our business. Finally, we may be subject to additional healthcare, statutory and regulatory requirements and enforcement by foreign regulatory authorities in jurisdictions in which we conduct our business. The U.S. laws that may affect our ability to operate include:

- the U.S. federal Anti-Kickback Statute, which prohibits, among other things, persons or entities from knowingly and willfully soliciting, offering, receiving or paying any remuneration (including any kickback, bribe, or certain rebates), directly or indirectly, overtly or covertly, in cash or in kind, to induce or reward either the referral of an individual for, or the purchase, lease, order or recommendation of, any good, facility, item or service, for which payment may be made, in whole or in part, under U.S. federal and state healthcare programs such as Medicare and Medicaid. A person or entity does not need to have actual knowledge of the statute or specific intent to violate it in order to have committed a violation;

- the U.S. federal false claims and civil monetary penalties laws, including the civil False Claims Act, which, among other things, impose criminal and civil penalties, including through civil whistleblower or qui tam actions, against individuals or entities for knowingly presenting, or causing to be presented, to the U.S. federal government, claims for payment or approval that are false or fraudulent, knowingly making, using or causing to be made or used, a false record or statement material to a false or fraudulent claim, or from knowingly making a false statement to avoid, decrease or conceal an obligation to pay money to the U.S. federal government. In addition, the government may assert that a claim including items and services resulting from a violation of the U.S. federal Anti-Kickback Statute constitutes a false or fraudulent claim for purposes of the False Claims Act;

- the U.S. federal Health Insurance Portability and Accountability Act of 1996 (“HIPAA”) which imposes criminal and civil liability for, among other things, knowingly and willfully executing, or attempting to execute, a scheme to defraud any healthcare benefit program, or knowingly and willfully falsifying, concealing or covering up a material fact or making any materially false statement, in connection with the delivery of, or payment for, healthcare benefits, items or services; similar to the U.S. federal Anti-Kickback

Statute, a person or entity does not need to have actual knowledge of the statute or specific intent to violate it in order to have committed a violation;

- HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act ("HITECH") enacted as part of the American Recovery and Reinvestment Act of 2009, and its implementing regulations, and as amended again by the Modifications to the HIPAA Privacy, Security, Enforcement and Breach Notification Rules Under HITECH and the Genetic Information Nondiscrimination Act; Other Modifications to the HIPAA Rules, commonly referred to as the Final HIPAA Omnibus Rule, published in January 2013, which imposes certain obligations, including mandatory contractual terms, on covered entities subject to HIPAA (i.e., health plans, healthcare clearinghouses and certain healthcare providers), as well as their business associates that perform certain services for or on their behalf involving the use or disclosure of individually identifiable health information, to safeguard the privacy, security and transmission of individually identifiable health information from any unauthorized use or disclosure;

- the FDCA, which prohibits, among other things, the adulteration or misbranding of drugs, biologics and medical devices;

- the U.S. federal Physician Payments Sunshine Act, enacted as part of the ACA, and its implementing regulations, which require certain manufacturers of drugs, devices, biologics and medical supplies that are reimbursable under Medicare, Medicaid, or the Children's Health Insurance Program to report annually to CMS information related to certain payments and other transfers of value to physicians (defined to include doctors, dentists, optometrists, podiatrists and chiropractors) and teaching hospitals, as well as ownership and investment interests held by the physicians described above and their immediate family members;

- analogous state laws and regulations, including: state anti-kickback and false claims laws, which may apply to our business practices, including, but not limited to, research, distribution, sales and marketing arrangements and claims involving healthcare items or services reimbursed by any third-party payor, including private insurers;

- state laws that require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the U.S. federal government, or otherwise restrict payments that may be made to healthcare providers and other potential referral sources;

- state laws and regulations that require drug manufacturers to file reports relating to pricing and marketing information, which requires tracking gifts and other remuneration and items of value provided to healthcare professionals and entities;

- state and local laws that require the registration of pharmaceutical sales representatives; and

- state laws governing the privacy and security of personal information, including personal health information in certain circumstances, many of which differ from each other in significant ways and often are not preempted by HIPAA, thus complicating compliance efforts.

Ensuring that our internal operations and current and future business arrangements with third parties comply with applicable healthcare laws and regulations will involve substantial costs. It is possible that governmental authorities will conclude that our business practices do not comply with current or future statutes, regulations, agency guidance or case law involving applicable fraud and abuse or other healthcare laws and regulations. If our operations are found to be in violation of any of the laws described above or any other governmental laws and regulations that may apply to us, we may be subject to significant penalties, including civil, criminal and administrative penalties, damages, fines, exclusion from U.S. government funded healthcare programs, such as Medicare and Medicaid, or similar programs in other countries or jurisdictions, disgorgement, individual imprisonment, contractual damages, reputational harm, diminished profits, additional reporting requirements and oversight if we become subject to a corporate integrity agreement or similar agreement to resolve allegations of non-compliance with these laws and the curtailment or restructuring of our operations. Further, defending against any such actions can be costly, time-consuming and may require significant financial and personnel resources. Therefore, even if we are successful in defending against any such actions that may be brought against us, our business may be impaired. If any of the physicians or other providers or entities with whom we expect to do business is found to not be in compliance with applicable laws, they may be subject to criminal, civil or administrative sanctions, including exclusions from government funded healthcare programs and imprisonment. If any of the above occur, it could adversely affect our ability to operate our business and our results of operations.

We may not be able to obtain or maintain orphan drug designation or exclusivity for our product candidates.

We have obtained orphan drug designation in the United States for troriluzole in SCA and for taldefgrobep alfa in SMA and in the EU for taldefgrobep alfa in SMA. We may seek orphan drug designation for other product candidates in the future. Regulatory authorities in some jurisdictions, including the United States and the EU, may designate drugs for relatively small patient populations as orphan drugs. Under the Orphan Drug Act, the FDA may designate a product as an orphan drug

if it is a drug intended to treat a rare disease or condition, which is generally defined as a patient population of fewer than 200,000 individuals in the United States.

Generally, if a product with an orphan drug designation subsequently receives the first marketing approval for the indication for which it has such designation, the product is entitled to a period of marketing exclusivity, which precludes the FDA or the EMA from approving another marketing application for the same drug for the same indication during that time period. The applicable period is seven years in the United States and ten years in the EU. The European exclusivity period can be reduced to six years if a drug no longer meets the criteria for orphan drug designation or if the drug is sufficiently profitable so that market exclusivity is no longer justified. Orphan drug exclusivity may be lost if the FDA or the EMA determines that the request for designation was materially defective or if the manufacturer is unable to assure sufficient quantity of the drug to meet the needs of patients with the rare disease or condition.

We cannot assure you that any future application for orphan drug designation with respect to any other product candidate will be granted. If we are unable to obtain orphan drug designation with respect to other product candidates in the United States, we will not be eligible to obtain the period of market exclusivity that could result from orphan drug designation or be afforded the financial incentives associated with orphan drug designation. Even when we obtain orphan drug exclusivity for a product, that exclusivity may not effectively protect the product from competition because different drugs can be approved for the same condition. Even after an orphan drug is approved, the FDA can subsequently approve a later drug for the same condition if the FDA concludes that the later drug is clinically superior in that it is shown to be safer, more effective or makes a major contribution to patient care.

We are subject to changing law and regulations regarding regulatory matters, corporate governance and public disclosure that have increased both our costs and the risk of non-compliance.

We are subject to rules and regulations by various governing bodies, including, for example, the SEC, which is charged with the protection of investors and the oversight of companies whose securities are publicly traded, and to new and evolving regulatory measures under applicable law, including the laws of the BVI. Our efforts to comply with new and changing laws and regulations have resulted in and are likely to continue to result in, increased general and administrative expenses and a diversion of management time and attention from revenue-generating activities to compliance activities.

Moreover, because these laws, regulations and standards are subject to varying interpretations, their application in practice may evolve over time as new guidance becomes available. This evolution may result in continuing uncertainty regarding compliance matters and additional costs necessitated by ongoing revisions to our disclosure and governance practices. If we fail to address and comply with these regulations and any subsequent changes, we may be subject to penalty and our business may be harmed.

Changes to legislation or regulations in the British Virgin Islands could lead to increased costs for us to comply with additional regulatory and reporting requirements.

As the global regulatory and tax environment evolves, we may be subject to new or different statutory and regulatory requirements. For example, on January 1, 2019, the Economic Substance (Companies and Limited Partnerships) Act, 2018 of the British Virgin Islands (the "Economic Substance Act") came into force and was amended on October 1, 2019 and June 29, 2021 and remains subject to further amendments, additional regulations and guidance on interpretation from the regulator. We strive to conduct our business in a manner that is in compliance with the Economic Substance Act. However, the imposition of additional requirements due to further amendments, additional regulations or new guidance on interpretation of these laws may create additional costs that may be borne by us or otherwise affect our management and operation.

Risks Related to Our Intellectual Property

We could lose market exclusivity earlier than expected.

We own or license patents in the U.S. and foreign countries that protect our products, their methods of use and manufacture, as well as other innovations relating to the advancement of our science to help bring new therapies to patients. We also develop brand names and trademarks for our products to differentiate them in the marketplace. We consider the overall protection of our patents, trademarks, licenses and other intellectual property rights to be of material value and act to protect these rights from infringement. We also rely on trade secrets to protect aspects of our business that are not amenable to, or that we do not consider appropriate for, patent protection. Our success will depend significantly on our ability to obtain and maintain patent and other proprietary protection for commercially important technology, inventions and know-how related to our business, defend and enforce our patents, preserve the confidentiality of our trade secrets and operate without infringing the valid and enforceable patents and other proprietary rights of third parties. We also rely on know-how, continuing technological innovation and in-licensing opportunities to develop, strengthen and maintain the proprietary position of our products and development programs.

In the biopharmaceutical industry, a substantial portion of an innovative product's commercial value is usually realized during the period in which the product has market exclusivity. A product's market exclusivity is

generally determined by two forms of intellectual property: patent rights held by the innovator company and any regulatory forms of exclusivity to which the innovative drug is entitled.

Patents are a key determinant of market exclusivity for most pharmaceuticals. Patents provide the innovator with the right to exclude others from practicing an invention related to the medicine. Patents may cover, among other things, the active ingredient(s), various uses of a drug product, discovery tools, pharmaceutical formulations, drug delivery mechanisms and processes for (or intermediates useful in) the manufacture of products. Protection for individual products extends for varying periods in accordance with the expiration dates of patents in the various countries. The protection afforded, which may also vary from country to country, depends upon the type of patent, its scope of coverage and the availability of meaningful legal remedies in the country.

Market exclusivity can also be influenced by regulatory data protection ("RDP"). Many developed countries provide certain non-patent incentives for the development of medicines. For example, in the U.S., the EU, the United Kingdom, Japan, and certain other countries, RDP intellectual property rights are offered to: (i) provide a time period of data protection during which a generic company is not allowed to rely on the innovator's data in seeking approval; (ii) restore patent term lost during drug development and approval; and (iii) provide incentives for research on medicines for rare diseases, or orphan drugs, and on medicines useful in treating pediatric patients. These incentives can extend the market exclusivity period on a product beyond the patent term.

Product Exclusivity – United States

In the United States, biopharmaceutical products are protected by patents with varying terms depending on the type of patent and the filing date. A significant portion of a product's patent life, however, is lost during the time it takes an innovative company to develop and obtain regulatory approval of a new drug. As compensation, at least in part, for the lost patent term due to regulatory review periods, the innovator may, depending on a number of factors, apply to the government to restore lost patent term by extending the expiration date of one patent up to a maximum term of five years, provided that the extension cannot cause the patent to be in effect for more than 14 years from the date of drug approval. A company seeking to market an innovative pharmaceutical in the U.S. must submit a complete set of safety and efficacy data to the FDA. If the innovative pharmaceutical is a chemical product, the company files an NDA. If the medicine is a biological product, a BLA is filed. The type of application filed affects RDP exclusivity rights.

Small Molecule Products

A competitor seeking to launch a generic substitute of small molecule drug in the U.S. must file an ANDA with the FDA. In the ANDA, the generic manufacturer needs to demonstrate only "bioequivalence" between the generic substitute and the approved NDA drug. The ANDA relies upon the safety and efficacy data previously filed by the innovator in its NDA. An innovator company is required to list certain of its patents covering the medicine with the FDA in what is commonly known as the FDA's Orange Book. The FDA cannot approve an ANDA until after the innovator's listed patents expire unless there is a successful patent challenge. However, after the innovator has marketed its product for four years, a generic manufacturer may file an ANDA and allege that one or more of the patents listed in the Orange Book under an innovator's NDA is either invalid or not infringed (a Paragraph IV certification). The innovator then must decide whether to file a patent infringement suit against the generic manufacturer. From time to time, ANDAs, including Paragraph IV certifications, are filed with respect to certain of our products.

In addition to patent protection, certain innovative pharmaceutical products can receive periods of regulatory exclusivity. An NDA that is designated as an orphan drug can receive seven years of exclusivity for the orphan indication. During this time period, neither NDAs nor ANDAs for the same drug product can be approved for the same orphan use. A company may also earn six months of additional exclusivity for a drug where specific clinical studies are conducted at the written request of the FDA to study the use of the medicine to treat pediatric patients, and submission to the FDA is made prior to the loss of basic exclusivity. Medicines approved under an NDA can also receive several types of RDP. An innovative chemical pharmaceutical product is entitled to five years of RDP in the U.S., during which the FDA cannot approve generic substitutes. If an innovator's patent is challenged, as described above, a generic manufacturer may file its ANDA after the fourth year of the five-year RDP period. A pharmaceutical drug product that contains an active ingredient that has been previously approved in an NDA, but is approved in a new formulation, but not for the drug itself, or for a new indication on the basis of new clinical studies, may receive three years of RDP for that formulation or indication.

Biologic products

The U.S. healthcare legislation enacted in 2010 created an approval pathway for biosimilar versions of innovative biological products that did not previously exist. Prior to that time, innovative biologics had essentially unlimited regulatory exclusivity. Under the new regulatory mechanism, the FDA can approve products that are similar to (but not generic copies of) innovative biologics on the basis of less extensive data than is required by a full Biologics License Application

("BLA"). After an innovator has marketed its product for four years, any manufacturer may file an application for approval of a “biosimilar” version of the innovator product. However, although an application for approval of a biosimilar version may be filed four years after approval of the innovator product, qualified innovative biological products will receive 12 years of regulatory exclusivity, meaning that the FDA may not approve a biosimilar version until 12 years after the innovative biological product was first approved by the FDA. The law also provides a mechanism for innovators to enforce the patents that protect innovative biological products and for biosimilar applicants to challenge the patents. Such patent litigation may begin as early as four years after the innovative biological product is first approved by the FDA.

In the U.S., the increased likelihood of generic and biosimilar challenges to innovators’ intellectual property has increased the risk of loss of innovators’ market exclusivity. First, generic companies have increasingly sought to challenge innovators’ basic patents covering major pharmaceutical products. Second, statutory and regulatory provisions in the U.S. limit the ability of an innovator company to prevent generic and biosimilar drugs from being approved and launched while patent litigation is ongoing. As a result of all of these developments, it is not possible to predict the length of market exclusivity for a particular product with certainty based solely on the expiration of the relevant patent(s) or the current forms of regulatory exclusivity.

Foreign Regulation

In order to market any product outside of the United States, we would need to comply with numerous and varying regulatory requirements of other countries and jurisdictions regarding quality, safety and efficacy and governing, among other things, clinical trials, marketing authorization, commercial sales and distribution of our products. Although many of the issues discussed above with respect to the United States apply similarly in the context of the EU and other geographies, the approval process varies between countries and jurisdictions and can involve additional product testing and additional administrative review periods. The time required to obtain approval in other countries and jurisdictions might differ from and be longer than that required to obtain FDA approval. Regulatory approval in one country or jurisdiction does not ensure regulatory approval in another, but a failure or delay in obtaining regulatory approval in one country or jurisdiction may negatively impact the regulatory process in others.

European Union

A typical route used by innovator companies to obtain marketing authorization of pharmaceutical products in the EU is through the “centralized procedure.” A company seeking to market an innovative pharmaceutical product through the centralized procedure must file a complete set of safety data and efficacy data as part of a MAA with the EMA. After the EMA evaluates the MAA, it provides a recommendation to the EC and the EC then approves or denies the MAA. It is also possible for new chemical products to obtain marketing authorization in the EU through a “mutual recognition procedure,” in which an application is made to a single member state, and if the member state approves the pharmaceutical product under a national procedure, then the applicant may submit that approval to the mutual recognition procedure of some or all other member states. After obtaining marketing authorization approval, a company must obtain pricing and reimbursement for the pharmaceutical product, which is typically subject to member state law. In certain EU countries, this process can take place simultaneously while the product is marketed but in other EU countries, this process must be completed before the company can market the new product. The pricing and reimbursement procedure can take months and sometimes years to complete. Throughout the EU, all products for which marketing authorizations have been filed after October/November 2005 are subject to an “8+2+1” regime. Eight years after the innovator has received its first community authorization for a medicinal product, a generic company may file a MAA for that product with the health authorities. If the MAA is approved, the generic company may not commercialize the product until after either 10 or 11 years have elapsed from the initial marketing authorization granted to the innovator. The possible extension to 11 years is available if the innovator, during the first eight years of the marketing authorization, obtains an additional indication that is of significant clinical benefit in comparison with existing treatments. For products that were filed prior to October/November 2005, there is a 10-year period of data protection under the centralized procedures and a period of either six or 10 years under the mutual recognition procedure (depending on the member state). In contrast to the U.S., patents in the EU are not listed with regulatory authorities. Generic versions of pharmaceutical products can be approved after data protection expires, regardless of whether the innovator holds patents covering its drug. Thus, it is possible that an innovator may be seeking to enforce its patents against a generic competitor that is already marketing its product. Also, the European patent system has an opposition procedure in which generic manufacturers may challenge the validity of patents covering innovator products within nine months of grant. In general, EU law treats chemically-synthesized drugs and biologically-derived drugs the same with respect to intellectual property and data protection. In addition to the relevant legislation and annexes related to biologic medicinal products, the EMA has issued guidelines that outline the additional information to be provided for biosimilar products, also known as generic biologics, in order to review an application for marketing approval.

Japan

In Japan, medicines of new chemical entities are generally afforded eight years of data exclusivity for approved indications and dosage. Patents on

pharmaceutical products are enforceable. Generic copies can receive regulatory approval after data exclusivity and patent expirations. As in the U.S., patents in Japan may be extended to compensate for the patent term lost during the regulatory review process. In general, Japanese law treats chemically-synthesized and biologically-derived drugs the same with respect to intellectual property and market exclusivity.

Rest of the World

In countries outside of the U.S., the EU and Japan, there is a wide variety of legal systems with respect to intellectual property and market exclusivity of pharmaceuticals. Most other developed countries utilize systems similar to either the U.S. or the EU. Among developing countries, some have adopted patent laws and/or regulatory exclusivity laws, while others have not. Some developing countries have formally adopted laws in order to comply with World Trade Organization ("WTO") commitments, but have not taken steps to implement these laws in a meaningful way. Enforcement of WTO actions is a long process between governments, and there is no assurance of the outcome.

We are dependent on licensed intellectual property in our business. If we are unable to obtain licenses from third parties on commercially reasonable terms or lose our rights to such licensed intellectual property, or if our rights are determined to be narrower than we understand them to be, we may not be able to continue developing or commercializing our product candidates.

We are a party to a number of license agreements under which we are granted rights to intellectual property that are important to our business, including, for example, an agreement with ALS Biopharma and Fox Chase Chemical Diversity Center, Inc., pursuant to which we were assigned intellectual property rights relating to troriluzole, a license agreement with Yale University, pursuant to which we were granted certain patent rights to develop and commercialize riluzole-based products, another license agreement with Yale University pursuant to which we acquired exclusive, worldwide rights to Yale's intellectual property directed to its MoDE platform, a license agreement with Highlightll, pursuant to which we were granted exclusive rights to develop and commercialize Highlightll's brain penetrant dual TYK2/JAK1 inhibitor program, a license agreement with Bristol-Myers Squibb, pursuant to which we were granted exclusive rights to develop and commercialize taldefgrobep alfa, license agreements with AstraZeneca, pursuant to which we were granted exclusive licenses relating to BHV-5500 and verdiperstat, a license agreement with AstraZeneca, pursuant to which we were granted an exclusive license to BHV-2200, and a license agreement with KU Leuven, pursuant to which we were granted an exclusive license to develop and commercialize the TRPM3 antagonist platform. We may enter into additional license agreements in the future. Our existing license agreements impose, and we expect that future license agreements will impose on us, various development, regulatory and/or commercial diligence obligations, payment of milestones and/or royalties and other obligations, such as non-compete periods for certain collaboration targets and rights of first negotiation for development of certain programs. Typically, in our licenses, we have control over the filing, prosecution, maintenance and enforcement of the licensed intellectual property. However, in some cases, we do not control prosecution of the licensed intellectual property, or do not have the first right to enforce such intellectual property. In those cases, we may not be able to adequately influence patent prosecution or enforcement, or prevent inadvertent lapses of coverage due to failure to pay maintenance fees.

If we fail to comply with any of our obligations under a current or future license agreement, the licensor may allege that we have breached our license agreement, and may seek to terminate our license. Termination of any of our current or future licenses could result in our loss of the right to use the licensed intellectual property, which could materially adversely affect our ability to develop, manufacture or commercialize a product candidate or product, if approved, as well as harm our competitive business position and our business prospects. Under some license agreements, termination may also result in the transfer of or granting of rights under certain of our intellectual property and information related to the product candidate being developed under the license, such as regulatory information. If our licensors fail to comply with their obligations under these agreements, such as, for example, by failing to maintain or enforce patents licensed to us, exclusivity relating to the products covered by the license may be diminished or lost. Our rights under license agreements could be determined to be narrower than we understand them to be. Also, if it is found that our licensors were not the original inventors of the licensed intellectual property, or were not the first to file patent applications, then we may lose rights to the licensed intellectual property.

Licensing of intellectual property is important to our business and involves complex legal, business and scientific issues. Disputes between us and our licensors have arisen and may arise in the future. For example, disputes may arise between us and our licensors regarding intellectual property subject to a license agreement, including:

- the scope of rights granted under the license agreement and other interpretation-related issues, including our right to sublicense patents and other rights to third parties;
- whether and the extent to which our technology and processes infringe on intellectual property of the licensor that is not subject to the licensing agreement;
- our diligence obligations with respect to the use of the licensed technology in relation to our development and commercialization of our product candidates, and what activities satisfy those diligence obligations;

- the ownership of inventions and know-how resulting from the joint creation or use of intellectual property by our licensors and us and our partners;
- our right to transfer or assign the license; and
- the effects of termination.

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

It may be necessary or desirable for us to use the patented or proprietary technology of third parties to commercialize our products, in which case we would seek to obtain a license from these third parties. If we are unable to license such technology, or if we are forced to license such technology on unfavorable terms, our business could be harmed. If we are unable to obtain a necessary license, we may be unable to develop or commercialize the affected product candidates, which could materially harm our business and the third parties owning such intellectual property rights could seek either an injunction prohibiting our sales or payment of royalties and/or other forms of compensation. Even if we are able to obtain a license, it may be non-exclusive, thereby giving our competitors access to the same technologies licensed to us.

Patent terms may not provide exclusivity for our product candidates for an adequate amount of time for us to realize commercial benefits.

Patents have a limited lifespan. In the United States and most of the world, the statutory expiration of a patent is generally 20 years from the first filing date. Even if patents covering our product candidates are obtained, once the patent life has expired for a product candidate, we may be open to competition from competitive products, including generic products. Given the amount of time required for the development, testing and regulatory review of new product candidates, patents protecting such product candidates may not provide us with exclusivity for an adequate amount of time for us to realize commercial benefits.

Depending upon the timing, duration and conditions of FDA marketing approval of our product candidates, one or more of our U.S. patents may be eligible for limited patent term restoration under the Drug Price Competition and Patent Term Restoration Act of 1984, referred to as the Hatch-Waxman Amendments, and similar legislation in the EU. The Hatch-Waxman Amendments permit a patent term extension of up to five years for a patent covering an approved product as compensation for effective patent term lost during product development and the FDA regulatory review process, subject to a statutory maximum of fourteen (14) years from the regulatory approval and an additional six months of pediatric exclusivity if available. Similar regulations regarding patent term extensions, or supplementary protection certificates, are available in some countries such as the EU, United Kingdom, Japan and Korea.

However, we may not receive a patent term restoration, a supplementary protection certificate or extension if we fail to satisfy applicable requirements. Moreover, the length of the extension could be less than we request. If we are unable to obtain a patent term restoration, a supplementary protection certificate or extension, or the term is less than we request, the period during which we can enforce our patent rights for that product will be shortened and our competitors may obtain approval to market competing products sooner. As a result, our revenue from applicable products could be reduced.

Third parties may seek to invalidate our patents.

Once granted, patents may remain open to invalidity challenges including opposition, interference, re-examination, post-grant review, inter partes review, nullification or derivation actions in court or before patent offices or similar proceedings for a given period after allowance or grant, during which time third parties can raise objections against such grant. In the course of such proceedings, which may continue for a protracted period of time, the patent owner may be compelled to limit the scope of the allowed or granted claims which are the subject of the challenge, or may lose the allowed or granted claims altogether.

Generic manufacturers seeking to launch a generic substitute of small molecule drug in the U.S. typically engage in patent challenges. We expect that as early as four (4) years after the approval of our products, one or more generic manufactures may allege that one or more of the patents listed in the Orange Book under our NDA is either invalid or not infringed (a Paragraph IV certification). We then must decide whether to file a patent infringement suit against such generic manufacturer(s). Some claimants may have substantially greater resources than we do and may be able to sustain the costs of complex intellectual property litigation to a greater degree and for longer periods of time than we could.

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

Third parties may assert infringement claims against us based on existing patents or patents that may be granted in the future, regardless of their merit. There is a risk that third parties may choose to engage in litigation with us to enforce or to otherwise assert their patent rights against us. Even if we believe such claims are without merit, a court of competent jurisdiction could hold that these third-party patents are valid, enforceable and infringed, and the holders of any such patents may be able to block our ability to commercialize such product candidate unless we obtained a license under the applicable patents, or until

such patents expire or are finally determined to be invalid or unenforceable. Similarly, if any third-party patents were held by a court of competent jurisdiction to cover aspects of our technology, such as our compositions, formulations, or methods of treatment, prevention or use, the holders of any such patents may be able to block our ability to develop and commercialize the applicable product candidate unless we obtained a license.

Parties making claims against us may obtain injunctive or other equitable relief, which could effectively block our ability to further develop and commercialize one or more of our product candidates. If, in the context of seeking approval for one of our product candidates subject to approval via Section 505(b)(2), we were required to file a Paragraph IV certification against any patents of a third party, we would additionally be at risk of an automatic stay if litigation is initiated, thereby potentially delaying our approval or market entry. Defense of these claims, regardless of their merit, would involve substantial litigation expense and would be a substantial diversion of resources from our business.

In the event of a successful claim of infringement against us, we may have to pay substantial damages, pay royalties, redesign our infringing products or obtain one or more licenses from third parties, which may be impossible or require substantial time and monetary expenditure.

In addition to claims of infringement made by third parties against us, we have in the past and may again in the future file claims of infringement and/or trade secret misappropriation against third parties who infringe, or misappropriate, our patents and/or trade secrets or those of our licensors. This can occur as a counter claim in an infringement suit against us or as a direct claim against the third party. Our adversaries in these proceedings may have the ability to dedicate substantially greater resources to prosecuting these legal actions than we can. In addition, in an infringement proceeding, a court may decide that a patent of ours or our licensors is not valid or is unenforceable, or may refuse to stop the other party from using the technology at issue on the grounds that our patents do not cover the technology in question. An adverse result in any litigation or defense proceedings could put one or more of our patents at risk of being invalidated or interpreted narrowly and could put our patent applications at risk of not issuing. The initiation of a claim against a third party may also cause the third party to bring counter claims against us such as claims asserting that our patents are invalid or unenforceable or claims challenging the scope of the intellectual property rights we own or control. The outcome following legal assertions of invalidity and unenforceability is unpredictable.

During the course of any intellectual property litigation, there could be public announcements of the results of hearings, rulings on motions or other interim proceedings in the litigation. If securities analysts or investors regard these announcements as negative, the perceived value of our product candidates, programs or intellectual property could be diminished and the market price of our common stock could decline. Such announcements could also harm our reputation or the market for our future products, which could have a material adverse effect on our business.

We are subject to other risks relating to our intellectual property.

In addition to the risk factors described above, we consider the items below to be relevant for consideration in the assessment of the Company's intellectual property position.

- Changes in intellectual property laws or regulations in the U.S. or other countries could negatively affect our business. Similarly, changes in the interpretation of such laws or regulations could have an impact on our business. For example, U.S. Supreme Court has ruled on several patent cases in recent years, such as Impression Products, Inc. v. Lexmark International, Inc., Association for Molecular Pathology v. Myriad Genetics, Inc., Mayo Collaborative Services v. Prometheus Laboratories, Inc., and Alice Corporation Pty. Ltd. v. CLS Bank International, either narrowing the scope of patent protection available in certain circumstances or weakening the rights of patent owners in certain situations. In addition to increasing uncertainty with regard to our ability to obtain patents in the future, decisions by courts may lead to legislation impacting our ability to obtain or enforce our intellectual property.

- Our ability to enforce our intellectual property outside of the U.S. is dependent on the laws of jurisdiction in which the alleged infringement occurred, the ability to engage in discovery to obtain evidence and the availability of meaningful recoveries, e.g., damages and injunctions. The laws of certain countries, particularly certain developing countries, do not favor the enforcement of patents, trade secrets and other intellectual property, particularly those relating to biotechnology products, which could make it difficult for us to stop the infringement of our patents or marketing of competing products in violation of our proprietary rights. As a result, our business may be harmed by limitations on our ability to protect our technology through the enforcement of our intellectual property in certain countries outside the U.S.

- The U.S. government may seek to exercise its rights under the Bayh-Dole Act of 1980 in programs that have received government funding. This exercise of rights could require us to grant exclusive, partially exclusive, or non-exclusive licenses to any of these inventions to a third party the U.S. Government determines that: (i) adequate steps have not been taken to commercialize the invention; (ii) government action is necessary to meet public health or safety needs; or (iii) government action is necessary to meet requirements

for public use under federal regulations (also referred to as “march-in rights”).

• We rely on trade secrets, including unpatented know-how, technology and other proprietary information, to maintain our competitive position. We seek to protect these trade secrets, in part, by entering into confidentiality agreements with parties who have access to them, such as our employees, third party collaborators, contract manufacturers, consultants, advisors and other third parties. An unauthorized disclosure or use of our trade secrets can have an adverse impact on our business.

• Other innovator companies may independently develop alternative technologies to our technologies without infringing our intellectual property rights, such as, for example, by developing compounds that function according to the same mechanism of action as our compounds, but are chemically distinct from ours and are not covered by the claims of the patents that we own or control.

• Litigation involving intellectual property can be generally time consuming and expensive. Litigation or other legal proceedings relating to intellectual property claims is unpredictable and generally expensive and time-consuming and is likely to divert significant resources from our core business, including distracting our technical and management personnel from their normal responsibilities. Furthermore, because of the substantial amount of discovery required in connection with intellectual property litigation, there is a risk that some of our confidential information could be compromised by disclosure during this type of litigation. In addition, there could be public announcements of the results of hearings, motions or other interim proceedings or developments and if securities analysts or investors perceive these results to be negative, it could have a substantial adverse effect on our valuation.

Risks Related to Our Business Operations, Employee Matters and Managing Growth

Our future growth and ability to compete depends on retaining our key personnel and recruiting additional qualified personnel.

We are highly dependent on the management, development, clinical, financial and business development experience of our senior management. Although we have entered into employment agreements with our executive officers, each of them may terminate their employment with us at any time. We do not maintain “key person” insurance for any of our executives or employees.

The competition for qualified personnel in the biopharmaceutical field is intense, and our future success depends upon our ability to attract, retain and motivate highly-skilled scientific, technical and managerial employees. We face competition for personnel from other companies, universities, public and private research institutions and other organizations. If our recruitment and retention efforts are unsuccessful in the future, it may be difficult for us to implement business strategy, which could harm our business.

In addition, we rely on consultants and advisors, including scientific and clinical advisors, to assist us in formulating our development and commercialization strategy. Our consultants and advisors may be employed by employers other than us and may have commitments under consulting or advisory contracts with other entities that may limit their availability to us. If we are unable to continue to attract and retain high quality personnel, our ability to pursue our growth strategy will be limited.

Our future growth depends, in part, on our ability to penetrate foreign markets, where we would be subject to additional regulatory burdens and other risks and uncertainties.

Our future profitability will depend, in part, on our ability to commercialize our product candidates in markets outside of the United States and the EU. If we commercialize our product candidates in foreign markets, we will be subject to additional risks and uncertainties, including:

• economic weakness, including inflation, or political instability in particular economies and markets;

• the burden of complying with complex and changing foreign regulatory, tax, accounting and legal requirements, many of which vary between countries;

• different medical practices and customs in foreign countries affecting acceptance in the marketplace;

• tariffs and trade barriers;

• other trade protection measures, import or export licensing requirements or other restrictive actions by U.S. or foreign governments;

• longer accounts receivable collection times;

• longer lead times for shipping;

• compliance with tax, employment, immigration and labor laws for employees living or traveling abroad;

• workforce uncertainty in countries where labor unrest is common;

• language barriers for technical training;

• reduced protection of intellectual property rights in some foreign countries, and related prevalence of generic alternatives to therapeutics;

• foreign currency exchange rate fluctuations and currency controls;

• differing foreign reimbursement landscapes;

- uncertain and potentially inadequate reimbursement of our products; and
- the interpretation of contractual provisions governed by foreign laws in the event of a contract dispute.

Foreign sales of our products could also be adversely affected by the imposition of governmental controls, political and economic instability, trade restrictions and changes in tariffs.

Our business and operations may be materially adversely affected in the event of computer system failures or security breaches.

Despite the implementation of security measures, our internal computer systems, and those of our CROs, CMOs and other third parties on which we rely, are vulnerable to damage from computer viruses, unauthorized access, cyberattacks, natural disasters, fire, terrorism, war and telecommunication and electrical failures. If such an event were to occur and interrupt our operations, it could result in a material disruption of our development programs. For example, the loss of clinical trial data from ongoing or planned clinical trials could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data. Although we have not experienced a material security breach or disruption to date, we may experience a material security breach or disruption in the future in the event such security breach or disruption results in a loss of or damage to our data or applications, loss of trade secrets or inappropriate disclosure of confidential or proprietary information, including individually identifiable health information or the personal data of employees or former employees, access to our clinical data or disruption of the manufacturing process. We are also vulnerable to cybersecurity incidents through cyberattacks by hackers, user error, phishing scams or other malfeasance, as well as cybersecurity incidents involving our employees, business partners, collaborators or other third parties. This type of breach of our cybersecurity may compromise our confidential information or our financial information and adversely affect our business, reputation, financial condition, results of operations or result in legal proceedings.

Additionally, the collection, use, disclosure, transfer, or other processing of personal data regarding individuals in the EU, including date concerning health, is subject to the EU General Data Protection Regulation, or GDPR, which became effective on May 25, 2018. The GDPR is wide-ranging in scope and imposes numerous requirements on companies that process personal data, including requirements relating to processing data concerning health and other sensitive data, obtaining consent of the individuals to whom the personal data relates to process their personal data, providing information to individuals regarding data processing activities, implementing safeguards to protect the security and confidentiality of personal data, providing notification of data breaches, and taking certain measures when engaging third-party processors. The GDPR also imposes strict rules on the transfer of personal data to countries outside the EU, including the United States, and permits data protection authorities to impose large penalties for violations of the GDPR, including potential fines of up to €20 million or 4% of annual global turnover, whichever is greater. The GDPR also confers a private right of action on data subjects and consumer associations to lodge complaints with supervisory authorities, seek judicial remedies, and obtain compensation for damages resulting from violations of the GDPR.

Various U.S. states and other governmental authorities around the world have imposed or are considering similar types of laws and regulations, data breach reporting and penalties for non-compliance and increasing security requirements. These laws and regulations are broad in scope and are subject to evolving interpretation and we have in the past been, and in the future could be, required to incur substantial costs to monitor compliance or to alter our practices. Moreover, these new laws and regulations could diverge and conflict with each other in certain respects. As new privacy-related laws and regulations are implemented, the time and resources needed for us to comply with such laws and regulations, as well as our potential liability for non-compliance and reporting obligations in the case of data breaches, have increased and may further increase.

Laws and regulations governing our international operations may preclude us from developing, manufacturing and selling certain product candidates and products outside of the United States and require us to develop and implement costly compliance programs.

As we expand our operations outside of the United States, we will be required to dedicate additional resources to comply with numerous laws and regulations in each jurisdiction in which we plan to operate. We are subject to U.S. laws governing international business activities, including U.S. economic sanctions, export controls and anti-corruption laws, including the Foreign Corrupt Practices Act (the "FCPA"), compliance with which is expensive and difficult, particularly in countries in which corruption is a recognized problem. As a result, these laws may preclude us from developing, manufacturing or selling certain product candidates outside of the United States, which could limit our growth potential and increase our development costs. If our employees or agents violate our policies or we fail to maintain adequate record keeping and internal accounting practices to accurately record our transactions, we may be subject to regulatory sanctions. The failure to comply with laws governing international business practices may result in substantial civil and criminal penalties and suspension or debarment from government contracting. The SEC also may suspend or bar issuers from trading securities on U.S. exchanges for violations of the FCPA's accounting provisions. Violations of U.S. economic

sanctions, export controls and anti-corruption laws, or allegations of such acts, could damage our reputation and subject us to civil or criminal investigations in the United States and in other jurisdictions and related shareholder lawsuits, could lead to substantial civil and criminal, monetary and nonmonetary penalties and could cause us to incur significant legal and investigatory fees which could adversely affect our business, consolidated financial condition and results of operations.

We expect to expand our development and regulatory capabilities and potentially implement sales, marketing and distribution capabilities, and as a result, we may encounter difficulties in managing our growth, which could disrupt our operations.

As our clinical development progresses, we expect to experience growth in the number of our employees and the scope of our operations, particularly in the areas of clinical operations, regulatory affairs and, if any of our product candidates receives marketing approval, sales, marketing and distribution. To manage our anticipated future growth, we must continue to implement and improve our managerial, operational and financial systems, expand our facilities and continue to recruit and train additional qualified personnel. Due to our limited financial resources and the limited experience of our management team in managing a company with such anticipated growth, we may not be able to effectively manage the expansion of our operations or recruit and train additional qualified personnel. The expansion of our operations may lead to significant costs and may divert our management and business development resources. Any inability to manage growth could delay the execution of our business plans or disrupt our operations.

Our employees, independent contractors, consultants, commercial collaborators, principal investigators, CROs and vendors may engage in misconduct or other improper activities, including non-compliance with regulatory standards and requirements.

We are exposed to the risk that our employees, independent contractors, consultants, commercial collaborators, principal investigators, CROs and vendors may engage in fraudulent conduct or other illegal activity. Misconduct by these parties could include intentional, reckless or negligent conduct or unauthorized activities that violates (1) the laws and regulations of the FDA, the EMA and other similar regulatory authorities, including those laws requiring the reporting of true, complete and accurate information to such authorities, (2) manufacturing standards, (3) federal and state data privacy, security, fraud and abuse and other healthcare laws and regulations in the United States and abroad and (4) laws that require the true, complete and accurate reporting of financial information or data. In particular, sales, marketing and business arrangements in the healthcare industry are subject to extensive laws and regulations intended to prevent fraud, misconduct, kickbacks, self-dealing and other abusive practices. These laws and regulations may restrict or prohibit a wide range of pricing, discounting, marketing and promotion, sales commission, customer incentive programs and other business arrangements. Misconduct by these parties could also involve the improper use of individually identifiable information, including information obtained in the course of clinical trials, creating fraudulent data in our preclinical studies or clinical trials or illegal misappropriation of product candidates, which could result in regulatory sanctions and serious harm to our reputation.

Although we have adopted a code of business conduct and ethics, it is not always possible to identify and deter misconduct by employees and other third parties, and the precautions we take to detect and prevent this activity may not be effective in controlling unknown or unmanaged risks or losses or in protecting us from governmental investigations or other actions or lawsuits stemming from a failure to be in compliance with such laws or regulations. Additionally, we are subject to the risk that a person or government could allege such fraud or other misconduct, even if none occurred. If any such actions are instituted against us, and we are not successful in defending ourselves or asserting our rights, those actions could have a significant impact on our business, including the imposition of significant civil, criminal and administrative penalties, including damages, fines, disgorgement, imprisonment, exclusion from participation in government healthcare programs, such as Medicare and Medicaid, contractual damages, reputational harm, additional reporting requirements and oversight if we become subject to a corporate integrity agreement or similar agreement to resolve allegations of non-compliance with these laws and the curtailment or restructuring of our operations.

We may be subject to securities litigation, which is expensive and could divert management attention.

Our share price may be volatile, and in the past companies that have experienced volatility in the market price of their shares have been subject to securities class action litigation. This risk is especially relevant for us because biotechnology companies have experienced significant share price volatility in recent years. Securities litigation against us could result in substantial costs and divert our management's attention from other business concerns, which could seriously harm our business.

Risks Related to Ownership of Our Common Shares

An active trading market for our common shares may not be sustained, or be liquid enough for investors to resell our common shares quickly or at the market price.

Our common shares began trading on the NYSE on October 4, 2022. Although trading in our common shares has developed, we cannot assure you that an active trading market will be sustained or that any trading

market will continue to be liquid. If an active market for our common shares is not sustained, it may be difficult for our shareholders to sell shares without depressing the market price for the shares or to sell their shares at all. An inactive market may also impair our ability to raise capital to continue to fund operations by selling our common shares and may impair our ability to acquire other companies or technologies by using our common shares as consideration.

The trading price of our common shares has in the past been and could in the future be volatile and fluctuate due to factors beyond our control, and purchasers of our common shares could incur substantial losses.

Our share price has in the past been and could in the future be volatile. The stock market in general and the market for biopharmaceutical companies in particular have experienced extreme volatility that has often been unrelated to the operating performance of particular companies. As a result of this volatility, our shareholders and investors may not be able to sell their common shares at or above the price paid for the shares. The market price for our common shares may be influenced by many factors, including:

- positive or negative results, including preliminary or topline results, of preclinical studies and clinical trials reported by us, strategic partners or competitors;
- any progress or delay in the commencement, enrollment and the ultimate completion of clinical trials;
- technological innovations or commercial product introductions by us or competitors;
- failure to successfully develop and commercialize any of our product candidates;
- developments, announcements or changes in government regulations relating to drug products, including related to drug pricing, reimbursement and healthcare coverage;
- delays in in-licensing or acquiring additional complementary product candidates;
- developments concerning proprietary rights, including patents and litigation matters;
- public concern relating to the commercial value or safety of any of our product candidates;
- financing or other corporate transactions, or inability to obtain additional funding;
- announcements relating to our arrangements with strategic partners;
- failure to meet or exceed expectations of the investment community;
- actual or anticipated variations in our operating results;
- changes in financial estimates by us or by any securities analysts who might cover our shares;
- announcements by therapeutic drug product providers related to pricing of therapeutics;
- announcements of significant licenses, acquisitions, strategic partnerships or joint ventures by us or our competitors;
- publication of research reports or comments by securities or industry analysts;
- failure to attract or retain of key personnel;
- sales of our common shares, including sales by our directors and officers or specific shareholders;
- general market or regulatory conditions in the pharmaceutical industry or in the economy as a whole;
- other events and factors, many of which are beyond our control; and
- other factors described in this “Risk Factors” section and elsewhere in this Annual Report on Form 10-K.

These and other market and industry factors may cause the market price and demand for our securities to fluctuate substantially, regardless of our actual operating performance, which may limit or prevent investors from selling their common shares at or above the price paid for the shares and may otherwise negatively affect the liquidity of our common shares.

Some companies that have experienced volatility in the trading price of their shares have been the subject of securities class action litigation. Any lawsuit to which we are a party, with or without merit, may result in an unfavorable judgment. We also may decide to settle lawsuits on unfavorable terms. Any such negative outcome could result in payments of substantial damages or fines, damage to our reputation or adverse changes to our offerings or business practices. Defending against litigation is costly and time-consuming, and could divert our management’s attention and resources. Furthermore, during the course of litigation, there could be negative public announcements of the results of hearings, motions or other interim proceedings or developments, which could have a negative effect on the market price of our common shares.

If securities or industry analysts do not publish research, or publish inaccurate or unfavorable research, about our business, the price of our common shares and our trading volume could decline.

The trading market for our common shares will depend in part on the research and reports that securities or industry analysts publish about us or our business. Equity research analysts may elect not to initiate, and our current equity research analysts may not elect to continue to provide research coverage of our

common shares, and such lack of research coverage may adversely affect the market price of our common shares. Even if we do have equity research analyst coverage, we will not have any control over the analysts or the content and opinions included in their reports. The price of our shares could decline if one or more equity research analysts downgrade our shares or issue other unfavorable commentary or research. If one or more equity research analysts ceases coverage of our company or fails to publish reports on us regularly, demand for our shares could decrease, which in turn could cause our share price or trading volume to decline.

Anti-takeover provisions in our amended memorandum and articles of association ("Amended Memorandum and Articles of Association") could make an acquisition of us, which may be beneficial to our shareholders, more difficult and may prevent attempts by our shareholders to replace or remove our current management and limit the market price of our common shares.

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

- establish a classified Board such that not all members of the Board are elected at one time;
- allow the authorized number of our directors to be changed only by resolution of our Board;
- limit the manner in which shareholders can remove directors from the Board;
- establish advance notice requirements for shareholder proposals that can be acted on at shareholder meetings and nominations to our Board;
- require that shareholder actions must be effected at a duly called shareholder meeting and prohibit actions by our shareholders by written consent;
- limit the ability of members to requisition and convene general meetings of members; and
- authorize our Board to issue preferred shares in one or more series and to designate the price, rights, preferences, privileges and restrictions of such preferred shares without any further vote or action by our members without shareholder approval, which could be used to institute a shareholder rights plan, or so-called "poison pill," that would work to dilute the stock ownership of a potential hostile acquirer, effectively preventing acquisitions that have not been approved by our Board.

Any provision of our Amended Memorandum and Articles of Association or BVI law that has the effect of delaying or deterring a change of control could limit the opportunity for our shareholders to receive a premium for their common shares, and could also affect the price that some investors are willing to pay for our common shares.

Substantially all of our total outstanding shares may be sold freely into the market. This could cause the market price of our common shares to drop significantly, even if our business is doing well.

Sales of substantially all of our common shares in the public market, or the perception that these sales might occur, could depress the market price of our common shares and could impair our ability to raise capital through the sale of additional equity securities. Substantially all of our common shares are freely tradable, without restrictions or further registration under the Securities Act, subject to certain restrictions applicable to shares held by our affiliates as defined in Rule 144 under the Securities Act.

Because we do not expect to pay dividends on our common shares in the foreseeable future, capital appreciation, if any, would be your sole source of gain.

We have never declared or paid any dividends on our common shares. We currently anticipate that we will retain future earnings for the development, operation and expansion of our business and do not anticipate declaring or paying any cash dividends for the foreseeable future. The decision to pay future dividends to shareholders will be at the discretion of our Board after taking into account various factors including our business prospects, cash requirements, financial performance and new product development. Accordingly, investors cannot rely on dividend income from our common shares and any returns on an investment in our common shares will likely depend entirely upon any future appreciation in the price of our common shares.

Effective December 31, 2023, we are a large accelerated filer and no longer qualify as a smaller reporting company or emerging growth company, which will increase our costs and demands on management.

Based on the Company's public float as of June 30, 2023, we became a "large accelerated filer" and lost "emerging growth company" status on December 31, 2023. Additionally, due to the Company's public float as of June 30, 2023, we no longer qualify as a "smaller reporting company." However, we are not required to reflect the change in our "smaller reporting company" status, or comply with the associated increased

disclosure obligations, until our quarterly report for the three-month period ended March 31, 2024. Due to this upcoming transition, we are devoting significant time and efforts to implement and comply with the additional standards, rules and regulations that will apply to us upon becoming a large accelerated filer and losing our smaller reporting company and emerging growth company status, diverting such time from the day-to-day conduct of our business operations. Compliance with the additional requirements of being a large accelerated filer have increased our legal, accounting and financial compliance costs as of year-end, and these costs will continue to increase. These requirements include, but are not limited to:

- compliance with the auditor attestation requirements in the assessment of our internal control over financial reporting;
- compliance with any requirement that is adopted by the Public Company Accounting Oversight Board regarding mandatory audit firm rotation or a supplement to the auditor's report providing additional information about the audit and the financial statements;
- full disclosure obligations regarding executive compensation in our periodic reports and proxy statements; and
- compliance with the requirements of holding a non-binding advisory vote on executive compensation and stockholder approval of any golden parachute payments not previously approved.

Due to the complexity and logistical difficulty of implementing the standards, rules and regulations that apply to a large accelerated filer, there is an increased risk that we may be found to be in non-compliance with such standards, rules and regulations or to have significant deficiencies or material weaknesses in our internal controls over financial reporting. Any failure to maintain effective disclosure controls and internal control over financial reporting could materially and adversely affect our business, results of operations, and financial condition and could cause a decline in the trading price of our common shares.

We are a BVI business company limited by shares and, the holders of our common shares may have fewer protections as a shareholder of our company, because judicial precedent regarding the rights of shareholders is more limited under BVI law than that under U.S. law.

Our corporate affairs are governed by our Amended Memorandum and Articles of Association as amended and restated from time to time, the BVI Business Companies Act (As Revised) (the “BVI Act”) and the common law of the BVI. The rights of shareholders to take legal action against our directors, actions by minority shareholders and the fiduciary responsibilities of our directors under BVI law are to a large extent governed by the common law of the BVI. The common law of the BVI is derived in part from comparatively limited judicial precedent in the BVI as well as from English common law, which has persuasive, but not binding, authority on a court in the BVI. The rights of our shareholders and the fiduciary responsibilities of our directors under BVI law therefore are not as clearly established as they would be under statutes or judicial precedents in some jurisdictions in the United States. In particular, the BVI has a less exhaustive body of securities laws as compared to the United States, and some states, such as Delaware, have more fully developed and judicially interpreted bodies of corporate law than the BVI. There is no statutory recognition in the BVI of judgments obtained in the U.S., although the courts of the BVI will in certain circumstances recognize and enforce a non-penal judgment of a foreign court of competent jurisdiction without retrial on the merits.

As a result of all of the above, holders of our common shares may have more difficulty in protecting their interests through actions against our management, directors or controlling shareholders than they would as shareholders of a U.S. company. They may have greater difficulty securing legal advice about the law of the BVI than they would U.S. and state law, and the relatively less developed nature of the BVI’s securities law may leave investors with less certainty about the validity and strength of any claims they believe they may have against us. In addition, other differences between BVI and U.S. law, as well as the terms of our Amended Memorandum and Articles of Association, may result in shareholders having different potential influence than they would under various U.S. state laws with respect to matters such as officer and director actions, mergers and acquisitions, dispositions of assets, takeover efforts, and other corporate decision making.

Shareholders in BVI business companies may not be able to initiate shareholder derivative actions, thereby depriving a shareholder of the ability to protect its interests.

While statutory provisions do exist in BVI law for derivative actions to be brought in certain circumstances, shareholders in BVI business companies may not have standing to initiate a shareholder derivative action in a federal court of the United States. The circumstances in which any such action may be brought, and the procedures and defenses that may be available in respect to any such action, may result in the rights of shareholders of a BVI business company being more limited than those of shareholders of a company organized in the United States. Accordingly, shareholders may have fewer alternatives available to them if they believe that corporate wrongdoing has occurred. The BVI courts are also unlikely to: (i) recognize or enforce against us judgments of courts in the United States based on certain civil liability provisions of U.S. securities law; or (ii) to impose liabilities against us, in original actions brought in the BVI, based on certain civil liability provisions of U.S. securities laws that are penal in nature or that relate to taxes or similar fiscal or revenue obligations or would be

viewed as contrary to BVI public policy or the proceedings pursuant to which judgment was obtained were contrary to natural justice.

There is no statutory recognition in the BVI of judgments obtained in the United States. However, the courts of the BVI will in certain circumstances recognize such a foreign judgment and treat it as a cause of action in itself which may be sued upon as a debt at common law so that no retrial of the issues would be necessary, provided that:

- the U.S. court issuing the judgment had jurisdiction in the matter and the company either submitted to such jurisdiction or was resident or carrying on business within such jurisdiction and was duly served with process;
- the judgment is final and for a liquidated sum;
- the judgment given by the U.S. court was not in respect of penalties, taxes, fines or similar fiscal or revenue obligations of the company;
- in obtaining judgment there was no fraud on the part of the person in whose favor judgment was given or on the part of the court;
- recognition or enforcement of the judgment in the British Virgin Islands would not be contrary to public policy; and
- the proceedings pursuant to which judgment was obtained were not contrary to natural justice.

The British Virgin Islands courts are unlikely:

- to recognize or enforce against the Company, judgments of courts of the U.S. predicated upon the civil liability provisions of the securities law of the U.S.; and
- to impose liabilities against the Company, predicated upon the certain civil liability provisions of the securities laws of the U.S. so far as the liabilities imposed by those provisions are penal in nature.

The laws of the BVI relating to the protection of minority shareholders differ from those under U.S. law and, in some circumstances, may offer less protection.

The BVI Act includes the following statutory remedies which minority shareholders in the company can rely upon:

- If the company or a director of the company engages in or proposes to engage in conduct, that contravenes the BVI Act or our Amended Memorandum and Articles of Association, a shareholder may apply to the BVI court for an order directing the company or its director(s) to comply with or restraining the company or a director from engaging in conduct that contravenes the BVI Act or our Amended Memorandum and Articles of Association.
- Under the BVI Act, minority shareholders have a statutory right to bring a derivative action in the name of and on behalf of the company in circumstances where the company has a cause of action against its directors. This remedy is available at the discretion of the BVI court which will take a number of factors into account before granting or refusing a leave to proceed to the relevant shareholder, including whether such action is in the interests of the company, the cost of such action and whether there are alternative remedies that the shareholder concerned may rely upon.
- A shareholder of the company may bring an action against the company for breach of duty owed to him or her as a shareholder. This would typically be relevant in a situation where a shareholder is aggrieved by the company for breach of an entitlement or right under the company's memorandum and articles of association.
- A shareholder of the company who considers that the affairs of the company have been, are being or likely to be, conducted in a manner that is, or any act or acts of the company have been, or are, likely to be oppressive, unfairly discriminatory, or unfairly prejudicial to him in that capacity, may apply to the BVI court for an order to remedy the situation. Again, this is a discretionary remedy and the BVI court will only award it if they are satisfied that it is just and equitable to do so.
- A shareholder may, in certain circumstances, apply for liquidators to be appointed over the affairs of a company under the BVI's Insolvency Act 2003 (as amended) (the "BVI Insolvency Act"). Shareholders can also by resolution appoint a liquidator of a BVI business company under the BVI Act if the company is solvent or under the BVI Insolvency Act if the company is insolvent.

In addition to the statutory rights outlined above, there are common law rights for the protection of shareholders that may be invoked, largely dependent on English common law. Under the general rule pursuant to English common law known as the rule in Foss v. Harbottle, a court will generally refuse to interfere with the management of a company at the insistence of a minority of its shareholders who express dissatisfaction with the conduct of the company's affairs by the majority or the Board. However, every shareholder is entitled to have the affairs of the company conducted properly according to law and the constituent documents of the company. As such, if those who control the company have persistently disregarded the requirements of company law or the provisions of the company's Amended Memorandum and Articles of Association, then the courts will grant relief. Generally, the areas in which the courts will intervene are the following: (1) an act complained of which is outside the scope of the authorized business or is illegal or not capable of ratification by the majority; (2) acts that constitute fraud on the minority where the wrongdoers control the company; (3) acts that infringe on the personal rights of the shareholders, such as the right to vote; and (4) where the company has not complied with provisions requiring approval of the shareholders, which are more

limited than the rights afforded minority shareholders under the laws of many states in the United States.

Having regard to the above, the protection available to minority shareholders under BVI law may be more limited than under the laws of some jurisdictions in the United States.

It may be difficult to enforce a U.S. or foreign judgment against us, our directors and our officers outside the United States, or to assert U.S. securities laws claims outside of the United States.

As a BVI business company, it may be difficult for a shareholder to effect service of process within the United States upon us, our directors and officers, or to enforce against us, or them, judgments obtained in U.S. courts, including judgments predicated upon the civil liability provisions of the securities laws of the United States or any state therein. Additionally, it may be difficult to assert U.S. securities law claims in actions originally instituted outside of the United States. Foreign courts may refuse to hear a U.S. securities law claim because foreign courts may not be the most appropriate forums in which to bring such a claim. Even if a foreign court agrees to hear a claim, it may determine that the law of the jurisdiction in which the foreign court resides, and not U.S. law, is applicable to the claim. Further, if U.S. law is found to be applicable, the content of applicable U.S. law must be proved as a fact, which can be a time-consuming and costly process, and certain matters of procedure would still be governed by the law of the jurisdiction in which the foreign court resides. Accordingly, it may be difficult or impossible for you to bring an action against us in the BVI if you believe your rights under the U.S. securities laws have been infringed. In addition, there is uncertainty as to whether the courts of the BVI would recognize or enforce judgments of U.S. courts against us or such persons predicated upon the civil liability provisions of the securities laws of the U.S. or any state and it is uncertain whether such British Virgin Islands courts would hear original actions brought in the British Virgin Islands against us or such persons predicated upon the securities laws of the U.S. or any state.

Changes in tax law, determinations by tax authorities or changes in our effective tax rates may adversely affect our business and financial results.

Under current law, we expect to be treated as a non-U.S. corporation for U.S. federal income tax purposes. The tax laws applicable to our business activities, however, are subject to change and uncertain interpretation. Our tax position could be adversely impacted by changes in tax rates, tax laws, tax practice, tax treaties or tax regulations or changes in the interpretation thereof by the tax authorities in jurisdictions in which we do business. Our actual tax rate may vary from our expectation and that variance may be material. A number of factors may increase our future effective tax rates, including: (1) the jurisdictions in which profits are determined to be earned and taxed; (2) the resolution of issues arising from any future tax audits with various tax authorities; (3) changes in the valuation of our deferred tax assets and liabilities; (4) our ability to use net operating loss carryforwards to offset future taxable income and any adjustments to the amount of the net operating loss carryforwards we can utilize; and (5) changes in tax laws or the interpretation of such tax laws, and changes in generally accepted accounting principles. We may also become subject to income, withholding or other taxes in jurisdictions by reason of our activities and operations, and it is possible that taxing authorities in such jurisdictions could assert that we are subject to greater taxation than we currently anticipate. Since 2017, the G20/OECD Inclusive Framework has been working on addressing the tax challenges arising from the digitalization of the economy and has proposed a two-pillar tax approach with pillar one referring to the re-allocation of taxing rights, addressing issues such as where tax should be paid and on what basis (i.e., where sustained and significant business is conducted, regardless of a physical presence), and pillar two ensuring a minimum tax to be paid by multinational enterprises. We are unable to predict when and how the Inclusive Framework agreement will be enacted into law in the countries in which we operate, and it is possible that the implementation of the Inclusive Framework agreement, including the global minimum corporate tax rate, could have a material effect on our liability for corporate taxes and our consolidated effective tax rate when we fall into the scope of the rules.

If we are or become a passive foreign investment company, there could be adverse U.S. federal income tax consequences to U.S. holders.

If we are or become a passive foreign investment company (“PFIC”) for any taxable year during which a U.S. holder holds our shares, the U.S. holder would be subject to adverse tax consequences regardless of whether we continue to qualify as a PFIC, including ineligibility for any preferred tax rates on capital gains or on actual or deemed dividends, interest charges on certain taxes treated as deferred, and additional reporting requirements.

Under the Code, we would be a PFIC for any taxable year in which (1) 75% or more of our gross income consisted of passive income or (2) 50% or more of the average quarterly value of our assets consisted of assets that produce, or are held for the production of, passive income. For purposes of these tests, passive income includes, but is not limited to, dividends, interest, gains from the sale or exchange of investment property and certain rents and royalties. In addition, for purposes of the above calculations and subject to certain exceptions, a non-U.S. corporation that directly or indirectly owns at least 25% by value of the shares of another corporation is treated as if it held its proportionate share of the assets and received directly its proportionate share of the income of such other corporation.

Although we believe our common shares should not currently be stock of a PFIC for U.S. federal income tax purposes and do not expect to become a PFIC in the foreseeable future, we cannot provide any assurances regarding our PFIC status for any current or future taxable years. The determination of whether we are a PFIC is a fact-intensive determination made on an annual basis applying principles and methodologies which in some circumstances are unclear and subject to varying interpretation. In particular, the determination of whether we are a PFIC and the characterization of our assets as active or passive may depend in part on (i) our current and intended future business plans which are subject to change, and (ii) the application of certain "look-through" rules. For our current and future taxable years, the total value of our assets for PFIC testing purposes may fluctuate considerably from time to time, and is dependent on our application (which inherently involves an element of judgment) of the relevant valuation assumptions and methodologies. Under the income test, our status as a PFIC depends on the composition of our income which, in our current and future taxable years, we may not be able to fully control, for example, with respect to income attributed to us from entities owned 25% or more by us. The composition of our income and assets is also affected by how, and how quickly, we spend the cash we raise in any offering. Therefore, we cannot provide any assurance regarding our PFIC status for any past, current or future taxable years.

In certain circumstances, a U.S. holder of shares in a PFIC may alleviate some of the adverse tax consequences described above by making a "qualified electing fund" ("QEF") election to include in income its pro rata share of the corporation's income on a current basis. However, a U.S. holder may make a QEF election with respect to our common shares only if we agree to furnish such U.S. holder annually with a PFIC annual information statement as specified in the applicable U.S. Treasury Regulations. We currently do not intend to prepare or provide the information that would enable U.S. holders to make a QEF election if we are treated as a PFIC for any taxable year, and U.S. holders of our common shares should assume that a QEF election will not be available.

U.S. holders should consult their own tax advisors with respect to the operation of the PFIC rules and related reporting requirements in light of their particular circumstances, including the advisability of making any election that may be available.

Mail addressed to us may not reach us in a timely manner.

Mail addressed to the Company and received at its registered office will be forwarded unopened to the forwarding address supplied by Company to be dealt with. None of the Company, its directors, officers, advisors or service providers (including the organization which provides registered office services in the BVI) will bear any responsibility for any delay howsoever caused in mail reaching the forwarding address. Such risk will be borne solely by the Company's shareholders.

Our Amended Memorandum and Articles of Association provide that unless we consent in writing to the selection of an alternative forum, the courts of the British Virgin Islands shall, with certain limited exceptions, be the sole and exclusive forum for certain disputes between us and our shareholders, which could limit our shareholders' ability to obtain a favorable judicial forum for disputes with us or our directors, officers or employees.

Our Amended Memorandum and Articles of Association provide that unless we consent in writing to the selection of an alternative forum, the courts of the British Virgin Islands shall be the sole and exclusive forum for (i) any derivative action or proceeding brought on behalf of the Company, (ii) any action asserting a claim of breach of a fiduciary duty owed by any director, officer or other employee of the Company to the Company or the Company's members, (iii) any action asserting a claim arising pursuant to any provision of British Virgin Islands law or the Amended Memorandum and Articles of Association, or (iv) any action asserting a claim against the Company governed by the internal affairs doctrine, and that each shareholder consents to the exclusive jurisdiction of the courts of the British Virgin Islands over all such claims or disputes. Section 22 of the Securities Act creates concurrent jurisdiction for federal and state courts over actions brought under the Securities Act or the rules and regulations promulgated thereunder. Furthermore, Section 27 of the Exchange Act creates exclusive federal jurisdiction over all suits brought to enforce any duty or liability created by the Exchange Act or the rules and regulations thereunder. As a result, the forum selection provision in our Amended Memorandum and Articles of Association will not apply to actions or suits brought to enforce any liability or duty created by the Securities Act, Exchange Act or any claim for which the federal district courts of the United States of America are, as a matter of the laws of the United States of America, the sole and exclusive forum for determination of such a claim.

This choice of forum provision may increase a shareholder's cost, impose additional litigation costs and limit the shareholder's ability to bring a claim in a judicial forum that it finds favorable for disputes with us or our directors, officers or other employees, which may discourage lawsuits against us and our directors, officers and other employees, although our shareholders will not be deemed to have waived our compliance with federal securities laws and the rules and regulations thereunder and may therefore bring certain claims in another appropriate forum. Any person or entity purchasing or otherwise acquiring any of our shares or other securities, whether by transfer, sale, operation of law or otherwise, shall be deemed to have notice of and have irrevocably agreed and consented to these provisions. It is possible that a court could find such a choice of forum provision to be inapplicable or unenforceable, and if a court were to find this provision in our Amended Memorandum and Articles of Association to be inapplicable or unenforceable in an

action, we may incur additional costs associated with resolving the dispute in other jurisdictions, which could have an adverse effect on our business, results of operations and financial condition.

The market price and trading volume of our common shares may be volatile.

The market price of our common shares could fluctuate significantly for many reasons, including in response to the risk factors listed in this Annual Report on Form 10-K or for reasons unrelated to our specific performance, such as reports by industry analysts, investor perceptions, or negative developments for our customers, competitors or suppliers, as well as general economic and industry conditions.

If we are unable to satisfy the requirements of Section 404 of the Sarbanes-Oxley Act, or our internal control over financial reporting is not effective, the reliability of our financial statements may be questioned and our share price may suffer.

Section 404 of the Sarbanes-Oxley Act requires any company subject to the reporting requirements of the U.S. securities laws to do a comprehensive evaluation of its and its consolidated subsidiaries' internal control over financial reporting. To comply with this statute, we are required to document and test our internal control procedures, our management is required to assess and issue a report concerning our internal control over financial reporting, and our independent auditors is required to issue an opinion on the Company's internal controls over financial reporting. The rules governing the standards that must be met for management to assess our internal control over financial reporting are complex and require significant documentation, testing and possible remediation to meet the detailed standards under the rules. During the course of its testing, our management may identify material weaknesses or deficiencies which may not be remedied in time to meet the deadline imposed by the Sarbanes-Oxley Act. If our management cannot favorably assess the effectiveness of our internal control over financial reporting or our auditors identify material weaknesses in our internal controls, investor confidence in our financial results may weaken, and our share price may suffer.

Item 1B. Unresolved Staff Comments

None.

Item 1C. Cybersecurity

Risk Management and Strategy

We have strategically integrated cybersecurity risk management into our broader risk management framework to promote a company-wide culture of cybersecurity risk management. Our cybersecurity program is aligned with industry standards and best practices, such as the National Institute of Standards and Technology ("NIST") Cybersecurity Framework. Our measures to prevent, detect and mitigate cyber threats include training for employees, multi-factor authentication, backup servers, threat monitoring, periodic strategy review and penetration testing performed by a third-party advisory firm, and coverage under an information security risk insurance policy. Third-party suppliers are pre-screened as part of our vendor onboarding process to evaluate their cybersecurity programs and assess risk. As a part of our pre-screening process, we evaluate each vendor's overall cybersecurity risk profile relative to the services they provide for Biohaven, including questions relating to cybersecurity insurance, security operations and access and management of our data. Cybersecurity incidents involving third-party supplier systems are evaluated for their impact on the Company and managed through our cyber incident management process. In addition, our employees are required to complete an annual cybersecurity training managed through our internal compliance training system.

Governance

The Board recognizes the importance of cybersecurity in maintaining the trust and confidence of our stakeholders, patients and employees. Our Audit Committee is central to the Board's oversight of cybersecurity and bears the primary responsibility for overseeing risks associated with our information systems and technology, including cybersecurity.

Our Chief Technology Officer ("CTO") is tasked with updating the Board and Audit Committee on cybersecurity risks. Our CTO provides comprehensive briefings to our Audit Committee on a quarterly basis regarding our strategy for managing and mitigating cybersecurity and technology-related risks. Beginning in 2024, our CTO will also provide such comprehensive briefings to the Board on an annual basis. Our CTO has been in the position for over three years and has over 35 years of industry experience focusing on large scale business transformations leveraging technology, cybersecurity, and technical operations excellence. Our CTO has held various senior technology leadership roles at other companies prior to joining the Company.

Cybersecurity Risks

Although cybersecurity risks have not materially affected us, including our business strategy, results of operations or financial condition, to date, we are subject to various cybersecurity risks, which could, in the future, be material. For more information about the cybersecurity risks we face, see risk factor entitled "Our business and operations may be materially adversely affected in the event of computer system failures or security breaches" in Item 1A, "Risk Factors."

Item 2. Properties

Our U.S. headquarters is located in New Haven, Connecticut. Details of our leased and owned facilities, which include our U.S. headquarters and consist of

office, lab, research, chemistry, and discovery facilities, are as follows:

Location	Type	Approximate Square Feet	Lease Expiration
New Haven, Connecticut	Office Space	42,000	N/A
Cambridge, Massachusetts	Office & Lab Space	27,000	October, 2032
Yardley, Pennsylvania	Office Space	21,000	September, 2027
Pittsburgh, Pennsylvania	Office & Research Space	20,000	October, 2024
New Haven, Connecticut	Office Space	10,000	N/A
New Haven, Connecticut	Chemistry & Discovery Facilities	10,000	December, 2024
Dublin, Ireland	Office Space	6,000	April, 2027

We believe that our current facilities are suitable and adequate to meet our current needs and we believe that suitable additional or substitute space will be available as needed to accommodate any future expansions.

Item 3. Legal Proceedings

From time to time, in the ordinary course of business, the Company is subject to litigation and regulatory examinations as well as information gathering requests, inquiries and investigations. We are not currently a party to any material legal proceedings, and we are not aware of any pending or threatened legal proceeding against us that we believe could have a material adverse effect on our business, operating results, cash flows or financial condition.

Item 4. Mine Safety Disclosures

Not applicable.

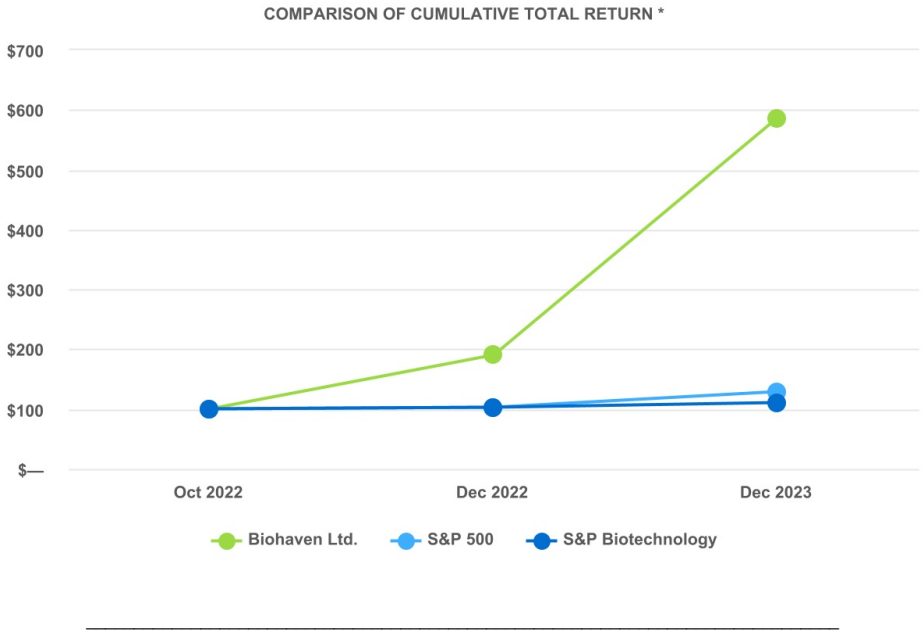
PART II

Item 5. Market for Registrant's Common Equity, Related Shareholder Matters and Issuer Purchases of Equity Securities

Market Information

Our common shares began trading on the New York Stock Exchange under the symbol "BHVN" on October 4, 2022.

Stock Performance Graph



* $100 invested on October 4, 2022 in stock or index, including reinvestment of dividends. Fiscal year ending December 31.

Shareholders

As of February 26, 2024, there were 51 shareholders of record of our common shares. The actual number of holders of our common shares is greater than this number of record holders, and includes shareholders who are beneficial owners, but whose shares are held in street name by brokers or held by other nominees. This number of holders of record also does not include shareholders whose shares may be held in trust by other entities.

Dividend Policy

We have never declared or paid dividends on our share capital. We do not expect to pay any cash dividends on our common shares in the foreseeable future. All decisions regarding the payment of dividends will be made by our Board of Directors from time to time in accordance with applicable law.

Recent Sales of Unregistered Securities

Agreement with Hangzhou Highlightll Pharmaceutical Co. Ltd.

In March 2023, we entered into an exclusive, worldwide (excluding People's Republic of China and its territories and possessions) license agreement with Hangzhou Highlightll Pharmaceutical Co. Ltd. ("Highlightll") (the "Highlightll Agreement"), pursuant to which we obtained the right to research, develop, manufacture and commercialize Highlightll's brain penetrant dual TYK2/JAK1 inhibitor program. In December 2023, we entered into a second amendment to the Highlightll Agreement, which granted us an exclusive option and right of first refusal to any Selective TYK2 Inhibitor being developed by or on behalf of Highlightll or its affiliates. As a result of the second amendment, we issued 721,136 of our common shares, valued at $21.8 million, that were not registered under the Securities Act to Highlightll in December 2023.

Highlightll represented that, among other things, it is an institutional accredited investor as defined in Rule 501(a) of Regulation D of the Securities Act, and the foregoing shares were issued in reliance on the private offering exemption provided by Section 4(a)(2) of the Securities Act. See Note 11, "License Agreements," to the Consolidated Financial Statements appearing elsewhere in this report for additional details on this transaction.

Pyramid Acquisition

In January 2024, we acquired Pyramid Biosciences, Inc. ("Pyramid"), pursuant to an Agreement and Plan of Merger, dated January 7, 2024 (the "Pyramid Agreement"). In consideration for the Pyramid acquisition, we made an upfront payment of $10.0 million of our common shares. We also agreed to make additional success-based payments up to $40.0 million upon achievement of certain regulatory milestones, which we may elect to pay in cash or our common shares. In January 2024, a $5.0 million payment became due to Pyramid related to achievement of a regulatory milestone under the Pyramid Agreement, which we elected to pay in our common shares. The shares related to both of these payments were not registered under the Securities Act.

Pyramid represented that, among other things, it is an institutional accredited investor as defined in Rule 501(a) of Regulation D of the Securities Act. The foregoing shares shall be issued in reliance on the private offering exemption provided by Section 4(a)(2) of the Securities Act. See Note 6, "Acquisitions," to the Consolidated Financial Statements appearing elsewhere in this report for additional details on this transaction.

Securities Authorized for Issuance Under Equity Compensation Plans

See Part III, Item 12 "Security Ownership of Certain Beneficial Owners and Management and Related Shareholder Matters" of this report.

Issuer Purchases of Equity Securities

We did not purchase any of our registered equity securities during the period covered by this report.

Item 6. Reserved

Item 7. Management's Discussion and Analysis of Financial Condition and Results of Operations

You should read the following discussion and analysis of our financial condition and results of operations together with our financial statements and related notes appearing elsewhere in this Annual Report on Form 10-K, or this Annual Report. Discussions of 2021 items and year-to-year comparisons between the years ended December 31, 2022 and 2021 that are not included in this Form 10-K can be found within "Item 7. Management's Discussion and Analysis of Financial Condition and Results of Operations" in our Annual Report on Form 10-K for the year ended December 31, 2022. In addition to historical information, this discussion and analysis contains forward-looking statements that involve risks, uncertainties and assumptions. Our actual results may differ materially from those anticipated in these forward-looking statements as a result of certain factors. We discuss factors that we believe could cause or contribute to these differences below and elsewhere in this report, including those set forth under Item 1A. "Risk Factors" and under "Cautionary Note Regarding Forward-Looking Statements" in this Annual Report.

Overview

We are a biopharmaceutical company focused on the discovery, development, and commercialization of life-changing treatments in key therapeutic areas, including immunology, neuroscience, and oncology. We are advancing our innovative portfolio of therapeutics, leveraging our proven drug development experience and multiple proprietary drug development platforms. Our extensive clinical and preclinical programs include Kv7 ion channel modulation for epilepsy and mood disorders; extracellular protein degradation for immunological diseases; TRPM3 antagonism for migraine and neuropathic pain; TYK2/JAK1 inhibition for neuroinflammatory disorders; glutamate modulation for OCD and SCA; myostatin inhibition for neuromuscular and metabolic diseases, including SMA and obesity; and antibody recruiting, bispecific molecules and ADCs for cancer.

For a full discussion of our programs, including recent developments, refer to "Item 1. Business" included in this Annual Report on Form 10-K.

Separation from Biohaven Pharmaceutical Holding Company Ltd.

On October 3, 2022, the Former Parent completed the Separation from Biohaven Ltd. As a result of the Separation, Biohaven Ltd. became an independent, publicly traded company as of October 3, 2022, and commenced regular way trading under the symbol "BHVN" on the NYSE on October 4, 2022.

Biohaven is a British Virgin Islands ("BVI") corporation and was a wholly owned subsidiary of the Former Parent prior to the Separation.

Prior to the Separation, the historical combined financial statements of the Company prior to the distribution were prepared on a stand-alone basis and are derived from the consolidated financial statements and accounting records of the Former Parent. The financial statements for all periods presented, including the historical results of the Company prior to October 3, 2022, are now referred to as "Consolidated Financial Statements." Our financial statements are presented in conformity with generally accepted accounting principles in the United States ("GAAP").

The financial position, results of operations and cash flows of the Company historically operated as part of the Former Parent's financial position, results of operations and cash flows up until the Distribution. These historical combined financial statements may not be indicative of the future performance of the Company and do not necessarily reflect what our consolidated results of operations, financial condition and cash flows would have been had we operated as a separate, publicly traded company during the periods presented.

Where we describe historical business activities in this Annual Report on Form 10-K, we do so as if these transfers had already occurred and the Former Parent's activities related to such assets and liabilities had been performed by Biohaven.

Refer to Note 1, "Nature of the Business and Basis of Presentation," of the Notes to the Consolidated Financial Statements appearing elsewhere in this Annual Report on Form 10-K for further discussion of the underlying basis used to prepare the consolidated financial statements.

Transition from the Former Parent and Costs to Operate as an Independent Company

The consolidated financial statements reflect the operating results and financial position of the Company as it was operated by the Former Parent prior to the Separation, rather than as an independent company. We have incurred and will continue to incur ongoing operating expenses to operate as an independent company. These costs will include the cost of various corporate headquarters functions, information technology-related costs and costs to operate stand-alone accounting, legal and other administrative functions. We will also incur non-recurring expenses and non-recurring capital expenditures. As an independent company, our information technology operating costs may be higher than the costs allocated in the historical combined financial statements. It is not practicable to estimate the costs that would have been incurred in each of the periods presented in the historical combined financial statements for the functions described above. Actual costs that would have been incurred if we operated as a stand-alone company during these periods would have depended on various factors, including the chosen organizational structure, what corporate functions the Company might have performed directly or outsourced and strategic

decisions the Company might have made in areas such as executive management, legal and other professional services, and certain corporate overhead functions.

Agreements with the Former Parent

We had entered into a Distribution Agreement and various other agreements relating to transition services, licenses and certain other matters with the Former Parent. These agreements govern our relationship with the Former Parent and include the allocation of employee benefits, taxes and certain other liabilities and obligations attributable to periods prior to, at and after the Separation. For additional information regarding these agreements, see Note 14, "Related Party Transactions," of the Notes to the Consolidated Financial Statements appearing elsewhere in this Annual Report on Form 10-K.

Components of Our Results of Operations

Revenue

To date, we have not generated any revenue from product sales and we do not expect to generate any revenue from the sale of products in the near future. If our development efforts for our product candidates are successful and result in regulatory approval or additional license agreements with third parties, then we may generate revenue in the future from product sales.

Operating Expenses

Research and Development Expenses

Research and development ("R&D") expenses consist primarily of costs incurred in connection with the development of our product candidates. We expense research and development costs as incurred. These expenses include:

- expenses incurred under agreements with contract research organizations (“CROs”) or contract manufacturing organizations (“CMOs”), as well as investigative sites and consultants that conduct our clinical trials, preclinical studies and other scientific development services;
- manufacturing scale-up expenses and the cost of acquiring and manufacturing preclinical and clinical trial materials and commercial materials, including manufacturing validation batches;
- employee-related expenses, including salaries, benefits, travel and non-cash share-based compensation expense for employees engaged in research and development functions;
- costs related to compliance with regulatory requirements;
- development milestone payments incurred prior to regulatory approval of the product candidate;
- rent and operating expenses incurred for leased lab facilities and equipment; and
- payments made in cash, equity securities or other forms of consideration under third-party licensing or other agreements prior to regulatory approval of the product candidate.

We recognize external development costs based on an evaluation of the progress to completion of specific tasks using estimates from our clinical personnel and information provided to us by our service providers.

Our external direct research and development expenses are tracked on a program-by-program basis for our product candidates and consist primarily of external costs, such as fees paid to outside consultants, CROs, CMOs, and central laboratories in connection with our preclinical development, process development, manufacturing and clinical development activities. Our direct research and development expenses by program also include fees and certain development milestones incurred under license agreements. We do not allocate employee costs, or other indirect costs, to specific programs because these costs are deployed across multiple programs and, as such, are not separately classified. We use internal resources primarily to oversee the research and development as well as for managing our preclinical development, process development, manufacturing and clinical development activities.

Product candidates in later stages of clinical development generally have higher development costs than those in earlier stages of clinical development, primarily due to the increased size and duration of later-stage clinical trials. We expect that our research and development expenses will remain significant over the next several years as we increase personnel costs, conduct late-stage clinical trials, and prepare regulatory filings for our product candidates. We also expect to incur additional expenses related to milestones payable to third parties with whom we have entered into license agreements to acquire the rights to our product candidates.

The successful development and commercialization of our product candidates is highly uncertain. At this time, we cannot reasonably estimate or know the nature, timing and costs of the efforts that will be necessary to complete the preclinical and clinical development of any of our product candidates or when, if ever, material net cash inflows may commence from any of our product candidates. This uncertainty is due to the numerous risks and uncertainties associated with product development and commercialization, including the uncertainty of:

- the scope, progress, outcome and costs of our preclinical development activities, clinical trials and other research and development activities;

- establishment of an appropriate safety profile with IND-enabling studies;
- successful patient enrollment in, and the initiation and completion of, clinical trials;
- the timing, receipt and terms of any marketing approvals from applicable regulatory authorities;
- establishment of commercial manufacturing capabilities or making arrangements with third-party manufacturers;
- development and timely delivery of commercial-grade drug formulations that can be used in our clinical trials and for commercial launch;
- acquisition, maintenance, defense and enforcement of patent claims and other intellectual property rights;
- significant and changing government regulation;
- initiation of commercial sales of our product candidates, if and when approved, whether alone or in collaboration with others; and
- maintenance of a continued acceptable safety profile of the product candidates following approval.

General and Administrative Expenses

General and administrative ("G&A") expenses consist primarily of personnel costs, including salaries, benefits and travel expenses for our executive, finance, business, corporate development and other administrative functions; and non-cash share-based compensation expense. General and administrative expenses also include facilities and other related expenses, including rent, depreciation, maintenance of facilities, insurance and supplies; and for public relations, audit, tax and legal services, including legal expenses to pursue patent protection of our intellectual property.

We anticipate that our general and administrative expenses, including payroll and related expenses, will remain significant in the future as we continue to support our research and development activities and prepare for potential commercialization of our product candidates, if successfully developed and approved. We also anticipate increased expenses associated with general operations, including costs related to accounting and legal services, director and officer insurance premiums, facilities and other corporate infrastructure, office-related costs, such as information technology costs, and certain costs to establish ourself as a standalone public company, as well as ongoing additional costs associated with operating as an independent, publicly traded company.

Other Income (Expense)

Other Income (Expense), Net

Other income (expense), net during the year ended December 31, 2023 primarily consists of net investment income and service revenue from the Transition Service Agreement we entered into with the Former Parent. Refer to Note 14, "Related Party Transactions," for further discussions of agreements entered into with the Former Parent. Net investment income is comprised of interest income and net accretion and amortization on investments in addition to realized gains and losses. Refer to Note 3, "Marketable Securities," for further discussion of our investments.

Other income (expense), net during the year ended December 31, 2022 primarily consisted of a $10.0 million impairment loss recognized during the fourth quarter on our Artizan Series A-2 Preferred Stock Investment, partially offset by net investment income and service revenue from the Transition Service Agreement we entered into with the Former Parent.

Provision for Income Taxes

The income tax expense in the consolidated financial statements was calculated on a separate return method and presented as if the Company’s operations were separate taxpayers in the respective jurisdictions up to and including the Separation. Cash tax payments, income taxes receivable and deferred taxes, net of valuation allowance, are reflective of our actual tax balances prior and subsequent to the Separation.

As a company incorporated in the BVI, we are principally subject to taxation in the BVI. Under the current laws of the BVI, the Company and all dividends, interest, rents, royalties, compensation and other amounts paid by the Company to persons who are not resident in the BVI and any capital gains realized with respect to any shares, debt obligations, or other securities of the Company by persons who are not resident in the BVI are exempt from all provisions of the Income Tax Ordinance in the BVI.

We have historically outsourced all of the research and clinical development for our programs under a master services agreement with Biohaven Pharmaceuticals, Inc. ("BPI"). As a result of providing services under this agreement, BPI was profitable during the years ended December 31, 2023, 2022, and 2021, and BPI is subject to taxation in the United States. As such, in each reporting period, the tax provision includes the effects of the results of operations of BPI.

At December 31, 2023 and 2022, we continued to maintain a full valuation allowance against our net deferred tax assets, comprised primarily of research and development tax credit carryforwards, and net operating loss carryforwards, based on management’s assessment that it is more likely than not that the deferred tax assets will not be realized.

Our income tax provision/benefit primarily represents Federal and state taxes related to the profitable operations of our subsidiaries in the United States and Ireland. The income tax benefit recorded during the year ended December 31, 2023 was primarily attributable to our adoption of the guidance contained in a Notice of Proposed Rule Making issued by the United States Internal Revenue Service during the third quarter of 2023 ("the Notice"). The Notice indicates that BPI has the ability to immediately deduct R&D expenditures which were incurred in the US and reimbursed by our foreign parent. Previously these expenditures were capitalized, as was generally understood to be required under the Tax Cuts and Jobs Act, which was effective for tax years beginning on or after January 1, 2022. Based on this guidance and its application to our specific facts, we deducted these expenditures on our 2022 tax return, substantially reducing our taxable income in the US and capitalized R&D expenditures, resulting in an increase to our federal net operating loss carryforward of $598.7 million that can be carried forward indefinitely. Our adoption of the Notice in 2023 also increased our State net operating losses, resulting in the reversal of $0.8 million of state income taxes recorded in 2022.

Results of Operations

Comparison of the Years Ended December 31, 2023 and 2022

The following table summarizes our results of operations for the years ended December 31, 2023 and 2022:

	Year Ended December 31,		
In thousands	**2023**	**2022**	**Change**
Operating expenses:			
Research and development	$ 373,281	$ 437,072	$ (63,791)
General and administrative	62,770	130,860	(68,090)
Total operating expenses	436,051	567,932	(131,881)
Loss from operations	(436,051)	(567,932)	131,881
Other income (expense):			
Other income (expense), net	26,500	(1,909)	28,409
Total other income (expense), net	26,500	(1,909)	28,409
Loss before (benefit) provision for income taxes	(409,551)	(569,841)	160,290
(Benefit) provision for income taxes	(1,383)	438	(1,821)
Net loss	$ (408,168)	$ (570,279)	$ 162,111

Research and Development Expenses

	Year Ended December 31,		
In thousands	**2023**	**2022***	**Change**
Direct research and development expenses by program:			
BHV-7000 & BHV-7010	$ 47,327	$ 131,956	$ (84,629)
BHV-8000	39,025	—	39,025
BHV-2100 (TRPM3)	12,864	8,255	4,609
Troriluzole	73,080	58,769	14,311
BHV-2000	40,870	16,799	24,071
BHV-1300	22,239	—	22,239
BHV-1100	1,951	852	1,099
BHV-1200 (COVID 19)	—	6,033	(6,033)
Verdiperstat	3,064	13,654	(10,590)
Other programs	600	668	(68)
Unallocated research and development costs:			
Personnel related (including non-cash share-based compensation)	78,608	170,122	(91,514)
Preclinical research programs	35,093	18,116	16,977
Other	18,560	11,848	6,712
Total research and development expenses	$ 373,281	$ 437,072	$ (63,791)

**Certain prior year amounts have been reclassified to conform to current year presentation*

R&D expenses, including non-cash share-based compensation costs, were $373.3 million for the year ended December 31, 2023, compared to $437.1 million for the year ended December 31, 2022. The decrease of $63.8 million was primarily due to a one-time $93.7 million expense to BHV-7000 and BHV-7010 during 2022 for our Kv7 Platform acquisition, a $25.0 million milestone relating to BHV-7000, a decrease of $91.5 million in personnel related costs, and reduced program spend for BHV-1200 and verdiperstat in 2023 compared to 2022. The decrease was partially offset by increases in direct program spend for additional and advancing clinical trials, including late Phase 2/3 studies and preclinical research programs in 2023, as compared to the same period in the prior year. The increased program spend in 2023 included recognition of one-time expenses of a $10.0 million cash payment and a $21.8 million non-cash issuance of common shares to acquire rights related to our Highlightll Agreement. Refer to

Note 11, "License Agreements" for further discussion of the payments to Highlightll.

Non-cash share-based compensation expense was $16.0 million for the year ended December 31, 2023, a decrease of $100.4 million as compared to the same period in 2022. Non-cash share-based compensation expense for the year ended December 31, 2022 included $108.7 million of expense allocated from the Former Parent, including $61.7 million of expense recognized in connection with the settlement of outstanding Former Parent stock options and RSUs upon the effectiveness of the Separation in the fourth quarter of 2022. Non-cash share-based compensation expense was higher in the year ended December 31, 2022 primarily because expense allocated from the Former Parent equity plan was based on equity awards with higher grant date fair value.

General and Administrative Expenses

G&A expenses, including non-cash share-based compensation costs, were $62.8 million for the year ended December 31, 2023, compared to $130.9 million for the year ended December 31, 2022. The decrease of $68.1 million was primarily due to decreased non-cash share-based compensation costs.

Non-cash share-based compensation expense was $12.8 million for the year ended December 31, 2023, a decrease of $64.4 million as compared to the same period in 2022. Non-cash share-based compensation expense for the year ended December 31, 2022 included $70.6 million of expense allocated from the Former Parent, including $39.7 million of expense recognized in connection with the settlement of each outstanding Former Parent stock option and RSU upon the effectiveness of the Separation in the fourth quarter of 2022. Non-cash share-based compensation expense was higher in the year ended December 31, 2022 primarily because expense allocated from the Former Parent equity plan was based on equity awards with higher grant date fair value.

Other Income (Expense), Net

Other income (expense), net was other income of $26.5 million for the year ended December 31, 2023, compared to other expense of $1.9 million for the year ended December 31, 2022. The increase of $28.4 million in other income (expense), net was primarily due to increased net investment income of $14.5 million and increased service revenue from the Transition Service Agreement we entered into with the Former Parent of $5.2 million in 2023, as compared to the same period in the prior year, as well as a $10.0 million impairment loss recognized during the fourth quarter of 2022 on our Artizan Series A-2 Preferred Stock Investment.

(Benefit) Provision for Income Taxes

We recorded a benefit for income taxes of $1.4 million for the year ended December 31, 2023, compared to a provision for income taxes of $0.4 million for the year ended December 31, 2022. The decrease in the income tax provision as compared to 2022 was primarily attributable to the Company adopting the guidance contained in the Notice. See further discussion of the Notice in "Components of Our Results of Operations" included in Item 7, "Management's Discussion and Analysis of Financial Condition and Results of Operations" of this report.

Liquidity and Capital Resources

Since our inception as a business of the Former Parent, we have not generated any revenue and have incurred significant operating losses and negative cash flows from operations. We will not generate revenue from product sales unless and until we successfully complete clinical development and obtain regulatory approval for our product candidates. We expect to continue to incur significant expenses for at least the next several years as we advance our product candidates from discovery through preclinical development and clinical trials and seek regulatory approval and pursue commercialization of any approved product candidate. In addition, if we obtain marketing approval for any of our product candidates, we expect to incur significant commercialization expenses related to product manufacturing, marketing, sales and distribution, regulatory and commercial milestones and royalty payments. We may also incur expenses in connection with the in-license or acquisition of additional product candidates.

Historically, for periods prior to the Separation, we have funded our operations primarily with proceeds allocated to our business from financing arrangements entered into by the Former Parent and through the one-time issuance of contingently redeemable non-controlling interests.

From the Separation through February 29, 2024, we have funded our operations primarily with the cash contribution received from the Former Parent at the Separation and proceeds from the public offerings of our common shares. Pursuant to the Distribution Agreement, immediately prior to the Separation, the Former Parent made a cash contribution to the Company which resulted in a cash balance of approximately $257.8 million as of October 3, 2022. We have incurred recurring losses since our inception and expect to continue to generate operating losses for the foreseeable future.

As of December 31, 2023, we had cash and cash equivalents of $248.4 million and marketable securities of $133.4 million. Cash in excess of immediate requirements is invested in marketable securities and money market funds with a view to liquidity and capital preservation. We continuously assess our working capital needs, capital expenditure requirements, and future investments or acquisitions.

Cash Flows

The following table summarizes our cash flows for each of the periods presented:

	Year Ended December 31,		
In thousands	2023	2022	2021
Net cash used in operating activities	$ (331,725)	$ (297,689)	$ (145,840)
Net cash provided by (used in) investing activities	129,830	(304,790)	944
Net cash provided by financing activities	211,908	767,597	138,447
Effect of exchange rate changes on cash and cash equivalents and restricted cash	(497)	429	—
Net increase (decrease) in cash, cash equivalents and restricted cash	$ 9,516	$ 165,547	$ (6,449)

Operating Activities

Net cash used in operating activities was $331.7 million in 2023, $297.7 million in 2022, and $145.8 million in 2021. The $34.0 million increase in net cash used in operating activities in 2023 was driven primarily by an increase in payments for clinical operations, clinical supply, discovery operations and personnel costs to support our acquired and late-stage programs partially offset by an increase in collections of income tax refunds, collections related to the transition services agreement with Pfizer, and interest received on cash and restricted cash, cash equivalents, and debt securities.

Investing Activities

Net cash provided by investing activities was $129.8 million in 2023, and $0.9 million in 2021, and net cash used in investing activities was $304.8 million in 2022. The $429.7 million increase in net cash provided by investing activities in 2023 was driven primarily by an increase in proceeds from sales and maturities of marketable securities, a decrease in purchases of marketable securities (see Note 3, "Marketable Securities," to the Consolidated Financial Statements), and a decrease in cash payments for IPR&D asset acquisition.

Financing Activities

Net cash provided by financing activities was $211.9 million in 2023, $767.6 million in 2022, and $138.4 million in 2021. The $555.7 million decrease in net cash provided by financing activities in 2023 was driven primarily by a decrease in proceeds from net transfers from Parent due to the Company operating as a standalone entity for the year ended December 31, 2023 and a decrease in restricted cash held in connection with the execution of the United States Distribution Services Agreement which is legally payable to the Former Parent (see Note 14, "Related Party Transactions," to the Consolidated Financial Statements).

October 2023 Public Offering

On October 5, 2023, we closed an underwritten public offering of 11,761,363 of our common shares, which included the exercise in full of the underwriters' option to purchase additional shares, at a price to the public of $22.00 per share. The net proceeds raised in the offering, after deducting underwriting discounts and expenses of the offering payable by us, were approximately $242.4 million. The net proceeds received from the offering are being used for general corporate purposes.

Equity Distribution Agreement

In October 2023, we entered into an equity distribution agreement pursuant to which we may offer and sell common shares having an aggregate offering price of up to $150.0 million from time to time through or to the sales agent, acting as our agent or principal (the "Equity Distribution Agreement"). Sales of our common shares, if any, will be made in sales deemed to be “at-the-market offerings”. The sales agent is not required to sell any specific amount of securities but will act as our sales agent using commercially reasonable efforts consistent with its normal trading and sales practices, on mutually agreed terms between the sales agent and us. We currently plan to use the net proceeds from any at-the-market offerings of our common shares for general corporate purposes.

As of December 31, 2023, we have issued and sold no common shares under the Equity Distribution Agreement.

October 2022 Public Offering

On October 25, 2022, we completed a public offering of 28,750,000 of our common shares, including the full exercise of the underwriters' option to purchase additional shares, at a public offering price of $10.50 per share. The offering resulted in net proceeds, after deducting underwriting discounts and expenses of the offering payable by Biohaven, of approximately $282.8 million. The net proceeds from the offering are being used for general corporate purposes.

Funding Requirements

We expect our expenses to increase in connection with our ongoing activities, particularly as we advance and expand preclinical activities, clinical trials and

potential commercialization of our product candidates. Our costs will also increase as we:

- continue to advance and expand the development of our discovery programs and clinical-stage assets;
- continue to initiate and progress other supporting studies required for regulatory approval of our product candidates, including long-term safety studies, drug-drug interaction studies, preclinical toxicology and carcinogenicity studies;
- initiate preclinical studies and clinical trials for any additional indications for our current product candidates and any future product candidates that we may pursue;
- continue to build our portfolio of product candidates through the acquisition or in-license of additional product candidates or technologies;
- continue to develop, maintain, expand and protect our intellectual property portfolio;
- pursue regulatory approvals for our current and future product candidates that successfully complete clinical trials;
- establish and support our sales, marketing and distribution infrastructure to commercialize any future product candidates for which we may obtain marketing approval;
- hire additional clinical, medical, commercial, and development personnel; and
- incur additional legal, accounting and other expenses in operating as a public company.

We expect that our cash, cash equivalents and marketable securities, as of the date of this Annual Report on Form 10-K, will be sufficient to fund our current forecast for operating expenses, financial commitments and other cash requirements for more than one year. We expect we will need to raise additional capital until we are profitable. If no additional capital is raised through either public or private equity financings, debt financings, strategic relationships, alliances and licensing agreements, or a combination thereof, we may delay, limit or reduce discretionary spending in areas related to research and development activities and other general and administrative expenses in order to fund our operating costs and working capital needs.

We have based these estimates on assumptions that may prove to be wrong, and we could utilize our available capital resources sooner than we expect. We expect that we will require additional capital to pursue in-licenses or acquisitions of other product candidates. If we receive regulatory approval for our product candidates, we expect to incur commercialization expenses related to product manufacturing, sales, marketing and distribution, depending on where we choose to commercialize or whether we commercialize jointly or on our own.

Because of the numerous risks and uncertainties associated with research, development and commercialization of pharmaceutical product candidates, we are unable to estimate the exact amount of our working capital requirements. Our future funding requirements will depend on and could increase significantly as a result of many factors, including:

- the scope, progress, results and costs of researching and developing our product candidates, and conducting preclinical studies and clinical trials;
- the costs, timing and outcome of regulatory review of our product candidates;
- the costs and timing of hiring new employees to support our continued growth;
- the costs 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;
- the timing, receipt and amount of sales of, or milestone payments related to or royalties on, our current or future product candidates, if any; and
- other capital expenditures, working capital requirements, and other general corporate activities.

Until such time, if ever, that we can generate product revenue sufficient to achieve profitability, we expect to finance our cash needs through a combination of public and private equity offerings, debt financings, other third-party funding, strategic alliances, licensing arrangements or marketing and distribution arrangements. To the extent that we raise additional capital through the sale of equity or convertible debt securities, the ownership interest of our existing shareholders will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect the rights of our existing shareholders. 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, making capital expenditures or declaring dividends. If we raise additional funds through other third-party funding, strategic alliances, licensing arrangements or marketing and distribution arrangements, we may have to relinquish valuable rights to our technologies, future revenue streams, research programs or product candidates or grant licenses on terms that may not be favorable to us.

Contractual Obligations and Commitments

The following table summarizes certain estimated future obligations by period under our various contractual obligations as of December 31, 2023 and the effects that such obligations are expected to have on our liquidity and cash flows in future periods:

	Payments Due by Period				
In thousands	**Total**	**2024**	**2025-2026**	**2027-2028**	**Thereafter**
Operating leases[1]	$ 39,780	$ 5,020	$ 9,687	$ 8,454	$ 16,619
Purchase obligations					
Research commitments[2]	36,539	36,539		—	—
Total	$ 76,319	$ 41,559	$ 9,687	$ 8,454	$ 16,619

(1) Refer to Note 12, "Commitments and Contingencies," to the consolidated financial statements included in this 10-K for additional information on future minimum rental commitments under non-cancelable operating leases.

(2) Research commitments are primarily CRO agreements that are enforceable and legally binding on us and that specify all significant terms, including fixed or minimum quantities to be purchased; fixed, minimum or variable price provisions; and the approximate timing of the transaction. For obligations with cancellation provisions, the amounts included in the preceding table are limited to the non-cancelable portion of the agreement terms or the minimum cancellation fee. In addition to the amounts above, as of December 31, 2023, the Company had remaining maximum research commitments in excess of one year of approximately $9,025, which are variable based on number of trial participants and contingent upon the achievement of certain milestones of the clinical trials covered under the agreements. Since the achievement of these milestones is uncertain and the timing unpredictable, the Company did not include the additional research commitments in the table above. If all related milestones are achieved, the Company expects these amounts to be paid over approximately one year.

In addition to the contractual obligations in the table above, under various agreements with third-party licensors and collaborators, we have agreed to make milestone payments and pay royalties and annual maintenance fees to third parties and to meet due diligence requirements based upon specified milestones. We have not included any contingent payment obligations, such as milestones, royalties, or due diligence, in the table above as the amount, timing and likelihood of such payments are not known. We have not included any of the annual license maintenance fee payments in the above table, as although the amount and timing are known, we cannot currently determine the final termination dates of the agreements and, as a result, we cannot determine the total amounts of such payments we will be required to make under the agreements. We do not anticipate making material annual license maintenance payments related to our license agreements in the next 12 months.

Critical Accounting Policies and Significant Judgments and Estimates

Our consolidated financial statements are prepared in accordance with generally accepted accounting principles in the United States ("GAAP"). The preparation of our consolidated financial statements and related disclosures requires us to make estimates, assumptions, and judgments that affect the reported amounts of assets, liabilities, expenses, and related disclosures at the date of the consolidated financial statements. We evaluate our estimates and judgments on an ongoing basis. We base our estimates on historical experience and on various other factors that we believe are reasonable under the circumstances, the results of which form the basis for making judgments about the carrying value of assets and liabilities that are not readily apparent from other sources. Actual results could therefore differ materially from these estimates under different assumptions or conditions.

While our significant accounting policies are described in more detail in Note 2, "Summary of Significant Accounting Policies," in the notes to our financial statements appearing at the end of this Annual Report, we believe that the following accounting policies are those most critical to the judgments and estimates used in the preparation of our financial statements.

Accrued Research and Development Expenses

As part of the process of preparing the consolidated financial statements, we are required to estimate accrued research and development expenses. This process involves reviewing open contracts and purchase orders, communicating with our personnel to identify services that have been performed on its behalf and estimating the level of service performed and the associated cost incurred for the service when it has not yet been invoiced or otherwise notified of actual costs. The majority of our service providers invoice in arrears for services performed, on a pre-determined schedule or when contractual milestones are met; however, some require advance payments. We make estimates of our accrued expenses as of each balance sheet date in the consolidated financial statements based on facts and circumstances known to us at that time. We periodically confirm the accuracy of these estimates with the service providers and make adjustments if necessary. Examples of estimated accrued research and development expenses include fees paid to:

- vendors, including central laboratories, in connection with preclinical development activities;
- CROs and investigative sites in connection with preclinical and clinical studies; and

• CMOs in connection with drug substance and drug product formulation of preclinical and clinical trial materials.

We base expenses related to preclinical studies and clinical trials on our estimates of the services received and efforts expended pursuant to quotes and contracts with multiple research institutions and CROs that conduct and manage preclinical studies and clinical trials on our behalf as well as estimates of services and materials consumed by CMOs that manufacture drug substances and products on our behalf. The financial terms of these agreements are subject to negotiation, vary from contract to contract and may result in uneven payment flows. There may be instances in which payments made to our vendors will exceed the level of services provided and result in a prepayment of the expense. Payments under some of these contracts depend on factors such as the successful enrollment of patients and the completion of clinical trial milestones. In accruing service fees, we estimate the time period over which services will be performed and the level of effort to be expended in each period. If the actual timing of the performance of services or the level of effort varies from the estimate, we adjust the accrual or the amount of prepaid expenses accordingly. Although we do not expect our estimates to be materially different from amounts actually incurred, our understanding of the status and timing of services performed relative to the actual status and timing of services performed may vary and may result in reporting amounts that are too high or too low in any particular period. To date, there have not been any material adjustments to our prior estimates of accrued research and development expenses.

Cost Allocations

Prior to the distribution on October 3, 2022, we have historically operated as part of the Former Parent and not as separate, publicly traded company. Accordingly, for periods prior to the distribution, certain shared costs and non-cash share-based compensation expenses have been allocated to us and are reflected as expenses in the accompanying consolidated statement of operations. Management considers the expense methodology and resulting allocation to be reasonable for all periods presented; however, the allocations may not be indicative of actual expenses that would have been incurred had we operated as an independent, publicly traded company for the periods presented. Actual costs that we may have incurred had we been a stand-alone company would depend on a number of factors, including the organizational structure, what corporate functions the Company might have performed directly or outsourced and strategic decisions the Company might have made in areas such as executive management, legal and other professional services, and certain corporate overhead functions.

Recently Issued Accounting Pronouncements

A description of recently issued accounting pronouncements that may potentially impact our financial position and results of operations is disclosed in Note 2, "Summary of Significant Accounting Policies," to our consolidated financial statements appearing at the end of this Annual Report.

Emerging Growth Company Status

The Jumpstart Our Business Startups (JOBS) Act (the "JOBS Act") permits an “emerging growth company” to take advantage of an extended transition period to comply with new or revised financial accounting standards applicable to public companies until those standards would otherwise apply to nonpublic companies. As an emerging growth company, we also were exempt from Section 404(b) of the Sarbanes-Oxley Act of 2002 (Sarbanes-Oxley), which would require independent auditors to report on the effectiveness of the Company's internal control over financial reporting.

As of June 30, 2023, the market value of our common stock that was held by non-affiliates exceeded $700 million, and as a result, we no longer qualified as an emerging growth company as of December 31, 2023 when we became a large accelerated filer. Therefore, we are required to comply with new or revised financial accounting standards as of the effective dates applicable to public companies that are not emerging growth companies. In addition, we are required to comply with Section 404(b) of Sarbanes-Oxley.

Smaller Reporting Company Status

A “smaller reporting company,” as defined in Rule 12b-2 under the Exchange Act, is eligible for exemptions from various reporting requirements applicable to other public companies that are not smaller reporting companies, including, but not limited to, reduced disclosure obligations regarding executive compensation.

As of June 30, 2023, the aggregate market value of our common shares held by non-affiliates exceeded $700 million. As a result, we became a “large accelerated filer” and no longer qualify as a “smaller reporting company.” However, we are not required to reflect the change in our “smaller reporting company” status, or comply with the associated increased disclosure obligations, until our quarterly report for the three-month period ended March 31, 2024. We may continue to take advantage of certain reduced disclosures available to smaller reporting companies through the filing of this Annual Report on Form 10-K for the year ending December 31, 2023.

Item 7A. Quantitative and Qualitative Disclosures about Market Risks

Foreign Currency Translation

Our operations include activities in countries outside the U.S. As a result, our financial results are impacted by factors such as changes in foreign currency exchange rates or weak economic conditions in the foreign markets where we operate. Our monetary exposures on our consolidated balance sheets were immaterial to our financial position as of December 31, 2023 and 2022.

We do not engage in any hedging activities against changes in foreign currency exchange rates.

Interest Rate Risk

As of December 31, 2023, we invest our excess cash balances in marketable securities of highly rated financial institutions and investment-grade debt instruments. We seek to diversify our investments and limit the amount of investment concentrations for individual institutions, maturities and investment types. Most of our interest-bearing securities are subject to interest rate risk and could decline in value if interest rates fluctuate. Based on the type of securities we hold, we do not believe a change in interest rates would have a material impact on our financial statements. If interest rates were to increase or decrease by 1.00%, the fair value of our investment portfolio would (decrease) increase by approximately $(0.3) million and $0.3 million, respectively. For further discussion of our investments in marketable securities, refer to Note 3, "Marketable Securities," of the Notes to the Consolidated Financial Statements appearing elsewhere in this Annual Report on Form 10-K.

We do not engage in any hedging activities against changes in interest rates.

Credit Risk

Financial instruments that potentially expose the Company to concentrations of credit risk consist of cash, cash equivalents, and short-term debt securities. The Company maintains a portion of its cash deposits in government insured institutions in excess of government insured limits. The Company deposits its cash in financial institutions that it believes have high credit quality and has not experienced any losses on such accounts. The Company's cash management policy permits investments in U.S. federal government and federal agency securities, corporate bonds or commercial paper, supranational and sovereign obligations, certain qualifying money market mutual funds, certain repurchase agreements, and places restrictions on credit ratings, maturities, and concentration by type and issuer. The Company is exposed to credit risk in the event of a default by the financial institutions holding its cash in excess of government insured limits and in the event of default by corporations and governments in which it holds investments in cash equivalents and short-term debt securities, to the extent recorded on the consolidated balance sheet.

We have not experienced any credit losses or recorded any allowance for credit losses related to our cash, cash equivalents, and short-term debt securities.

ITEM 8. FINANCIAL STATEMENTS AND SUPPLEMENTARY DATA

The consolidated financial statements and supplementary data of the Company required in this item are set forth beginning on page F-1.

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

None.

ITEM 9A. CONTROLS AND PROCEDURES

Evaluation of Disclosure Controls and Procedures

The term "disclosure controls and procedures," as defined in Rules 13a-15(e) and 15d-15(e) under the Securities Exchange Act of 1934, as amended (the "Exchange Act"), refers to controls and procedures 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 rules and forms. Disclosure controls and procedures include, without limitation, controls and procedures designed to ensure that such information is accumulated and communicated to a company's management, including its principal executive and principal financial officers, as appropriate to allow timely decisions regarding required disclosure.

In designing and evaluating our disclosure controls and procedures, management recognizes that disclosure controls and procedures, no matter how well conceived and operated, can provide only reasonable, not absolute, assurance that the objectives of the disclosure controls and procedures are met. Additionally, in designing disclosure controls and procedures, our management necessarily was required to apply its judgment in evaluating the cost-benefit relationship of possible disclosure controls and procedures. The design of any system of controls also is based in part upon certain assumptions about the likelihood of future events, and there can be no assurance that any design will succeed in achieving its stated goals under all potential future conditions; over time, controls may become inadequate because of changes in conditions, or the degree of compliance with policies or procedures may deteriorate. Because of the inherent limitations in a control system, misstatements due to error or fraud may occur and not be detected.

Based on the evaluation of our disclosure controls and procedures, our Chief Executive Officer and Chief Financial Officer have concluded that, as of December

31, 2023, our disclosure controls and procedures were effective at the reasonable assurance level.

Management's Report on Internal Control over Financial Reporting

Management of the Company is responsible for establishing and maintaining adequate internal control over financial reporting as defined in Rules 13a-15(f) and 15d-15(f) under the Exchange Act. Our internal control over financial reporting is a process designed 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. Because of its inherent limitations, internal control over financial reporting may not prevent or detect misstatements. Also, projections of any evaluation of effectiveness to future periods are subject to the risk that controls may become inadequate because of changes in conditions, or that the degree of compliance with the policies or procedures may deteriorate.

Management conducted an evaluation of the effectiveness of our internal control over financial reporting as of December 31, 2023 based on the framework in Internal Control-Integrated Framework (2013) issued by the Committee of Sponsoring Organizations of the Treadway Commission ("COSO"). Based on that evaluation, management has concluded that the Company's internal control over financial reporting was effective as of December 31, 2023.

Report of Independent Registered Public Accounting Firm

The effectiveness of our internal control over financial reporting as of December 31, 2023 has been audited by Ernst & Young LLP., an independent registered public accounting firm, as stated in their report, which is included below.

Report of Independent Registered Public Accounting Firm

To the Shareholders and the Board of Directors of Biohaven Ltd.

Opinion on Internal Control Over Financial Reporting

We have audited Biohaven Ltd.'s internal control over financial reporting as of December 31, 2023, based on criteria established in Internal Control—Integrated Framework issued by the Committee of Sponsoring Organizations of the Treadway Commission (2013 framework) (the COSO criteria). In our opinion, Biohaven Ltd. (the Company) maintained, in all material respects, effective internal control over financial reporting as of December 31, 2023, based on the COSO criteria.

We also have audited, in accordance with the standards of the Public Company Accounting Oversight Board (United States) (PCAOB), the consolidated balance sheets of the Company as of December 31, 2023 and 2022, the related consolidated statements of operations, comprehensive loss, shareholders' equity and cash flows for each of the three years in the period ended December 31, 2023, and the related notes and our report dated February 29, 2024 expressed an unqualified opinion thereon.

Basis for Opinion

The Company's management is responsible for maintaining effective internal control over financial reporting and for its assessment of the effectiveness of internal control over financial reporting included in the accompanying Management's Report on Internal Control over Financial Reporting. Our responsibility is to express an opinion on the Company's internal control over financial reporting based on our audit. We are a public accounting firm registered with the PCAOB and are required to be independent with respect to the Company in accordance with the U.S. federal securities laws and the applicable rules and regulations of the Securities and Exchange Commission and the PCAOB.

We conducted our audit in accordance with the standards of the PCAOB. Those standards require that we plan and perform the audit to obtain reasonable assurance about whether effective internal control over financial reporting was maintained in all material respects.

Our audit included obtaining an understanding of internal control over financial reporting, assessing the risk that a material weakness exists, testing and evaluating the design and operating effectiveness of internal control based on the assessed risk, and performing such other procedures as we considered necessary in the circumstances. We believe that our audit provides a reasonable basis for our opinion.

Definition and Limitations of Internal Control Over Financial Reporting

A company's internal control over financial reporting is a process designed 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. A company's internal control over financial reporting includes those policies and procedures that (1) pertain to the maintenance of records that, in reasonable detail, accurately and fairly reflect the transactions and dispositions of the assets of the company; (2) provide reasonable assurance that transactions are recorded as necessary to permit preparation of financial statements in accordance with generally accepted accounting principles, and that receipts and expenditures of the company are being made only in accordance with authorizations of management and directors of the company; and (3) provide reasonable assurance regarding prevention or timely detection of unauthorized acquisition, use, or disposition of the company's assets that could have a material effect on the financial statements.

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

/s/ Ernst & Young LLP

Hartford, Connecticut

February 29, 2024

Changes in Internal Controls over Financial Reporting

There have been no changes in our internal control over financial reporting (as defined in Rules 13a-15(f) and 15d-15(f) under the Exchange Act) that occurred during the three months ended December 31, 2023 that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

ITEM 9B. OTHER INFORMATION

During the quarter ended December 31, 2023, none of our directors or officers adopted or terminated a "Rule 10b5-1 trading arrangement" or "non-Rule 10b5-1 trading arrangement," as each term is defined in Item 408 of Regulation S-K.

ITEM 9C. DISCLOSURE REGARDING FOREIGN JURISDICTIONS THAT PREVENT INSPECTIONS

Not applicable.

PART III

We will file a definitive Proxy Statement for our 2024 Annual Meeting of Shareholders (the "2024 Proxy Statement") with the SEC, pursuant to Regulation 14A, not later than 120 days after the end of our fiscal year. Accordingly, certain information required by Part III has been omitted under General Instruction G(3) to Form 10-K. Only those sections of the 2024 Proxy Statement that specifically address the items set forth herein are incorporated by reference.

Item 10. Directors, Executive Officers and Corporate Governance

The information required by this Item is incorporated herein by reference to the information that will be contained in our 2024 Proxy Statement under the captions "Information Regarding the Board of Directors and Corporate Governance," "Election of Directors," "Executive Officers" and "Delinquent Section 16(a) Reports."

Item 11. Executive Compensation

The information required by this Item is incorporated herein by reference to the information that will be contained in the 2024 Proxy Statement under the captions "Executive Compensation" and "Director Compensation."

Item 12. Security Ownership of Certain Beneficial Owners and Management and Related Shareholder Matters

The information required by this Item is incorporated herein by reference to the information that will be contained in the 2024 Proxy Statement under the captions "Security Ownership of Certain Beneficial Owners and Management" and "Securities Authorized for Issuance under Equity Compensation Plans."

Item 13. Certain Relationships and Related Transactions, and Director Independence

The information required by this Item is incorporated herein by reference to the information that will be contained in the 2024 Proxy Statement under the captions "Transactions with Related Persons" and "Independence of the Board of Directors."

Item 14. Principal Accountant Fees and Services

The information required by this Item is incorporated herein by reference to the information that will be contained in the 2024 Proxy Statement under the caption "Ratification of Selection of Independent Auditors."

PART IV

Item 15. Exhibit and Financial Statement Schedules

a. The following documents are filed as part of this report:

(1) Financial Statements:

The financial statements required by this item are submitted in a separate section beginning on page F-1 of this report.

(2) Financial Statement Schedules:

All other financial statement schedules have been omitted because they are not applicable, not required or the information required is shown in the consolidated financial statements or the notes thereto.

(3) Exhibits.

Exhibit Number		Description of Document
2.1		Separation and Distribution Agreement, dated as of May 9, 2022, by and among Pfizer Inc., Bulldog (BVI) Ltd. and Biohaven Pharmaceutical Holding Company Ltd. (incorporated by reference to Exhibit 2.1 to the Company's Form 10 (File No. 001-41477) filed on August 10, 2022).
2.2		Agreement and Plan of Merger, dated as of May 9, 2022, by and among Pfizer Inc., Bulldog (BVI) Ltd. and Biohaven Pharmaceutical Holding Company Ltd. (incorporated by reference to Exhibit 2.2 to the Company's Form 10 (File No. 001-41477) filed on August 10, 2022).
2.3	#	Membership Interest Purchase Agreement, dated as of February 24, 2022, by and among Biohaven Therapeutics LTD., Knopp Biosciences LLC, Channel Biosciences, LLC and Biohaven Pharmaceutical Holding Company Ltd., solely for the purpose of Section 9.14 (incorporated by reference to Exhibit 2.3 to the Company's Form 10 (File No. 001-41477) filed on August 10, 2022).
3.1		Amended & Restated Memorandum and Articles of Association of Biohaven Ltd. (incorporated by reference to Exhibit 3.1 to the Company's Current Report on Form 8-K (File No. 001-41477) filed on October 3, 2022).
10.1	#	Amended and Restated Agreement, by and between the Registrant and Yale University, dated as of May 6, 2019 (incorporated by reference to Exhibit 10.1 to the Company's Form 8-K (File No. 001-41477) filed on October 3, 2022).
10.2	#	License Agreement, by and between the Registrant and AstraZeneca AB, dated as of September 4, 2018 (incorporated by reference to Exhibit 10.2 to the Company's Form 10 (File No. 001-41477) filed on August 10, 2022).
10.3	#	License Agreement between Biohaven Therapeutics LTD. and Bristol-Myers Squibb Company, dated as of December 23, 2021 (incorporated by reference to Exhibit 10.3 to the Company's Form 10 (File No. 001-41477) filed on August 10, 2022).
10.4	#	ALS Biopharma Agreement, by and among the registrant, ALS Biopharma, LLC and Fox Chase Chemical Diversity Center Inc., dated as of August 10, 2015 (incorporated by reference to Exhibit 10.4 to the Company's Form 10 (File No. 001-41477) filed on August 10, 2022).
10.5		Amendment and Assignment, by and among the Registrant, ALS Biopharma, LLC, Fox Chase Chemical Diversity Center and Biohaven Therapeutics Ltd, dated as of May 29, 2019 (incorporated by reference to Exhibit 10.5 to the Company's Form 10 (File No. 001-41477) filed on August 10, 2022).
10.6	+	Employment Agreement dated May 9, 2017 by and between Biohaven Pharmaceuticals, Inc. and Vlad Coric (incorporated by reference to Exhibit 10.6 to the Company's Form 10 (File No. 001-41477) filed on August 10, 2022).
10.7	+	Employment Agreement, dated December 8, 2021, between Biohaven Pharmaceuticals, Inc. and Matthew Buten (incorporated by reference to Exhibit 10.7 to the Company's Form 10 (File No. 001-41477) filed on August 10, 2022).
10.8	+	Employment Agreement dated February 1, 2014 by and between Biohaven Pharmaceuticals, Inc. and Kimberly A. Gentile (incorporated by reference to Exhibit 10.9 to the Company's Form 10 (File No. 001-41477) filed on August 10, 2022).
10.9	+	Employment Agreement, dated March 29, 2016, between Biohaven Pharmaceutical Holding Company Ltd. and John Tilton (incorporated by reference to Exhibit 10.10 to the Company's Form 10 (File No. 001-41477) filed on August 10, 2022).
10.10	+	2022 Equity Incentive Plan (incorporated by reference to Exhibit 4.2 to Company's Registration Statement on Form S-8 filed on October 11, 2022).
10.11	+	Form of Restricted Share Unit Grant Notice and Restricted Share Unit Award Agreement under 2022 Equity Incentive Plan (incorporated by reference to Exhibit 10.12 to the Company's Form 10 (File No. 001-41477) filed on September 7, 2022).
10.12	+	Form of Share Option Grant Notice and Share Option Agreement under 2022 Equity Incentive Plan (incorporated by reference to Exhibit 10.13 to the Company's Form 10 (File No. 001-41477) filed on September 7, 2022).
10.13	+	Legacy Equity Award Settlement Plan (incorporated by reference to Exhibit 4.4 to Company's Registration Statement on Form S-8 filed on October 11, 2022).

10.14	+	2022 Employee Share Purchase Plan (incorporated by reference to Exhibit 4.3 to Company's Registration Statement on Form S-8 filed on October 11, 2022).
10.15	+	Form of Employment Agreement by and between Biohaven Ltd. and Vladimir Coric Form of Employment Agreement by and between Biohave Ltd. and Matthew Buten (incorporated by reference to Exhibit 10.16 to the Company's Form 10 (File No. 001-41477) filed on September 7, 2022).
10.16	+	Form of Employment Agreement by and between Biohaven Ltd. and Matthew Buten (incorporated by reference to Exhibit 10.17 to the Company's Form 10 (File No. 001-41477) filed on September 7, 2022).
10.17	+	Amended and Restated Offer of Employment, by and between Biohaven Pharmaceuticals, Inc. and Bruce Car (Incorporated by reference to Exhibit 10.18 to the Company's Form 10-K (File No. 001-41477) filed on March 23, 2023).
10.18	#	Development and License Agreement, dated as of March 21, 2023, by and between Hangzhou Highlightll Pharmaceutical Co. Ltd. and Biohaven Therapeutics LTD. (incorporated by reference to Exhibit 10.1 to the Company's Form 10-Q (File No. 001-41477) filed on May 12, 2023).
10.19	#	Amendment to Development and License Agreement dated March 21, 2023, dated as of April 14, 2023, by and between Hangzhou Highlightl Pharmaceutical Co. Ltd. and Biohaven Therapeutics LTD. (incorporated by reference to Exhibit 10.2 to the Company's Form 10-Q (File No. 001-41477) filed on May 12, 2023).
10.20	+	Form of Nonstatutory Share Option Grant Notice (Early Exercise) and Share Option Agreement under the 2022 Equity Incentive Plan (incorporated by reference to Exhibit 10.3 to the Company's Form 10-Q (File No. 001-41477) filed on May 12, 2023).
10.21	+	Form of Amendment to Share Option Grant Notice and Option Agreement under the 2022 Equity Incentive Plan(incorporated by reference to Exhibit 10.4 to the Company's Form 10-Q (File No. 001-41477) filed on May 12, 2023).
10.22	#	Second Amendment to Development and License Agreement dated March 21, 2023, dated as of December 10, 2023, by and between Hangzhou Highlightll Pharmaceutical Co. Ltd. and Biohaven Therapeutics LTD.
21.1		Subsidiaries of the Registrant.
23.1		Consent of Ernst & Young LLP.
24.1		Power of Attorney (contained on signature page hereto).
31.1		Certification of Principal Executive Officer under Section 302 of the Sarbanes-Oxley Act.
31.2		Certification of Principal Financial Officer under Section 302 of the Sarbanes-Oxley Act.
32.1	*	Certifications of Principal Executive Officer and Principal Financial Officer under Section 906 of the Sarbanes-Oxley Act.
97		Incentive Compensation Recovery Policy.
101		The following materials from the Registrant's Annual Report on Form 10-K for the year ended December 31, 2023 formatted in Inline XBRL (Extensible Business Reporting Language): (i) the Consolidated Balance Sheets, (ii) the Consolidated Statements of Operations, (iii) the Consolidated Statements of Comprehensive Loss, (iv) the Consolidated Statements of Shareholders' Equity, (v) the Consolidated Statements of Cash Flows, (vi) Notes to Consolidated Financial Statements, and (vi) Cover Page, tagged as blocks of text.
104		The cover page from the Registrant's Annual Report on Form 10-K for the fiscal year ended December 31, 2023, formatted in Inline XBRL (included as Exhibit 101).

Portions of this exhibit (indicated by asterisks) have been omitted as such information is (i) not material and (ii) would likely cause competitive harm to the Registrant if publicly disclosed.

+ Indicates management contract or compensatory plan.

* These certifications are being furnished solely to accompany this Annual Report pursuant to 18 U.S.C. Section 1350, and are not being filed for purposes of Section 18 of the Securities Exchange Act of 1934, as amended, and are not to be incorporated by reference into any filing of the Registrant, whether made before or after the date hereof, regardless of any general incorporation language in such filing.

Item 16. Form 10-K Summary

Not applicable.

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.

BIOHAVEN LTD.

Dated: February 29, 2024

By: /s/ VLAD CORIC, M.D.

Vlad Coric, M.D.

Chief Executive Officer

(On behalf of the Registrant and as the Principal Executive Officer)

By: /s/ MATTHEW BUTEN

Matthew Buten

Chief Financial Officer

(Principal Financial Officer)

KNOW ALL PERSONS BY THESE PRESENTS, that each person whose signature appears below constitutes and appoints Vlad Coric as his or her true and lawful attorney-in-fact and agent, with full power of substitution and resubstitution, for him or her and in his or her name, place and stead, in any and all capacities, to sign this Annual Report on Form 10-K of Biohaven Ltd., and any or all amendments thereto, and to file the same, with all exhibits thereto, and other documents in connection therewith, with the Securities and Exchange Commission, granting unto said attorney-in-fact and agent full power and authority to do and perform each and every act and thing requisite or necessary to be done in and about the premises hereby ratifying and confirming all that said attorney-in-fact and agent, or his substitute or substitutes, may lawfully do or cause to be done by virtue hereof.

Pursuant to the requirements of the Securities Exchange Act of 1934, as amended, this report has been signed below by the following persons on behalf of the registrant and in the capacities and on the dates indicated.

Signature	Title	Date
/s/ VLAD CORIC, M.D. Vlad Coric, M.D.	Chief Executive Officer and Director (Principal Executive Officer)	February 29, 2024
/s/ MATTHEW BUTEN Matthew Buten	Chief Financial Officer (Principal Financial Officer)	February 29, 2024
/s/ GEORGE C. CLARK George C. Clark	Chief Accounting Officer (Principal Accounting Officer)	February 29, 2024
/s/ GREGORY H. BAILEY, M.D. Gregory H. Bailey, M.D.	Director	February 29, 2024
/s/ JOHN W. CHILDS John W. Childs	Director	February 29, 2024
/s/ JULIA P. GREGORY Julia P. Gregory	Director	February 29, 2024
/s/ MICHAEL HEFFERNAN Michael Heffernan	Director	February 29, 2024
/s/ ROBERT J. HUGIN Robert J. Hugin	Director	February 29, 2024
/s/ KISHEN MEHTA Kishen Mehta	Director	February 29, 2024
/s/ IRINA ANTONIJEVIC Irina Antonijevic	Director	February 29, 2024

Biohaven Ltd.

Financial Statements

For the Years Ended December 31, 2023, 2022 and 2021

Report of Independent Registered Public Accounting Firm

To the Shareholders and the Board of Directors of Biohaven Ltd.

Opinion on the Financial Statements

We have audited the accompanying consolidated balance sheets of Biohaven Ltd. (the Company) as of December 31, 2023 and 2022, the related consolidated statements of operations, comprehensive loss, shareholders' equity and cash flows for each of the three years in the period ended December 31, 2023, and the related notes (collectively referred to as the “consolidated financial statements”). In our opinion, the consolidated financial statements present fairly, in all material respects, the financial position of the Company at December 31, 2023 and 2022, and the results of its operations and its cash flows for each of the three years in the period ended December 31, 2023, in conformity with U.S. generally accepted accounting principles.

We also have audited, in accordance with the standards of the Public Company Accounting Oversight Board (United States) (PCAOB), the Company's internal control over financial reporting as of December 31, 2023, based on criteria established in Internal Control-Integrated Framework issued by the Committee of Sponsoring Organizations of the Treadway Commission (2013 framework), and our report dated February 29, 2024 expressed an unqualified opinion thereon.

Basis for Opinion

These financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on the Company’s financial statements based on our audits. We are a public accounting firm registered with the PCAOB and are required to be independent with respect to the Company in accordance with the U.S. federal securities laws and the applicable rules and regulations of the Securities and Exchange Commission and the PCAOB.

We conducted our audits in accordance with the standards of the PCAOB. Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the financial statements are free of material misstatement, whether due to error or fraud. Our audits included performing procedures to assess the risks of material misstatement of the financial statements, whether due to error or fraud, and performing procedures that respond to those risks. Such procedures included examining, on a test basis, evidence regarding the amounts and disclosures in the financial statements. Our audits also included evaluating the accounting principles used and significant estimates made by management, as well as evaluating the overall presentation of the financial statements. We believe that our audits provide a reasonable basis for our opinion.

Critical Audit Matter

The critical audit matter communicated below is a matter arising from the current period audit of the financial statements that was communicated or required to be communicated to the audit committee and that: (1) relates to accounts or disclosures that are material to the financial statements and (2) involved our especially challenging, subjective or complex judgments. The communication of the critical audit matter does not alter in any way our opinion on the consolidated financial statements, taken as a whole, and we are not, by communicating the critical audit matter below, providing a separate opinion on the critical audit matter or on the accounts or disclosures to which it relates.

Accounting for Research and Development Costs and Related Prepaid and Accrued Costs

Description of the Matter	As discussed in Note 2 to the consolidated financial statements, the Company recognizes estimated ongoing research costs based on management's analysis of the progress of the studies or clinical trials, including the phase or completion of events, invoices received and contracted costs. During 2023, the Company incurred $373.3 million in research and development expense. As of December 31, 2023, the Company recorded prepaid expenses of $35.2 million which included prepayment for certain clinical trial costs and recorded $29.5 million for accrued clinical trial costs. Auditing management's accounting for research and development costs was challenging due to the estimation required by management to determine the cost incurred for the services rendered on or prior to the balance sheet date for preclinical development activities, preclinical and clinical studies and drug substance and drug product formulation for preclinical and clinical trial material. The Company has contracts with multiple clinical research organizations ("CROs") that conduct and manage studies on its behalf, as well as contract manufacturing organizations ("CMOs") that perform manufacturing activities. The financial terms of these agreements are subject to negotiation, vary from contract to contract and may result in uneven payment flows during the period.
How We Addressed the Matter in Our Audit	We obtained an understanding, evaluated the design, and tested the operating effectiveness of relevant controls over the Company's accounting for research and development costs, including controls over the determination of estimates of the progress completed, expected milestones or expected service period and the completeness and accuracy of the data used in determining prepaid and accrued research and development expenses estimate. To evaluate the completeness, measurement and valuation of the research and development costs and related prepaid and accrued costs, our audit procedures included, among others, reading certain contracts with CROs and CMOs to evaluate the financial and other relevant contractual terms, testing the accuracy and completeness of the underlying data used by management to estimate the recorded balances, and testing the mathematical accuracy of the calculation of the accrued or prepaid balances. We also evaluated management's estimates of progress, expected milestones, or expected service period for a sample of clinical trials and manufacturing efforts by making direct inquiries of the Company's research and development personnel who oversee the research and development program. Further, we requested direct confirmation of total contract value, costs incurred, amounts invoiced, invoices unpaid and expected service period as of December 31, 2023, from a sample of CROs and CMOs. To evaluate the completeness of the accrued research and development costs we also examined invoices received from a sample of vendors and a sample of cash disbursements made to third-party service providers subsequent to December 31, 2023, to the extent such invoices were received, or payments were made prior to the date that the financial statements were issued.

/s/ Ernst & Young LLP

We have served as the Company's auditor since 2022.

Hartford, Connecticut

February 29, 2024

BIOHAVEN LTD.

CONSOLIDATED BALANCE SHEETS

(Amounts in thousands, except share amounts)

	December 31, 2023	December 31, 2022
Assets		
Current assets:		
Cash and cash equivalents	$ 248,402	$ 204,877
Marketable securities	133,417	260,464
Prepaid expenses	35,242	20,945
Income tax receivable	13,252	46,139
Restricted cash held on behalf of Former Parent	—	35,212
Other current assets	12,133	19,331
Total current assets	442,446	586,968
Property and equipment, net	17,191	17,512
Intangible assets	18,400	18,400
Goodwill	1,390	1,390
Other non-current assets	33,785	37,513
Total assets	$ 513,212	$ 661,783
Liabilities and Shareholders' Equity		
Current liabilities:		
Accounts payable	$ 15,577	$ 10,703
Due to Former Parent	—	35,212
Accrued expenses and other current liabilities	39,846	44,106
Total current liabilities	55,423	90,021
Long-term operating lease liability	27,569	30,581
Other non-current liabilities	2,245	2,410
Total liabilities	85,237	123,012
Commitments and contingencies (Note 12)		
Shareholders' Equity:		
Preferred shares, no par value; 10,000,000 shares authorized, no shares issued and outstanding as of December 31, 2023 and 2022	—	—
Common shares, no par value; 200,000,000 shares authorized as of December 31, 2023 and 2022; 81,115,723 and 68,190,479 shares issued and outstanding as of December 31, 2023 and 2022, respectively	887,528	615,742
Additional paid-in capital	39,804	13,869
Accumulated deficit	(499,292)	(91,124)
Accumulated other comprehensive (loss) income	(65)	284
Total shareholders' equity	427,975	538,771
Total liabilities and shareholders' equity	$ 513,212	$ 661,783

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

BIOHAVEN LTD.

CONSOLIDATED STATEMENTS OF OPERATIONS

(Amounts in thousands, except share and per share amounts)

	Year Ended December 31,		
	2023	2022	2021
Operating expenses:			
Research and development	$ 373,281	$ 437,072	$ 181,486
General and administrative	62,770	130,860	37,414
Total operating expenses	436,051	567,932	218,900
Loss from operations	(436,051)	(567,932)	(218,900)
Other income (expense):			
Gain from equity method investment	—	—	5,261
Other income (expense), net	26,500	(1,909)	1,209
Total other income (expense), net	26,500	(1,909)	6,470
Loss before (benefit) provision for income taxes	(409,551)	(569,841)	(212,430)
(Benefit) provision for income taxes	(1,383)	438	1,366
Net loss	$ (408,168)	$ (570,279)	$ (213,796)
Net loss per share — basic and diluted	$ (5.73)	$ (12.75)	$ (5.43)
Weighted average common shares outstanding—basic and diluted	71,200,527	44,741,316	39,375,944

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

BIOHAVEN LTD.

CONSOLIDATED STATEMENTS OF COMPREHENSIVE LOSS

(Amounts in thousands)

	Year Ended December 31,		
	2023	2022	2021
Net loss	$ (408,168)	$ (570,279)	$ (213,796)
Other comprehensive (loss) income, net of tax:			
Foreign currency translation adjustments	(497)	429	—
Net unrealized gain (loss) related to available-for-sale debt securities	148	(145)	—
Other comprehensive (loss) income	(349)	284	—
Comprehensive loss	$ (408,517)	$ (569,995)	$ (213,796)

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

BIOHAVEN LTD.

CONSOLIDATED STATEMENTS OF SHAREHOLDERS' EQUITY

(Amounts in thousands, except share amounts)

	Common Shares						
	Shares	Amount	Net Investment from Former Parent	Additional Paid-in Capital	Accumulated Deficit	Accumulated Other Comprehensive (Loss) Income	Total Shareholders' Equity
Balances as of December 31, 2020	—	$ —	$ 16,781	$ —	$ —	$ —	$ 16,781
Net loss	—	—	(213,796)	—	—	—	(213,796)
Net transfers from Former Parent			231,706				231,706
Balances as of December 31, 2021	—	—	34,691	—	—	—	34,691
Net loss	—	—	(479,155)	—	(91,124)	—	(570,279)
Net transfers from Former Parent, including separation related adjustments	—	—	776,630	—	—	—	776,630
Issuance of common shares in connection with the Separation and reclassification of Net investment from Former Parent	39,375,944	332,166	(332,166)	—	—	—	—
Issuance of common shares, net of offering costs	28,750,000	282,804	—	—	—	—	282,804
Issuance of common shares under 2022 Equity Incentive Plan	64,535	772	—	(321)	—	—	451
Non-cash share-based compensation expense	—	—	—	14,189	—	—	14,189
Other comprehensive income	—	—	—	—	—	284	284
Balances as of December 31, 2022	68,190,479	615,742	—	13,869	(91,124)	284	538,771
Net loss	—	—	—	—	(408,168)	—	(408,168)
Issuance of common shares, net of offering costs	11,761,363	242,425	—	—	—	—	242,425
Issuance of common shares as payment for license agreement	721,136	21,814	—	—	—	—	21,814
Issuance of common shares under 2022 Equity Incentive Plan and employee share purchase plan	442,745	7,547	—	(2,852)	—	—	4,695
Non-cash share-based compensation expense	—	—	—	28,787	—	—	28,787
Other comprehensive loss	—	—	—	—	—	(349)	(349)
Balances as of December 31, 2023	81,115,723	$ 887,528	$ —	$ 39,804	$ (499,292)	$ (65)	$ 427,975

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

BIOHAVEN LTD.

CONSOLIDATED STATEMENTS OF CASH FLOWS

(Amounts in thousands)

	Year ended December 31,		
	2023	2022	2021
Cash flows from operating activities:			
Net loss	$ (408,168)	$ (570,279)	$ (213,796)
Adjustments to reconcile net loss to net cash used in operating activities:			
Non-cash share-based compensation	28,787	193,556	65,639
Acquisition of IPR&D asset	—	93,747	—
Impairment of Artizan preferred stock	—	10,000	—
Depreciation and amortization	6,912	1,386	1,393
Issuance of Former Parent common shares as payment for license and consulting agreements	—	1,779	7,929
Issuance of common shares as payment for license agreement	21,814	—	—
Gain from equity method investment	—	—	(5,261)
Other non-cash items	(5,683)	726	(3,408)
Changes in operating assets and liabilities:			
Prepaid expenses, income tax receivable, and other current and non-current assets	27,176	(41,336)	(9,182)
Accounts payable	4,874	5,928	1,025
Accrued expenses and other current and non-current liabilities	(7,437)	6,804	9,821
Net cash used in operating activities	$ (331,725)	$ (297,689)	$ (145,840)
Cash flows from investing activities:			
Proceeds from maturities of marketable securities	322,079	—	—
Proceeds from sales of marketable securities	4,920	—	—
Purchases of marketable securities	(194,121)	(259,716)	—
Purchases of property and equipment	(3,048)	(6,074)	(938)
Payment for IPR&D asset acquisition	—	(35,000)	—
Cash acquired in business acquisition	—	—	1,882
Purchase of Artizan preferred stock	—	(4,000)	—
Net cash provided by (used in) investing activities	$ 129,830	$ (304,790)	$ 944
Cash flows from financing activities:			
Net transfers from Former Parent	—	449,130	138,052
Change in restricted cash due to Former Parent	(35,212)	35,212	—
Proceeds from issuance of common shares	243,225	283,804	—
Payments of issuance costs	(800)	(1,000)	—
Proceeds from issuance of common shares under 2022 Equity Incentive Plan and employee share purchase plan	4,695	451	395
Net cash provided by financing activities	$ 211,908	$ 767,597	$ 138,447
Effect of exchange rates on cash, cash equivalents and restricted cash	(497)	429	—
Net increase (decrease) in cash, cash equivalents and restricted cash	9,516	165,547	(6,449)
Cash, cash equivalents and restricted cash at beginning of period	242,604	77,057	83,506
Cash, cash equivalents and restricted cash at end of period	$ 252,120	$ 242,604	$ 77,057
Supplemental disclosure of cash flow information:			
Cash paid for interest	$ —	$ —	$ 107
Cash (received) paid for income taxes	$ (33,300)	$ 38,165	$ 16,594

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

BIOHAVEN LTD.

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS

(Amounts in thousands, except share and per share amounts)

1. Nature of the Business and Basis of Presentation

Biohaven Ltd. (“we,” “us," "our," "Biohaven" or the “Company”) was incorporated in Tortola, British Virgin Islands in May 2022. Biohaven is a biopharmaceutical company focused on the discovery, development, and commercialization of life-changing treatments in key therapeutic areas, including immunology, neuroscience, and oncology. The Company is advancing its innovative therapeutic portfolio of therapeutics, leveraging its proven drug development experience and multiple, proprietary drug development platforms. Biohaven's extensive clinical and preclinical programs include Kv7 ion channel modulation for epilepsy and mood disorders; extracellular protein degradation for immunological diseases; Transient Receptor Potential Melastatin 3 ("TRPM3") antagonism for migraine and neuropathic pain; Tyrosine Kinase 2/Janus Kinase 1 ("TYK2/JAK1") inhibition for neuroinflammatory disorders; glutamate modulation for obsessive-compulsive disorder (“OCD”); and spinocerebellar ataxia ("SCA"); myostatin inhibition for neuromuscular and metabolic diseases, including spinal muscular atrophy ("SMA") and obesity; and antibody recruiting, bispecific molecules and antibody drug conjugates ("ADCs") for cancer.

The Company is subject to risks and uncertainties common to companies in the biotechnology industry, including, but not limited to, development by competitors of new technological innovations, dependence on key personnel, protection of proprietary technology, compliance with government regulations and the ability to secure additional capital to fund operations. Product candidates currently under development will require significant additional research and development efforts, including preclinical and clinical testing and regulatory approval, prior to commercialization. These efforts may require additional capital, additional personnel and infrastructure, and further regulatory and other capabilities. Even if the Company’s product development efforts are successful, it is uncertain when, if ever, the Company will realize significant revenue from product sales.

Separation from Biohaven Pharmaceutical Holding Company Ltd.

On May 9, 2022, Biohaven Pharmaceutical Holding Company Ltd. (the "Former Parent"), Pfizer Inc. (“Pfizer”) and Bulldog (BVI) Ltd., a wholly owned subsidiary of Pfizer (“Merger Sub”), entered into an Agreement and Plan of Merger (the “Merger Agreement”), which provided for the acquisition by Pfizer of the Former Parent through the merger of Merger Sub with and into the Former Parent (the “Merger”). In connection with the Merger Agreement, the Former Parent and Biohaven entered into a Separation and Distribution Agreement, dated as of May 9, 2022 (the “Distribution Agreement”). In connection with the Distribution Agreement, the Board of Directors of the Former Parent approved and directed the Former Parent’s management to effect the Spin-Off (as defined below) of the business, operations, and activities that are not the calcitonin gene-related peptide ("CGRP") Business (as defined below), including the Kv7 ion channel activators, glutamate modulation, myeloperoxidase ("MPO") inhibition and myostatin inhibition platforms, preclinical product candidates, and certain corporate infrastructure owned by the Former Parent.

To implement the Spin-Off, the Former Parent transferred the related license agreements, intellectual property and corporate infrastructure, including certain non-commercial employee agreements, share based awards and other corporate agreements (the "Business") to Biohaven, through a series of internal restructuring transactions. Descriptions of historical business activities in these Notes to the Consolidated Financial Statements are presented as if these transfers had already occurred, and the Former Parent’s activities related to such assets and liabilities had been performed by the Company.

On October 3, 2022, the Former Parent completed the distribution (the “Distribution”) to holders of its common shares of all of the outstanding common shares of Biohaven and the spin-off of Biohaven from the Former Parent (the “Spin-Off”) described in Biohaven’s Information Statement (the “Information Statement”) attached as Exhibit 99.1 to Biohaven’s Registration Statement on Form 10, as amended (Reg. No. 001-41477), which was declared effective by the Securities and Exchange Commission (the "SEC") on September 22, 2022. Each holder of Former Parent common shares received one common share of Biohaven for every two Former Parent common shares held of record as of the close of business on September 26, 2022. In the Distribution, an aggregate of 35,840,459 Biohaven common shares were issued. The aggregate number of common shares issued in connection with the Distribution did not include 2,611,392 common shares issued in connection with Former Parent stock options that were exercised on October 3, 2022 and 924,093 common shares to be issued in connection with Former Parent restricted stock units that vested on October 3, 2022. See Note 9, "Non-Cash Share-Based Compensation," for discussion of the Legacy Equity Award Settlement Plan. As a result of the Distribution, Biohaven became an independent, publicly traded

1. Nature of the Business and Basis of Presentation (Continued)

company. Collectively, we refer to the Distribution and Spin-Off throughout this Annual Report on Form 10-K as the "Separation."

The Separation generally resulted in (a) the Company directly or indirectly owning, assuming, or retaining certain assets and liabilities of the Former Parent and its subsidiaries related to the Former Parent's pipeline assets and businesses and (b) the Former Parent directly or indirectly owning, assuming, or retaining all other assets and liabilities, including those associated with the Former Parent's platform for the research, development, manufacture and commercialization of calcitonin gene-related receptor antagonists, including rimegepant, zavegepant and the Heptares Therapeutics Limited preclinical CGRP portfolio and related assets (the "CGRP Business").

In connection with the Separation, the Company entered into various agreements relating to transition services, licenses and certain other matters with the Former Parent. For additional information regarding these agreements, see Note 14, "Related Party Transactions."

Basis of Presentation

The financial statements for all periods presented, including the historical results of the Company prior to October 3, 2022, are now referred to as "Consolidated Financial Statements," and have been prepared in accordance with accounting principles generally accepted in the United States of America ("GAAP") and pursuant to the rules and regulations of the SEC.

Periods Prior to the Separation

On October 3, 2022, the Company became a standalone publicly traded company, and its financial statements are now presented on a consolidated basis. Prior to the Separation on October 3, 2022, the Company's historical combined financial statements were prepared on a standalone basis and were derived from the Former Parent's consolidated financial statements and accounting records.

For periods prior to the Separation, the consolidated financial statements present, on a historical basis, the combined assets, liabilities, expenses and cash flows directly attributable to the Business, which have been prepared from the Former Parent's consolidated financial statements and accounting records, and are presented on a stand-alone basis as if the operations had been conducted independently from the Former Parent. The consolidated statements of operations for periods prior to the Separation include all costs directly related to the Business, including costs for facilities, functions and services utilized by the Company. The consolidated statements of operations for periods prior to the Separation also include allocations for various expenses related to the Former Parent's corporate functions, including research and development, human resources, information technology, facilities, tax, shared services, accounting, finance and legal. These expenses were allocated on the basis of direct usage or benefit when specifically identifiable, with the remainder allocated on a proportional cost allocation method primarily based on employee labor hours or direct expenses. Management believes the assumptions underlying the consolidated financial statements for periods prior to the Separation, including the expense methodology and resulting allocation, are reasonable for all periods presented. However, the allocations may not include all of the actual expenses that would have been incurred by the Company and may not reflect its consolidated results of operations, financial position and cash flows had it been a standalone company during the periods presented. It is not practicable to estimate actual costs that would have been incurred had the Company been a standalone company and operated as an unaffiliated entity during the periods presented. Actual costs that might have been incurred had the Company been a standalone company would depend on a number of factors, including the chosen organizational structure, what corporate functions the Company might have performed directly or outsourced and strategic decisions the Company might have made in areas such as executive management, legal and other professional services, and certain corporate overhead functions.

The income tax amounts in the consolidated financial statements for the periods prior to the Separation were calculated on a separate return method and is presented as if the Company's operations were separate taxpayers in the respective jurisdiction. Therefore, tax expense, cash tax payments, and items of current and deferred taxes may not be reflective of the Company's actual tax balances prior to or subsequent to the Distribution.

The consolidated balance sheets for periods prior to the Separation include assets and liabilities that have been determined to be specifically identifiable or otherwise attributable to the Company, including certain assets that were historically held at the corporate level in the Former Parent. All intracompany transactions within the Company have been eliminated. All intercompany transactions between the Company and the Former Parent are considered to be effectively settled in the consolidated financial statements at the time the transactions are recorded. The total net effect of these intercompany transactions considered to be settled is reflected in the consolidated statement of cash flows within financing activities as "Net transfers from Former Parent." See Note 14, "Related Party

1. Nature of the Business and Basis of Presentation (Continued)

Transactions," for additional information regarding related party transactions.

For periods prior to the Separation, the Company's equity balance in these consolidated financial statements represents the excess of total assets over liabilities. Net investment from Former Parent is primarily impacted by contributions from the Former Parent, which are the result of net funding provided by or distributed to the Former Parent. As a result of the Separation, the Company's Net investment from Former Parent balance was reclassified to common shares. The Net investment from Former Parent balance reclassified to common shares during the fourth quarter of 2022 included Separation-related adjustments of $ 27,811 . The adjustments related primarily to differences in the amount of assets and liabilities transferred to the Company upon the Separation and the amount of the transferred assets and liabilities reported in the Company's combined balance sheet as of September 30, 2022.

Going Concern

In accordance with Accounting Standards Codification ("ASC") 205-40, Going Concern, the Company has evaluated whether there are conditions and events, considered in the aggregate, that raise substantial doubt about the Company's ability to continue as a going concern within one year after the date that the consolidated financial statements are issued.

Through February 29, 2024, the Company has funded its operations primarily with funding from the Former Parent, proceeds from the public offerings of its common shares (refer to Note 7, "Shareholders' Equity"), and the cash contribution received from the Former Parent at the Separation (refer to Note 14, "Related Party Transactions.") The Company has incurred recurring losses since its inception and expects to continue to generate operating losses for the foreseeable future.

As of the date of issuance of these consolidated financial statements, the Company expects its existing cash, cash equivalents and marketable securities will be sufficient to fund operating expenses, financial commitments and other cash requirements for at least one year after the issuance date of these financial statements.

To execute its business plans, the Company will require funding to support its continuing operations and pursue its growth strategy. Until such time as the Company can generate significant revenue from product sales or royalties, if ever, it expects to finance its operations through the sale of public or private equity, debt financings or other capital sources, including collaborations with other companies or other strategic transactions. The Company may not be able to obtain financing on acceptable terms, or at all. The terms of any financing may adversely affect the holdings or the rights of the Company's shareholders. If the Company is unable to obtain funding, the Company could be forced to delay, reduce or eliminate some or all of its research and development programs, product portfolio expansion or commercialization efforts, which could adversely affect its business prospects, or the Company may be unable to continue operations.

2. Summary of Significant Accounting Policies

Use of Estimates

The preparation of consolidated financial statements in conformity with GAAP requires management to make estimates and assumptions that affect the reported amounts of assets and liabilities, the disclosure of contingent assets and liabilities at the date of the consolidated financial statements and the reported amounts of expenses during the reporting periods. Significant estimates and assumptions reflected in these consolidated financial statements include, but are not limited to, determining the allocations of costs and expenses from the Former Parent and the accrual for research and development expenses. In addition, management's assessment of the Company's ability to continue as a going concern involves the estimation of the amount and timing of future cash inflows and outflows. Estimates are periodically reviewed in light of changes in circumstances, facts and experience. Changes in estimates are recorded in the period in which they become known. Actual results could differ from those estimates.

Reclassifications

Certain items in the prior period's consolidated financial statements have been reclassified to conform to the current year presentation.

Cash and Cash Equivalents

The Company considers all highly liquid investments with an original maturity of 90 days or less at the date of purchase to be cash equivalents. The Company's cash equivalents are comprised of short-term money market funds and marketable securities that are highly liquid and readily convertible to known amounts of cash.

Restricted Cash

Restricted cash held on behalf of the Former Parent on the consolidated balance sheet represents cash held

(Amounts in thousands, except share and per share amounts)

2. Summary of Significant Accounting Policies (Continued)

by the Company on behalf of the Former Parent related to the execution of the United States Distribution Services Agreement (the “Distribution Services Agreement”). Pursuant to the terms of the Distribution Services Agreement, which was entered into by the Company and the Former Parent in connection with the Separation, the Company continued to serve as the Former Parent’s distributor and agent for the distribution of the pharmaceutical product Nurtec ODT in the United States. The Distribution Services Agreement was terminated during the second quarter of 2023, and, as such, restricted cash held on behalf of the Former Parent on the consolidated balance sheet was $ 0 as of December 31, 2023. Refer to Note 14, "Related Party Transactions," for further information on the agreements entered into by the Company and the Former Parent in connection with the Separation.

Restricted cash included in other current assets primarily includes employee contributions to the Company's employee share purchase plan held for future purchases of the Company's outstanding shares. See Note 9, "Non-Cash Share-Based Compensation," for additional information on the Company's employee share purchase plan.

Restricted cash included in other non-current assets in the consolidated balance sheets represents collateral held by banks for a letter of credit ("LOC") issued in connection with the leased office space in Yardley, Pennsylvania and a LOC issued in connection with the leased office space in Cambridge, Massachusetts. See Note 12, "Commitments and Contingencies," for additional information on the real estate leases.

The following represents a reconciliation of cash and cash equivalents in the consolidated balance sheets to total cash, cash equivalents and restricted cash for the years ended December 31, 2023, 2022 and 2021, respectively, in the consolidated statements of cash flows:

	December 31,		
	2023	2022	2021
Cash and cash equivalents	$ 248,402	$ 204,877	$ 76,057
Restricted cash held on behalf of Former Parent	—	35,212	—
Restricted cash (included in other current assets)	1,318	117	250
Restricted cash (included in other non-current assets)	2,400	2,398	750
Total cash, cash equivalents and restricted cash at the end of the period in the consolidated statement of cash flows	$ 252,120	$ 242,604	$ 77,057

Marketable Securities

We invest our excess cash balances in marketable securities of highly rated financial institutions, which consist of investment-grade debt instruments. We seek to diversify our investments and limit the amount of investment concentrations for individual institutions, maturities and investment types. We classify marketable debt securities as available-for-sale and, accordingly, record such securities at fair value on the consolidated balance sheets. We classify these securities as current assets as these investments are intended to be available to the Company for use in funding current operations.

Unrealized gains and losses on our marketable debt securities that are deemed temporary are included in accumulated other comprehensive income as a separate component of shareholders’ equity. If any adjustment to fair value reflects a significant decline in the value of the security, we evaluate the extent to which the decline is determined to be other-than-temporary and would mark the security to market through a charge to our consolidated statement of operations. Credit losses are identified when we do not expect to receive cash flows sufficient to recover the amortized cost basis of a security. In the event of a credit loss, only the amount associated with the credit loss is recognized in operating results, with the amount of loss relating to other factors recorded in accumulated other comprehensive income.

Acquisitions

Our consolidated financial statements include the operations of acquired businesses after the completion of the acquisitions. We account for acquired businesses using the acquisition method of accounting, which requires, among other things, that assets acquired and liabilities assumed be recognized at their estimated fair values as of the acquisition date and that the fair value of acquired In-Process Research and Development ("IPR&D") be recorded on the balance sheet. Transaction costs are expensed as incurred. Any excess of the consideration transferred over the assigned values of the net assets acquired is recorded as goodwill. Contingent consideration in a business acquisition is included as part of the consideration transferred and is recognized at fair value as of the acquisition date. Fair value of IPR&D and contingent consideration is generally estimated by using a probability-weighted discounted cash flow approach.

Property and Equipment

Property and equipment are recorded at cost and depreciated or amortized using the straight-line method over the estimated useful lives of the respective assets. As of December 31, 2023 and December 31, 2022, the

(Amounts in thousands, except share and per share amounts)

2. Summary of Significant Accounting Policies (Continued)

Company's property and equipment consisted of building and land, office and lab equipment, computer hardware and software, furniture and fixtures, and leasehold improvements.

The fixed assets have the following useful lives:

Building	30 years
Office equipment	3 - 5 years
Computer hardware and software	3 - 5 years
Lab equipment	3 years
Furniture and fixtures	3 years
Leasehold improvements	Shorter of the useful life or remaining lease term

Upon retirement or sale, the cost of assets disposed of and the related accumulated depreciation are removed from the accounts and any resulting gain or loss is included in loss from operations. Expenditures for repairs and maintenance are charged to expense as incurred. Property and equipment are monitored regularly for impairment whenever events or changes in business circumstances indicate that the carrying amount of the assets may not be fully recoverable.

Intangible Assets

Acquired In-Process Research and Development

IPR&D that the Company acquires in conjunction with the acquisition of a business represents the fair value assigned to incomplete research projects which, at the time of acquisition, have not reached technological feasibility. The amounts are capitalized and accounted for as indefinite-lived intangible assets, subject to impairment testing until completion or abandonment of the projects. Upon successful completion of each project, the asset is classified as a definite-lived intangible and the Company will make a determination as to the then-useful life of the intangible asset, generally determined by the period in which the substantial majority of the cash flows are expected to be generated, and begin amortization.

The Company reviews amounts capitalized as acquired IPR&D for impairment annually, as of November 30, and whenever events or changes in circumstances indicate that the carrying value of the assets might not be recoverable. If impairment indicators are present, the Company performs a quantitative test that compares the fair value of the IPR&D intangible asset with its carrying value. If the fair value is less than the carrying amount, an impairment loss is recognized in operating results.

The Company did not record any impairment charges relating to its acquired IPR&D for the years ended December 31, 2023, 2022 or 2021.

If we acquire an asset or group of assets that do not meet the definition of a business under applicable accounting standards, the acquired IPR&D is expensed on its acquisition date, unless it has an alternative future use. Future costs to develop these assets are recorded to research and development expense as they are incurred.

In January 2021, in connection with our acquisition of Kleo Pharmaceuticals, Inc. (“Kleo”), we recorded intangible assets consisting of IPR&D assets of $ 18,400 , which included an oncology therapeutic candidate entering Phase I clinical trials and a Multimodal Antibody Therapy Enhancer ("MATE") conjugation asset in the planning stage for clinical development, and goodwill of $ 1,390 .

Impairment of Long-lived Assets

The Company reviews its long-lived assets for indicators of impairment annually, as of November 30, and whenever events or changes in circumstances indicate that the carrying value of an asset may not be recoverable. If such indicators are present, the Company assesses the recoverability of affected assets by determining whether the carrying value of such assets is less than the sum of the undiscounted future cash flows of the assets. If such assets are found not to be recoverable, the Company measures the amount of such impairment by comparing the carrying value of the assets to the fair value of the assets, with the fair value generally determined based on the present value of the expected future cash flows associated with the assets.

The Company did not record any impairment charges relating to its long-lived assets for the years ended December 31, 2023, 2022 or 2021.

Fair Value Measurements

Certain assets of the Company are carried at fair value under GAAP. Fair value is defined as the exchange price that would be received for an asset or paid to transfer a liability (an exit price) in the principal or most advantageous market for the asset or liability in an orderly transaction between market participants on the measurement date. Valuation techniques used to measure fair value must maximize the use of observable inputs and minimize the use of unobservable inputs. Financial assets and liabilities carried at fair value are to be classified and disclosed in one of the following three levels of the fair value hierarchy, of which the first two

2. Summary of Significant Accounting Policies (Continued)

are considered observable and the last is considered unobservable:

- *Level 1*—Quoted prices in active markets for identical assets or liabilities.

- *Level 2*—Observable inputs (other than Level 1 quoted prices), such as quoted prices in active markets for similar assets or liabilities, quoted prices in markets that are not active for identical or similar assets or liabilities, or other inputs that are observable or can be corroborated by observable market data.

- *Level 3*—Unobservable inputs that are supported by little or no market activity that are significant to determining the fair value of the assets or liabilities, including pricing models, discounted cash flow methodologies and similar techniques.

The carrying values of other current assets, accounts payable, and accrued expenses approximate their fair values due to the short-term nature of these assets and liabilities.

Leases

The Company determines if an arrangement contains a lease at the inception of a contract. Right-of-use assets represent the Company's right to use an underlying asset for the lease term and lease liabilities represent the Company's obligation to make lease payments arising from the lease. Right-of-use assets and lease liabilities are recognized at the commencement date based on the present value of the remaining future minimum lease payments. If the interest rate implicit in the Company's leases is not readily determinable, the Company utilizes an estimate of its incremental borrowing rate based on market sources including interest rates for companies with similar credit quality for agreements of similar duration, determined by class of underlying asset, to discount the lease payments. The right-of-use assets also include lease payments made before commencement and exclude lease incentives. The Company's lease terms may include options to extend or terminate the lease when it is reasonably certain that the Company will exercise that option. Leases with an initial term of 12 months or less are not recorded on the balance sheet. Lease expense for these short-term leases are expensed as incurred over the term of the lease.

Certain real estate leases require additional payments including reimbursement for real estate taxes, common area maintenance and insurance, which are expensed as incurred as variable lease costs. Other real estate leases contain one fixed lease payment that includes real estate taxes, common area maintenance and insurance. These fixed payments are considered part of the lease payment and included in the right-of-use assets and lease liabilities.

For its real estate leases, which are accounted for as operating leases, the Company has elected the practical expedient to include both the lease and non-lease components as a single component. In addition, payments made by the Company for improvements to the underlying asset, if the payment relates to an asset of the lessor, are recorded as prepaid rent within other non-current assets in the consolidated balance sheets prior to lease commencement and on commencement, reclassified to the right-of-use asset. As of December 31, 2023, the Company had restricted cash of $ 2,400 included in other non-current assets and $ 250 included in other current assets in the consolidated financial statements, which represents collateral held by banks for letters of credit issued in connection with the leased office space in Yardley, Pennsylvania and Cambridge, Massachusetts. Restricted cash is generally invested in time deposits and short-term money market funds. See Note 12, "Commitments and Contingencies," for additional information on real estate leases.

Research and Development Costs

Research and development costs are expensed as incurred. Research and development expenses consist of costs incurred in performing research and development activities, including salaries, non-cash share-based compensation and benefits, third-party license fees, rent and operating expenses for leased lab facilities, and external costs of vendors engaged to conduct clinical development activities and clinical trials as well as to manufacture clinical trial materials. Prepayments for goods or services that will be used or rendered for future research and development activities are deferred and capitalized. Such amounts are recognized as an expense as the goods are delivered or the related services are performed, or until it is no longer expected that the goods will be delivered or the services rendered.

The Company has entered into various research and development-related contracts. These agreements are cancellable, and related expenses are recorded as research and development expenses are incurred. The Company records accruals for estimated ongoing research costs. When evaluating the adequacy of the accrued liabilities, the Company analyzes progress of the studies or clinical trials, including the phase or completion of events, invoices received and contracted costs. Certain judgments and estimates are made in determining the accrued balances at the end of any reporting period. Actual results could differ from the Company's estimates. The Company's historical accrual estimates have not been materially different from the actual costs.

2. Summary of Significant Accounting Policies (Continued)

Foreign Currency

The financial statements of our subsidiaries with functional currencies other than the U.S. dollar are translated into U.S. dollars using period-end exchange rates for assets and liabilities, historical exchange rates for shareholders' equity and weighted average exchange rates for operating results. The resulting translation gains and losses are included in accumulated other comprehensive (loss) income, net of tax, in shareholders' equity.

For U.S. dollar functional currency subsidiaries, foreign currency assets and liabilities are remeasured into U.S. dollars at end-of-period exchange rates, except for nonmonetary balance sheet accounts which are remeasured at historical exchange rates. Revenues and expenses are remeasured at average exchange rates in effect during each period, except for those expenses related to the nonmonetary balance sheet amounts which are remeasured at historical exchange rates. Gains or losses from foreign currency remeasurement are included in other income (expense), net in the consolidated statement of operations.

The Company's aggregate foreign currency transaction gains and losses and effects of foreign currency remeasurements were immaterial for the years ended December 31, 2023, 2022 and 2021.

Share-Based Compensation

The Company measures share options and restricted share unit awards granted to employees, non-employees, and directors based on the fair value on the date of the grant and recognizes compensation expense of those awards, over the requisite service period, which is generally the vesting period of the respective award. Forfeitures are accounted for as they occur. Generally, the Company issues share options and restricted share units with only service-based vesting conditions and records the expense for these awards using the straight-line method. The Company also issues share options with performance-based vesting conditions and records expense for these awards when the Company concludes that it is probable that the performance condition will be achieved.

The Company classifies non-cash share-based compensation expense in its consolidated statement of operations in the same manner in which the award recipient's payroll costs are classified or in which the award recipient's service payments are classified.

The fair value of each share option grant is estimated on the date of grant using the Black-Scholes option-pricing model. The Company lacks a sufficient history of company-specific historical and implied volatility information for its shares. Therefore, it estimates its expected share price volatility based on the historical volatility of publicly traded peer companies and expects to continue to do so until such time as it has adequate historical data regarding the volatility of its own traded share price. The expected term of all of the Company's share options has been determined utilizing the "simplified" method. The risk-free interest rate is determined by reference to the U.S. Treasury yield curve in effect at the time of grant of the award for time periods approximately equal to the expected term of the award. Expected dividend yield is based on the fact that the Company has never paid cash dividends on common shares and does not expect to pay any cash dividends in the foreseeable future.

Management evaluates its award grants and modifications and will adjust the fair value if any are determined to be spring-loaded.

Share-Based Compensation for Periods Prior to the Separation

Prior to the Separation from the Former Parent on October 3, 2022, certain of the Company's employees have historically participated in the Former Parent's non-cash share-based compensation plans. Non-cash share-based compensation expense for periods prior to the Separation has been allocated to the Company based on a combination of specific identification and a proportionate cost allocation method.

Income Taxes

The Company accounts for income taxes using the asset and liability method, which requires the recognition of deferred tax assets and liabilities for the expected future tax consequences of events that have been recognized in the consolidated financial statements or in the Company's tax returns. Deferred tax assets and liabilities are determined on the basis of the differences between the consolidated financial statements and tax basis of assets and liabilities using enacted tax rates in effect for the year in which the differences are expected to reverse. Changes in deferred tax assets and liabilities are recorded in the provision for income taxes.

The Company assesses the likelihood that its deferred tax assets will be recovered from future taxable income and, to the extent it believes, based upon the weight of available evidence, that it is more likely than not that all or a portion of the deferred tax assets will not be realized, a valuation allowance is established through a charge to income tax expense. Potential for recovery of deferred tax assets is evaluated by estimating the future taxable profits expected and considering prudent and feasible tax planning strategies. The provision for income taxes includes the

2. Summary of Significant Accounting Policies (Continued)

effects of applicable tax reserves, or unrecognized tax benefits, as well as the related net interest and penalties.

Net Loss per Share

Basic net loss per common share is calculated by dividing net loss by the weighted-average shares outstanding during the period. For purposes of the diluted net loss per share calculation, common share options are considered to be common share equivalents but have been excluded from the calculation of diluted net loss per share, as their effect would be anti-dilutive for all periods presented. Therefore, basic and diluted net loss per share were the same for all periods presented.

Net Loss per Share for Periods Prior to the Separation

Net loss per share for periods prior to the Separation from the Former Parent was calculated based on the 39,375,944 shares of the Company's common shares distributed to the Former Parent's shareholders at the time of the Distribution, including common shares issued in connection with Former Parent share options that were exercised on October 3, 2022 and common shares issued in connection with Former Parent restricted share units that vested on October 3, 2022. The same number of shares is being utilized for the calculation of basic and diluted earnings per share for all periods presented prior to the Spin-Off.

Concentrations of Credit Risk

Financial instruments that potentially expose the Company to concentrations of credit risk consist of cash, cash equivalents, and short-term debt securities. The Company maintains a portion of its cash deposits in government insured institutions in excess of government insured limits. The Company deposits its cash in financial institutions that it believes have high credit quality and has not experienced any losses on such accounts. The Company's cash management policy permits investments in U.S. federal government and federal agency securities, corporate bonds or commercial paper, supranational and sovereign obligations, certain qualifying money market mutual funds, certain repurchase agreements, and places restrictions on credit ratings, maturities, and concentration by type and issuer. The Company is exposed to credit risk in the event of a default by the financial institutions holding its cash in excess of government insured limits and in the event of default by corporations and governments in which it holds investments in cash equivalents and short-term debt securities, to the extent recorded on the consolidated balance sheets.

Segment Information

The Company manages its operations as a single segment, the development of therapies targeting areas including neuroscience, immunology and oncology. for the purposes of assessing performance and making operating decisions. Consistent with our operational structure, the Company's chief decision maker manages and allocates resources at a consolidated level. Therefore, results of our operations are reported on a consolidated basis for the purposes of assessing performance and making operating decisions. In 2023 and 2022, materially all the Company's long-lived assets were held in the United States.

Recently Issued Accounting Pronouncements

In November 2023, the FASB issued ASU No. 2023-07, Segment Reporting—Improvements to Reportable Segment Disclosures, which improves reportable segment disclosure requirements, primarily through enhanced disclosures about significant segment expenses. The amendments in ASU No. 2023-07 apply to public entities, including those with a single reportable segment, and are effective for fiscal years beginning after December 15, 2023, and interim periods within fiscal years beginning after December 15, 2024, with early adoption permitted. The Company is currently evaluating the impact ASU No. 2023-07 will have on its consolidated financial statements

In December 2023, the FASB issued ASU No. 2023-09, Income Taxes (Topic 740): Improvements to Income Tax Disclosures, to improve the transparency of income tax disclosures by requiring consistent categories and greater disaggregation of information in the rate reconciliation and income taxes paid disaggregated by jurisdiction. The ASU also includes certain other amendments to improve the effectiveness of income tax disclosures. The amendments in ASU 2023-09 are effective for fiscal years beginning after December 15, 2024, with early adoption permitted for annual financial statements that have not yet been issued or made available for issuance. The Company is currently evaluating the impact ASU No. 2023-09 will have on its consolidated financial statements.

(Amounts in thousands, except share and per share amounts)

3. Marketable Securities

The amortized cost, gross unrealized holding gains, gross unrealized holding losses and fair value of debt securities available-for-sale by type of security at December 31, 2023 and December 31, 2022 is as follows:

	Amortized Cost	Allowance for Credit Losses	Net Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Value
December 31, 2023						
Debt securities						
U.S. corporate bonds	$ 46,228	$ —	$ 46,228	$ 7	$ (24)	$ 46,211
Foreign corporate bonds	7,180	—	7,180	—	(7)	7,173
U.S. treasury bills	113,908	—	113,908	27	—	113,935
Total	$ 167,316	$ —	$ 167,316	$ 34	$ (31)	$ 167,319
December 31, 2022						
Debt securities						
U.S. corporate bonds	$ 142,697	$ —	$ 142,697	$ 25	$ (135)	$ 142,587
Foreign corporate bonds	36,766	—	36,766	9	(32)	36,743
U.S. treasury bills	89,308	—	89,308	17	(5)	89,320
U.S. agency bonds	41,734	—	41,734	—	(24)	41,710
Total	$ 310,505	$ —	$ 310,505	$ 51	$ (196)	$ 310,360

The fair values of debt securities available-for-sale by classification in the consolidated balance sheets were as follows:

	December 31, 2023	December 31, 2022
Cash and cash equivalents	$ 33,902	$ 49,896
Marketable securities	133,417	260,464
Total	$ 167,319	$ 310,360

The net amortized cost and fair value of debt securities available-for-sale at December 31, 2023 and December 31, 2022 are shown below by contractual maturity. Actual maturities may differ from contractual maturities because securities may be restructured, called or prepaid, or the Company intends to sell a security prior to maturity.

	December 31, 2023		December 31, 2022	
	Net Amortized Cost	Fair Value	Net Amortized Cost	Fair Value
Due to mature:				
Less than one year	$ 167,316	$ 167,319	$ 310,505	$ 310,360

(Amounts in thousands, except share and per share amounts)

3. Marketable Securities (Continued)

Summarized below are the debt securities available-for-sale the Company held at December 31, 2023 and December 31, 2022 that were in an unrealized loss position, aggregated by the length of time the investments have been in that position:

	Less than 12 months		
	Number of Securities	Fair Value	Unrealized Losses
December 31, 2023			
Debt securities			
U.S. corporate bonds	6	$ 29,537	$ (24)
Foreign corporate bonds	1	7,173	(7)
Total	7	$ 36,710	$ (31)
December 31, 2022			
Debt securities			
U.S. corporate bonds	16	$ 104,508	$ (135)
Foreign corporate bonds	3	31,886	(32)
U.S. treasury bills	1	9,762	(5)
U.S. agency bonds	4	41,710	(24)
Total	24	$ 187,866	$ (196)

The Company did no t have any investments in a continuous unrealized loss position for more than twelve months as of December 31, 2023 or December 31, 2022.

The Company reviewed the securities in the table above and concluded that they are performing assets generating investment income to support the needs of the Company's business. In performing this review, the Company considered factors such as the credit quality of the investment security based on research performed by external rating agencies and the prospects of realizing the carrying value of the security based on the investment's current prospects for recovery. As of December 31, 2023, the Company did not intend to sell these securities and did not believe it was more likely than not that it would be required to sell these securities prior to the anticipated recovery of their amortized cost basis.

Net Investment Income

Gross investment income includes interest income from debt securities available-for-sale, money-market funds, cash, and restricted cash. Net investment income included in other income (expense), net in the consolidated statements of operations for the years ended December 31, 2023 and December 31, 2022 were as follows:

	2023	2022
Debt securities (including realized losses)	$ 10,490	$ 1,454
Other investments	7,432	$ 1,781
Gross investment income (including realized losses)	17,922	$ 3,235
Investment expenses	(236)	(27)
Net investment income	$ 17,686	$ 3,208

The Company had no net investment income during the year ended December 31, 2021.

(Amounts in thousands, except share and per share amounts)

3. Marketable Securities (Continued)

We utilize the specific identification method in computing realized gains and losses on sales of debt securities. The proceeds from the sale of debt securities available-for-sale and the related gross realized losses for the year ended December 31, 2023 were as follows:

	2023
Proceeds from sales	$ 4,920
Gross realized losses	$ 39

The Company had no sales of debt securities during the years ended December 31, 2022 or 2021.

4. Fair Value of Financial Assets and Liabilities

The preparation of the Company's consolidated financial statements in accordance with GAAP requires certain assets and liabilities to be reflected at their fair value and others to be reflected on another basis, such as an adjusted historical cost basis. In this note, the Company provides details on the fair value of financial assets and liabilities and how it determines those fair values.

Financial Instruments Measured at Fair Value on the Consolidated Balance Sheets

Certain of the Company's financial instruments are measured at fair value on the consolidated balance sheets on a recurring basis. The fair values of these instruments are based on valuations that include inputs that can be classified within one of three levels of a hierarchy established by GAAP. See Fair Value Measurements in Note 2, "Summary of Significant Accounting Policies," for a brief description of the type of valuation information ("valuation inputs") that qualifies a financial asset or liability for each level.

Financial assets measured at fair value on a recurring basis on the consolidated balance sheets at December 31, 2023 and December 31, 2022 were as follows:

		Fair Value Measurement Using:			
Balance Sheet Classification	Type of Instrument	Level 1	Level 2	Level 3	Total
December 31, 2023					
Assets:					
Cash equivalents	Money market funds	$ 59,199	$ —	$ —	$ 59,199
Cash equivalents	U.S. treasury bills	—	27,901	—	27,901
Cash equivalents	U.S. corporate bonds	—	6,001	—	6,001
Marketable securities	U.S. treasury bills	9,874	76,160	—	86,034
Marketable securities	U.S. corporate bonds	—	40,210	—	40,210
Marketable securities	Foreign corporate bonds	—	7,173	—	7,173
Other non-current assets	Money market funds	1,900	—	—	1,900
Total assets		$ 70,973	$ 157,445	$ —	$ 228,418
December 31, 2022					
Assets:					
Cash equivalents	Money market funds	$ 72,866	$ —	$ —	$ 72,866
Cash equivalents	U.S. treasury bills	—	39,948	—	39,948
Cash equivalents	U.S. corporate bonds	—	9,948	—	9,948
Marketable securities	U.S. treasury bills	—	49,372	—	49,372
Marketable securities	U.S. corporate bonds	—	132,639	—	132,639
Marketable securities	U.S. agency bonds	—	41,710	—	41,710
Marketable securities	Foreign corporate bonds	—	36,743	—	36,743
Total assets		$ 72,866	$ 310,360	$ —	$ 383,226

(Amounts in thousands, except share and per share amounts)

4. Fair Value of Financial Assets and Liabilities (Continued)

The Company had no financial liabilities measured at fair value on a recurring basis as of December 31, 2023 or December 31, 2022.

There were no securities transferred between Level 1, 2, and 3 during the years ended December 31, 2023 or December 31, 2022.

The following is a description, including valuation methodology, of the financial assets and liabilities measured at fair value on a recurring basis:

Cash Equivalents

Cash equivalents at December 31, 2023 consisted of cash invested in short-term money market funds and debt securities with an original maturity of 90 days or less at the date of purchase. The carrying value of cash equivalents approximates fair value as maturities are less than three months. When quoted prices are available in an active market, cash equivalents are classified in Level 1 of the fair value hierarchy. Fair values of cash equivalent instruments that do not trade on a regular basis in active markets are classified as Level 2.

Marketable Securities

Quoted prices for identical assets in active markets are considered Level 1 and consist of on-the-run U.S. Treasury bills. The fair values of the Company's Level 2 debt securities are obtained from quoted market prices of debt securities with similar characteristics, quoted prices from identical assets in inactive markets, or discounted cash flows to estimate fair value.

5. Balance Sheet Components

Property and Equipment, Net

Property and equipment, net consisted of the following:

	As of December 31, 2023	As of December 31, 2022
Building and land	$ 11,728	$ 12,297
Leasehold improvements	802	—
Computer hardware and software	875	780
Office and lab equipment	9,961	5,501
Furniture and fixtures	1,550	1,202
	$ 24,916	$ 19,780
Accumulated depreciation	(8,283)	(4,914)
	16,633	14,866
Equipment not yet in service	558	2,646
Property and equipment, net	$ 17,191	$ 17,512

Depreciation expense was $ 3,369 , $ 1,361 and $ 673 for the years ended December 31, 2023, 2022 and 2021, respectively.

Equipment not yet in service primarily consisted of lab equipment that had not been placed into service as of December 31, 2023 or 2022.

Other Non-current Assets

Other non-current assets consisted of the following:

	As of December 31, 2023	As of December 31, 2022
Operating lease right-of-use assets	$ 31,385	$ 34,928
Other	2,400	2,585
Other non-current assets	$ 33,785	$ 37,513

Accrued Expenses and Other Current Liabilities

Accrued expenses and other current liabilities consisted of the following:

	As of December 31, 2023	As of December 31, 2022
Accrued employee compensation and benefits	$ 837	$ 14,603
Accrued clinical trial costs	29,501	17,788
Operating lease liability - current portion	3,308	3,019
Other accrued expenses and other current liabilities	6,200	8,696
Accrued expenses and other current liabilities	$ 39,846	$ 44,106

6. Acquisitions

Kv7 Platform Acquisition

In April 2022, the Company closed the acquisition from Knopp Biosciences LLC ("Knopp") of Channel Biosciences, LLC ("Channel"), a wholly owned subsidiary of Knopp owning the assets of Knopp's Kv7 channel targeting platform (the "Kv7 Platform Acquisition"), pursuant to a Membership Interest Purchase Agreement (the "Purchase Agreement"), dated February 24, 2022.

In consideration for the Kv7 Platform Acquisition, on April 4, 2022, the Company made an upfront payment

6. Acquisitions (Continued)

comprised of $ 35,000 in cash and 493,254 common shares of the Former Parent, valued at approximately $ 58,747 , issued through a private placement. The Company has also agreed to pay additional success-based payments comprised of (i) up to $ 325,000 based on developmental and regulatory milestones through approvals in the United States, EMEA and Japan for the lead asset, BHV-7000 (formerly known as KB-3061), (ii) up to an additional $ 250,000 based on developmental and regulatory milestones for the Kv7 pipeline development in other indications and additional country approvals, and (iii) up to $ 562,500 for commercial sales-based milestones of BHV-7000. Additionally, the Company has agreed to make scaled royalty payments in cash for BHV-7000 and the pipeline programs, starting at high single digits and peaking at low teens for BHV-7000 and starting at mid-single digits and peaking at low tens digits for the pipeline programs.

The Company accounted for this purchase as an asset acquisition as substantially all of the fair value of the gross assets acquired was concentrated in a single identifiable asset, IPR&D. The IPR&D asset has no alternative future use and relates to intellectual property rights related to the Kv7 platform. There was no material value assigned to any other assets or liabilities acquired in the acquisition. As such, during the second quarter of 2022, the Company recorded a charge to research and development ("R&D") expense in the accompanying consolidated statement of operations of $ 93,747 .

During the year ended December 31, 2022, the Company recorded $ 25,000 to R&D expense in the consolidated statement of operations for a regulatory milestone payment which became due and was paid to Knopp during 2022.

Excluding the milestone payment noted above, the Company has not recorded any of the possible contingent consideration payments to Knopp as a liability in the accompanying consolidated balance sheet, as none of the future events which would trigger a milestone payment were considered probable of occurring at December 31, 2023.

Pyramid Acquisition

In January 2024, the Company acquired Pyramid Biosciences, Inc. ("Pyramid"), pursuant to an Agreement and Plan of Merger, dated January 7, 2024 ("the Pyramid Agreement"). In consideration for the Pyramid acquisition, Biohaven made an upfront payment of $ 10,000 in common shares of the Company.

Biohaven has also agreed to make additional success-based payments comprised of (i) up to $ 10,000 based on developmental and regulatory milestones for the lead asset, BHV-1510 (formerly known as PBI-410), (ii) up to an additional $ 30,000 based on developmental and regulatory milestones for a second asset (formerly known as PBI-200) and (iii) up to $ 40,000 for commercial sales-based milestones of BHV-1510. Contingent developmental and regulatory milestone payments may be paid in cash or Biohaven common shares at the election of Biohaven and commercial sales-based milestones are to be made in cash.

During the first quarter of 2024, the Company incurred $ 5,000 of R&D expense related to a regulatory milestone which became due under the Pyramid Agreement.

7. Shareholders' Equity

Issuance of common shares for the Highlightll Agreement

In March 2023, the Company and Hangzhou Highlightll Pharmaceutical Co. Ltd. ("Highlightll") entered into an exclusive, worldwide (excluding People's Republic of China and its territories and possessions) license agreement (the "Highlightll Agreement") pursuant to which Biohaven obtained the right to research, develop, manufacture and commercialize Highlightll's brain penetrant dual TYK2/JAK1 inhibitor program. In connection with the Highlightll Agreement, in December 2023, the Company issued 721,136 common shares valued at $ 21,814 . See Note 11, "License Agreements," for further detail on the Highlightll Agreement.

Issuance of Common Shares for the October 2023 Offering

In October 2023, the Company completed an underwritten public offering of 11,761,363 of its common shares, including the exercise in full of the underwriters' option to purchase additional common shares, at a price to the public of $ 22.00 per share, pursuant to a registration statement on Form S-3 filed with the SEC. The net proceeds raised in the offering, after deducting underwriting discounts and expenses of the offering payable by the Company, were approximately $ 242,425 .

Equity Distribution Agreement

In October 2023, the Company entered into an equity distribution agreement pursuant to which the Company may offer and sell common shares having an aggregate offering price of up to $ 150,000 from time to time through or to the sales agent, acting as its agent or principal (the "Equity Distribution Agreement"). Sales of the Company's common shares, if any, will be made in sales deemed to be “at-the-market offerings”. The sales agent is not required to sell any specific amount of securities but will act as the Company's sales agent

(Amounts in thousands, except share and per share amounts)

7. Shareholders' Equity (Continued)

using commercially reasonable efforts consistent with its normal trading and sales practices, on mutually agreed terms between the sales agent and the Company. The Company currently plans to use the net proceeds from any at-the-market offerings of its common shares for general corporate purposes.

As of December 31, 2023, the Company has issued and sold no common shares under the Equity Distribution Agreement.

Issuance of Common Shares for the October 2022 Offering

In October 2022, the Company commenced a public offering of 25,000,000 of its common shares at a price of $ 10.50 per share, pursuant to a registration statement on Form S-1 filed with the SEC, which was declared effective by the SEC on October 20, 2022. The Company also granted the underwriters a 30-day option to purchase up to an additional 3,750,000 common shares. On October 25, 2022, the Company closed the offering, including a full exercise of the underwriters' option to purchase additional shares. The net proceeds raised in the offering, after deducting underwriting discounts and expenses of the offering payable by the Company, were approximately $ 282,804 .

Issuance of Common Shares in connection with the Separation from the Former Parent

On October 3, 2022, the Former Parent completed the Distribution to holders of its common shares and the spin-off of Biohaven. Each holder of Former Parent common shares received one common share of Biohaven for every two Former Parent common shares held of record as of the close of business, New York City time, on September 26, 2022.

In the Distribution, an aggregate of 35,840,459 common shares of the Company were issued. The aggregate number of common shares issued in connection with the Distribution did not include 2,611,392 common shares issued in connection with Former Parent share options that were exercised on October 3, 2022 and 924,093 common shares issued in connection with Former Parent restricted share units that vested on October 3, 2022. See Note 9, "Non-Cash Share-Based Compensation," for discussion of the Legacy Equity Award Settlement Plan.

8. Accumulated Other Comprehensive (Loss) Income

Shareholders' equity included the following activity in accumulated other comprehensive (loss) income for the years ended December 31, 2023 and 2022:

	2023	2022
Net unrealized investment gains (losses):		
Beginning of period balance	$ (145)	$ —
Other comprehensive income (loss)[1]	109	(145)
Amounts reclassified from accumulated other comprehensive income (loss)[2]	39	—
Other comprehensive income (loss)	148	(145)
End of period balance	3	(145)
Foreign currency translation adjustments:		
Beginning of period balance	429	—
Other comprehensive (loss) income[1]	(497)	429
End of period balance	(68)	429
Total beginning of period accumulated other comprehensive income	284	—
Total other comprehensive (loss) income	(349)	284
Total end of period accumulated other comprehensive (loss) income	$ (65)	$ 284

(1) There was no tax on other comprehensive income (loss) and immaterial tax on amounts reclassified from accumulated other comprehensive (loss) income during the period.

(2) Amounts reclassified from accumulated other comprehensive (loss) income for specifically identified debt securities are included in other income (expense), net in the consolidated statements of operations.

No amounts were reclassified from accumulated other comprehensive (loss) income during the years ended December 31, 2022 or 2021. The Company had no accumulated other comprehensive (loss) income included in shareholders' equity as of December 31, 2021.

9. Non-Cash Share-Based Compensation

2022 Equity Incentive Plan

In September 2022, the Company's shareholders approved the 2022 Equity Incentive Plan (the "2022 Plan"), which became effective on October 3, 2022. The

9. Non-Cash Share-Based Compensation (Continued)

2022 Plan provides for the grant of incentive share options, nonstatutory share options, share appreciation rights, restricted share awards, restricted share unit awards ("RSUs"), performance-based share awards and other share-based awards.

Upon the effectiveness of the 2022 Plan, there were 9,190,000 common shares reserved for issuance under the 2022 Plan. As of December 31, 2023, the 2022 Plan allows for a maximum of 11,917,619 shares of the Company's common shares to be reserved and available for grants. Also as of December 31, 2023, there were 173,899 shares of the Company's common shares available for future grants under the 2022 Plan. The number of shares reserved for issuance under the 2022 Plan automatically increases on January 1 of each calendar year by 4 % of total common shares outstanding as of December 31 of the prior year, beginning October 3, 2022 through January 1, 2032. In January 2024, the number of common shares reserved for future issuance under the 2022 Plan automatically increased by 3,244,628 common shares.

Legacy Equity Award Settlement Plan

In September 2022, the Company's shareholders approved the Legacy Equity Award Settlement Plan (the "Legacy Plan"), which became effective on September 29, 2022. The Legacy Plan is intended solely to provide for the grant and settlement of nonstatutory share options and RSUs, issued in respect of share options and RSUs originally granted pursuant to the Former Parent's 2017 Equity Incentive Plan and 2014 Equity Incentive Plan.

On October 3, 2022, the Former Parent completed the Distribution and the Separation. Each Former Parent share option and RSU outstanding prior to the Distribution was converted into .5 stock options and RSUs in the Company. In total, 4,057,121 share options and 924,093 RSUs were granted and settled into 2,611,392 and 924,093 common shares, respectively, under the Legacy Plan on October 3, 2022.

Non-Cash Share-Based Compensation Expense

For periods prior to the Separation from the Former Parent, non-cash share-based compensation has been allocated to the Company by using a combination of specific identification and a proportionate cost allocation method based on employee hours or directly identified operating expenses, depending on the employee’s function. The amounts presented are not necessarily indicative of future awards and do not necessarily reflect the costs that the Company would have incurred as an independent company for the periods presented.

Upon the effectiveness of the Company's Spin-off from the Former Parent, each Former Parent share option and RSU outstanding vested immediately (the "Acceleration") and converted into .5 share options and RSUs in the Legacy Plan in the Company. The non-cash share-based compensation expense related to the Acceleration was then allocated to the Company using the same methodology described above and recognized immediately, resulting in $ 101,440 of non-cash share-based compensation expense recognized related to to the Acceleration in the fourth quarter of 2022. The share options and RSUs granted under the Legacy Plan also immediately vested and settled as a result of the Spin-off, and the Company determined that no incremental compensation should be recognized related to these awards.

Non-cash share-based compensation under the Former Parent’s non-cash share-based compensation plans was measured at the grant date based on the fair value of the award and was recognized as expense over the requisite service period of the award (generally three to four years) using the straight-line method.

The Company measures non-cash share-based compensation at the grant date based on the fair value of the award and is recognized as expense over the requisite service period of the award (generally three years) using the straight-line method. Non-cash share-based compensation expense, consisting of expense for stock options, RSUs, and the 2022 Employee Share

(Amounts in thousands, except share and per share amounts)

9. Non-Cash Share-Based Compensation (Continued)

Purchase Plan (the "ESPP"), was classified in the consolidated statements of operations as follows:

	Year Ended December 31,		
	2023	2022	2021
Research and development expenses			
Allocated from the Former Parent excluding the Acceleration	$ —	$ 46,976	$ 39,381
Allocated from the Former Parent related to the Acceleration	—	61,749	—
2022 Equity Incentive Plan	15,985	7,654	—
Total research and development expenses	15,985	116,379	39,381
General and administrative expenses			
Allocated from the Former Parent excluding the Acceleration	—	30,951	26,258
Allocated from the Former Parent related to the Acceleration	—	39,691	—
2022 Equity Incentive Plan	12,802	6,535	—
Total general and administrative expenses	12,802	77,177	26,258
Total non-cash share-based compensation expense	$ 28,787	$ 193,556	$ 65,639

As of December 31, 2023, total unrecognized compensation cost related to the unvested share-based awards was $ 52,190 , which is expected to be recognized over a weighted average period of 2.36 years.

Share Options

All share option grants are awarded at fair value on the date of grant. The fair value of share options is estimated using the Black-Scholes option pricing model. Share options generally expire 10 years after the grant date.

The aggregate intrinsic value of share options is calculated as the difference between the exercise price of the share options and the fair value of the Company's common shares for those share options that had exercise prices lower than the fair value of the Company's common shares at December 31, 2023. The total intrinsic value of outstanding share options for the years ended December 31, 2023 and 2022 was $ 356,369 and $ 61,639 , respectively. The total intrinsic value of share options exercised for the year ended December 31, 2023 was $ 7,756 . The tax benefit from share options exercised for the years ended December 31, 2023 and 2022 was not material.

The assumptions that the Company used to determine the grant-date fair value of share options granted under the 2022 Plan were as follows, presented on a weighted average basis:

	Year Ended December 31,	
	2023	2022
Risk-free interest rate	4.32 %	3.86 %
Expected term (in years)	5.93	5.75
Expected volatility	84.60 %	82.05 %
Expected dividend yield	— %	— %
Exercise price	$ 24.57	$ 7.00

(Amounts in thousands, except share and per share amounts)

9. Non-Cash Share-Based Compensation (Continued)

The following table is a summary of the Company's share option activity for the year ended December 31, 2023:

	Number of Shares	Weighted Average Exercise Price	Weighted Average Remaining Contractual Term (in years)	Aggregate Intrinsic Value
Outstanding as of December 31, 2022	8,959,215	$ 7.00		
Granted	2,929,616	$ 24.57		
Exercised	(299,756)	$ 7.68		
Forfeited	(209,646)	$ 8.17		
Outstanding as of December 31, 2023	11,379,429	$ 11.48	8.99	$ 356,369
Options exercisable as of December 31, 2023	4,746,876	$ 9.46	8.88	$ 158,284
Vested and expected to vest as of December 31, 2023	11,379,429	$ 11.48	8.99	$ 356,369

The weighted average grant date fair value per share of share options granted under the Company’s share option plans during the years ended December 31, 2023 and 2022 was $ 18.01 and $ 4.97 , respectively. The Company expects approximately 6,632,553 of the unvested share options to vest over the requisite service period.

Restricted Share Units

As discussed above, in connection with the Distribution, on October 3, 2022, 924,093 RSUs were granted and settled into 924,093 common shares under the Legacy Equity Award Settlement Plan. No additional RSUs were granted under the 2022 Plan during the years ended December 31, 2023 or 2022.

The Company’s RSUs are considered nonvested share awards and require no payment from the employee. For each RSU, employees receive one common share at the end of the vesting period. The employee can elect to receive the one common share net of taxes or pay for taxes separately and receive the entire share. Compensation cost is recorded based on the market price of the Company’s common shares on the grant date and is recognized on a straight-line basis over the requisite service period.

There were no unvested restricted share units outstanding as of December 31, 2023 and 2022. No restricted share units were granted, forfeited, or vested, in the year ended December 31, 2023.

Employee Share Purchase Plan

In September 2022, the Company's board of directors approved the rules and procedures of the 2022 Employee Share Purchase Plan approved by shareholders of the Company on September 28, 2022.

The ESPP allows each eligible employee who is participating in the plan to purchase shares by authorizing payroll deductions of up to 15 % of eligible earnings. Unless the participating employee has previously withdrawn from the offering, accumulated payroll deductions will be used to purchase shares on the last business day of the offering period at a price equal to 85% of the fair market value of the shares on the first business day or the last business day of the offering period, whichever is lower. Under applicable tax rules, an employee may purchase no more than $25 worth of ordinary shares, valued at the start of the offering period, under the ESPP in any calendar year. There is no minimum holding period associated with shares purchased pursuant to this plan. An employee’s purchase rights terminate immediately upon termination of employment.

The number of shares reserved for issuance under the ESPP automatically increases on January 1 of each calendar year by 1 % of total common shares outstanding as of December 31 of the prior year, beginning October 3, 2022 through January 1, 2032. As of December 31, 2023, the ESPP allows for a maximum of 1,075,673 shares of the Company's common shares to be reserved and available for issuance under the ESPP. As of December 31, 2023, 916,484 shares remained available for future issuance under the ESPP. In January 2024, 811,157 additional shares were authorized to be issued under the ESPP.

The Company accounts for employee share purchases made under its ESPP using an estimate of the grant date fair value, which is determined in accordance with ASC 718, Stock Compensation. The purchase price discount and the look-back feature cause the ESPP to be compensatory and the Company to recognize compensation expense. The compensation

(Amounts in thousands, except share and per share amounts)

9. Non-Cash Share-Based Compensation (Continued)

cost is recognized on a straight-line basis over the requisite service period. The Company values ESPP shares using the Black-Scholes model. The Company recognized compensation expense of $ 1,341 for the year ended December 31, 2023. The Company did not recognize material compensation expense related to the ESPP for the year ended December 31, 2022.

As of December 31, 2023, there was $ 638 of unrecognized non-cash share-based compensation expense related to the ESPP, which is expected to be recognized over the remaining offering period ending May 31, 2024. During the year ended December 31, 2023, 159,189 shares were issued under the ESPP.

10. Net Loss Per Share

Basic and diluted net loss per share attributable to common shareholders of Biohaven was calculated as follows:

	Year Ended December 31,		
	2023	2022	2021
Numerator:			
Net loss	$ (408,168)	$ (570,279)	$ (213,796)
Denominator:			
Weighted average common shares outstanding—basic and diluted [1]	71,200,527	44,741,316	39,375,944
Net loss per share—basic and diluted	$ (5.73)	$ (12.75)	$ (5.43)

(1) Prior to the Spin-Off from the Former Parent on October 3, 2022, Biohaven Ltd. did not operate as an independent company. At the time of the Distribution, 39,375,944 shares of the Company's common stock were distributed to the Former Parent's shareholders, including common shares issued in connection with Former Parent share options that were exercised on October 3, 2022 and common shares issued in connection with Former Parent restricted share units that vested on October 3, 2022. This number of shares is being utilized for the calculation of basic and diluted earnings per share for all periods presented prior to the Spin-Off.

The Company's potential dilutive securities include share options which have been excluded from the computation of diluted net loss per share as the effect would be to reduce the net loss per share. Therefore, the weighted average number of common shares outstanding used to calculate both basic and diluted net loss per share attributable to common shareholders of the Company is the same. The Company excluded the following potential common shares, presented based on amounts outstanding at each period end, from the computation of diluted net loss per share attributable to common shareholders for the periods indicated because including them would have had an anti-dilutive effect:

	Year Ended December 31,	
	2023	2022
Options to purchase common shares	11,379,429	8,959,215

11. License Agreements

The following is a summary of all license agreements that the Company has entered into. As of December 31, 2023, the Company has potential future developmental, regulatory, and commercial milestone payments under these agreements of up to approximately $ 101,800 , $ 547,350 , and $ 1,270,450 , respectively.

Yale Agreements

In September 2013, the Company entered into an exclusive license agreement (the "Yale Agreement") with Yale University to obtain a license to certain patent rights for the commercial development, manufacture, distribution, use and sale of products and processes resulting from the development of those patent rights, related to the use of riluzole in treating various neurological conditions, such as general anxiety disorder, post-traumatic stress disorder and depression.

The Yale Agreement was amended and restated in May 2019. As amended, the Company agreed to pay Yale University up to $ 2,000 upon the achievement of specified regulatory milestones and annual royalty payments of a low single-digit percentage based on net sales of riluzole-based products from the licensed patents or from products based on troriluzole. Under the amended and restated agreement, the royalty rates are reduced as compared to the original agreement. In addition, under the amended and restated agreement, the Company may develop products based on riluzole or troriluzole. The amended and restated agreement retains a minimum annual royalty of up to $ 1,000 per year, beginning after the first sale of product under the agreement. If the Company grants any sublicense rights under the Yale Agreement, it must pay Yale University a low single-digit percentage of sublicense income that it receives.

For the years ended December 31, 2023, 2022 and 2021, the Company did not record any material expense or make any milestone or royalty payments under the Yale Agreement.

In January 2021, the Company entered into a worldwide, exclusive license agreement with Yale University for the development and commercialization of

11. License Agreements (Continued)

a novel Molecular Degrader of Extracellular Protein ("MoDE") platform (the "Yale MoDE Agreement"). Under the Yale MoDE Agreement, the Company acquired exclusive, worldwide rights to Yale University's intellectual property directed to its MoDE platform. The platform pertains to the clearance of disease-causing protein and other biomolecules by targeting them for lysosomal degradation using multi-functional molecules. As part of consideration for this license, the Company paid Yale University an upfront cash payment of $ 1,000 and 11,668 common shares of the Former Parent valued at approximately $ 1,000 . Under the Yale MoDE Agreement, the Company may develop products based on the MoDE platform. The Yale MoDE Agreement includes an obligation to pay a minimum annual royalty of up to $ 1,000 per year, and low single digit royalties on the net sales of licensed products. If the Company grants any sublicense rights under the Yale MoDE Agreement, it must pay Yale University a low single-digit percentage of sublicense income that it receives. In addition, Yale University will be eligible to receive additional development milestone payments of up to $ 800 and commercial milestone payments of up to $ 2,950 . The Yale MoDE Agreement terminates on the later of twenty years from the effective date, twenty years from the filing date of the first investigational new drug application for a licensed product or the last to expire of a licensed patent.

Under the Yale MoDE Agreement, the Company entered into a sponsored research agreement (the “Yale MoDE SRA”), which included funding of up to $ 4,000 over the life of the agreement.

The Company recorded research and development expense related to the Yale MoDE SRA of $ 1,333 , $ 2,666 and $ 150 for the years ended December 31, 2023, 2022 and 2021, respectively. For the years ended December 31, 2023, 2022 and 2021, the Company did not make any milestone or royalty payments under the Yale MoDE Agreement.

In May 2023, the Company entered into an additional sponsored research agreement with Yale University (the "2023 Yale SRA"), which included funding of up to $ 612 over the life of the agreement. For the year ended December 31, 2023, the company recorded $ 367 in research and development expense related to the 2023 Yale SRA.

ALS Biopharma Agreement

In August 2015, the Company entered into an agreement (the "ALS Biopharma Agreement") with ALS Biopharma and Fox Chase Chemical Diversity Center Inc. ("FCCDC"), pursuant to which ALS Biopharma and FCCDC assigned the Company their worldwide patent rights to a family of over 300 prodrugs of glutamate modulating agents, including troriluzole, as well as other innovative technologies. Under the ALS Biopharma Agreement, the Company is obligated to use commercially reasonable efforts to commercialize and develop markets for the patent products. The Company is obligated to pay $ 3,000 upon the achievement of specified regulatory milestones with respect to the first licensed product and $ 1,000 upon the achievement of specified regulatory milestones with respect to subsequently developed products, as well as royalty payments of a low single-digit percentage based on net sales of products licensed under the ALS Biopharma Agreement, payable on a quarterly basis.

The ALS Biopharma Agreement terminates on a country-by-country basis as the last patent rights expire in each such country. If the Company abandons its development, research, licensing or sale of all products covered by one or more claims of any patent or patent application assigned under the ALS Biopharma Agreement, or if the Company ceases operations, it has agreed to reassign the applicable patent rights back to ALS Biopharma.

For the years ended December 31, 2023, 2022 and 2021, the Company did no t record any expense or make any milestone or royalty payments under the ALS Biopharma Agreement.

2016 AstraZeneca Agreement

In October 2016, the Company entered into an exclusive license agreement (the "2016 AstraZeneca Agreement") with AstraZeneca, pursuant to which AstraZeneca granted the Company a license to certain patent rights for the commercial development, manufacture, distribution and use of any products or processes resulting from development of those patent rights, including BHV-5000 and BHV-5500. In exchange for these rights, the Company agreed to pay AstraZeneca an upfront payment, milestone payments and royalties on net sales of licensed products under the agreement. The regulatory milestones due under the 2016 AstraZeneca Agreement depend on the indication of the licensed product being developed as well as the territory where regulatory approval is obtained.

Regulatory milestones due under the 2016 AstraZeneca Agreement with respect to Rett syndrome total up to $ 30,000 , and, for any indication other than Rett syndrome, total up to $ 60,000 . Commercial milestones are based on net sales of all products licensed under the 2016 AstraZeneca Agreement and total up to $ 120,000 . The Company has also agreed to pay royalties in two tiers, with each tiered royalty in the range from 0 - 10 % of net sales of products licensed under the 2016 AstraZeneca Agreement. If the Company receives revenue from sublicensing any of its rights

11. License Agreements (Continued)

under the 2016 AstraZeneca Agreement, the Company is also obligated to pay a portion of that revenue to AstraZeneca. The Company is also required to reimburse AstraZeneca for any fees that AstraZeneca incurs related to the filing, prosecution, defending, and maintenance of patent rights licensed under the 2016 AstraZeneca Agreement.

The 2016 AstraZeneca Agreement expires upon the expiration of the patent rights under the agreement or on a country-by-country basis ten years after the first commercial sale and can also be terminated if certain events occur, e.g., material breach or insolvency.

For the years ended December 31, 2023, 2022 and 2021, the Company did no t record any expense or make any milestone or royalty payments under the 2016 AstraZeneca Agreement.

2018 AstraZeneca License Agreement

In September 2018, the Company entered into an exclusive license agreement (the "2018 AstraZeneca Agreement") with AstraZeneca, pursuant to which AstraZeneca granted the Company a license to certain patent rights for the commercial development, manufacture, distribution and use of any products or processes resulting from development of those patent rights, including BHV-3241 (verdiperstat). Under the 2018 AstraZeneca Agreement, the Company paid AstraZeneca an upfront cash payment of $ 3,000 and 109,523 shares valued at $ 4,080 on the date of settlement and is obligated to pay milestone payments to AstraZeneca totaling up to $ 55,000 upon the achievement of specified regulatory and commercial milestones and up to $ 50,000 upon the achievement of specified sales-based milestones. In addition, the Company will pay AstraZeneca royalties in three tiers, with each tiered royalty in the range from 0 - 10 % of net sales of specified approved products, subject to specified reductions.

In November 2021, the Company completed enrollment in a Phase 3 clinical trial of this product candidate, which is now referred to as verdiperstat, for the treatment of Amyotrophic Lateral Sclerosis ("ALS").In September 2022, the Company announced negative topline results from the Phase 3 clinical trial of verdiperstat for ALS. ALS is a progressive, life-threatening, and rare neuromuscular disease for which there are currently limited treatment options and no cure. The Company is solely responsible, and has agreed to use commercially reasonable efforts, for all development, regulatory and commercial activities related to verdiperstat. The Company may sublicense its rights under the agreement and, if it does so, will be obligated to pay a portion of any milestone payments received from the sublicense to AstraZeneca in addition to any milestone payments it would otherwise be obligated to pay.

The 2018 AstraZeneca Agreement terminates on a country-by-country basis and product-by-product basis upon the expiration of the royalty term for such product in such country and can also be terminated if certain events occur, e.g., material breach or insolvency.

For the years ended December 31, 2023, 2022 and 2021, the Company did no t record any material expense or make any milestone or royalty payments under the 2018 AstraZeneca Agreement.

Fox Chase Chemical Diversity Center Inc. Agreement

In May 2019, the Company entered into an agreement with FCCDC (the "FCCDC Agreement") pursuant to which the Company purchased certain intellectual property relating to the TDP-43 protein from FCCDC. The FCCDC Agreement provides the Company with a plan and goal to identify one or more new chemical entity candidates for preclinical development for eventual clinical evaluation for the treatment of one or more TDP-43 proteinopathies. As consideration, the Company issued 100,000 of the Former Parent's common shares to FCCDC valued at $ 5,646 .

In addition, the Company is obligated to pay FCCDC milestone payments totaling up to $ 3,000 with $ 1,000 for each additional NDA filing. The Company also issued a warrant to FCCDC, granting FCCDC the option to purchase up to 100,000 of the Former Parent’s common shares, at a strike price of $ 56.46 per share, subject to vesting upon achievement of certain milestones in development of TDP-43. In connection with the Separation, the warrants issued to FCCDC were vested and settled, resulting in $ 4,245 being recorded as research and development expense for the year ended December 31, 2022.

In connection with the FCCDC Agreement, the Company and FCCDC have established a TDP-43 Research Plan, which was amended in November 2020, under which the Company will pay FCCDC an earned royalty equal to 0 % to 10 % of net sales of any TD-43 patent products with a valid claim as defined in the FCCDC Agreement. The Company may also license the rights developed under the FCCDC Agreement and, if it does so, will be obligated to pay a portion of any payments received from such licensee to FCCDC in addition to any milestones it would otherwise be obligated to pay. The Company is also responsible for the prosecution and maintenance of the patents related to the TDP-43 assets.

The FCCDC Agreement terminates on a country-by-country basis and product-by-product basis upon expiration of the royalty term for such product in such

11. License Agreements (Continued)

country and can also be terminated if certain events occur, e.g., material breach or insolvency.

The Company did no t record any material research and development expense or make any milestone payments related to the FCCDC Agreement in the consolidated statements of operations during the years ended December 31, 2023, 2022 and 2021.

UConn

In October 2018, the Company announced it had signed an exclusive, worldwide option and license agreement (the “UConn Agreement”) with the University of Connecticut (“UConn”) for the development and commercialization rights to UC1MT, a therapeutic antibody targeting extracellular metallothionein. Under the UConn Agreement, the Company had the option to acquire an exclusive, worldwide license to UC1MT and its underlying patents to develop and commercialize throughout the world in all human indications (the "UConn Option"). In September 2022, the Company exercised the UConn Option in exchange for a payment of $ 400 . Under the UConn Agreement, UConn is entitled to milestone payments upon the achievement of specified developmental and regulatory milestones of up to $ 30,100 and commercial milestones of up to $ 50,000 , and royalties of a low single-digit percentage of net sales of licensed products.

Excluding the payment made in connection with the exercise of the UConn Option in September 2022, for the years ended December 31, 2023, 2022 and 2021, the Company did no t record any research and development expense or make any milestone payments related to the UConn Agreement.

Artizan Agreement

In December 2020, the Company entered into an Option and License Agreement (the "2020 Artizan Agreement") with Artizan Biosciences Inc. ("Artizan"). Pursuant to the 2020 Artizan Agreement, the Company acquired an option (“Biohaven Option”) to obtain a royalty-based license from Artizan to manufacture, use and commercialize certain products in the United States for the treatment of diseases, including, for example, inflammatory bowel disease and other gastrointestinal inflammatory disorders, e.g., Crohn’s disease. The Biohaven Option is exercisable throughout the development phase of the products at an exercise price of approximately $ 4,000 to $ 8,000 , which varies based on the market potential of the products. The Company and Artizan have also formed a joint steering committee to oversee, review and coordinate the product development activities with regard to all products for which we have exercised (or will exercise in the future) the Biohaven Option.

In December 2020, simultaneously with the 2020 Artizan Agreement, the Company entered into a Series A-2 Preferred Stock Purchase Agreement with Artizan. Under the agreement, the Company paid Artizan 61,494 of the Former Parent’s common shares valued at $ 6,000 , which were issued in January 2021. In exchange, the Company acquired 34,472,031 shares of series A-2 preferred stock of Artizan.

In June 2021, the Company entered into a Development and License Agreement with Artizan Biosciences Inc (the "2021 Artizan Agreement"). Pursuant to the 2021 Artizan Agreement, the Company acquired an exclusive, worldwide license under Artizan’s IgA-SEQ patented technology and know-how to develop, manufacture and commercialize certain of Artizan’s compounds for use in Parkinson’s Disease. Under the 2021 Artizan Agreement, the Company is responsible for funding the development of the compounds, obtaining regulatory approvals, manufacturing the compounds and commercializing the compounds. the Company is also responsible for the prosecution, maintenance and enforcement of Artizan’s patents. The Company will pay Artizan development milestones of $ 20,000 for the first licensed compound to achieve U.S. marketing authorization and $ 10,000 for each subsequent U.S. approval. In addition, the Company will pay Artizan commercialization milestones totaling up to $ 150,000 and royalties in the low- to mid-single digits. The 2021 Artizan Agreement terminates on a country-by-country basis on the later of 10 years from the first commercial sale of licensed product in such country or the expiration of Artizan’s patents in such country and can also be terminated if certain events occur, e.g., material breach or insolvency. In June 2023, the 2020 Artizan Agreement and 2021 Artizan Agreement were both terminated.

In June 2022, the Company entered into an amendment (the "Amendment") to the Series A-2 Preferred Stock Purchase Agreement with Artizan. Under the Amendment, the Company made a cash payment of $ 4,000 in exchange for 22,975,301 shares of series A-2 preferred stock of Artizan out of a total of 45,950,601 shares of series A-2 preferred stock of Artizan for a total raise of $ 8,000 (the “A2 Extension Raise”). Along with the Amendment, the Company and Artizan executed a non-binding indication of interest (“Artizan Side Letter”), which describes terms under which the Company and Artizan would amend the 2020 Artizan Agreement to eliminate certain milestone payments required by us in exchange for limiting our option to the selection of the first licensed product. The Artizan Side Letter required Artizan to commit at least 80 % of the funds raised in the A-2 Extension Raise to a certain program and to raise $ 35,000 of additional capital within a certain time.

11. License Agreements (Continued)

As of December 31, 2022, due to concerns related to Artizan's inability to fund its future operations, the Company determined its investment in Artizan to be fully impaired. Accordingly, during the fourth quarter of 2022 the Company recognized an impairment loss of $ 10,000 in other income (expense),net on the consolidated statements of operations.

For the years ended December 31, 2023, 2022 and 2021, excluding the upfront payments above, the Company did no t record any research and development expense or make any material milestone payments related to the 2020 Artizan Agreement and the 2021 Artizan Agreement.

Reliant Agreement

In July 2021, the Company entered into a development and licensing agreement (the "Reliant Agreement") with Reliant Glycosciences LLC ("Reliant"), pursuant to which the Company and Reliant have agreed to collaborate on a program with Biohaven Labs' multifunctional molecules to develop and commercialize conjugated antibodies for therapeutic uses relating to IgA nephropathy and treatment of other diseases and conditions. Under the Reliant Agreement, the Company paid Reliant an upfront payment in the form of issuance of common shares of the Former Parent valued at approximately $ 3,686 , which the Company recorded as research and development expense on its consolidated statement of operations. In addition, Reliant will be eligible to receive development and regulatory milestone payments of up to $ 36,500 , and royalties of a low single-digit percentage of net sales of licensed products.

Excluding the upfront payment discussed above, for the years ended December 31, 2023, 2022 and 2021, the Company did no t record any material research and development expense related to the Reliant Agreement.

KU Leuven Agreement

In January 2022, the Company and Katholieke Universiteit Leuven ("KU Leuven") entered into an Exclusive License and Research Collaboration Agreement (the "KU Leuven Agreement") to develop and commercialize TRPM3 antagonists to address the growing proportion of people worldwide living with chronic pain disorders. The TRPM3 antagonist platform was discovered at the Centre for Drug Design and Discovery and the Laboratory of Ion Channel Research at KU Leuven. Under the KU Leuven Agreement, the Company receives exclusive global rights to develop, manufacture and commercialize KU Leuven's portfolio of small-molecule TRPM3 antagonists. The portfolio includes the lead candidate, henceforth known as BHV-2100, which is being evaluated in preclinical pain models and will be the first to advance towards Phase 1 studies. The Company will support further basic and translational research at KU Leuven on the role of TRPM3 in pain and other disorders. As consideration, KU Leuven received an upfront cash payment of $ 3,000 and 15,340 shares of the Former Parent valued at $ 1,779 , and is eligible to receive additional development, regulatory, and commercialization milestones payments of up to $ 327,750 . In addition, KU Leuven will be eligible to receive mid-single digit royalties on net sales of products resulting from the collaboration.

For the year ended December 31, 2023, the Company recorded $ 3,250 to R&D expense in the consolidated statements of operations related to developmental milestones which became due to KU Leuven during 2023. Excluding the upfront payments discussed above, for the year ended December 31, 2022, the Company did no t record any material research and development expense related to the KU Leuven Agreement.

Taldefgrobep Alfa License Agreement

In February 2022, following the transfer of intellectual property, the Company announced that it entered into a worldwide license agreement with BMS for the development and commercialization rights to taldefgrobep alfa (also known as BMS-986089), a novel, Phase 3-ready anti-myostatin adnectin (the "Taldefgrobep Alfa License Agreement"). Under the terms of the Taldefgrobep Alfa License Agreement, the Company will receive worldwide rights to taldefgrobep alfa and BMS will be eligible for regulatory approval milestone payments of up to $ 200,000 , as well as tiered, sales-based royalty percentages from the high teens to the low twenties. There were no upfront or contingent payments to BMS related to the Taldefgrobep Alfa License Agreement.

For the years ended December 31, 2023 and 2022, the Company did no t record any material expense or make any milestone or royalty payments under the Taldefgrobep Alfa License Agreement.

Agreement with Hangzhou Highlightll Pharmaceutical Co. Ltd.

In March 2023, the Company entered into the Highlightll Agreement pursuant to which Biohaven obtained the right to research, develop, manufacture and commercialize Highlightll's brain penetrant dual TYK2/JAK1 inhibitor program. In connection with the Highlightll Agreement, the Company was obligated to pay Highlightll a cash payment of $ 10,000 and 721,136 common shares (collectively, "the Highlightll Upfront Payments"), upon the completion of certain post-closing activities. In December 2023, the Company entered into

11. License Agreements (Continued)

a second amendment to the Highlightll Agreement, which granted the Company an exclusive option and right of first refusal to any Selective TYK2 Inhibitor being developed by or on behalf of Highlightll or its affiliates and provided for the payment of the Highlightll Upfront Payments. As a result, the Company made a $ 10,000 cash payment and issued 721,136 shares, valued at $ 21,814 , to Highlightll during the fourth quarter of 2023, which was recorded as R&D expense during the year ended December 31, 2023.

Under the Highlightll Agreement, the Company is obligated to make milestone payments to Highlightll totaling up to $ 200,000 upon the achievement of specified developmental, regulatory and commercial milestones for a first indication, up to $ 100,000 upon the achievement of pre-specified developmental, regulatory and commercial milestones for a second indication, and up to $ 650,000 upon the achievement of specified sales-based milestones. Additionally, the Company has agreed to make tiered royalty payments as a percentage of net sales starting at mid single digits and peaking at low teens digits. During the royalty term, if the Company offers to include China clinical sites in its Phase 3 study sufficient for submission to Chinese National Medical Products Administration and Highlightll, at its sole discretion, agrees, then Highlightll will pay royalties in the low tens digits to the Company on China sales upon approval.

The Highlightll Agreement terminates on a country-by-country basis upon expiration of the royalty term and can also be terminated if certain events occur, e.g., material breach or insolvency.

Excluding the Highlightll Upfront Payments discussed above, for the year ended December 31, 2023, the Company did not record any material milestone or royalty payments related to the Highlightll Agreement.

12. Commitments and Contingencies

All consideration paid by the Former Parent in association with the following agreements, certain of which were assigned by the Former Parent to the Company in connection with the Spin-Off, during the periods prior to the Separation is recorded in the consolidated financial statements of the Company.

Lease Agreements

The Company's leases primarily consist of lab and office space for use in its operations. Its leases generally have lease terms of 1 to 10 years, some of which include options to extend for up to 5 to 10 years or on a month-to-month basis. The Company includes extension options that are reasonably certain to be exercised as part of determination of lease terms. As of December 31, 2023, none of the Company's lease terms included the extension option as the Company has determined that it is unlikely to exercise the extension option. For periods prior to Separation, lease costs were allocated to the Company based on a proportional cost allocation method. Allocated operating lease cost for periods prior to Separation and actual operating lease cost was $ 5,874 , $ 1,158 and $ 264 for the years ended December 31, 2023, 2022 and 2021, respectively.

The Company currently has one short-term real estate leases with immaterial lease expense. The Company had immaterial sublease income and there are no sale-leaseback transactions. Certain of the Company's lease agreements contain variable lease payments that are adjusted for actual operating expense true-ups compared with estimated amounts; however, these amounts are immaterial. The Company's lease agreements do not contain any material residual value guarantees or material restrictive covenants.

The following table summarizes supplemental cash flow information:

	Years Ended December 31,		
	2023	2022	2021
Right-of-use assets obtained in exchange for new operating lease liabilities	$ —	$ 31,342	$ 478

Operating cash flows paid for operating leases were immaterial for all periods prior to the Separation, and were $ 4,854 and $ 780 for the years ended December 31, 2023 and December 31, 2022.

Supplemental balance sheet information related to leases is as follows:

In thousands, except remaining lease term and discount rate	December 31, 2023	December 31, 2022
Assets		
Other non-current assets	$ 31,385	$ 34,928
Liabilities		
Other current liabilities	$ 3,308	$ 3,019
Long-term operating lease liability	27,569	30,581
	$ 30,877	$ 33,600
Weighted average remaining lease term (in years)	8.21	9.08
Weighted average discount rate	6.47 %	6.55 %

(Amounts in thousands, except share and per share amounts)

12. Commitments and Contingencies (Continued)

The following table summarizes maturities of operating lease liabilities as of December 31, 2023:

	Operating leases
2024	$ 5,020
2025	4,782
2026	4,905
2027	4,509
2028	3,945
Thereafter	16,619
Total lease payments	39,780
Less: imputed interest	(8,903)
Total lease liabilities	$ 30,877

West Palm Beach Lease Agreement

In June 2022, the Company entered into a lease agreement in West Palm Beach, Florida for office space (the "West Palm Beach Lease"), which will be used for general office purposes. The lease is expected to commence in late 2024 after substantial completion of building improvements, and has a term of 120 months, with an option to extend for two additional periods of 60 months each. The Company expects to record the West Palm Beach Lease as an operating lease. The Company has annual commitments relating to the West Palm Beach Lease ranging from $ 1,263 to $ 1,649 .

Research Commitments

The Company has entered into agreements with several CROs to provide services in connection with the Company's preclinical studies and clinical trials. As of December 31, 2023, the Company had remaining maximum research commitments in excess of one year of approximately $ 9,025 , which are variable based on number of trial participants, and contingent upon the achievement of certain milestones of the clinical trials covered under the agreements. If all related milestones are achieved, the Company expects these amounts to be paid over approximately one year .

Indemnification Agreements

In the ordinary course of business, the Company may provide indemnification of varying scope and terms to vendors, lessors, business partners and other parties with respect to certain matters including, but not limited to, losses arising out of breach of such agreements or from intellectual property infringement claims made by third parties. In addition, the Company has entered into indemnification agreements with members of its board of directors and executive officers that will require the Company, among other things, to indemnify them against certain liabilities that may arise by reason of their status or service. The maximum potential amount of future payments the Company could be required to make under these indemnification agreements is, in many cases, unlimited. The Company's amended and restated memorandum and articles of association also provide for indemnification of directors and officers in specific circumstances. To date, the Company has not incurred any material costs as a result of such indemnification provisions. The Company does not believe that the outcome of any claims under indemnification arrangements will have a material effect on its financial position, results of operations or cash flows, and it has not accrued any liabilities related to such obligations in its consolidated financial statements as of December 31, 2023 or December 31, 2022.

License Agreements

The Company has entered into license agreements with various parties for which it is obligated to make contingent and non-contingent payments. See Note 11, "License Agreements," for additional details.

Other Agreements

On January 1, 2021, the Company entered into a consulting services agreement (the "Moda Agreement") with Moda Pharmaceuticals LLC ("Moda") to further the scientific advancement of technology, drug discovery platforms (including the technology licensed under the Yale MoDE Agreement), product candidates and related intellectual property owned or controlled by the Company.

Under the Moda Agreement, the Company paid Moda an upfront cash payment of $ 2,700 and 37,836 shares of the Former Parent valued at approximately $ 3,243 . In addition, Moda will be eligible to receive additional development milestone payments of up to $ 81,612 and commercial milestone payments of up to $ 30,171 . The Moda Agreement has a term of four years and may be terminated earlier by the Company or Moda under certain circumstances including, for example, the Company's discontinuation of research on the MoDE platform or default.

For the years ended December 31, 2023, 2022 and 2021, excluding the upfront payments above, the Company did no t record any material research and development expense or make any milestone payments related to the Moda Agreement.

Legal Proceedings

From time to time, in the ordinary course of business, the Company is subject to litigation and regulatory examinations as well as information gathering requests, inquiries and investigations. As of

(Amounts in thousands, except share and per share amounts)

12. Commitments and Contingencies (Continued)

December 31, 2023, there were no matters which would have a material impact on the Company's financial results.

13. Income Taxes

The income tax expense in the consolidated financial statements has been calculated on a separate return method and is presented as if the Company's operations were separate taxpayers in the respective jurisdictions up to and including the Separation. Cash tax payments, income taxes receivable and deferred taxes, net of valuation allowance, are reflective of its actual tax balances prior and subsequent to the Distribution.

As a company incorporated in the British Virgin Islands ("BVI"), the Company is principally subject to taxation in the BVI. Under the current laws of the BVI, the Company and all dividends, interest, rents, royalties, compensation and other amounts paid by the Company to persons who are not resident in the BVI and any capital gains realized with respect to any shares, debt obligations, or other securities of the Company by persons who are not resident in the BVI are exempt from all provisions of the Income Tax Ordinance in the BVI.

The Company, including the Former Parent for periods prior to the Separation, has historically outsourced all of the research and clinical development for its programs under a master services agreement with Biohaven Pharmaceuticals, Inc. ("BPI"). As a result of providing services under this agreement, BPI was profitable during the years ended December 31, 2023, 2022 and 2021, and BPI is subject to taxation in the United States. As such, in each reporting period, the Company's tax provision includes the effects of consolidating the results of operations of BPI.

At December 31, 2023 and 2022, the Company continued to maintain a full valuation allowance against its net deferred tax assets, which are comprised primarily of capitalized research and development deductions, research and development tax credit carryforwards and net operating loss carryforwards, based on management's assessment that it is more likely than not that the deferred tax assets will not be realized. The Company will continue to evaluate the need for a valuation allowance on its deferred tax assets until there is sufficient positive evidence to support the reversal of all or some portion of these allowances.

The Company recorded an income tax benefit during the year ended December 31, 2023 of $ 1,383 and income tax provisions of $ 438 and $ 1,366 during the years ended December 31, 2022 and 2021, respectively, which primarily represent Federal and state taxes related to the Company's profitable operations in the U.S. and Ireland.

Loss before provision for income taxes consisted of the following:

	Year Ended December 31,		
	2023	2022	2021
BVI	$ (424,647)	$ (598,046)	$ (211,334)
Foreign	15,096	28,205	(1,096)
Loss before provision for income taxes	$ (409,551)	$ (569,841)	$ (212,430)

The provision for income taxes consisted of the following:

	Year Ended December 31,		
	2023	2022	2021
Current income tax (benefit) provision:			
BVI	$ —	$ —	$ —
Foreign	(1,383)	438	1,366
Total current income tax (benefit) provision	(1,383)	438	1,366
Deferred income tax provision (benefit):			
BVI	—	—	—
Foreign	—	—	—
Total deferred income tax provision (benefit)	—	—	—
Total (benefit) provision for income taxes	$ (1,383)	$ 438	$ 1,366

A reconciliation of the BVI statutory income tax rate of 0% to the Company's effective income tax rate is as follows:

	Year Ended December 31,		
	2023	2022	2021
BVI statutory income tax rate	0.0 %	0.0 %	0.0 %
Foreign tax rate differential	1.3	(12.6)	0.0
Tax credits	(12.7)	(5.8)	(5.0)
Change in valuation allowance	10.6	18.5	7.0
Other	0.5	0.0	(1.0)
Effective income tax rate	(0.3)%	0.1 %	1.0 %

(Amounts in thousands, except share and per share amounts)

13. Income Taxes (Continued)

The Company's income tax (benefit) provision primarily represents Federal and state taxes related to the profitable operations of its subsidiaries in the United States and Ireland. The income tax benefit recorded during the year ended December 31, 2023 was primarily attributable to the adoption of the guidance contained in a Notice of Proposed Rule Making issued by the United States Internal Revenue Service during the third quarter of 2023 ("the Notice"). The Notice indicates that BPI has the ability to immediately deduct R&D expenditures which were incurred in the US and reimbursed by its foreign parent. Previously these expenditures were capitalized, as was generally required under the Tax Cuts and Jobs Act, which was effective for tax years beginning on or after January 1, 2022. Based on this guidance and its application to the Company's specific facts, Biohaven deducted these expenditures on its 2022 tax return, substantially reducing its taxable income in the US and capitalized R&D expenditures, resulting in an increase to its federal net operating loss carryforward of $ 598.7 million that can be carried forward indefinitely. The Company's adoption of the Notice in 2023 also increased its State net operating losses, resulting in the reversal of $ 0.8 million of state income taxes recorded in 2022.

Net deferred tax assets (liabilities) consisted of the following:

	December 31,	
	2023	2022
Deferred tax assets:		
Foreign net operating loss carryforwards	$ 138,480	$ 8,895
Tax credits	111,660	60,340
R&D capitalization	887	142,269
Other	15,944	5,205
Valuation allowance	(259,448)	(216,061)
Total deferred tax assets	7,523	648
Deferred tax liabilities:		
Other	(7,523)	(648)
Total deferred tax liabilities	(7,523)	(648)
Net deferred tax asset (liability)	$ —	$ —

As of December 31, 2023, and 2022, the Company had foreign net operating loss carryforwards of $ 847,582 and $ 69,214 , respectively. As of December 31, 2023, and 2022, the Company had federal and state research and development and orphan drug credits of $ 111,660 and $ 60,340 , respectively, which begin to expire in 2039.

Changes in the valuation allowance for deferred tax assets during the years ended December 31, 2023 and 2022 were due primarily to generation of net operating losses and tax credit carryforwards. Changes in the valuation allowance for deferred tax assets during the years ended December 31, 2022 and 2021 were due primarily to generation of nondeductible research expenses and tax credit carryforwards.

	Year Ended December 31,		
	2023	2022	2021
Valuation allowance as of beginning of year	$ 216,061	$ 54,224	$ 32,97
Increases recorded to Purchase Accounting and Net Investment from Former Parent	—	50,905	6,449
Increases recorded to income tax provision	43,387	110,932	14,805
Valuation allowance as of end of year	$ 259,448	$ 216,061	$ 54,22

The Company followed the authoritative guidance for recognizing and measuring uncertainty in income taxes for tax positions taken or expected to be taken in a tax return.

The beginning and ending amounts of unrecognized tax benefits reconciles as follows:

	Year Ended December 31,		
	2023	2022	2021
Beginning of period balance	$ 2,200	$ 3,800	$ 2,700
Increase for tax positions taken during the current period	—	—	50
(Decreases) increases recorded to Purchase Accounting and Net Investment from Former Parent	—	(1,600)	1,050
End of period balance	$ 2,200	$ 2,200	$ 3,800

The unrecognized tax benefits relate primarily to issues common among multinational corporations. All of these unrecognized tax benefits, if recognized, would impact the Company's effective income tax rate. The Company's policy is to record interest and penalties related to income taxes, if any, as part of its income tax provision. As of December 31, 2023 and 2022, the total amount of accrued interest and penalties was not significant.

BPI and Kleo file income tax returns in the U.S. and certain state jurisdictions. BPI's U.S. federal and state income tax returns are subject to tax examinations for the tax year ended December 31, 2019 and

13. Income Taxes (Continued)

subsequent years. The federal tax return for BPI is currently under audit by the IRS for the period ended December 31, 2019.

14. Related Party Transactions

Relationship with the Former Parent

Upon the effectiveness of the Separation on October 3, 2022, the Former Parent ceased to be a related party to the Company and accordingly, no related party transactions or balances are reported subsequent to October 3, 2022.

On October 3, 2022, the Company entered into agreements with the Former Parent in connection with the Separation, including the following:

Transition Services Agreement. The Company entered into a Transition Services Agreement with the Former Parent (the "Transition Services Agreement") under which the Company or one of its affiliates will provide the Former Parent, and the Former Parent or one of its affiliates will provide the Company, with certain transition services for a limited time to ensure an orderly transition following the Spin-Off. The services that the Company and the Former Parent agreed to provide to each other under the Transition Services Agreement include certain finance, information technology, clinical study support, human resources and compensation, facilities, financial reporting and accounting and other services. The Company will pay the Former Parent, and the Former Parent will pay the Company, for any such services received by the Former Parent or the Company, as applicable, at agreed amounts as set forth in the Transition Services Agreement.

Amounts received in connection with the Transition Services Agreement are recorded as other income on the consolidated statement of operations, as they are outside of the normal operating business of the Company. For the year ended December 31, 2023, the Company recorded $ 9,250 in other income (expense), net on the consolidated statements of operations reflecting transition services provided to the Former Parent, of which $ 396 was included as a receivable in other current assets on the consolidated balance sheet as of December 31, 2023. For the year ended December 31, 2022, the Company recorded $ 4,024 in other income (expense), net on the consolidated statements of operations reflecting transition services provided to the Former Parent, of which $ 2,748 was included as a receivable in other current assets on the consolidated balance sheet as of December 31, 2022.

United States Distribution Services Agreement. The Company entered into a United States Distribution Services Agreement with the Former Parent, pursuant to which the Company shall continue to serve as the Former Parent's distributor and agent for the distribution of the pharmaceutical product Nurtec ODT in the United States for a limited period of time following the Spin-Off. Under the Distribution Services Agreement, the Former Parent and Pfizer Inc. have agreed to indemnify the Company for, among other things, losses resulting from the conduct of the distribution business or actions taken at the direction of the Former Parent.

As the Company was acting as an agent of the Former Parent for services performed under the Distribution Services Agreement, no amounts for revenues or expenses relating to the services performed thereunder were included on the Company's consolidated financial statements. As of December 31, 2022, the Company recorded restricted cash held on behalf of Former Parent of $ 35,212 and Due to Former Parent of $ 35,212 on the consolidated balance sheet primarily relating to cash held in connection with the execution of the Distribution Services Agreement which was legally payable to the Former Parent. The Company did not hold any restricted cash on behalf of the Former Parent as of December 31, 2023.

Outsourcing & Employee Transfer Agreements. The Company entered into Outsourcing & Employee Transfer Agreements, one with Pfizer Inc., Bulldog (BVI) Ltd., the Former Parent and Biohaven Pharmaceuticals, Inc. ("U.S. Employer"), and the other with Pfizer Inc., Bulldog (BVI) Ltd., the Former Parent, and BioShin (Shanghai) Consulting Services Co., Ltd. ("Chinese Employer"), pursuant to which the Chinese Employer and the U.S. Employer will, among other things, provide Pfizer Inc. with the services of, and remain the employers of, certain of their employees for a limited period of time following the Spin-Off. During such period, Pfizer Inc. or one of its affiliates will pay the U.S. Employer for employee-related expenses for its employees (including the cost of salary and wages) and will pay the Chinese Employer a service fee based on employee-related expenses for its employees (including the cost of salary and wages).

Amounts received in connection with the Outsourcing & Employee Transfer Agreements are recorded against their related operating expenses as they represent reimbursements for operating expenses incurred by the Company on behalf of the Former Parent.

Relationship with the Former Parent prior to the Separation

Pursuant to the Distribution Agreement, immediately prior to the Separation the Former Parent made a cash contribution to the Company which

(Amounts in thousands, except share and per share amounts)

14. Related Party Transactions (Continued)

resulted in a cash balance of approximately $ 257,799 as of October 3, 2022.

Prior to the Separation, the Company did not historically operate as a standalone business and the consolidated financial statements are derived from the consolidated financial statements and accounting records of the Former Parent. The following disclosure summarizes activity between the Company and the Former Parent prior to the Separation, including the affiliates of the Former Parent that were not part of the Spin-Off.

Cost Allocations

The consolidated financial statements for periods prior to the Separation reflect allocations of certain expenses from the financial statements of the Former Parent, including research and development expenses and general and administrative expenses. These allocations include, but are not limited to, executive management, employee compensation and benefits, facilities and operations, information technology, business development, financial services (such as accounting, audit, and tax), legal, insurance, and non-cash share-based compensation.

For periods prior to the Separation, these allocations to the Company are reflected in the consolidated statement of operations as follows:

	Year Ended December 31,	
	2022	2021
Research and development	$ 146,521	$ 70,929
General and administrative	82,744	33,928
Total	$ 229,265	$ 104,857

Management believes these cost allocations are a reasonable reflection of services provided to, or the benefit derived by, the Company during the periods presented. The allocations may not, however, be indicative of the actual expenses that would have been incurred had the Company operated as a standalone public company. Actual costs that may have been incurred if the Company had been a standalone public company would depend on a number of factors, including the chosen organizational structure, what corporate functions the Company might have performed directly or outsourced and strategic decisions the Company might have made in areas such as executive management, legal and other professional services, and certain corporate overhead functions.

Non-Cash Share-Based Compensation

As discussed in Note 9, "Non-Cash Share-Based Compensation," prior to the Separation, Biohaven employees participated in the Former Parent's non-cash share-based compensation plans, the costs of which, including those related to the Acceleration, have been allocated to the Company and recorded in research and development and general and administrative expenses in the consolidated statements of operations for periods prior to the Separation.

(Amounts in thousands, except share and per share amounts)

14. Related Party Transactions (Continued)

Net Transfers From Former Parent

Net transfers from Former Parent represent the net effect of transactions between the Company and the Former Parent prior to the Separation. The components of net transfers from Former Parent are as follows:

	Year Ended December 31,	
	2022[1]	2021
General financing activities	$ 399,231	$ 98,834
Corporate cost allocations, excluding non-cash share-based compensation	49,899	39,218
Net transfers from Former Parent as reflected in the Consolidated Statement of Cash Flows	449,130	138,052
Non-cash share-based compensation	179,367	65,639
Issuance of Former Parent common shares to repurchase non-controlling interest in a subsidiary	60,000	—
Issuance of Former Parent common shares for building purchase	—	4,871
Issuance of Former Parent common shares as payment for IPR&D asset acquisition	58,747	—
Issuance of Former Parent common shares as payment for business acquisition	—	10,673
Issuance of Former Parent common shares as payment for Artizan investment	—	6,000
Issuance of Former Parent common shares as payment for license and consulting agreements	1,779	7,929
Separation related adjustments[2]	27,811	—
Other non-cash adjustments	(204)	(1,458)
Net transfers from Former Parent as reflected in the Consolidated Statement of Changes in Equity	$ 776,630	$ 231,706

(1) The amounts for the year ended December 31, 2022 represent activity through the date of Separation.

(2) Refer to Note 1, "Nature of the Business and Basis of Presentation," for further details on separation related adjustments.

Related Party Agreements

License Agreements with Yale

On September 30, 2013, the Company entered into the Yale Agreement with Yale (see Note 11, "License Agreements," for details). The Company's Chief Executive Officer is one of the inventors of the patents that the Company has licensed from Yale and, as such, is entitled to a specified share of the glutamate product-related royalty revenues that may be received by Yale under the Yale Agreement.

In January 2021, the Company entered into the Yale MoDE Agreement with Yale (see Note 11, "License Agreements," for details). Under the license agreement, the Company acquired exclusive, worldwide rights to Yale's intellectual property directed to its MoDE platform. As part of consideration for this license, the Company paid Yale University an upfront cash payment of $ 1,000 and 11,668 common shares of the Former Parent valued at approximately $ 1,000 . Under the Yale MoDE Agreement, the Company entered into the Yale MoDE SRA (see Note 11, "License Agreements," for details), which included funding of up to $ 4,000 over the life of the agreement. In May 2023, the Company entered into an additional sponsored research agreement with Yale University (the "2023 Yale SRA"), which includes funding of up to $ 612 over the life of the agreement.

For the years ended December 31, 2023, 2022 and 2021, the Company recorded $ 2,942 , $ 3,420 and $ 458 , respectively, in research and development expense, including certain administrative expenses, related to the Yale MoDE Agreement and the Yale MoDE SRA, the Yale Agreement, and the 2023 Yale SRA (the "Yale Agreements"). As of December 31, 2023, the Company did not owe any amounts to Yale.

Exhibit 10.22

Execution Version

December 10, 2023

Hangzhou HighlightII Pharmaceutical Co. Ltd.
RM 301/302, BLDG 4, Hexiang Sci & Tech Center,
Qiantang District
Hangzhou 310018, China

Re: Amendment No. 2 to Development and License Agreement dated March 21, 2023

Dear Sirs;

We refer to the Development and License Agreement dated March 21, 2023 between Biohaven Therapeutics Ltd. ("Biohaven") and Hangzhou HighlightII Pharmaceutical Co. Ltd. ("HighlightII") relating to dual TYK2/JAK1 tyrosine kinase inhibitors, as amended ("TYK2/JAK1 License Agreement"). Capitalized terms shall have the meanings ascribed to them in the TYK2/JAK1 License Agreement unless otherwise defined herein.

On October 18, 2023, the FDA issued a letter ("October FDA Letter") to Biohaven indicating [***].

The issuance of the October FDA Letter [***] set forth in Sections 4.1 and 4.2 of theTYK2/JAK1 License Agreement regarding the payment of the upfront fee and issuance of the common stock in Biohaven Ltd. (NYSE:BHVN). The Parties desire to provide for the payment of the upfront fee and issuance of the License Shares, upon execution of this Amendment No. 2 [***].

The Parties also desire to grant Biohaven an option to acquire a license to Highlightll's Selective TYK2 Inhibitor (defined below) and clarify the scope of the competitive activities with respect to the Selective TYK2 Inhibitors.

Accordingly, we propose to amend the TYK2/JAK1 License Agreement as follows.

New Section 1.66 is added as follows:

1.66 "**Selective TYK2 Inhibitor**" means a tyrosine kinase ("TKY2") inhibitor with: (i) Ki <200nM activity inhibition in the Assay (for both allosteric and non-allosteric Tyk2 inhibitors, (ii) Brain Penetration greater than or equal to ten percent (10%) and (iii) 20x times greater affinity to TYK2 in a cell free assay (ATP dependent and allosteric assays) over Jak1, Jak2 and Jak3, and over 50x all other off-targets (including kinome and Eurofins secondary pharmacology panels).

New Section 2.1.4. is added as follows:

2.1.4. Highlightll hereby grants to Biohaven an exclusive option and right of first refusal to acquire from Highlightll an exclusive license to any Selective TYK2 Inhibitors that are being developed by or on behalf of Highlightll or its Affiliates ("the TYK2 License"). The terms and conditions for the TYK2 License (including but not limited to financial terms, the Field and Territory, and non-compete clauses) shall be equivalent to the terms and conditions under TYK2/JAK1 License Agreement. The option shall begin [***] days after the date that Highlightll [***], and the option shall expire [***] days thereafter [***].

From the effective date of this Amendment No. 2 to the expiration of the [***] neither Biohaven nor its Affiliates shall itself or through any Third Party, or in collaboration with any Third Party, engage, directly or indirectly in the Development of a Selective TYK2 Inhibitor prior to the expiration of the [***].

From the effective date of this Amendment No. 2 to the expiration of the [***] neither Highlightll nor its Affiliates shall itself or through any Third Party, or in collaboration with any Third Party, engage, directly or indirectly in the clinical Development of a Selective TYK2 Inhibitor prior to the expiration of the [***].

In consideration of making the above-described amendments to the TYK2/JAK1 License Agreement, the Parties further agree to the following:

- **Upfront Payment**. Within two (2) Business Days after execution of this Amendment No. 2 by the Parties, Biohaven shall cause its Affiliate, Biohaven Pharmaceuticals, Inc. ("BPI"), to provide instructions to the Escrow Agent (defined in the Escrow Agreement between BPI, Highlightll and JPMorgan Chase Bank dated April 14, 2023, "Escrow Agreement") to release the Fund (as defined in the Escrow Agreement) to Highlightll.
- **Equity.** Biohaven shall cause its Affiliate, Biohaven Ltd., to issue to HighlightII or its designated Affiliate, within fifteen (15) Business Days after execution of this Amendment No. 2 by the Parties, 721,136 Biohaven Ltd.'s common shares ("BHVN Shares"). The issuance of the BHVN Shares to HighlightII shall be governed by a private placement agreement between Biohaven Ltd. and HighlightII pursuant to Rule 144 under the Securities Act of 1933.

Please indicate your agreement to this Amendment No. 2 by countersigning this letter below, effective as of the date of this letter.

Very truly yours;

/s/ Warren Volles

Biohaven Therapeutics Ltd.

By: Warren Volles

Title: Chief Legal Officer

Agreed:

/s/ Chris Liang

Hangzhou Highlightll Pharmaceutical Co. Ltd.

By: Chris Liang, Ph.D

Title: Chief Executive Officer

Exhibit 21.1

SUBSIDIARIES OF BIOHAVEN LTD.

As of December 31, 2023

Name	Jurisdiction of Incorporation
Biohaven Specialty Pharmaceutical Ltd.	British Virgin Islands
Biohaven Therapeutics Ltd.	British Virgin Islands
Biohaven Pharmaceuticals, Inc.	Delaware
BioShin Limited	Cayman Islands
BioShin Hong Kong Limited	Hong Kong
BioShin (Shanghai) Consulting Services Co., Limited	China
Biohaven Bioscience Ireland Limited	Ireland
Biohaven Therapeutics IP Ltd.	British Virgin Islands
Biohaven CGRP IP Ltd.	British Virgin Islands
BioShin (Singapore) PTE. LTD.	Singapore
Kleo Pharmaceuticals, Inc.	Delaware
Kleo Pharmaceuticals Pty Ltd.	Australia
PharmaHaven Ltd.	British Virgin Islands

Exhibit 23.1

CONSENT OF INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM

We consent to the incorporation by reference in the following Registration Statements:

1. Registration Statement (Form S-3 No. 333-274822) of Biohaven Ltd.,
2. Registration Statement (Form S-8 No. 333-267818) pertaining to the Biohaven Ltd. 2022 Equity Incentive Plan, Biohaven Ltd. 2022 Employee Share Purchase Plan, and Biohaven Ltd. Legacy Equity Award Settlement Plan, and
3. Registration Statement (Form S-8 No. 333-271886) pertaining to the Biohaven Ltd. 2022 Equity Incentive Plan and Biohaven Ltd. 2022 Employee Share Purchase Plan;

of our reports dated February 29, 2024, with respect to the consolidated financial statements of Biohaven Ltd. and the effectiveness of internal control over financial reporting of Biohaven Ltd. included in this Annual Report (Form 10-K) of Biohaven Ltd. for the year ended December 31, 2023.

/s/ Ernst & Young LLP

Hartford, Connecticut
February 29, 2024

Exhibit 31.1

CERTIFICATION OF PRINCIPAL EXECUTIVE OFFICER
PURSUANT TO SECTION 302 OF THE SARBANES-OXLEY ACT OF 2002

I, Vlad Coric, certify that:

1. I have reviewed this Annual Report on Form 10-K for the year ended December 31, 2023 of Biohaven Ltd. (the "registrant");

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(s) 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(s) and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

    a. All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

    b. Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: February 29, 2024

/s/ VLAD CORIC, M.D.

Vlad Coric, M.D.
President and Chief Executive Officer
(principal executive officer)

Exhibit 31.2

CERTIFICATION OF PRINCIPAL FINANCIAL OFFICER
PURSUANT TO SECTION 302 OF THE SARBANES-OXLEY ACT OF 2002

I, Matthew Buten, certify that:

1. I have reviewed this Annual Report on Form 10-K for the year ended December 31, 2023 of Biohaven Ltd. (the "registrant");

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(s) 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(s) and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

    a. All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

    b. Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: February 29, 2024

/s/ MATTHEW BUTEN

Matthew Buten

Chief Financial Officer

(principal financial officer)

Exhibit 32.1

CERTIFICATIONS OF
PRINCIPAL EXECUTIVE OFFICER AND PRINCIPAL FINANCIAL OFFICER
PURSUANT TO 18 U.S.C. SECTION 1350,
AS ADOPTED PURSUANT TO
SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002

Pursuant to the requirement set forth in Rule 13a-14(b) of the Securities Exchange Act of 1934, as amended, (the "Exchange Act") and Section 1350 of Chapter 63 of Title 18 of the United States Code (18 U.S.C. §1350), Vlad Coric, M.D., President and Chief Executive Officer of Biohaven Ltd. (the "Company"), and Matthew Buten, Chief Financial Officer of the Company, each hereby certifies that, to the best of his knowledge:

1. The Company's Annual Report on Form 10-K for the year ended December 31, 2023, to which this Certification is attached as Exhibit 32.1 (the "Periodic Report"), fully complies with the requirements of Section 13(a) or Section 15(d) of the Exchange Act; and

2. The information contained in the Periodic Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

IN WITNESS WHEREOF, the undersigned have set their hands hereto as of the 29 day of February 2024.

/s/ VLAD CORIC, M.D.	/s/ MATTHEW BUTEN
Vlad Coric, M.D.	Matthew Buten
President and Chief Executive Officer	*Chief Financial Officer*
(principal executive officer)	*(principal financial officer)*

* This certification accompanies the Form 10-K to which it relates, is not deemed filed with the Securities and Exchange Commission and is not to be incorporated by reference into any filing of the Company under the Securities Act of 1933, as amended, or the Exchange Act (whether made before or after the date of the Form 10-K), irrespective of any general incorporation language contained in such filing.

BIOHAVEN LTD.

INCENTIVE COMPENSATION RECOVERY POLICY

I. Purpose

The Board of Directors (the "**Board**") of Biohaven Ltd. (the "**Company**") believes that it is in the best interests of the Company and its shareholders to create and maintain a culture that emphasizes integrity and accountability. The Board therefore adopts this Incentive Compensation Recovery Policy (this "**Policy**") which requires the recoupment of certain executive compensation in accordance with the terms herein. This Policy is designed to comply with, and shall be interpreted in light of, the requirements of Section 10D of the Securities Exchange Act of 1934, as amended (the "**Exchange Act**"), Rule 10D-1 adopted thereunder and the applicable listing rules of the New York Stock Exchange ("**NYSE**").

II. Administration

This Policy shall be administered by the Compensation Committee of the Board or, in the discretion of the Board, any other committee or body of the Board consisting only of independent directors (the "**Committee**"). All determinations and interpretations made by the Committee shall be final, binding and conclusive.

III. Covered Persons

This Policy is applicable to any person who Receives Excess Compensation (each, a "**Covered Person**"). For the avoidance of doubt, a Cover Person includes any person who Received Excess Compensation during the Applicable Period but is no longer an employee of the Company at the time the determination to recoup compensation is made.

IV. Defined Terms

The following terms shall have the meanings set forth below for purposes of this Policy:**"Accounting Restatement"** means an accounting restatement of any of the Company's financial statements due to the Company's material noncompliance with any financial reporting requirement under U.S. securities laws, including any required accounting restatement to correct an error in previously issued financial statements that is material to the previously issued financial statements (often referred to as a "Big R" restatement), or that would result in a material misstatement if the error were corrected in the current period or left uncorrected in the current period (often referred to as a "little r" restatement). An Accounting Restatement does not include situations in which financial statement changes did not result

from material non-compliance with financial reporting requirements, such as, but not limited to, retrospective: (i) application of a change in accounting principles; (ii) revision to reportable segment information due to a change in the structure of the Company's internal organization; (iii) reclassification due to a discontinued operation; (iv) application of a change in reporting entity, such as from a reorganization of entities under common control; or (v) revision for stock splits, stock dividends, reverse stock splits or other changes in capital structure. An Accounting Restatement also does not include out-of-period adjustments that are immaterial to both the current and prior periods. The determination of whether the restatement is due to material non-compliance with any financial reporting requirement shall be based on facts and circumstances and existing judicial and administrative interpretations.

"**Applicable Period**" means the three completed fiscal years preceding the earlier of: (i) the date that the Board, a committee of the Board, or the officer or officers of the Company authorized to take such action if Board action is not required, concludes, or reasonably should have concluded, that the Company is required to prepare an Accounting Restatement; or (ii) the date a court, regulator, or other legally authorized body directs the Company to prepare an Accounting Restatement. To the extent required under Section 303A.14 of the NYSE Listed Company Manual or other Exchange listing rules, the Applicable Period also includes any transition period that results from a change in the Company's fiscal year within or immediately following those three completed fiscal years.

"**Excess Compensation"** means all Incentive-based Compensation (calculated on a pre- tax basis) Received by a person: (i) after beginning service as an Executive Officer; (ii) who served as an Executive Officer at any time during the performance period for that Incentive- based Compensation; (iii) while the Company had a class of securities listed on an Exchange; and (iv) during the Applicable Period, that exceeded the amount of Incentive-based Compensation that otherwise would have been Received had the amount been determined based on the Financial Performing Measures as reflected in the Accounting Restatement. With respect to Incentive-based Compensation based on stock price or total shareholder return ("TSR"), when the amount of erroneously awarded compensation is not subject to mathematical recalculation directly from the information in an accounting restatement, the amount will be based on the Committee's reasonable estimate of the effect of the Accounting Restatement on the stock price or TSR upon which the Incentive-based Compensation Received by the Covered Person originally was based.

"**Exchange**" means any national securities exchange or national securities association on which the Company has a class of securities listed.

"Executive Officer" means the Company's president, principal financial officer, principal accounting officer (or if there is no such accounting officer, the controller), any vice-president of the Company in charge of a principal business unit, division, or function (such as sales, administration, or finance), any other officer who performs a policy-making function, or any other person (including any executive officer of the Company's subsidiaries or affiliates) who performs similar policy-making functions for the Company.

"Impracticable" means, after exercising a normal due process review of all the relevant facts and circumstances and taking all steps required by Exchange Act Rule 10D-1 and any applicable Exchange listing standard, the Committee determines that recovery of the Excess Compensation is impracticable because: (i) it has determined that the direct expense that the Company would pay to a third party to assist in enforcing this Policy and recovering the Excess Compensation otherwise recoverable would exceed the amount to be recovered; (ii) it has concluded that the recovery of the Excess Compensation would violate home country law adopted prior to November 28, 2022; or (iii) it has determined that the recovery of the Excess Compensation would cause an otherwise tax-qualified retirement plan, under which benefits are broadly available to the Company's employees, to fail to meet the requirements of 26 U.S.C. 401(a)(13) or 26 U.S.C. 411(a) and regulations thereunder.

"**Incentive-based Compensation**" means any compensation that is granted, earned, or vested based wholly or in part upon the attainment of a Financial Reporting Measure; however it does not include: (i) base salaries; (ii) discretionary cash bonuses; (iii) awards (either cash or equity) that are based upon subjective, strategic or operational standards; and (iv) equity awards that vest solely based on the passage of time.

"**Financial Reporting Measure**" means a measure that is determined and presented in accordance with the accounting principles used in preparing the Company's financial statements, and any measure that is derived wholly or in part from such measures (including "non-GAAP" financial measures, such as those appearing in earnings releases); provided, however, that any such measure need not be presented within the Company's financial statements or included in a filing made with the Securities and Exchange Commission. Examples of Financial Reporting Measures include measures based on: revenues, net income, operating income, financial ratios, EBITDA, liquidity measures (such as free cash flow), return measures (such as return on assets or return on invested capital), profitability of one or more segments, and cost per employee. Stock price and TSR also are Financial Reporting Measures.

"**Received**": Incentive-based Compensation is deemed "Received" in any Company fiscal period during which the Financial Reporting Measure specified in the Incentive-based Compensation award is attained, even if the payment or grant of the Incentive-based Compensation occurs after the end of that period.

V. Recoupment

In the event that the Company is required to prepare an Accounting Restatement, then the Committee shall, unless the Committee determines it to be Impracticable, take reasonably prompt action to recover all Excess Compensation from any Covered Person, such recovery to be on a "no fault" basis and without regard to whether any misconduct occurred or to a Covered Person's responsibility for the noncompliance that lead to the Accounting Restatement. The Company's obligation to recover Recoverable Compensation is not dependent on if or when the restated financial statements are filed.

VI. Method of Recovery

The Committee will determine, in its sole discretion, the form and method for recovering Excess Compensation hereunder which may include, without limitation: (a) requiring reimbursement in cash of Excess Compensation previously paid; (b) seeking recovery of any gain realized on the vesting, exercise, settlement, transfer or other disposition of any equity- based awards; (c) offsetting the recovered amount from any compensation otherwise owed by the Company to the Covered Person; (d) cancelling outstanding vested or unvested equity awards; (e) reducing future compensation or (f) taking any other remedial and recovery action or combination of actions permitted by law, as determined by the Committee.

VII. No Indemnification

Notwithstanding the terms of any of the Company's organizational documents, any corporate policy or any contract, no Covered Person shall be indemnified against the loss of any Excess Compensation.

VIII. Other Recoupment Rights; Prior Policy

Any right of recovery under this Policy is in addition to, and not in lieu of, any other remedies or rights of recovery or recoupment that may be available to the Company pursuant to the terms of any similar policy in any employment agreement, severance or change in control agreement, equity award agreement or similar agreement and any other legal remedies available to the Company.

This Policy supersedes and replaces the Company's Clawback Policy approved by the Board on September 29, 2022 (the "**Prior Policy**"), except that the Prior Policy and not this Policy shall continue to apply to Incentive-based Compensation Received prior to October 2, 2023.

IX. Successors

This Policy shall be binding and enforceable against all Covered Persons and their beneficiaries, heirs, executors, administrators or other legal representatives.

Adopted by the Board of Directors on August 8, 2023.