UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
Washington, D.C. 20549

FORM 10-K

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

For the Fiscal Year Ended September 30, 2022

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 001-35963

NEUBASE THERAPEUTICS, INC.

(Exact name of registrant as specified in its charter)

Delaware	**46-5622433**
(State or other jurisdiction of incorporation or organization)	(I.R.S. Employer Identification No.)

350 Technology Drive, Pittsburgh, PA 15219
(Address of principal executive offices and zip code)

(412) 763-3350
(Registrant's telephone number, including area code)

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

Title of each class	Trading Symbol(s)	Name of each exchange on which registered
Common Stock, $0.0001 par value per share	NBSE	The Nasdaq Stock Market LLC

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 ☐ No ☒

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

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

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

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

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

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

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

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

The aggregate market value of the voting stock held by non-affiliates of the registrant on the last business day of the registrant's most recently completed second fiscal quarter: $ 57.9 million based upon the closing sale price of our common stock of $1.88 on that date. Common stock held by each officer and director and by each person known to own in excess of 10% of outstanding shares of our common stock has been excluded in that such persons may be deemed to be affiliates. The determination of affiliate status for this purpose is not necessarily a conclusive determination for other purposes.

As of December 16, 2022, 33,008,657 shares of the common stock, par value $0.0001, of the registrant were outstanding.

DOCUMENTS INCORPORATED BY REFERENCE

None.

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As previously disclosed on July 12, 2019, Ohr Pharmaceutical, Inc., a Delaware corporation ("Ohr"), completed a Merger with NeuBase Therapeutics, Inc., a Delaware corporation ("Legacy NeuBase"), in accordance with the terms of the Agreement and Plan of Merger Reorganization (the "Merger Agreement") entered into on January 2, 2019. Pursuant to the Merger Agreement, (i) a subsidiary of Ohr merged with and into Legacy NeuBase, with Legacy NeuBase (renamed as "NeuBase Corporation") continuing as a wholly-owned subsidiary of Ohr and the surviving corporation of the merger and (ii) Ohr was renamed as "NeuBase Therapeutics, Inc." (the "Merger").

Unless the context otherwise requires, references to the "Company," the "combined company," "we," "our" or "us" in this Form 10-K refer to NeuBase Therapeutics, Inc. and its subsidiaries, references to "NeuBase" refer to the Company following the completion of the Merger and references to "Ohr" refer to the Company prior to the completion of the Merger.

Except as otherwise noted, references to "common stock" in this Form 10-K refer to common stock, par value $0.0001 per share, of the Company.

PART I.

Cautionary Note Regarding Forward-Looking Statements

This Form 10-K contains "forward-looking statements" that involve risks and uncertainties, as well as assumptions that, if they never materialize or prove incorrect, could cause our results to differ materially and adversely from those expressed or implied by such forward-looking statements. The forward-looking statements are contained principally in Item 1—"Business," Item 1.A—"Risk Factors" and Item 7—"Management's Discussion and Analysis of Financial Condition and Results of Operations," but appear throughout this Form 10-K. Examples of forward-looking statements include, but are not limited to, our expectations, beliefs or intentions regarding our potential product offerings, business, financial condition, results of operations, strategies or prospects and other matters that do not relate strictly to historical facts or statements of assumptions underlying any of the foregoing. These statements are often identified by the use of words such as "anticipate," "believe," "continue," "could," "estimate," "expect," "intend," "may," "ongoing," "opportunity," "plan," "potential," "predicts," "seek," "should," "will," or "would," and similar expressions and variations or negatives of these words. These forward-looking statements are based on the expectations, estimates, projections, beliefs and assumptions of our management based on information currently available to management, all of which are subject to change. Such forward-looking statements are subject to risks, uncertainties and other factors that are difficult to predict and could cause our actual results and the timing of certain events to differ materially and adversely from future results expressed or implied by such forward-looking statements. Factors that could cause or contribute to such differences include, but are not limited to, those identified below and those discussed under Item 1.A—"Risk Factors" in this Form 10-K. Furthermore, such forward-looking statements speak only as of the date of this Form 10-K. We undertake no obligation to update or revise publicly any forward-looking statements to reflect events or circumstances after the date of such statements for any reason, except as otherwise required by law.

ITEM 1. BUSINESS

Overview

NeuBase Therapeutics, Inc. ("NeuBase","Company", "we", "us" and "our") is a biotechnology company focused on significantly reducing the burden of untreatable morbidity and mortality across the globe caused by rare and common diseases.

To achieve this goal, we have designed, built, and validated a new precision genetic medicines platform technology able to uniquely drug the double-stranded human genome and address disease at the root of causality without many of the limitations of early precision genetic medicine technologies.

Overview

Most diseases remain undruggable with current therapeutic modalities, leaving millions of patients with limited options. These include rare diseases, cancers, common chronic and infectious diseases. Most diseases are genetic, in whole or in part, underscoring the critical importance of new medicines that can drug the human genome for the future of health. Yet the genome has been difficult to target with therapies due to its double-stranded structure, which evolved to protect the fidelity of this essential blueprint of life.

Genes in the genome are "transcribed" into RNA which are then usually "translated" into proteins. As a result of the historical inability to drug the genome directly to address root causality, the pharmaceutical industry moved downstream, initially working to drug the protein products of genes. The complexity associated with drugging proteins, each of which is a unique and often dynamic molecular entity, has resulted in a drug development process that is commonly inefficient, time-consuming, and expensive with low probabilities of success. This strategy has, in part, resulted in high drug prices and a high remaining burden of unmet patient need.

These issues could potentially be resolved by targeting the genetic material itself, instead of downstream protein products. Precision genetic medicines represent a relatively new class of therapies that target genetic sequences that are the root cause of diseases. While early precision genetic medicines companies have proven that human disease can be addressed at the genetic level, the nascent field remains fragmented with most technologies only able to address a single causal genetic mechanism (either gain-of-function, change-of-function, or loss-of-function of a gene). Most technologies still act downstream at the RNA level and exhibit limitations relating to delivery, tolerability, selectivity, manufacturability, durability of effect and scalability.

We have designed, built, and validated a new technology platform that can uniquely Drug the Genome™ to address the three disease-causing mechanisms (i.e., gain-of-function, change-of-function, or loss-of-function of a gene) without the limitations of early precision genetic medicines. Our technology is predicated on synthetic peptide-nucleic acid ("PNA") chemistry and can directly engage the genome in a sequence-specific manner and potentially address root causality of diseases. These compounds operate by temporarily engaging the genome (or single and double-stranded RNA targets, if desired) and interacting with cellular machinery that process mutant genes to halt their ability to manifest a disease. We have now repeatedly demonstrated, in proof-of-concept preclinical animal studies the ability to address multiple disease-causing genes and different causal mechanisms to resolve the disease state without the limitations of early genetic medicine technologies. These limitations, and the data that illustrates that we have likely engineered them out of our platform to potentially unlock broad impact across many diseases, are:

- **Delivery.** Most early precision genetic medicine technologies are large and heavily negatively charged, making it difficult for them to broadly distribute throughout the body to address tissues that are affected by many diseases. This size and charge profile often requires these early genetic medicines to be locally injected, such as into the brain, likely limiting their ability for broad-based impact. The Company has developed a new type of genetic medicine that has low molecular weight, is neutrally charged, and water-soluble, which are features that facilitate transport throughout tissues. We have designed and developed a proprietary delivery technology that allows these compounds to be administered using a patient-friendly route, such as subcutaneous injection, and achieve broad biodistribution, including into the deep brain and nuclei of cells.

Validating data. Preclinical non-human primate ("NHP") data presented in FY2020 illustrated the ability of a 5mg/kg systemic injection of the delivery shuttle to distribute into every tissue examined at therapeutically relevant concentrations, defined as concentrations which show activity in patient-derived cell lines when coupled to a pharmacophore. In FY2021, we presented data illustrating that the delivery shuttle conjugated to a NT-0200 program compound could be injected subcutaneously into the HSA^{LR} transgenic animal model of myotonic dystrophy type 1 ("DM1") and achieve both molecular and functional rescue of myotonia in the distant tibialis anterior muscle group in the transgenic animal. Also, in FY2021 we presented data that illustrate that subcutaneous injection of the same delivery shuttle conjugated to a compound for the NT-0100 program into the zQ175 transgenic animal model of Huntington's disease ("HD") could, within 7 days from first dose, cross the blood-brain barrier in sufficient concentrations to engage the mutant gene target and reduce the resultant protein levels from whole brain isolates to a statistically significant degree. Delivery of drugs across the blood-brain barrier and into muscle via a systemic route has been a significant challenge in the industry. More recently, in FY2022 we presented data illustrating that a single 30 mg/kg intravenous injection of our lead DM1 development candidate (NT-0231.F) in wild-type BALB/c mice was cleared rapidly from the systemic compartment and demonstrated rapid and wide distribution into the tibialis anterior muscle, heart muscle, and brain tissues. NT-0231.F rapidly cleared the plasma, and each tissue evaluated displayed an extended elimination phase with tissue concentrations measurable for at least four weeks following a single intravenous dose administration. Additionally, in the fourth quarter of calendar year 2022 we completed a three-month pharmacokinetic ("PK") study of NT-0231.F in NHPs illustrating that we could achieve pharmacologically active concentrations to skeletal muscles after a single intravenous (IV) injection of 6 mg/kg and 14 mg/kg. Also in FY2022, we presented data illustrating that twice-weekly subcutaneous injections over an 8-week period of a NT-0100 program compound into the R6/2 Huntington's disease transgenic animal model were able to penetrate into the brain and reduce mutant Huntingtin protein levels throughout all brain regions with statistical significance.

- **Tolerability.** Most early precision genetic medicine technologies trigger the innate and/or acquired immune system, limiting their ability to achieve pharmacologic doses or to be used repeatedly. For example, delivery of negatively charged nucleic acid therapies often trigger the innate immune system and delivery of proteins often trigger the acquired immune system. Our technology is comprised of fully synthetic compounds that have been shown to be "immunologically inert", potentially allowing them to be administered chronically to temporarily Drug the Genome™ over a patient's lifetime.

  Validating data. We have illustrated good tolerability of a variety of compounds across our various programs in rodent and NHP animal models across FY2020, FY2021 and FY2022, with both single and multi-dose administration. This data includes a lack of an obvious acute immune response in NHPs which has been seen with other early precision genetic medicines. Most recently, we completed a series of exploratory toxicology studies with our lead DM1 candidate, NT-0231.F, that illustrated a lack of cytokine activity in NHPs after IV injection at and below the maximum tolerated dose, negative Ames and *in vitro* micronucleus tests, and a maximum tolerated dose in NHPs and rats projected to be in excess of the projected human equivalent dose.

- **Selectivity.** Many technologies in the early precision genetic medicines industry cannot discriminate between mutant gene sequences and their healthy ("wild-type") counterparts, nor between other highly similar target sequences in the cell. This potentially limits these technologies in their ability to address small disease-causing mutations such as single nucleotide changes ("point mutations"), which account for a large fraction of disease-causing mutations and functional variants. Our technology can discriminate point mutations, which increases the program opportunity space. This capability comes from the "rigid" nature of the backbone, which does not tolerate imperfect target engagement. In addition, this single-base selectivity reduces the likelihood that our compounds will engage with genes elsewhere in the genome that are similar but not identical, potentially reducing any adverse events triggered by off-target engagement ("OTEs").

  Validating data. In FY2021, we presented data illustrating that we could achieve near-perfect selectivity in engaging the mutant G12D copy of the *KRAS* gene vs. the wild-type copy and inhibiting transcription and translation of the resultant mutant protein production*in vitro*. When these compounds are delivered to xenograft animal models with *KRAS*-mutation driven tumors, significant tumor growth reductions and signaling through downstream effectors of the KRAS pathway were measured. In FY2021, we also presented data that illustrated statistically significant allele-selective knock-down of mutant Huntingtin ("HTT") protein vs. the healthy wild-type protein after subcutaneous injection into the zQ175 transgenic animal model.

- **Manufacturability.** Many technologies in the early precision genetic medicines industry require significant investments in custom manufacturing infrastructure, and thus are limited in their potential impact and scalability. Our technology utilizes established and fully commoditized manufacturing processes, both for small molecule and synthetic peptide synthesis (the combination of which are required to manufacture our compounds) that are available with high redundancy and at commercial scale.

  Validating data. In FY2021, we nominated a development candidate for our DM1 program and initiated manufacturing scale up with several contract manufacturing organizations ("CMOs") across the globe. Within a period of months, for relatively low cost compared to the investment required to build bespoke manufacturing facilities, we established redundancy in manufacturing for GLP and GMP materials at scale.

- **Durability.** Many technologies in the early precision genetic medicines industry can only be dosed a single time, are often cleared by the immune system, or are otherwise not durable in their efficacy.

  Validating data. We illustrated in preclinical studies in FY2020 that our delivery shuttle, when delivered systemically into NHPs, is rapidly taken up into tissues across the body and is very slowly eliminated via the kidneys. Elimination of the delivery shuttle occurred at approximately 4% of the administered dose over the course of one week, promising an enduring response. More recently, in FY2022 we presented data illustrating that a single 30 mg/kg intravenous injection of NT-0231.F in wild-type BALB/c mice was cleared rapidly from the systemic compartment and demonstrated rapid and wide distribution into the tibialis anterior muscle, heart muscle, and brain tissues. NT-0231.F rapidly cleared the plasma, and each tissue evaluated displayed an extended elimination phase with tissue concentrations measurable for at least four weeks following a single intravenous dose administration. Most recently, we completed a single dose 3-month NHP PK study, which illustrated rapid uptake from the systemic compartment, robust exposures across a variety of tissues consistent with our mouse PK results, and tissue-specific elimination rates with near full clearance in most tissues examined.

- **Scalability.** Many technologies in the early precision genetic medicines industry are not truly scalable across a variety of indications, for the reasons described above. As our goal is to provide solutions to those suffering from a wide variety of diseases across the globe, we have purpose-built a scalable platform. We always address a single target type for all therapeutic programs (the genome), utilize predominantly the same chemistry, likely yielding similar therapeutic indices for the pharmacophore, are able to predict OTEs *a priori* using bioinformatics and engineer around them before beginning development, and leverage manufacturing process development across programs such that ongoing platform learnings have already created increasing speed and efficiency.

  Validating data. In FY2021, we launched a new set of programs in oncology targeting the most prevalent and currently undruggable *KRAS* mutations. The development of a series of hits was achieved with an order of magnitude higher efficiency than with our first two programs based on a deeper understanding of the structure-activity relationships uncovered in our initial programs and use of the same delivery shuttle for seamless *in vivo* pharmacologic testing.

As further validation of the PATrOL™ platform's capabilities, in FY2021, we described data illustrating that our first-in-class platform technology can address various types of causal insults by Drugging the Genome™ in animal models of a variety of human diseases after patient-friendly routes of administration and did so in a well-tolerated manner. In FY2022, we presented data illustrating that a single intramuscular dose of NT-0231.F is pharmacologically active in the muscle and drives molecular and functional rescue in the HSA^{LR} model, including splice rescue, nuclear aggregate resolution, and myotonia (delayed muscle relaxation after contraction) reversal. A single intravenous dose of NT-0231.F or multiple subcutaneous doses over a 28-day period broadly rescued splicing, including the chloride channel (Clcn1) transcript, and reversed myotonia in the model. A single intravenous dose of NT-0231.F provided initial splice rescue at around two weeks, with significant splice rescue around three weeks. Myotonia reversal was achieved at around four weeks, with effects enduring to at least six weeks. A time course of multiple subcutaneous doses across increasing concentrations of NT-0231.F was also investigated and showed splice rescue and myotonia reversal in a dose-responsive manner, illustrating feasibility of the differentiated and patient-friendly subcutaneous route.

The NeuBase Opportunity

Many diseases remain undruggable with current therapeutic modalities. These include rare diseases, cancers, common, chronic and infectious diseases. Most diseases are driven by functional genetic variants (including mutations). We are advancing first-in-class

ultra-precision genetic medicines to Drug the Genome™ and address disease at its root cause with the goal of helping potentially millions of patients who currently have no therapeutic options.

Historically, malfunctioning proteins (the products of malfunctioning genes) have been targeted by small molecules, antibodies, or protein replacement therapies to address disease states. The fundamental challenge with these strategies has been related to a lack of an ability to accurately model protein structures and the associated structural dynamics in an easy and high-throughput manner so that drugs could be intelligently designed. In addition, biologics have been hampered by delivery challenges, such as accessibility to the organ, cell type or subcellular compartment where the therapy is required to resolve functionality and by immunogenicity related to delivering a foreign antigenic therapy into the body. Recent advances in computational methods promise to enable accurate protein structure modeling in a high-throughput manner allowing intelligently designed protein-targeting medicines. While this potentially accelerates early target engagement activities, challenges related to accurately predicting and addressing structural dynamics of the target and off-target engagement with the multitude of other molecules in the body remain daunting. Delivery challenges and immunogenicity with large molecular weight biologics persist. The challenges of drug development targeting proteins have manifested themselves in high drug pricing in the market and large remaining unmet needs for patients.

In almost every human disease, a genetic variant is introduced into a cell, either through inheritance or *de novo* (for example, by a mutagen) at some point in development that causes the normal cellular processes to go awry. Diseases can either be completely driven by such a single genetic variant (termed a "mutation") or by individual or combinations of several variants (often referred to as "functional variants") often with environmental triggers.

Sequencing of the human genome set the stage for the advent of drugs that can target nucleic acids (DNA or RNA) upstream of proteins and closer to the root of disease causality. The world's collective knowledge of the human genome, and our ability to apply that knowledge to the development of medicines that can cure disease and mitigate suffering, has resulted in a new category of therapeutic commonly referred to as "precision genetic medicines." In the approximately 20 years since the scientific community has delivered the first draft of the human genome sequence, efforts across the globe have identified the genetic variants that cause thousands of different diseases and consolidated this variant information into accessible databases. In parallel, a molecular diagnostics industry has matured to enable many patients to get access to the precise genetic variant that causes their disease. Today, because of these advances, it is possible to know the gene and sequence variants that must be targeted for a specific disease indication, and it is also possible to identify patients who will benefit from these therapies, thus giving rise to the precision genetic medicines industry.

In parallel, a computational infrastructure has been developed that allows precision genetic medicines companies to house and query the genome to design medicines that precisely target genes that cause disease and reduce or eliminate engagement of these drugs with other highly similar sequences. Precision genetic medicines can target either DNA or RNA. Each cell in the human body contains two copies of the approximately 3 billion letter "haploid" genetic code, one copy inherited from each parent. At conception, a single fertilized egg, containing a complete approximately 6 billion letter "diploid" genome will contain all the necessary hereditary information to give rise to a complete human being. Each cell divides into daughter cells as an individual grows and develops and, through the cell division process, receives a replicate of the genome. Upon adulthood, a human being is comprised of trillions of individual nucleated cells, each of which is operating with a diploid genome that controls its function in the complete organism by activating various genes in various combinations at various times. Computational expertise and infrastructure have enabled the industry.

The human genome is double-stranded, an evolutionary mechanism thought to have arisen to protect this essential molecule against insult and degradation. This same protective mechanism has made directly drugging the genome difficult. Over the past decade, tremendous progress has been made in targeting genes, instead of proteins, to address diseases. Targeting nucleic acids allows one to utilize nature's own digital information encoding system, Watson-Crick nucleobase binding, which forms the basis of the double-helix of DNA, to engage an exact genetic target with a genetic medicine. Most early strategies target disease processes downstream of the double-stranded human genome primarily because early technologies cannot access the double-stranded genome. Thus, most precision genetic medicines focus on drugging RNA and are constrained to stretches of accessible single stranded portions of a target RNA.

Genes in the genome are "transcribed" into RNA molecules, which are processed by splicing events that remove intervening sequences ("introns") and often various combinations of protein coding sequences ("exons"), into mature messenger RNAs ("mRNA") to form a diversity of molecules from each gene. In addition, the rate of transcription of each gene in each cell type and at each time in development, balanced with the metabolism of each mRNA molecule, which often differs between transcripts, adds additional complexity to targeting RNA in disease conditions. These mRNA species are then generally translated into proteins. Each

mRNA molecule can be translated into many protein molecules before it is eventually degraded. After a protein is made, it often goes through a process of "post-translational" modification, which often trims the protein and/or adds modifications such as sugar or phosphate groups onto the proteins. Thus, a single mutant gene (present in either one or two copies in most diseases) outputs many different mRNA molecules and in turn, a diversity of protein molecules with additional structural and functional complexities derived from alternative splicing. Thus, we believe that grappling with disease at the protein or RNA level is inherently more complex than targeting the DNA itself, and due to our unique ability to engage the double-stranded genome to modulate gene output and/or function, we believe that we have a more scalable and differentiated opportunity.

Efforts to target genes that contain disease-causing variants at the RNA and DNA level have illustrated human clinical benefit in altering the natural history of several diseases. Genetic medicines generally fall into three separate categories, each being developed by various separate companies: gene silencing, gene replacement and gene editing. It is our belief that, unlike any other company to date, NeuBase has the potential to unify the field. Gene silencing is primarily centered on degrading the target transcript, most often using antisense oligonucleotides ("ASOs") or RNA interference ("RNAi"). Activating or replacing a gene is being done by gene therapy and mRNA replacement. Changing the function of a gene can be done using splice modification or emerging DNA and RNA editing technologies. These therapies have transformed care for a handful of genetic and infectious diseases and proven that precision genetic medicines can resolve disease upstream of proteins.

In general, addressing each of the three main pathogenic mechanisms is done using a distinct technology, though challenges have been encountered, such as delivery challenges due to the larger size and negatively charged backbones of first-generation genetic medicines therapies. For example, the scavenger receptors in the liver clear circulating negatively charged modified nucleic acids, and tolerability issues, such as immunogenicity, have been seen. While delivery modalities of first-generation genetic medicines continue to improve with, for example, antibody-mediated delivery, they also present new manufacturing and immunogenicity complexities. Delivery of non-permanent mRNA replacement technologies also continue to improve, promising the potential to eliminate the need to encapsulate transcripts to protect them from degradation during circulation, but tissue targeting challenges, immunogenicity and liver clearance issues will likely persist. Outside of delivery, tolerability and manufacturing challenges, first-generation genetic medicines have exhibited selectivity challenges as they are often unable to adequately discriminate between small mutations and normal gene sequences.

Medicines that target DNA are being developed in the community, with gene replacement and gene editing showing clinical successes. These approaches are meant to be permanent, which on the one hand would allow patients to live disease-free lives, but on the other hand pose challenges related to the level of rigor necessary to ensure mistakes do not occur. Gene replacement is generally currently done using viral vector-delivered gene therapies. Gene therapy can often be administered only once because patients exhibit an immune response against the viral delivery vectors. Further, gene therapy often results in heterogeneous cellular distribution. Non-viral delivery technologies are improving, which may allow DNA replacement strategies for loss-of-function conditions to mature more rapidly in the coming years. Gene editing technologies have also recently shown human *in vivo* clinical activity and early tolerability, but issues related to off-target editing and permanent modifications to the germ cells of patients warrant caution in developing gene editing solutions. Current strategies are also defined by delivering large molecular complexes, which are difficult to distribute and often include or likely produce antigenic protein complexes, relegating most gene editing activities to *ex vivo* cell editing and strategies, followed by cell therapy. Gene therapy and gene editing have limitations related to delivery of high molecular weight molecules and accessing tissues at the sites of pathology, immunogenicity, durability, and safety.

We have an opportunity, with our technology platform, to scale our proprietary genetic medicines that target mutant genes at their root cause by Drugging the Genome™. We have shown in preclinical proof-of-concept studies that the platform technology can address most causal mechanisms of disease at the genetic level by gene silencing, gene editing or gene activating, which leads to the potential of a unifying platform for the industry. Because we can drug the genome upstream of the complexity that occurs during transcription, splicing, translation and post-translational processes, and by using common chemistry to engage a common target type, there are opportunities to scale across indications.

In addition, we have demonstrated with preclinical data in several disease indications that our PATrOL™ platform-enabled precision genetic medicines have additional benefits that have limited other genetic medicines. We have successfully validated our PATrOL™ platform's ability to efficiently deliver genetic medicines with broad tissue distribution, including into the deep brain, and precisely engage DNA (and RNA) with a favorable safety profile and potential for sustained efficacy. These platform benefits include:

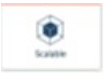
Broad biodistribution after systemic administration, including into all tissues examined at therapeutic concentrations: our compounds are water soluble, neutral in charge, which is tunable, and have low molecular weight, which facilitates broad distribution, including into the deep brain, after systemic administration.

Low potential for immunogenicity allowing for repeat chronic drugging of the genome: the chemistry that forms the workhorse of the platform has been extensively shown to be well tolerated in vivo at and above effective concentrations with no innate or acquired immune response.

Discriminates between mutant and healthy genes with single-base selectivity to reduce off-target effects: The ability to engage and bind to double-stranded DNA or single- and double-stranded RNA sequences enables better differentiation between mutant and healthy genes.

Leverages existing contract development and manufacturing organizations: our precision genetic medicines are made using commoditized small molecule and synthetic peptide manufacturing infrastructure, enabling cost-effective development and rapid commercial scaling.

Is stable in biological fluids and resistant to protease or nuclease digestion due to the synthetic chemistry, and when coupled to the proprietary delivery shuttle is rapidly taken up from the circulation into tissues where it resides and is slowly eliminated over time through the kidneys.



Most diseases are genetic, and the ability to reinvest process development and knowledge creates increasing speed and efficiencies.

We have designed and purpose-built a first-in-class technology platform and illustrated that the platform-enabled precision genetic medicines can precisely engage gene mutations and resolve root causality in three different diseases with different mechanisms of action, and in a well-tolerated manner.

Establishment of this novel platform has been our focus since initiation of the Company's operations in mid-CY2019. The process of building a first-in-class genetic medicines platform company is unique based on the technology being developed, and that process is articulated in Figure 1, which includes an overview of platform-related achievements in FY2022.

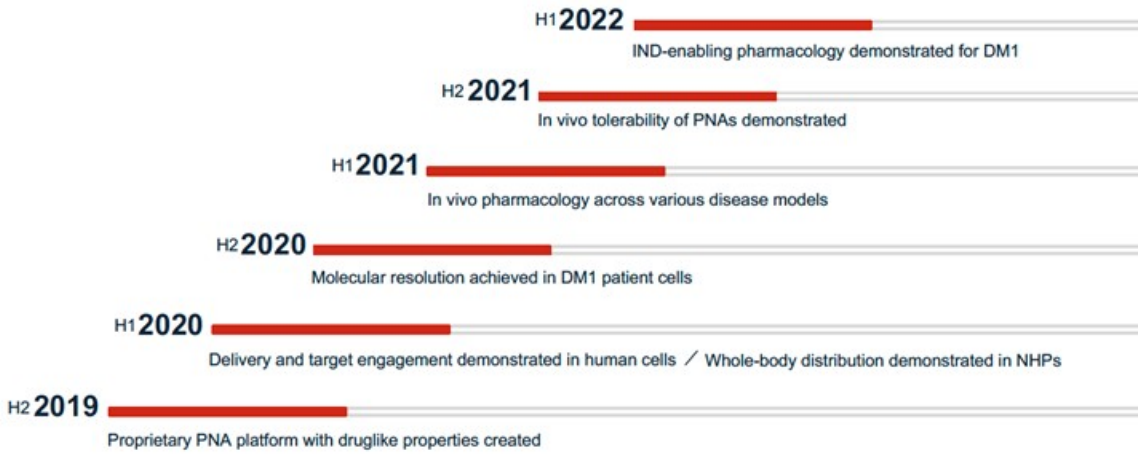


Figure 1. Major milestones achieved in establishing the first-in-class PATrOL™ platform, including development of a novel delivery shuttle that enables broad biodistribution including into the brain after systemic administration and culminating in multiple in vivo proof-of-concept data sets in high-value indications illustrating resolution of causality and tolerability. Each platform has a novel path to implementation and here we articulate the efforts over the first approximately 36 months of our operations.

While our precision genetic medicine pipeline currently includes programs in myotonic dystrophy type 1, Huntington's disease, and cancer (*KRAS* G12V and G12D mutations), in October 2022, we announced plans to expand the focus of our platform into gene editing, and we are identifying and evaluating multiple indications for potential development. Pipeline expansion into new disease areas uniquely addressable with our PATrOL™ platform is expected to continue.

The market need is enormous for a company that can address disease at a genetic level with a scalable technology platform. Globally, there are thousands of genetic diseases, most of which lack any therapeutic options. Most human diseases are genetic. Traditionally, therapeutic development for each disorder has been approached with a unique strategy, which is inefficient and has resulted in a large remaining unmet need and high drug prices in the marketplace. The collective population of people with untreatable diseases, numbering in the hundreds of millions, stands to potentially benefit profoundly from the emergence of a scalable and modular treatment development platform that allows for a more efficient discovery and delivery of drug product candidates to address these conditions cohesively.

In summary, as a result of the Human Genome Project, global efforts to catalog genetic variants that cause or predispose to human disease, and genetic testing infrastructure to enable patients to avail themselves of precision genetic medicines, the stage is now set for us to begin development efforts across a series of human disease with no currently available therapies and large addressable market sizes. The relatively new strategies of targeting DNA and RNA molecules upstream of proteins to address disease at the genetic level have achieved human proof-of-concept, yet these early technologies are fragmented in their ability to address the different causal mechanisms and suffer from limitations related to delivery, tolerability, selectivity, manufacturability, durability and scalability. We believe we have a unifying solution without the limitations of other precision genetic medicine technologies that will allow us to address many diseases, both rare and common.

The PATrOL™ Platform Technology

We have designed and purpose-built a novel technology platform to address disease-causing genetic variants without the limitations of early precision genetic medicines. At the surface, our platform-enabled compounds resemble short single strands of DNA (oligonucleotides) with extremely high biding affinity that allows them to uniquely invade the human double-stranded genome. Our compounds have nucleobases that are arranged in the correct sequence to engage a known gene target, a backbone which holds the nucleobases at the proper spacing to engage a target, and an additional module which is our proprietary delivery technology. These compounds are completely synthetic molecules. The pharmacophore's neutral charge is key to broad cell and tissue distribution, penetrating cell nuclei and the deep brain. Figure 2 below illustrates a conceptual overview of how our PATrOL™ platform technology functions.

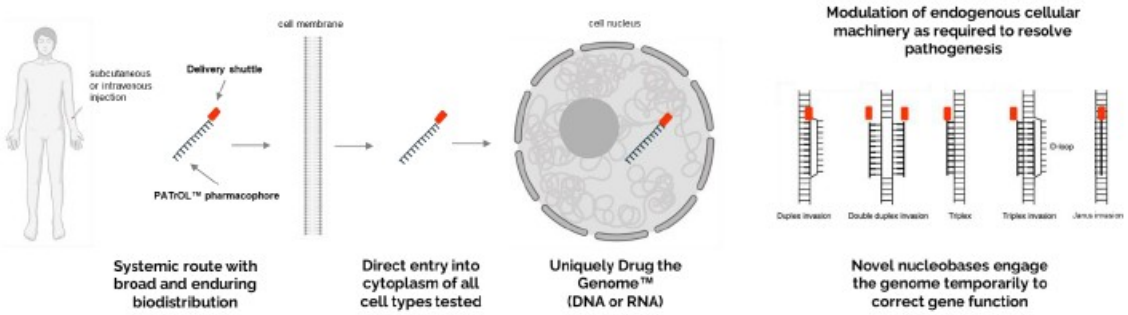


Figure 2. Conceptual overview of the process by which NeuBase compounds enter the cell after systemic administration, engage genes within the double-stranded DNA (or RNA) and resolve root causality. A proprietary delivery shuttle coupled to modified peptide-nucleic acid oligonucleotides allow systemic delivery with broad biodistribution including into the central nervous system, cellular entry in a non-cell type or species-specific manner, and single-base selective target engagement in the nucleus within 24 hours of dosing with a variety of binding modes that can be used to address various genetic lesions to modulate gene function based on steric interference with cellular machinery, such as RNA polymerase and transcription factors. Gene editing takes advantage of recruiting endogenous high-fidelity repair enzymes and does not require delivery of proteins nor induction of double-stranded DNA breaks. The technology is also able to engage single- and double-stranded RNA in the cytoplasm, and tropism for one type of nucleic acid versus another can be induced through backbone modifications.

The PATrOL™ platform is comprised of three main component modules: new nucleobases, a neutral- charged backbone, and a novel delivery shuttle (Figure 3). Each of these three component modules has been specifically designed to interact with one another to achieve the desired function and can be "tuned" to refine that function.

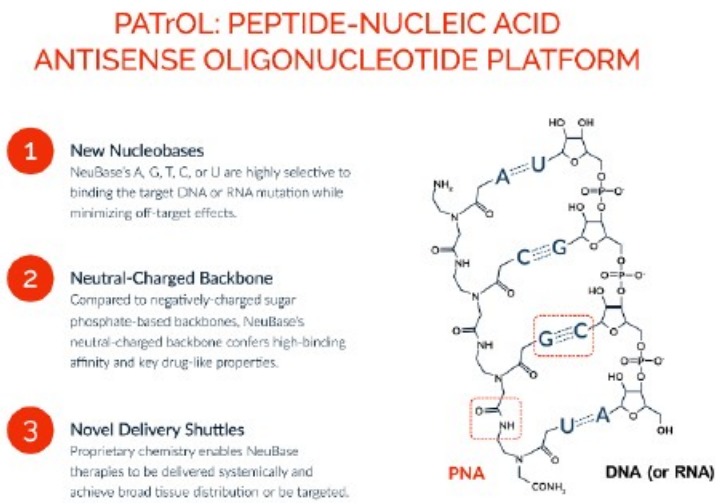


Figure 3. Conceptual overview of the PATrOL™ platform modules.

New Nucleobases. We have assembled, in-licensed, and invented a kit of nucleobases which can be arranged in a specific order onto a backbone so that they engage a genetic target. It is the order, or sequence, of the nucleobases that define the location within the target gene that the compound will ultimately engage. We can use natural nucleobases (that are used by the human body to construct its own genetic material); these are cytosine ("C"), guanine ("G"), adenine ("A"), thymine ("T") and uracil ("U"). These natural nucleobases are often used as their properties are well understood and they provide for standard Watson-Crick hydrogen binding between complementary nucleobases (A to T/U; G to C). These natural nucleobases can also be modified through chemical engineering to strengthen or weaken certain positions of engagement. The combination of natural nucleobases with the neutral-charge backbone and proprietary backbone modifications allows the fully constructed compound to "invade" the double helix (or a double-stranded RNA molecule) by out-competing the complementary DNA (or RNA) strand due to a higher binding affinity for the target sequence. This ability to invade double-stranded targets also allows increased efficiency when designing compounds against RNA targets as secondary structures are less likely to interfere with target engagement and confound optimal sequence targeting. Once the PATrOL™-enabled compound has invaded, it binds via standard Watson-Crick base pairing.

The second class of nucleobases are Hoogsteen nucleobases. When coupled to the proprietary neutral-charge backbone, Hoogsteen nucleobases allow a PATrOL™-enabled compound to scan the outside of the double helix by querying nucleobase content inside the double helix through the major groove. When a sequence match is found between these types of nucleobases and the fully Watson-Crick paired natural nucleobases inside the double helix, a triplex structure is formed.

A third class of nucleobases are termed Janus nucleobases. Janus bases are bi-specific nucleobases that can be considered natural nucleobases with a second binding face chemically engineered onto them to allow simultaneous engagement of two strands of nucleic acids. There are 16 possible Janus bases that will engage with any two strands of nucleic acids of interest. For example, Janus bases can be used in an invader use-case to double the number of hydrogen bonds that are engaged for a fixed sequence length when drugging the double helix, binding both the Watson and the Crick strands simultaneously and can increase the specificity of engagement. These bi-specific nucleobases have been shown to enable access to double stranded RNA targets comprised of secondary

structures, such as imperfect hairpins (double stranded RNA targets which are folded upon themselves). In programs where we are drugging an RNA, this allows us to potentially access regions of the target molecule which may be unique in secondary structure relative to the wild-type RNA to allow enhanced selectivity for the target.

Importantly, all these nucleobases which allow sequence-specific recognition of a gene within a target double-stranded nucleic acid are required to be used in combination with our neutral-charged backbone. Without the backbone, the nucleobases do not have the ability to engage utilizing these various binding modes.

Neutral-Charged Backbone. PNAs form the predicate chemistry for our backbones. The key differentiator between a PNA backbone and most traditional nucleic acid backbones (like the backbones in DNA and RNA) or modified backbones (like the backbones that are used by various companies in the genetic medicines space) is that the PNA backbone is neutral (not anionic) in charge and is helical in its secondary structure. PNAs use a synthetic backbone subunit ("monomer"), which in our construction is charge-neutral and characterized by high binding affinity to a nucleic acid target. The high binding affinity is due in part to the neutral charge that does not repulse its target nucleic acid, but also due to our ability to induce helicity through backbone modifications and thereby reduce the energy requirement necessary to engage a nucleic acid target. These features allow the nucleobases to perform in the required manner to either invade or form triplex structures. Another major differentiator of our backbone technology is, we believe, industry leading sequence selectivity of target engagement imparted by the "rigidity" of the backbone, which confers an intolerance for sequence mismatches. This manifests in an advantage to better differentiate mutant gene sequences from their wild-type counterparts, and in potentially reducing OTEs with highly similar sequences elsewhere in the genome. The backbones have high stability in biological fluids and are resistant to protease and nuclease digestion, contributing to their enduring presence in tissues after systemic administration and, we believe, to their ability to redistribute in and out of tissues over time via the circulatory system. PNA backbones have been described in the literature and by us to not trigger an innate nor acquired immune response, a key limiter of other precision genetic medicines, and potentially allow routine dosing.

We believe we are a leader in the development of the next generation of genetic medicines. We have exclusively licensed and developed proprietary innovations around PNAs that potentially enable them to be used as pharmaceuticals to address most causal mechanisms of disease by Drugging the Genome™. Our PNAs can modulate the machinery controlling gene function and fidelity, exhibit wide bio-distribution after systemic administration, including into the brain, a favorable tolerability profile, and high sequence selectivity. Our PATrOL™-enabled precision genetic medicines may be manufactured via well-commoditized peptide synthetic methods in a GMP setting. We have established a decentralized, outsourced, and cost-effective global supply and manufacturing infrastructure to allow rapid scaling across indications.

Novel Delivery Shuttles. The third component of our modular platform is a proprietary delivery technology that works in conjunction with the backbone chemistry to enable systemic administration and achieve broad biodistribution. The delivery shuttle is of low molecular weight, approximately 1.5 kD in size, synthetic in nature, likely non-immunogenic and is likely cell, tissue, and species agnostic in its ability to delivery our neutral water-soluble compounds into the cytoplasm or nucleus. We have previously (in FY2020) presented data with our first delivery shuttle in NHPs, which described systemic administration via an intravenous route having an in-circulation half-life of approximately 1.5 hours during which time the shuttle is taken up into every tissue in the body, including into the brain, within 4 hours post single-dose. Elimination is primarily through the renal system, and occurs slowly, with only approximately 4% of the administered dose excreted over the course of 7 days after single-dose injection. This delivery shuttle also appears to rapidly pass through the cell membranes of all cell types tested and, when coupled to a pharmacophore, engage the genetic target in less than 24 hours (earliest timepoint tested) in both cell culture and after systemic administration *in vivo*. We disseminated data illustrating this feature in both the DM1 and HD programs after a subcutaneous injection in FY2021. This delivery technology is currently being used for several of our therapeutic programs but does not preclude us from using various delivery technologies with our backbones.

The Therapeutic Pipeline

We are developing precision genetic medicines targeting rare, monogenic diseases for which there are no approved therapies, as well as more common genetic disorders, including cancers that are resistant to current therapeutic approaches. Our pipeline includes therapeutic candidates for the treatment of DM1, HD, and cancer-driving point mutations in *KRAS,* G12V and G12D, which are involved in many tumor types and have historically been "undruggable" (Figure 4).

Our capabilities to use this new modality to achieve gene silencing at the DNA and RNA levels have now been illustrated. In October 2022, we announced a strategic restructuring to expand our focus to include the advancement of the differentiated gene editing capabilities of our platform. As part of this expansion of our focus into gene editing, the Company will defer preclinical activities for its DM1, HD and KRAS programs, hold plans to submit an Investigational New Drug (IND) application for DM1 to the U.S. Food and Drug Administration (FDA), and pursue collaborative initiatives, including partnerships, for these programs. In the future, we expect to expand our focus once again to include gene activation use-cases.

On October 21, 2022, in line with our strategic restructuring to expand our focus to include the advancement of our platform into gene editing, we announced that we entered into a research agreement with a global healthcare company, pursuant to which such global healthcare company will evaluate our PATrOL™ platform for three monogenic genetic diseases and we will collaborate on the evaluation of drug candidates for three undisclosed indications.

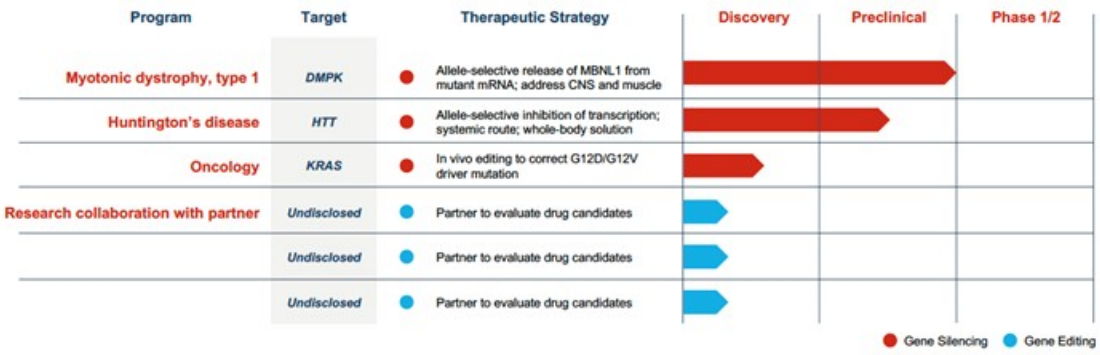


Figure 4. The initial pipeline in rare disease and oncology, with platform learnings increasing the efficiency and speed of additional undisclosed programs. Programs in gene editing added in October 2022.

Myotonic Dystrophy Type 1 (DM1): NT-0200 Program

DM1 exhibits whole-body manifestations. It is a multi-system, progressive disorder characterized by weakness of smooth and skeletal muscles that can range from mild to severe myotonia, cardiac conduction defects, cognitive deficits, respiratory distress, and shortened lifespan. Current treatment options include therapies to manage the symptoms, physical therapy, and assistive devices. No treatments that slow or stop the progression of DM1 are currently FDA approved. It is estimated that the global prevalence of DM1 is approximately 1 in 20,000 individuals.

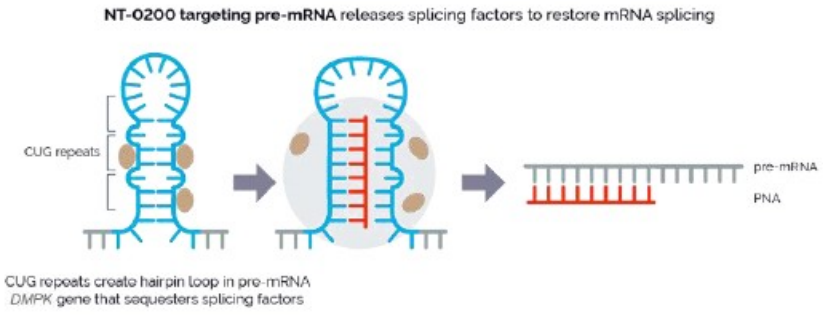


Figure 5. NT-0200 program mechanism of action to resolve root causality. Our DM1 drug candidate is designed to specifically target the mutated *DMPK* RNA. Restoring mRNA splicing and correct downstream protein production, including *DMPK*, our precision genetic approach has the potential to be disease-modifying.

DM1 is caused by a single inherited copy of a trinucleotide repeat expansion in the 3' untranslated region of the *DMPK* gene. Repeat length exceeding 34 repeats is abnormal, and patients often have hundreds or thousands of repeat units. When transcribed into RNA, the expanded trinucleotide repeat creates a molecular hairpin structure that is toxic in that it results in broad mRNA splicing abnormalities in hundreds to thousands of mRNAs in the cell, characterized by a reversion from adult gene/protein isoforms to embryonic isoforms, which affect protein function in some cases (for example, the skeletal muscle chloride channel). The toxic RNA hairpin sequesters muscleblind-like (MBNL) proteins making them unavailable for normal, developmentally appropriate splice regulation in the nucleus of affected cells. The altered protein isoforms result in DM1's numerous symptoms. DM1 is a multi-system disorder which would benefit from a whole-body solution.

Preclinical data previously presented in FY2021 demonstrated that our development candidate maintains *DMPK* protein levels in patient-derived cell lines after dosing and rescues the root genetic defect to normalize miss-spliced muscle mRNA transcripts in the HSA^{LR} transgenic mouse model. This has been illustrated via both an intravenous dose (single 29 mg/kg) and a subcutaneous dose (3 mg/kg multi-dose). We have also previously presented data that illustrates functional rescue of myotonia in the HSA^{LR} transgenic mouse after multi-subcutaneous dosing of 3 mg/kg.

During FY2022, we announced new preclinical data for our lead development candidate, NT-0231.F, to treat DM1 demonstrating splice rescue, nuclear aggregate resolution and myotonia reversal. A single intramuscular dose confirmed that NT-0231.F is pharmacologically active in the muscle and drives molecular and functional rescue in the HSA^{LR} model, including splice rescue, nuclear aggregate resolution, and myotonia (delayed muscle relaxation after contraction) reversal. A single intravenous (IV) dose of NT-0231.F or multiple subcutaneous (SC) doses over a 28-day period broadly rescued splicing, including the chloride channel *(Clcn1)* transcript, and reversed myotonia in the model. A single IV dose of NT-0231.F provided initial splice rescue at around two weeks, with significant splice rescue around three weeks. Myotonia reversal was achieved at around four weeks, with effects enduring to at least six weeks, the longest timepoint tested in the study. A time course of multiple SC doses across increasing concentrations of NT-0231.F was also investigated and showed splice rescue and myotonia reversal in a dose-responsive manner, illustrating feasibility of the differentiated and patient-friendly SC route. Figure 6 below illustrates splice rescue of *Clcn1* at day 21 and consequent reversal of myotonia at day 35 in tibialis anterior muscle following a single dose of NT-0231.F administered intravenously.

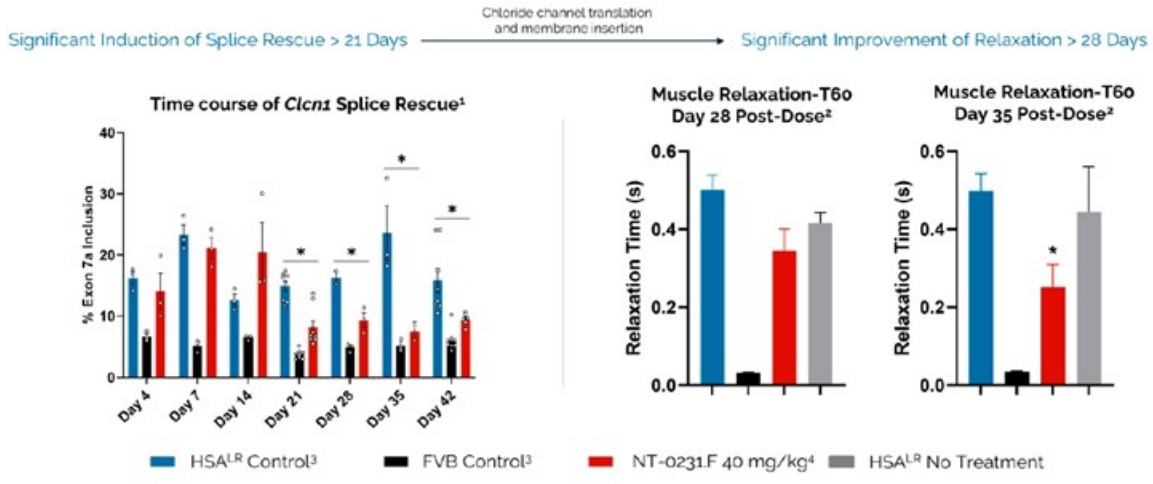


Figure 6. Rescue of Clcn1 adult cassette exon use and resultant rescue of myotonia after a single IV dose of NT-0231.F in the HSA^{LR} transgenic animal model. Murine transcript, relative usage by RT-PCR as per Wojtkowiak-Szlachcic A *et al.* Nucleic Acids Res. 2015 Mar 31;43(6):3318-31 and Klein AF *et al.* J Clin Invest. 2019 Nov 1;129(11):4739-4744; Myotonia as measured by time to 60% relaxation of gastrocnemius muscle at 2nd electrically-stimulated maximal contraction, no changes to muscle force production; Vehicle treated; Oligo mass; t-test comparison to HSA^{LR} Control. *p < 0.05.

We also presented preclinical PK and biodistribution data for NT-0231.F demonstrating wide tissue distribution and supporting a differentiated whole-body treatment solution for myotonic dystrophy type 1 (DM1). Following a single intravenous injection of 30 mg/kg in wild-type BALB/c mice, NT-0231.F was cleared rapidly from the systemic compartment and demonstrated rapid and wide distribution into tibialis anterior muscle, heart muscle, and brain tissues. NT-0231.F rapidly cleared the plasma, and each tissue evaluated displayed an extended elimination phase with tissue concentrations measurable for at least four weeks following a single IV dose administration. Figure 7 below represents the PK profile of NT-0231.F in wild-type BALB/c mice.

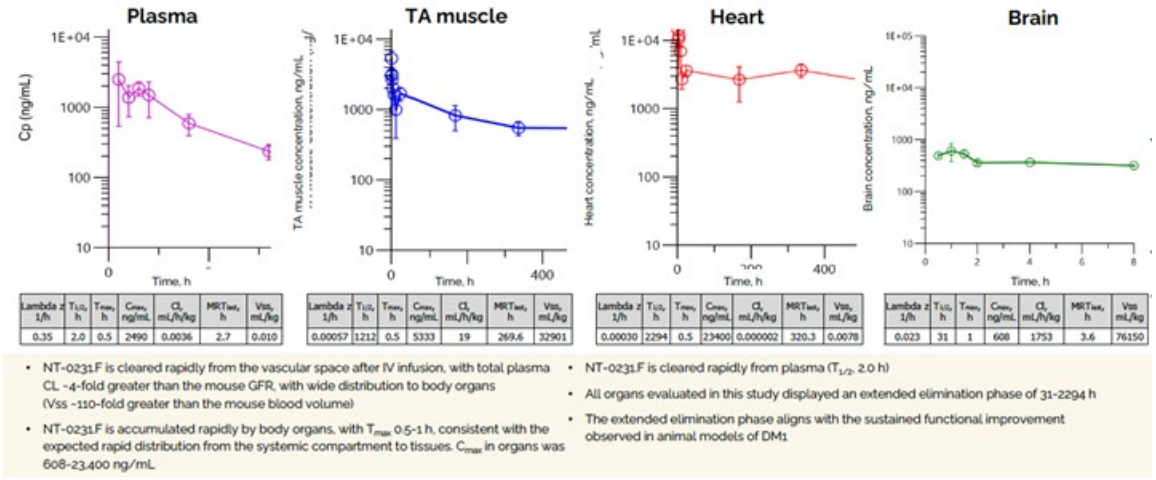


Figure 7. PK of NT-0231.F in BALB/c mice after single-dose intravenous injection of 30 mg/kg.

To begin to define the toxicology profile of NT-0231.F, we performed multispecies dose range finding exploratory toxicology studies in advance of GLP-toxicology studies. Single doses of NT-0231.F were administered IV to rats and NHPs. In rats, the toxic dose was 58 mg/kg, with the next lower dose of 29 mg/kg in this non-GLP exploratory study being defined as the maximum tolerated dose ("MTD"). At the MTD, a decrease in body weight growth within the first week after dose administration was observed, and minimal microscopic findings in the kidney (14 mg/kg and 29 mg/kg) and at the injection site (29 mg/kg) were observed. Of the three male and three female rats dosed, one male animal died during dose administration. In NHPs, the toxic dose was 29 mg/kg, with the next lower dose of 14 mg/kg being defined as the MTD. At the MTD, minimal renal tubular degeneration and regeneration was observed. No other test-article related histologic findings were observed at or below the MTDs in both species. No cytokine abnormalities were identified at or below the MTD. The MTD will be refined and potentially increased in subsequent GLP-toxicology studies. In addition, the Ames and *in vitro* micronucleus tests were negative for NT-0231.F, plasma protein binding of 96% was observed, and metabolism (stability & profiling) and drug interaction (cytochrome P450 inhibition) test panels were negative.

To project the pharmacologically active single dose in humans, and to model the multi-dose regimen based on elimination rates across various organs, we performed a 3-month PK study in NHPs. Tissue concentrations across various organs that manifest the pathologies in DM1 were measured by liquid chromatography followed by mass spectroscopy ("LC-MS") and compared against the concentration of NT-0231.F that was 50% effective ("EC50") in engaging the target *DMPK* mRNA in patient-derived differentiated myotubes in culture. Single dose IV injections were administered at either 6 mg/kg or 14 mg/kg into groups of NHPs, and blood and tissue samples were obtained at various timepoints up to three months. Like the prior BALB/c mouse 28-day PK studies we have previously

described, the compound was rapidly taken up out of the circulation ($T_{1/2}$ ~ 3 hours) and distributed broadly throughout the various organs in the body. The 6 mg/kg dose achieved an >EC50 in skeletal muscle up to day 15, and a single dose of 14 mg/kg achieved an >EC50 up to and including day 92. This data suggests there is a sufficient safety margin to warrant moving NT-0231.F forward into GLP-toxicology studies. The elimination rates from the three-month NHP study allow modelling of multi-dose regimens as the next step.

We believe that our therapeutic solution has the potential to offer patients a differentiated option in the marketplace, which includes:

- A whole-body solution to address muscle, heart, and brain pathologies based on NHP PK/biodistribution data
- Designed to maintain *DMPK* protein as opposed to explicitly degrade it, which is likely important for health
- A patient-friendly systemic route such as subcutaneous administration

The path forward in CY2023. As announced in October 2022, the Company plans to reduce activities and pursue partnerships for this gene silencing program.

Huntington's Disease (HD): NT-0100 Program

HD is a fatal neurodegenerative disease characterized by neuronal death in deep brain structures culminating in progressive impairments in movement and cognitive control followed by death. Current treatment options attempt to lessen involuntary movement and psychiatric symptoms, but disease-modifying treatments have yet to successfully advance through clinical trials.

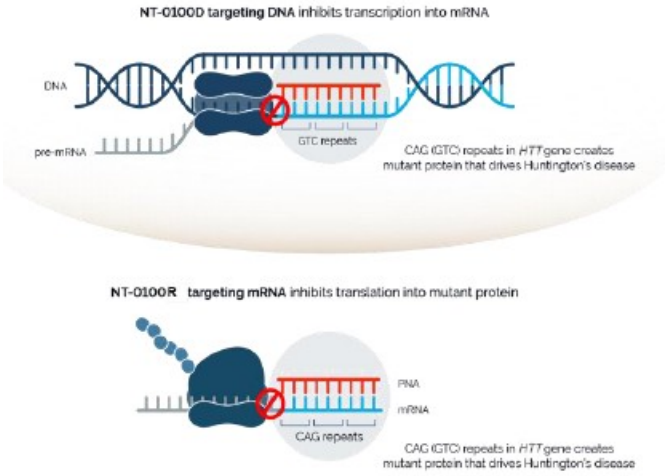


Figure 8. NT-0100 Mechanism of action to resolve root causality. DNA-binders (NT-0100D) and RNA-binders (NT-0100R) allele-selective compounds inhibit transcription and translation, respectively, to knock down mutant HTT protein while maintaining normal HTT.

HD is caused by a single inherited copy of a trinucleotide repeat expansion in the coding region of the Huntingtin gene. The expanded trinucleotide repeat is transcribed into RNA and translated into an abnormally sticky version of the Huntingtin protein, characterized by a poly-glutamine stretch of amino acids proximal to the amino-terminus of the protein. This mutant protein leads to toxic accumulation in neurons and eventual neuronal cell death, followed by death of cells throughout the body over time.

The wild-type *HTT* ("wtHTT") gene has a region in which a three-base DNA sequence, CAG, is repeated many times. When the DNA sequence CAG is repeated 26 or fewer times in this region, the resulting protein behaves normally. When the DNA sequence CAG is

repeated 40 times or more in this region, the resulting protein becomes toxic ("m*HTT*"). Every person has two copies, or alleles, of the *HTT* gene. Only one of the alleles (the "mutant" allele) needs to bear at least 40 CAG repeats for HD to occur. Current therapies for patients with HD can only manage individual symptoms. There is no approved therapy that has been shown to delay or halt disease progression. In the U.S. alone, there are likely more than 40,000 symptomatic patients and likely more than 200,000 at-risk of inheriting the disease.

The goal of the NT-0100 program is to advance a drug candidate that selectively binds the mutated DNA (or mRNA) to reduce or prevent production of the mutated protein and do so while maximally maintaining healthy wild-type protein, which we believe is important to normal function. One especially important advantage of the PATrOL™ platform that makes it promising for the treatment of repeat expansion disorders like HD is the ability of our investigational therapies to target the mutant double-stranded RNA hairpin in the mt*HTT* transcript.

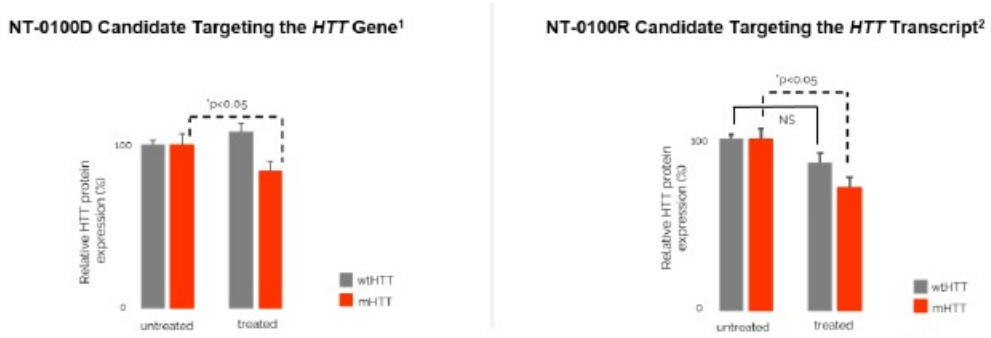


Figure 9. Subcutaneous injection of NT-0100 compounds cross the blood-brain barrier and knock down mutant HTT protein selectively after only 7 days. 1zQ175 mouse model with 190 CAG repeats in exon 1 of HTT; 60 mg/kg subcutaneous dose with NT-100D candidate on day 1 & 4; sacrifice day 7; 2zQ175 mouse model with 190 CAG repeats in exon 1 of HTT; 30 mg/kg subcutaneous dose with NT-0100R candidate on day 1 & 4; sacrifice day 7. For both NT-100D and NT-0100R, whole-brain homogenates from day 7 animals were used to prepare protein extracts for Western blotting using antibodies to detect both normal and mutant HTT protein.

The mechanism of our PATrOL™-enabled compounds is illustrated in Figure 8. The HTT protein is essential during embryogenesis and dependence upon HTT appears to decrease with age until adulthood, but the protein is known to be involved in a diverse range of functions and signaling pathways, so it is difficult to imagine complete loss of the protein would be tolerated.

Preclinical data from transgenic mouse zQ175 animal models demonstrate the ability of NT-0100 program candidate compounds to be administered subcutaneously, cross the blood-brain barrier, enter the parenchymal space, penetrate neuronal cell bodies and either act in the nucleus (DNA-targeting) or the cytoplasm (RNA-targeting) to reduce the production of toxic protein while maintaining production of normal HTT protein (Figure 9). In FY2020, we illustrated the ability of our technology to enrich for translational inhibition and resultant reduction of mutant protein formation in human patient-derived cell lines versus wild-type protein production and that our investigational therapies can inhibit ribosomal elongation via high-affinity binding to a target RNA. In FY2021, we presented data demonstrating selective reduction of mutant Huntingtin protein in the brain after subcutaneous dosing in the zQ175 Huntington's disease transgenic mouse model targeting both the mutant mRNA and inhibiting translation, but also now by targeting the DNA and inhibiting transcription after subcutaneous administration – not only illustrating passage across the blood-brain barrier after a patient-friendly route but also allele-selective knock-down.

In FY2022, we presented new data (set forth in Figure 10 below) illustrating that NT-100D crosses the blood-brain barrier after subcutaneous dosing and selectively reduces mutant Huntingtin protein mRNA levels and intraneuronal toxic protein aggregates, while avoiding wild-type Huntingtin protein, in the brains of R6/2 mice, which is an acute model of Huntington's disease. A dose-

related trend to improved functional rescue was also observed in R6/2 mice. NT-100D was well tolerated at pharmacologically active doses following subcutaneous administration.

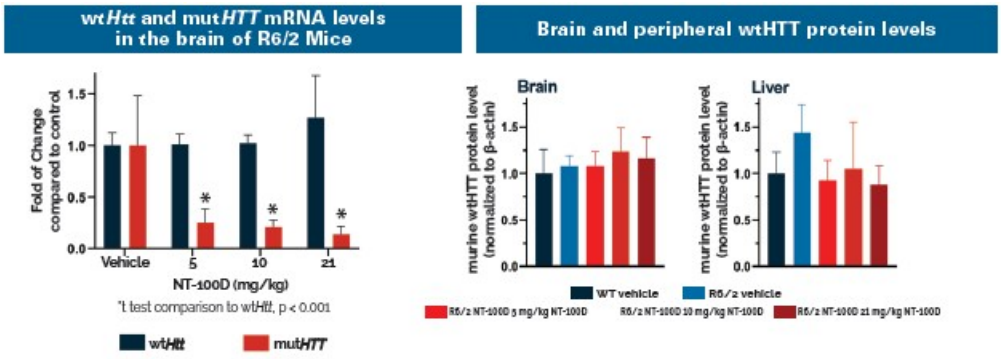


Figure 10. Allele-selective DNA-target engagement and inhibition of transcription. Left Panel: Wild type *Htt* and mutated *HTT* mRNA levels were measured by qRT-PCR in the brains of R6/2 mice treated with NT-100D b.i.w. x8 weeks. Statistical analysis was performed by t-test comparison to Vehicle Control, * = p <0.001. **Right Panel:** Western blot analysis of wild type HTT protein level in total brain and liver lysates. Wild type HTT protein was detected using the EPR5526 antibody and visualized using fluorescent secondary antibodies on the Li-Cor imager.

We believe the potential differentiators of our HD program are:

- Whole-body solution to address brain and body pathologies based on NHP PK/biodistribution studies
- Designed to maintain normal Huntingtin protein, which is likely important for health
- Patient-friendly systemic route such as subcutaneous administration

The path forward in CY2023. As announced in October 2022, the Company plans to reduce activities and pursue partnerships for this gene silencing program.

Oncology (KRAS G12D and G12V): NT-0300 Program

Our oncology program targets *KRAS* G12D and G12V gene mutations, which are the two most common and historically "undruggable" *KRAS* driver mutations. There are no approved therapies for KRAS G12D or G12V mutations, which account for approximately 55% of all KRAS mutations.

We have designed novel PATrOL™-enabled compounds to selectively engage with the mutant cancer driver mutation at either the DNA or RNA level to inhibit downstream mutant KRAS protein production and hyperactive mitotic signaling. Preclinical studies have shown tumor growth inhibition after intra-tumoral administration and reduction of downstream signaling, validating that the compounds can engage the target. We have also shown preclinical data illustrating that we achieve allele-selective engagement of the mutant allele with single-nucleotide precision. Figure 11 illustrates how the compounds access the *KRAS* mutations in codon 12 at the

DNA and RNA levels, and Figure 12 illustrates the *in vitro* and *in vivo* proof-of-concept data illustrating potency and selectivity of target engagement.

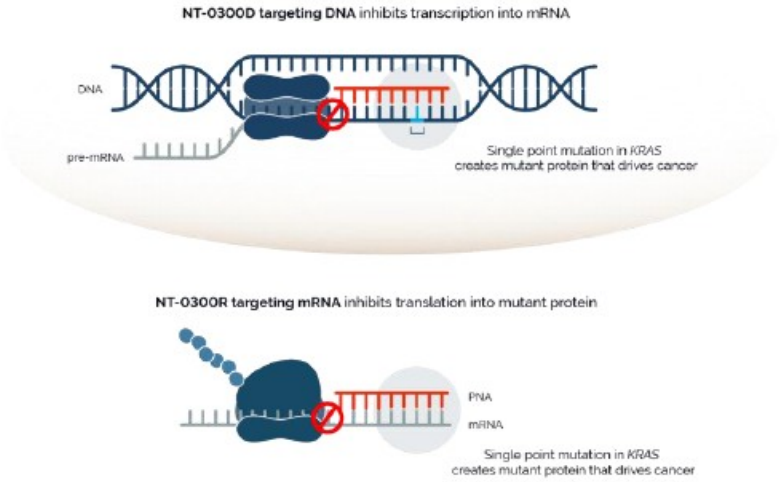


Figure 11. Systemic delivery (left panel) allows even perfusion of the CNS to access the striatum. Mechanism of DNA- (middle panel) and RNA-targeted (right panel) allele-selective compounds to knock down mutant HTT protein while maintaining normal HTT.

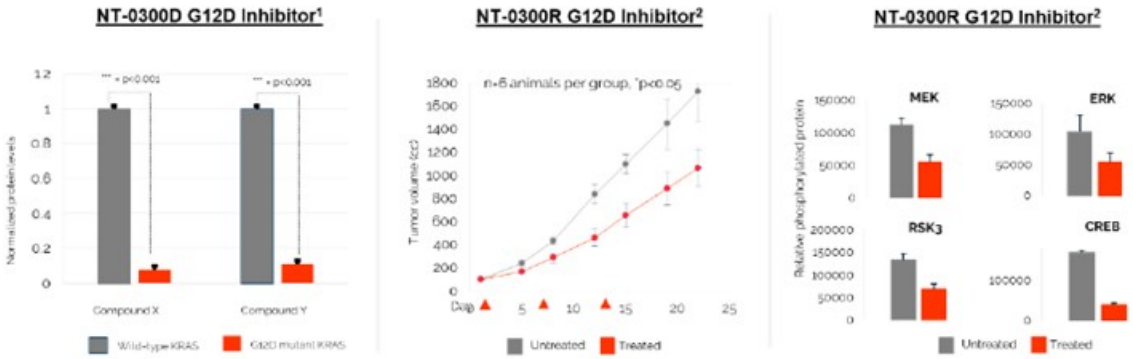


Figure 12. Representative results from in vitro and in vivo hits that selectively engage*KRAS* activating point mutations in exon 1 at the DNA and RNA levels and exhibit pharmacologic effects, reduce downstream signaling. [1]>90% knock-down of KRAS G12D mutant protein relative to wild-type KRAS protein in an *in vitro* transcription and translation assay utilizing a *KRAS* G12D-specific compound; 0.3 mg/kg intra-tumoral injections into HPAFII heterozygous pancreatic cancer xenografts on day 1, 7 and 14 (orange triangles).

While our KRAS program is in early development, we believe it illustrates that our investigational therapies can target the mutated gene at the level of DNA (as opposed to RNA or protein levels), which provides potential potency and dosing advantages, and the ability of the PATrOL™-enabled compounds to achieve single-base selectivity. We believe we can scale this across missense mutations and smaller mutational events in new targets that other genetic medicine modalities have difficulty accessing selectively.

We believe the potential advantages of our *KRAS* inhibitors include:

- Whole-body solution to address primary tumors and metastases
- Designed to maintain normal KRAS protein, which is likely important for health
- Patient-friendly systemic route such as intravenous or subcutaneous administration

The path forward in CY2023. As announced in October 2022, the Company plans to reduce activities and pursue partnerships for this gene silencing program.

Gene Editing Programs

The next wave of the Company's pipeline programs is focused on in vivo genome editing using PATrOL™-based synthetic genome-targeting chemistry, which can recruit the body's own editing machinery to correct mutations that cause disease. This approach harnesses millennia of evolution to enable high-fidelity genome editing. The human body's endogenous DNA repair machinery has been projected to edit up to one million acquired mutations per cell in each of the trillions of cells in the body, speaking to the potential robustness and fidelity of this gene editing approach versus other approaches.

Our exclusively licensed technology has been proven to achieve in vivo editing in various diseases and has been repeatedly published in top-tier journals such as Nature and Science. The PATrOL™ editors are differentiated from CRISPR/Cas-derived base editors (BEs) in the areas of fidelity by using three layers of sequence selectivity, tolerability without double-stranded breaks or bystander edits, versatility in non-viral delivery options, and in the overall type and thus number of mutations that can be resolved. PATrOL™ editors are like prime editors (PEs) in that they are not dependent on protospacer adjacent motif (PAM) flanking sequences, they can edit transversions as well as transitions, and they can repair insertions and deletions.

We project PATrOL™ editors can potentially address up to ~90% of all known human mutations. In contrast, BEs, in aggregate across all current versions of CRISPR/Cas nucleases (and all companies employing them), can currently only address ~ 20% of all know human mutations due to sequence and editing constraints.

The synthetic chemistry employed by PATrOL™ editors has low molecular weight and thus is more easily packaged for delivery. It is well described to be non-immunogenic, enabling re-dosing to increase editing efficiency and address tissue turnover. The ideal use case for PATrOL™ editors is for in vivo correction of prevalent causal variants that cannot be addressed by BEs, such as transversions and insertion / deletion events. Due to the lack of acquired immunogenicity that likely will plague BEs and PEs, our solution has the potential to address durability issues faced by other editors.

In summary, the differentiators of the PATrOL™ editing technology are:

- Better On-Target Fidelity – Technology utilizes three layers of sequence selectivity to identify a locus of interest and leverages the human body's own repair machinery (with performance criteria established across millions of mutations per day and trillions of cells per human) as opposed to bacterial enzymes (BEs/PEs).

- Lower Off-Target Edits – The approach induces single-stranded nicks (no double-stranded breaks) and does not result in bystander editing, which reduces the risk of off-target effects by orders of magnitude.

- Heightened Tolerability – Our synthetic chemistry has been demonstrated to be non-immunogenic, and the gene editing payloads can be delivered by validated non-viral delivery systems, enabling re-dosing to achieve higher clinical editing efficiencies and address tissue turnover across time.

- Versatile Delivery Options – Our editors have low molecular weight, are non-biodegradable, and are charge-tunable, which results in more opportunities for coupling to various non-viral delivery modalities, enabling a customizable option for partners with proprietary delivery technologies.

- More Addressable Clinical Mutations – The technology can address transversions as well as transitions, insertions and deletions. In addition, the editors do not require a flanking PAM sequence. Taken together, this increases the opportunity space to apply our editors by approximately five-fold over BEs, with nearly complete coverage of causal mutations.

On October 21, 2022, in line with our strategic restructuring to expand our focus to include the advancement of our platform into gene editing, we announced that we entered into a research agreement with a global healthcare company, pursuant to which such global healthcare company will evaluate our PATrOL™ platform for three monogenic genetic diseases and we will collaborate on the evaluation of drug candidates for three undisclosed indications. We are also identifying and evaluating multiple indications for possible future development, and we expect to provide further details during fiscal year 2023 regarding our gene editing pipeline.

Summary

We are currently focused on therapeutic areas in which we believe our drugs will provide the greatest benefit with a significant market opportunity. We intend to utilize our technology to build a pipeline of custom designed therapeutics for additional high-value disease targets. We plan to leverage appropriate partnering strategies based on the specific drug candidate, therapeutic area expertise, and resources potential partners may bring to a collaboration. For some drug candidates, we may choose to develop and, if approved, commercialize them ourselves or through our affiliates. For other drug candidates, we may form single or multi-asset partnerships leveraging our partners' global expertise and resources needed to support large commercial opportunities.

In October 2022, we announced a strategic restructuring to expand our focus to include the advancement of the differentiated gene editing capabilities of our platform. As part of the development pipeline shift to gene editing, we will defer preclinical activities for our DM1, HD, and KRAS programs, hold plans to submit an IND application for DM1 to the FDA, and pursue collaborative initiatives, including partnerships, for these programs.

As we focus on the advancement of our gene editing platform, we are currently identifying and evaluating multiple indications for potential future development. We are also working with a global healthcare company, pursuant to the research collaboration agreement the Company announced in October 2022, to evaluate drug candidates for three monogenic genetic diseases. We expect to provide further details during fiscal year 2023 regarding our gene editing pipeline.

We plan to attend various scientific and medical conferences in CY2023 where we plan to present the results of the foregoing activities described above.

Overall, using our PATrOL™ platform, we believe we can create therapies that may have distinct advantages over other chemical entities currently in the market or in development for precision genetic medicine applications to modulate mutant genes and improve a clinical trait or disorder. We employ a rational approach to selecting disease targets, considering many scientific, technical, business and indication-specific factors before choosing each indication. We intend to build a diverse portfolio of therapies to treat a variety of health conditions.

We believe the depth of our knowledge and expertise with PNAs, engineered nucleotides, genetics, genomics and therapeutic development of first-in-class modalities allows us to determine the optimal development and commercialization strategy to maximize the near and longer-term value of our therapeutic programs. We believe the breadth of the PATrOL™ platform gives us the ability to potentially address a multitude of diseases.

Intellectual Property

The initial intellectual property position behind our fundamental PATrOL™ technology was initially developed at Carnegie Mellon University ("CMU"). Our success depends, in part, on our ability to obtain patent protection for our product candidates in the United States and other countries. We have exclusively licensed patent applications, pursuant to our license agreement with CMU (as amended, the "CMU License Agreement"), protecting our platform for development and commercialization of therapeutics. We will continue to focus our resources on obtaining patents and filing new patent applications that drive value.

We have an exclusive license to patent applications pursuant to the CMU License Agreement that may provide exclusivity for product candidates in our pipeline and may provide exclusivity for our core technology. Our core technology patent applications are directed to chemically-modified nucleosides and peptide nucleic acids to form compounds of biological and clinical interest. We have

exclusively licensed patent applications pursuant to the CMU License Agreement to cover 16 Janus bases and treatment of repeat expansion disorders using this technology.

Peptide Nucleic Acids containing Modified Nucleobases

We have exclusively licensed patent applications pursuant to the CMU License Agreement covering peptide nucleic acid oligomers containing modified nucleobases, which can be used as a basis for therapeutics. Nucleosides and chemically modified nucleosides are the basic building blocks of our drug development platform. Therefore, claims that cover an oligonucleotide incorporating one of our proprietary modified nucleosides may apply to a wide array of mechanisms of action and therapeutic targets. Our modified nucleobases may comprise a divalent nucleobase in sequence with several other divalent nucleobases to create a PNA.

We have filed patent applications in this category in the United States and pursuant to the Patent Cooperation Treaty.

Methods of Producing Peptide Nucleic Acids with Modified Nucleobases

We have exclusively licensed a patent (with an estimated expiration date of April 11, 2034) in the United States pursuant to the CMU License Agreement disclosing a method of manufacturing a peptide nucleic acid oligomer containing a modified nucleobase.

Use of Peptide Nucleic Acids to Disrupt RNA Structure

We have exclusively licensed a provisional patent application (with an estimated expiration date of June 7, 2039) in the United States pursuant to the CMU License Agreement covering use of a peptide nucleic acid oligomer for disrupting a target RNA structure, to prevent translation of a target protein.

Use of Peptide Nucleic Acids to Treat Repeat Expansion Disorders

We have exclusively licensed patent applications (with an estimated expiration date of February 22, 2040) in the United States pursuant to the CMU License Agreement covering the use of a peptide nucleic acid oligomer for the treatment of repeat expansion disorders, including, for example, HD and DM1.

Patent Portfolio

We plan to seek patent protection in significant markets and/or countries for each product to be developed. We also seek to maximize patent terms. In some cases, the patent term can be extended to recapture a portion of the term lost during the FDA regulatory review. The patent exclusivity period for a drug may deter generic drugs from entering the market. Patent exclusivity depends on a number of factors including initial patent term and available patent term extensions based upon delays caused by the regulatory approval process. We also rely on trade secrets, proprietary know-how and continuing technological innovation to develop and maintain a competitive position in our field.

While we have obtained patents and have patent applications pending, the extent of effective patent protection in the United States and other countries is highly uncertain. No consistent policy addresses the breadth of claims allowed in or the degree of protection afforded under patents of medical and pharmaceutical companies. Patents we currently own or may obtain might not be sufficiently broad to protect us against competitors with similar technology. Any of our patents could be invalidated or circumvented.

The holders of competing patents could determine to commence a lawsuit against us and may even prevail in any such lawsuit. Litigation could result in substantial cost to and diversion of effort by us, which may harm our business. In addition, our efforts to protect or defend our proprietary rights may not be successful or, even if successful, may result in substantial cost to us.

License Agreement with Carnegie Mellon University

On December 17, 2018, we entered into the CMU License Agreement. Under the CMU License Agreement, CMU granted us an exclusive, worldwide right to the PATrOL™ technology. Our exclusive, worldwide right to the PATrOL™ technology is subject to CMU's right (which is exercisable only upon our written consent) to grant a non-exclusive license to a third party as a means of resolving disputes or to settle claims arising out of allegations that the licensed technology under the CMU License Agreement

infringes upon the intellectual property rights of such third party. On January 1, 2022, we entered into an amendment to the CMU License Agreement (the "CMU License Agreement Amendment") to amend certain terms and add additional patents and patent applications.

As partial consideration for the license right, we issued and delivered to CMU 835,625 shares of our common stock. Further, as partial consideration for the license right, we issued a warrant to CMU, and CMU exercised such warrant prior to the effective time of the Merger for an aggregate of 103,787 shares of our common stock. Under the CMU License Agreement, CMU has preemptive rights with respect to certain future sales of securities by us for capital-raising purposes, "piggyback" registration rights and co-sale rights with respect to certain resales of shares by our stockholders.

Pursuant to the CMU License Agreement, we paid CMU a one-time payment of approximately $54,000 for patenting and other intellectual property protection costs incurred by CMU prior to the effective date of the CMU License Agreement and relating to the licensed technology thereunder. In connection with entering into the CMU License Agreement Amendment, we paid CMU a one-time amendment fee of $10,000. Further, we must achieve certain milestones to demonstrate certain developments of the licensed product. Further, subject to certain conditions, we will pay to CMU royalties at a low single-digit percentage of aggregate annual net sales of licensed products and a percentage at the medium and higher ranges of the bottom third of sublicensing fees.

The term of the CMU License Agreement concludes at the end of 20 years from its effective date or on the expiration date of the last-to-expire patent licensed, whichever comes later, unless otherwise terminated. The CMU License Agreement may be terminated (or the exclusivity of the license may be terminated) before the term due to customary payment default and fundamental change default provisions and failure of performance obligations. In addition, CMU may terminate the CMU License Agreement if we or our affiliates challenge the validity of the intellectual property licensed thereunder in a judicial or administrative proceeding. In the event we or our affiliates successfully challenge the validity of the intellectual property licensed thereunder, the royalties payable to CMU increase by a single digit percentage. We may terminate the CMU License Agreement upon payment of termination fees, the amounts of which depend on the date of such termination, but only if at the time of such termination, a licensed patent contains a valid claim. If not earlier terminated, at the expiration of the term, the rights and licenses granted to us by CMU survive in perpetuity, subject to our compliance with indemnification and dispute resolution obligations.

Manufacturing

We currently manufacture our starting materials both in-house and by using third-party suppliers and our research-scale final products both in-house and externally. We intend to rely on third parties for larger scale manufacturing going forward. We currently contract for the manufacture of developmental quantities of our product candidates that we may develop. We currently employ internal resources and third-party consultants to manage our manufacturing contractors.

Sales and Marketing

We have not yet defined our sales, marketing or product distribution strategy for any of our future product candidates. Our commercial strategy may include the use of strategic partners, distributors, a contract sale force, or the establishment of our own commercial and specialty sales force, as well as similar strategies for regions and territories outside the United States. We plan to further evaluate these alternatives when it approaches approval for the use of our product candidates for one or more indications.

Competition

The biotechnology industry is highly competitive and involves a high degree of risk. Potential competitors in the United States and worldwide are numerous and include pharmaceutical and biotechnology companies, educational institutions and research foundations. We compete with many of these entities who, either alone or with their strategic partners, have far greater experience, capital resources, research and technical resources, marketing experience, clinical trial experience, and research and development staffs and facilities than we do. Some of our competitors may develop and commercialize products that compete directly with our product candidates, and they may introduce products to market earlier than our products or on a more cost-effective basis. These competitors also compete with us in recruiting and retaining qualified scientific and management personnel and, in the future, in recruiting clinical trial sites and subjects for our clinical trials.

We expect any products that we develop and commercialize to compete on the basis of, among other things, efficacy, safety, price and the availability of reimbursement from government and other third-party payors. Our commercial opportunity could be reduced or eliminated if our competitors develop and commercialize products that are viewed as safer, more effective, more convenient or less expensive than any products that we may develop. Our competitors also may obtain FDA or other regulatory approval for their competing products more rapidly than we may obtain approval for any of our product candidates, which could result in our competitors establishing a strong market position before we are able to enter the market.

The field of gene editing is broad and characterized by a diversity of approaches. These include but are not limited to: CRISPR/Cas9 nuclease technology, alternative nuclease-based genome editing technologies such as ZFNs, engineered meganucleases and TALENs, base editing, prime editing, epigenetic editing, gene therapy, oligonucleotides, cell therapy and DNA-cutting enzymes. Based on publicly available information, we are aware of numerous companies pursing such approaches, including Arbor Biotechnologies, Inc., Beam Therapeutics Inc., bluebird bio, Inc., Caribou Biosciences, Inc., Chroma Medicine, Inc., CRISPR Therapeutics AG, Editas Medicine, Inc., Graphite Bio., Inc., Intellia Therapeutics, Inc., Mammoth Biosciences, Inc., Metagenomi, Inc., Precision Biosciences, Inc., Prime Medicine, Inc., Sangamo Therapeutics, Inc., Scribe Therapeutics, Inc., Tune Therapeutics, Inc., and Verve Therapeutics, Inc.

There are no disease-modifying treatments available for DM1. Based on publicly available information, we are aware of several companies actively pursuing preclinical and clinical development on various approaches to treat DM1 including AMO Pharma Limited, Avidity Biosciences, Inc., Dyne Therapeutics, Inc., Design Therapeutics, Inc., PepGen Inc., Entrada Therapeutics, Inc., Vertex Pharmaceuticals Incorporated, and Expansion Therapeutics, Inc.

There are currently no approved treatments available to slow the progression of HD. Based on publicly available information, several companies have ongoing clinical and preclinical programs targeting the underlying disease in HD, including Wave Life Sciences, Ltd., Annexon, Inc., Sangamo Therapeutics, Inc., ProQR Therapeutics N.V., uniQure N.V., Spark Therapeutics, PTC Therapeutics, Inc., and Voyager Therapeutics, Inc. We are aware that a number of other companies are developing drugs focused on treating the symptoms associated with HD, including Teva Pharmaceutical Industries Ltd., and Azevan Pharmaceuticals, among others.

Legacy NeuBase Pre-Merger Programs

We have an equity interest in DepYmed, Inc. (“DepYmed”), a joint venture that Ohr entered into with Cold Spring Harbor Laboratory in 2014. DepYmed is a preclinical stage company focused on Wilson’s disease, Rett syndrome, and oncology applications. We also retain intellectual property, which has been licensed to DepYmed, and have no other ongoing obligations (monetary or otherwise) to DepYmed. In February 2021, the Company sold certain intellectual property to DepYmed in exchange for shares of Series A-4 preferred stock of DepYmed.

Governmental Regulation

Government authorities in the United States (including federal, state and local authorities) and in other countries, extensively regulate, among other things, the manufacturing, research and clinical development, marketing, labeling and packaging, storage, distribution, post-approval monitoring and reporting, advertising and promotion, pricing and export and import of pharmaceutical products, such as our product candidates. The process of obtaining regulatory approvals and the subsequent compliance with appropriate federal, state, local and foreign statutes and regulations require the expenditure of substantial time and financial resources. Moreover, failure to comply with applicable regulatory requirements may result in, among other things, warning letters, clinical holds, civil or criminal

penalties, recall or seizure of products, injunction, disbarment, partial or total suspension of production or withdrawal of the product from the market. Any agency or judicial enforcement action could have a material adverse effect on us.

Food and Drug Administration Regulation and Marketing Approval

In the United States, the FDA regulates drugs under the Federal Food, Drug, and Cosmetic Act (the "FDCA"), and related regulations. Under the FDCA, a drug is defined as "articles intended for use in the diagnosis, cure, mitigation, treatment, or prevention of disease and articles (other than food) intended to affect the structure or any function of the body of man or other animals." Drugs are also subject to other federal, state and local statutes and regulations, and when sold or used outside the United States are subject to regulation by other governmental authorities equivalent to the FDA. Failure to comply with the applicable U.S. regulatory requirements at any time during the drug development process, approval process or after approval may subject an applicant to administrative or judicial sanctions and non-approval of product candidates. These sanctions could include the imposition by the FDA or an Institutional Review Board ("IRB") of a clinical hold on clinical trials, the FDA's refusal to approve pending applications or related supplements, withdrawal of an approval, untitled or warning letters, product recalls, product seizures, total or partial suspension of production or distribution, injunctions, fines, restitution, disgorgement, civil penalties or criminal prosecution. Such actions by government agencies could also require us to expend a large amount of resources to respond to the actions. Any agency or judicial enforcement action could have a material adverse effect on us.

The FDA and comparable regulatory agencies in state and local jurisdictions and in foreign countries impose substantial requirements upon the clinical development, manufacture and marketing of pharmaceutical products.

These agencies and other federal, state and local entities regulate research and development activities and the testing, manufacture, quality control, safety, effectiveness, labeling, packaging, storage, distribution, record-keeping, approval, post-approval monitoring, sales, advertising, promotion, marketing, sampling and import and export of our products. Our drug candidates must be approved by the FDA through the New Drug Application ("NDA") process before they may be legally marketed in the United States, and would need approval from equivalent governmental authorities to be marketed in other countries.

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

- completion of nonclinical laboratory tests, animal studies and formulation studies conducted according to Good Laboratory Practice ("GLP") or other applicable regulations;
- submission of an Investigational New Drug application ("IND"), which allows clinical trials to begin unless the FDA objects within 30 days;
- adequate and well-controlled human clinical trials to establish the safety and efficacy of the proposed drug for its intended use or uses conducted in accordance with Good Clinical Practices ("GCPs"), which are international ethical and scientific quality standards meant to assure that the rights, safety and well-being of trial participants are protected, to define the roles of investigators, clinical trial sponsors, administrators and monitors, and to assure clinical trial data integrity;
- submission of a NDA based on the clinical trial data demonstrations of efficacy and safety;
- pre-approval inspection of manufacturing facilities and clinical trial sites; and
- FDA approval of an NDA, which must occur before a drug can be marketed or sold in the U.S.

IND and Clinical Trials

Prior to commencing the first clinical trial, an IND, which contains the results of preclinical studies along with other information, such as information about product chemistry, manufacturing and controls and a proposed protocol, must be submitted to the FDA. The IND automatically becomes effective 30 days after receipt by the FDA unless the FDA within the 30-day time period raises concerns or questions about the conduct of the clinical trial. In such a case, the IND sponsor must resolve any outstanding concerns with the FDA before the clinical trial may begin. A separate submission to the existing IND must be made for each successive clinical trial to be

conducted during drug development. Further, an independent IRB for each site proposing to conduct the clinical trial must review and approve the investigational plan for any clinical trial before it commences at that site. Informed written consent must also be obtained from each trial subject. Regulatory authorities, including the FDA, an IRB, a data safety monitoring board or the sponsor, may suspend or terminate a clinical trial at any time on various grounds, including a finding that the participants are being exposed to an unacceptable health risk or that the clinical trial is not being conducted in accordance with FDA requirements.

For purposes of NDA approval, human clinical trials are typically conducted in sequential phases that may overlap:

- *Phase I* - The drug is initially given to healthy human subjects or patients in order to determine metabolism and pharmacologic actions of the drug in humans, side effects and, if possible, to gain early evidence on effectiveness. During Phase I clinical trials, sufficient information about the investigational drug's pharmacokinetics and pharmacologic effects may be obtained to permit the design of well-controlled and scientifically valid Phase II clinical trials.

- *Phase II* - Clinical trials are conducted to evaluate the effectiveness of the drug for a particular indication or in a limited number of patients in the target population to identify possible adverse effects and safety risks, to determine the efficacy of the drug for specific targeted diseases and to determine dosage tolerance and optimal dosage. Multiple Phase II clinical trials may be conducted by the sponsor to obtain information prior to beginning larger and more expensive Phase III clinical trials.

- *Phase III* - When Phase II clinical trials demonstrate that a dosage range of the drug appears effective and has an acceptable safety profile and provide sufficient information for the design of Phase III clinical trials, Phase III clinical trials in an expanded patient population at multiple clinical sites may be undertaken. They are performed after preliminary evidence suggesting effectiveness of the drug has been obtained, and are intended to further evaluate dosage, effectiveness and safety, to establish the overall benefit-risk relationship of the investigational drug and to provide an adequate basis for product labeling and approval by the FDA. In most cases, the FDA requires two adequate and well-controlled Phase III clinical trials to demonstrate the efficacy of the drug in an expanded patient population at multiple clinical trial sites.

- *Phase IV* - The FDA may require, or companies may pursue, additional clinical trials after a product is approved. These Phase IV clinical trials may be made a condition to be satisfied for continuing drug approval. The results of Phase IV clinical trials can confirm the effectiveness of a product candidate and can provide important safety information.

All clinical trials must be conducted in accordance with FDA regulations, GCP requirements and their protocols in order for the data to be considered reliable for regulatory purposes.

An investigational drug product that is a combination of two different drugs in a single dosage form must comply with an additional rule that requires that each component make a contribution to the claimed effects of the drug product. This typically requires larger studies that test the drug against each of its components. In addition, typically, if a drug product is intended to treat a chronic disease, as is the case with some of our product candidates, safety and efficacy data must be gathered over an extended period of time, which can range from six months to three years or more. Government regulation may delay or prevent marketing of product candidates or new drugs for a considerable period of time and impose costly procedures upon our activities.

Disclosure of Clinical Trial Information

Sponsors of clinical trials of FDA-regulated products, including drugs, are required to register and disclose certain clinical trial information. Information related to the product, patient population, phase of investigation, study sites and investigators, and other aspects of the clinical trial, is then made public as part of the registration. Sponsors are also obligated to discuss the results of their clinical trials after completion. Under certain conditions, a sponsor may delay disclosure of the results of these trials until the new product or new indication being studied has been approved. Competitors may use this publicly available information to gain knowledge regarding the progress of development programs.

The NDA Approval Process

In order to obtain approval to market a drug in the United States, a marketing application must be submitted to the FDA that provides data establishing to the FDA's satisfaction the safety and effectiveness of the investigational drug for the proposed indication. Each NDA submission requires a substantial user fee payment (exceeding $3.1 million in fiscal year 2022) unless a waiver or exemption

applies. The application includes all relevant data available from pertinent nonclinical studies, or preclinical studies and clinical trials, including negative or ambiguous results as well as positive findings, together with detailed information relating to the product's chemistry, manufacturing, controls and proposed labeling, among other things. Data can come from company-sponsored clinical trials intended to test the safety and effectiveness of a use of a product, or from a number of alternative sources, including studies initiated by investigators that meet GCP requirements.

During the development of a new drug, sponsors may be given opportunities to meet with the FDA at certain points. These points may be prior to submission of an IND, at the end of Phase II clinical trials, and before an NDA is submitted. Meetings at other times may also be requested. These meetings can provide an opportunity for the sponsor to share information about the data gathered to date, for the FDA to provide advice and for the sponsor and the FDA to reach agreement on the next phase of development. Sponsors typically use the end-of-Phase II clinical trials meetings to discuss their Phase II clinical trials results and present their plans for the pivotal Phase III registration trial that they believe will support approval of the new drug.

Concurrent with clinical trials, companies usually complete additional preclinical safety studies and must also develop additional information about the chemistry and physical characteristics of the drug and finalize a process for the NDA sponsor's manufacturing the product in compliance with current Good Manufacturing Practice ("cGMP") requirements. The manufacturing process must be capable of consistently producing quality batches of the product candidate and the manufacturer must develop methods for testing the identity, strength, quality and purity of the final drugs. Additionally, appropriate packaging must be selected and tested, and stability studies must be conducted to demonstrate that the product candidate does not undergo unacceptable deterioration over its shelf-life.

The results of drug development, nonclinical studies and clinical trials, along with descriptions of the manufacturing process, analytical tests conducted on the chemistry of the drug, proposed labeling and other relevant information are submitted to the FDA as part of an NDA requesting approval to market the product. The FDA reviews all NDAs submitted to ensure that they are sufficiently complete for substantive review before it accepts them for filing. It may request additional information rather than accept an NDA for filing. In this event, the NDA must be resubmitted with the additional information. The resubmitted application is also subject to review before the FDA accepts it for filing. The FDA has 60 days from its receipt of an NDA to conduct an initial review to determine whether the application will be accepted for filing based on the FDA's threshold determination that the application is sufficiently complete to permit substantive review. If the NDA submission is accepted for filing, the FDA reviews the NDA 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 cGMP to assure and preserve the product's identity, strength, quality and purity. The FDA has agreed to specific performance goals on the review of NDAs and seeks to review standard NDAs within 12 months from submission of the NDA. The review process may be extended by the FDA for three additional months to consider certain late submitted information or information intended to clarify information already provided in the submission. After the FDA completes its initial review of an NDA, it will communicate to the sponsor that the drug will either be approved, or it will issue a complete response letter to communicate that the NDA will not be approved in its current form and inform the sponsor of changes that must be made or additional clinical, nonclinical or manufacturing data that must be received before the application can be approved. The FDA does not comment in a complete response letter regarding the ultimate approvability of the application or the timing of any such approval, if ever. If, or when, those deficiencies have been addressed to the FDA's satisfaction in a resubmission of the NDA, the FDA will issue an approval letter. The FDA has committed to reviewing such resubmissions in two to six months depending on the type of information included. The FDA may refer applications for novel drug products or drug products that present difficult questions of safety or effectiveness to an advisory committee, typically a panel that includes clinicians and other experts, for review, evaluation and a recommendation as to whether the application should be approved and, if so, under what conditions. The FDA is not bound by the recommendations of an advisory committee, but it considers such recommendations carefully when making decisions.

Before approving an NDA, the FDA typically will inspect the facilities at which the product is manufactured. The FDA will not approve the product unless it determines that the manufacturing processes and facilities are in compliance with cGMP requirements and adequate to assure consistent production of the product within required specifications. Additionally, before approving an NDA, the FDA may inspect one or more clinical sites to assure compliance with GCP regulations. If the FDA determines the application, manufacturing process or manufacturing facilities are not acceptable, it typically will outline the deficiencies and request additional testing or information. This may significantly delay further review of the application. If the FDA finds that a clinical site did not conduct the clinical trial in accordance with GLP regulations, the FDA may determine the data generated by the clinical site should be excluded from certain analyses provided in the NDA. Additionally, notwithstanding the submission of any requested additional information, the FDA ultimately may decide that the application does not satisfy the regulatory criteria for approval.

The FDA may require, or companies may pursue, Phase IV clinical trials, which are additional clinical trials performed after a product is approved. Phase IV clinical trials may be made a condition to be satisfied for continuing drug approval. The results of Phase IV clinical trials can confirm the effectiveness of a product candidate and can provide important safety information. In addition, the FDA now has express statutory authority to require sponsors to conduct post-marketing trials to specifically address safety issues identified by the agency.

The FDA also has authority to require a Risk Evaluation and Mitigation Strategy ("REMS"), from manufacturers to ensure that the benefits of a drug outweigh its risks. A sponsor may also voluntarily propose a REMS as part of the NDA submission. The need for a REMS is determined as part of the review of the NDA. Based on statutory standards, elements of a REMS may include "dear doctor letters," a medication guide, more elaborate targeted educational programs, and in some cases elements to assure safe use ("ETASU"), which is the most restrictive REMS. ETASU can include, but are not limited to, special training or certification for prescribing or dispensing, dispensing only under certain circumstances, special monitoring and the use of patient registries. These elements are negotiated as part of the NDA approval, and in some cases if consensus is not obtained until after the "review date" set forth under the Prescription Drug User Fee Act of 1992, as amended, the approval date may be delayed. FDA may also require a REMS after the NDA approval based on "new safety information." Once adopted, REMS are subject to periodic assessment and modification.

Changes to some of the conditions established in an approved application, including changes in indications, labeling, manufacturing processes or facilities, require submission and FDA approval of a new NDA or NDA supplement before the change can be implemented. An NDA supplement for a new indication typically requires clinical data similar to that in the original application, and the FDA uses the same procedures and actions in reviewing NDA supplements as it does in reviewing NDAs.

The FDA, under its Fraud, Untrue Statements of Material Facts, Bribery and Illegal Gratuities Policy, can significantly delay the approval of a marketing application, or seek to withdraw an approved application where it identifies fraud or discrepancies in regulatory applications or submissions. Such actions by the FDA may significantly delay or suspend substantive scientific review of a pending application during a validity assessment or remove approved products from the market until the assessment is complete and questions regarding reliability of the data are resolved.

Even if a product candidate receives regulatory approval, the approval may be limited to specific disease states, patient populations and dosages, or might contain significant limitations on use in the form of warnings, precautions or contraindications, or in the form of onerous risk management plans, restrictions on distribution or post-marketing trial requirements. Further, even after regulatory approval is obtained, later discovery of previously unknown problems with a product may result in restrictions on the product or even complete withdrawal of the product from the market. Delay in obtaining, or failure to obtain, regulatory approval for our products, or obtaining approval but for significantly limited use, would harm our business. In addition, we cannot predict what adverse governmental regulations may arise from future U.S. or foreign governmental action.

Orphan Designation and Exclusivity

The FDA may grant orphan drug designation to drugs intended to treat a rare disease or condition that affects fewer than 200,000 individuals in the United States, or if it affects more than 200,000 individuals in the United States and there is no reasonable expectation that the cost of developing and making the drug for this type of disease or condition will be recovered from sales in the United States.

Orphan drug designation entitles a party to financial incentives such as opportunities for grant funding towards clinical study costs, tax advantages, and user-fee waivers. Orphan drug designation does not convey any advantage in, or shorten the duration of, the regulatory review and approval process. In addition, the first NDA or Biologics License Application ("BLA") applicant to receive orphan drug designation for a particular drug is entitled to orphan drug exclusivity, which means the FDA may not approve any other application to market the same drug for the same indication for a period of seven years in the United States, except in limited circumstances. Orphan drug exclusivity does not prevent the FDA from approving a different drug for the same disease or condition, or the same drug for a different disease or condition. Our initial two programs in DM1 and HD are targeting orphan indications.

The Hatch-Waxman Amendments

Under the Drug Price Competition and Patent Term Restoration Act of 1984, as amended, commonly known as the Hatch-Waxman Amendments, a portion of a product's U.S. patent term that was lost during clinical development and regulatory review by the FDA may be restored. The Hatch-Waxman Amendments also provide a process for listing patents pertaining to approved products in the FDA's Approved Drug Products with Therapeutic Equivalence Evaluations (commonly known as the Orange Book) and for a competitor seeking approval of an application that references a product with listed patents to make certifications pertaining to such patents. In addition, the Hatch-Waxman Amendments provide for a statutory protection, known as non-patent exclusivity, against the FDA's acceptance or approval of certain competitor applications.

Patent Term Restoration

Patent term restoration can compensate for time lost during drug development and the regulatory review process by returning up to five years of patent life for a patent that covers a new product or its use. This period is generally one-half the time between the effective date of an IND (falling after issuance of the patent) and the submission date of an NDA, plus the time between the submission date of an NDA and the approval of that application, provided the sponsor acted with diligence. Patent term restorations, however, cannot extend the remaining term of a patent beyond a total of 14 years from the date of product approval and only one patent applicable to an approved drug may be extended and the extension must be applied for prior to expiration of the patent. The United States Patent and Trademark Office, in consultation with the FDA, reviews and approves the application for any patent term extension or restoration.

Orange Book Listing

In seeking approval for a drug through an NDA, applicants are required to list with the FDA each patent whose claims cover the applicant's product. Upon approval of a drug, each of the patents listed by the NDA holder listed in the drug's application or otherwise are then published in the FDA's Orange Book. Drugs listed in the Orange Book can, in turn, be cited by potential generic competitors in support of approval of an abbreviated new drug application ("ANDA"). An ANDA provides for marketing of a drug product that has the same active ingredients in the same strengths and dosage form as the listed drug and has been shown through bioequivalence testing to be therapeutically equivalent to the listed drug. Other than the requirement for bioequivalence testing, ANDA applicants are not required to conduct, or submit results of, preclinical studies or clinical trials to prove the safety or effectiveness of their drug product. Drugs approved in this way are commonly referred to as "generic equivalents" to the listed drug and can often be substituted by pharmacists under prescriptions written for the original listed drug.

The ANDA applicant is required to certify to the FDA concerning any patents listed for the approved product in the FDA's Orange Book. Specifically, the applicant must certify that: (1) the required patent information has not been filed; (2) the listed patent has expired; (3) the listed patent has not expired but will expire on a particular date and approval is sought after patent expiration; or (4) the listed patent is invalid or will not be infringed by the new product. The ANDA applicant may also elect to submit a Section VIII statement certifying that its proposed ANDA label does not contain (or carves out) any language regarding the patented method-of-use rather than certify to a listed method-of-use patent. If the applicant does not challenge the listed patents, the ANDA application will not be approved until all the listed patents claiming the referenced product have expired.

A certification that the new product will not infringe the already approved product's listed patents, or that such patents are invalid, is called a Paragraph IV certification. If the ANDA applicant has provided a Paragraph IV certification to the FDA, the applicant must also send notice of the Paragraph IV certification to the NDA and patent holders once the ANDA has been accepted for filing by the FDA. The NDA and patent holders may then initiate a patent infringement lawsuit in response to the notice of the Paragraph IV certification. The filing of a patent infringement lawsuit within 45 days of the receipt of a Paragraph IV certification automatically prevents the FDA from approving the ANDA until the earlier of 30 months, expiration of the patent, settlement of the lawsuit or a decision in the infringement case that is favorable to the ANDA applicant.

An applicant submitting an NDA under Section 505(b)(2) of the FDCA (a "Section 505(b)(2) NDA"), which permits the filing of an NDA 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, is required to certify to the FDA regarding any patents listed in the Orange Book for the approved product it references to the same extent that an ANDA applicant would.

Market Exclusivity

Market exclusivity provisions under the FDCA also can delay the submission or the approval of certain applications. The FDCA provides a five-year period of non-patent marketing exclusivity within the United States to the first applicant to gain approval of an NDA for a new chemical entity. A drug is a new chemical entity if the FDA has not previously approved any other new drug containing the same active moiety, which is the molecule or ion responsible for the action of the drug substance. During the exclusivity period, the FDA may not accept for review an ANDA or a Section 505(b)(2) NDA submitted by another company for another version of such drug where the applicant does not own or have a legal right of reference to all the data required for approval. However, an application may be submitted after four years if it contains a Paragraph IV certification. The FDCA also provides three years of marketing exclusivity for an NDA, a Section 505(b)(2) NDA or supplement to an existing NDA if new clinical investigations, other than bioavailability studies, that were conducted or sponsored by the applicant are deemed by the FDA to be essential to the approval of the application, for example, for new indications, dosages or strengths of an existing drug. This three-year exclusivity covers only the conditions associated with the new clinical investigations and does not prohibit the FDA from approving ANDAs for drugs containing the original active agent. Five-year and three-year exclusivity will not delay the submission or approval of a full NDA; however, an applicant submitting a full NDA would be required to conduct or obtain a right of reference to all of the nonclinical studies and adequate and well-controlled clinical trials necessary to demonstrate safety and effectiveness.

Post-Marketing Requirements.

Following approval of a new product, a pharmaceutical company and the approved product are subject to continuing regulation by the FDA, including, among other things, monitoring and record-keeping activities, reporting to the applicable regulatory authorities of

adverse experiences with the product, providing the regulatory authorities with updated safety and efficacy information, product sampling and distribution requirements, and complying with promotion and advertising requirements, which include, among others, standards for direct-to-consumer advertising, restrictions on promoting drugs for uses or in patient populations that are not described in the drug's approved labeling (known as "off-label use"), limitations on industry-sponsored scientific and educational activities and requirements for promotional activities involving the internet, including social media. Although physicians may prescribe legally available drugs for off-label uses, manufacturers may not market or promote such off-label uses. Modifications or enhancements to the product or its labeling or changes of the site of manufacture are often subject to the approval of the FDA and other regulators, who may or may not grant approval, or may include in a lengthy review process.

Prescription drug advertising is subject to federal, state and foreign regulations. In the United States, the FDA regulates prescription drug promotion, including direct-to-consumer advertising. Prescription drug promotional materials must be submitted to the FDA in conjunction with their first use. Any distribution of prescription drug products and pharmaceutical samples must comply with the U.S. Prescription Drug Marketing Act of 1987, as amended, a part of the FDCA.

In the United States, once a product is approved, its manufacture is subject to comprehensive and continuing regulation by the FDA. The FDA regulations require that products be manufactured in specific, approved facilities and in accordance with cGMP. We rely, and expect to continue to rely, on third parties for the production of clinical and commercial quantities of our products in accordance with cGMP regulations. cGMP regulations require, among other things, quality control and quality assurance as well as the corresponding maintenance of records and documentation and the obligation to investigate and correct any deviations from cGMP. Drug manufacturers and other entities involved in the manufacture and distribution of approved drugs are required to register their establishments with the FDA and certain state agencies and are subject to periodic unannounced inspections by the FDA and certain state agencies for compliance with cGMP and other laws. Accordingly, manufacturers must continue to expend time, money and effort in the area of production and quality control to maintain cGMP compliance. These regulations also impose certain organizational, procedural and documentation requirements with respect to manufacturing and quality assurance activities. NDA holders using contract manufacturers, laboratories or packagers are responsible for the selection and monitoring of qualified firms and, in certain circumstances, qualified suppliers to these firms. These firms and, where applicable, their suppliers are subject to inspections by the FDA at any time, and the discovery of violative conditions, including failure to conform to cGMP, could result in enforcement actions that interrupt the operation of any such product or may result in restrictions on a product, manufacturer, or holder of an approved NDA, including, among other things, recall or withdrawal of the product from the market.

The FDA also may require post-marketing testing, also known as Phase IV testing, to monitor the effects of an approved product or place conditions on an approval that could restrict the distribution or use of the product. Discovery of previously unknown problems with a product or the failure to comply with applicable FDA requirements can have negative consequences, including adverse publicity, judicial or administrative enforcement, untitled or warning letters from the FDA, mandated corrective advertising or communications with doctors, withdrawal of approval, and civil or criminal penalties, among others. Newly-discovered or developed safety or effectiveness data may require changes to a product's approved labeling, including the addition of new warnings and contraindications, and also may require the implementation of other risk management measures. Also, new government requirements, including those resulting from new legislation, may be established, or the FDA's policies may change, which could delay or prevent regulatory approval of our products in development.

Reimbursement

In the United States, the research, manufacturing, distribution, sale and promotion of drug products and medical devices are potentially subject to regulation by various federal, state and local authorities in addition to the FDA, including the Centers for Medicare & Medicaid Services ("CMS"), other divisions of the U.S. Department of Health and Human Services ("HHS") (*e.g*., the Office of Inspector General), or "OIG", the Drug Enforcement Administration, the Consumer Product Safety Commission, the Federal Trade Commission, the Occupational Safety & Health Administration, the Environmental Protection Agency, state Attorneys General and other state and local government agencies). For example, sales, marketing and scientific/educational grant programs must comply with the federal Anti-Kickback Statute (or "AKS"), the federal False Claims Act (or "FCA"), the privacy regulations promulgated under the Health Insurance Portability and Accountability Act of 1996 ("HIPAA"), and similar state laws. Pricing and rebate programs must comply with the Medicaid Drug Rebate Program requirements of the Omnibus Budget Reconciliation Act of 1990, as amended, and the Veterans Health Care Act of 1992, as amended. If products are made available to authorized users of the Federal Supply Schedule of the General Services Administration, additional laws and requirements apply. The handling of any controlled substances must comply with the U.S. Controlled Substances Act and Controlled Substances Import and Export Act. Products must meet

applicable child-resistant packaging requirements under the U.S. Poison Prevention Packaging Act. All of these activities are also potentially subject to federal and state consumer protection and unfair competition laws.

The Medicare Modernization Act ("MMA") established the Medicare Part D program ("Part D") to provide a voluntary prescription drug benefit to Medicare beneficiaries. Under Part D, Medicare beneficiaries may enroll in prescription drug plans offered by private entities which will provide coverage of outpatient prescription drugs. Unlike Medicare Part A (hospital insurance) and Part B (medical insurance), Part D coverage is not standardized. Part D prescription drug plan sponsors are not required to pay for all covered Part D drugs, and each drug plan can develop its own drug formulary that identifies which drugs it will cover and at what tier or level. However, Part D prescription drug formularies must include drugs within each therapeutic category and class of covered Part D drugs, though not necessarily all the drugs in each category or class. Any formulary used by a Part D prescription drug plan must be developed and reviewed by a pharmacy and therapeutic committee. Government payment for some of the costs of prescription drugs may increase demand for product candidates for which we receive regulatory approval. However, any negotiated prices for our product candidates covered by a Part D prescription drug plan will likely be lower than the prices we might otherwise obtain. Moreover, while the MMA applies only to drug benefits for Medicare beneficiaries, private payors often follow Medicare coverage policy and payment limitations in setting their own payment rates. Any reduction in payment that results from the MMA may result in a similar reduction in payments from non-government payors.

The distribution of pharmaceutical products is subject to additional requirements and regulations, including extensive record-keeping, licensing, storage and security requirements intended to prevent the unauthorized sale of pharmaceutical products.

The American Recovery and Reinvestment Act of 2009 provides funding for the federal government to compare the effectiveness of different treatments for the same illness. A plan for the research will be developed by the Department of Health and Human Services, the Agency for Healthcare Research and Quality and the National Institutes for Health, and periodic reports on the status of the research and related expenditures will be made to Congress. Although the results of the comparative effectiveness studies are not intended to mandate coverage policies for public or private payors, it is not clear what effect, if any, the research will have on the sales of our product candidates, if any such product or the condition that it is intended to treat is the subject of a clinical trial. It is also possible that comparative effectiveness research demonstrating benefits in a competitor's product could adversely affect the sales of our product candidates, if approved. If third-party payors do not consider our products to be cost-effective compared to other available therapies, they may not cover our product candidates after approval as a benefit under their plans or, if they do, the level of payment may not be sufficient to allow us to sell our product candidates on a profitable basis.

In addition, in some foreign countries, the proposed pricing for a drug must be approved before it may be lawfully marketed. The requirements governing drug pricing vary widely from country to country. For example, the European Union provides options for its member states to restrict the range of medicinal products for which their national health insurance systems provide reimbursement and to control the prices of medicinal products for human use. A member state may approve a specific price for the medicinal product or it may instead adopt a system of direct or indirect controls on the profitability of the company placing the medicinal product on the market. There can be no assurance that any country that has price controls or reimbursement limitations for pharmaceutical products will allow favorable reimbursement and pricing arrangements for any of our products. Historically, products launched in the European Union do not follow price structures of the United States and generally tend to be priced significantly lower than in the United States.

Fraud and Abuse

As noted above, in the United States, we are subject to complex laws and regulations pertaining to healthcare "fraud and abuse," including, but not limited to, the federal Anti-Kickback Statute, the federal False Claims Act, and other state and federal laws and regulations. Violating these fraud and abuse laws could result in significant criminal, civil, and administrative sanctions.

The federal Anti-Kickback Statute makes it illegal for any person, including a prescription drug manufacturer, or a party acting on its behalf, to knowingly and willfully solicit, receive, offer or pay any remuneration that is intended to induce the referral of business, including the purchase, order or prescription of a particular drug, or other good or service for which payment in whole or in part may be made under a federal healthcare program, such as Medicare or Medicaid. Violations of this law are punishable by up to five years in prison, criminal fines, administrative civil money penalties and exclusion from participation in federal healthcare programs. In addition, many states have adopted laws similar to the federal Anti-Kickback Statute. Some of these state prohibitions apply to the referral of patients for healthcare services reimbursed by any insurer, not just federal healthcare programs such as Medicare and Medicaid. Due to the breadth of these federal and state anti-kickback laws, the absence of substantive, practical guidance in the form

of regulations or court decisions and the potential for additional legal or regulatory change in this area, it is possible that our future sales and marketing practices or our future relationships with medical and healthcare professionals might be challenged under anti-kickback laws, which could significantly harm us. Because we intend to commercialize product candidates that could be reimbursed under a federal healthcare program and other governmental healthcare programs, we plan to develop a comprehensive compliance program that establishes internal controls to facilitate adherence to the rules and program requirements to which we will or may become subject.

The federal False Claims Act prohibits anyone from knowingly presenting, or causing to be presented, for payment to federal programs (including Medicare and Medicaid) claims for items or services, including drugs, that are false or fraudulent, claims for items or services not provided as claimed or claims for medically unnecessary items or services. The False Claims Act also allows a private individual (acting as a "whistleblower") to bring on action on behalf of the federal government and share in any monetary recoveries. Although we would not submit claims directly to payors, manufacturers can be held liable under these laws if they are deemed to "cause" the submission of false or fraudulent claims by, for example, providing inaccurate billing or coding information to customers or promoting a product off-label. In addition, our future activities relating to the reporting of wholesaler or estimated retail prices for our products, the reporting of prices used to calculate Medicaid rebate information and other information affecting federal, state and third-party reimbursement for our product candidates, and the sale and marketing of our product candidates, are subject to scrutiny under this law. For example, pharmaceutical companies have been found liable under the federal False Claims Act in connection with their off-label promotion of drugs. Penalties for a federal False Claims Act violations are particularly concerning because they include three times the actual damages sustained by the government, plus mandatory civil penalties of between currently $11,803 and $23,607 for each separate false claim, and the potential for exclusion from participation in federal healthcare programs and. Additionally, although the federal False Claims Act is a civil statute, conduct that results in a federal False Claims Act violation may also implicate various federal criminal statutes. If the government were to allege that we were, or convict us of, violating these false claims laws, we could be subject to a substantial fine. In addition, private individuals have the ability to bring actions under the federal False Claims Act and certain states have enacted laws modeled after the federal False Claims Act fines and/or penalties.

There are also an increasing number of state laws that require manufacturers to make reports to states on pricing and marketing information. Many of these laws contain ambiguities as to what is required to comply with the laws. In addition, as

The Civil Monetary Penalties Law allows for the imposition of civil monetary penalties against any person who offers or transfers anything of value to a federal health care program beneficiary that the person knows or should know is likely to influence the beneficiary's selection of a particular provider, practitioner, or supplier for the order or receipt of any item or service reimbursable by a federal health care program. Pharmaceutical manufacturers are typically not considered "providers, practitioners, or suppliers." However, offering anything of value to a beneficiary that is likely to influence the beneficiary to select a particular provider, practitioner, or supplier (e.g., a physician or pharmacy) would implicate the beneficiary inducement provisions of the Civil Monetary Penalties Law (Beneficiary Inducements CMP).

The federal criminal statute on false statements (related to health care matters) makes it a crime to knowingly and willfully falsify, conceal, or cover up any material fact, make any materially false, fictitious or fraudulent statements or representations, or make or use any materially false writing or document knowing the same to contain any materially false, fictitious, or fraudulent statement in connection with the delivery of or payment for health care benefits, items, or services.

As discussed below, a similar the federal requirement Physician Payments Sunshine Act (at times referred to as the Open Payments™ Program) requires manufacturers to track and report to the federal government (for disclosure to the public via a databases) certain payments (i.e., transfers of value) made to physicians and teaching hospitals in the previous calendar year. These laws, The Open Payments Program and its requirements may affect our sales, marketing and other promotional activities by imposing administrative and compliance burdens on us. In addition, given the lack of clarity with respect to these laws and their implementation, our reporting actions could be subject to the penalty provisions of the pertinent state, and soon federal, authorities public and government scrutiny and penalties.

The majority of states also have statutes akin to the federal Anti-Kickback Statute, False Claims Act, and the Physician Payments Sunshine Act that may apply (now or when we have a commercialized product), which, given certain ambiguities around application and execution, may impact the way we operate and increases the potential for non-compliance and risks (e.g., penalties).

Because we intend to commercialize product candidates that could be reimbursed under a federal, state healthcare program and other governmental healthcare programs, we plan to develop a comprehensive compliance program that establishes internal controls to facilitate adherence to the rules and program requirements to which we will or may become subject to. Notably, while we seek to comply with all applicable laws and regulations, we cannot guarantee that our forthcoming program and controls will always protect us from acts committed by employees or third-party vendors.

Federal and state agencies continue to expend significant attention, energy and resources to combat healthcare fraud and abuse within our industry. The failure to comply with regulatory requirements subjects companies to possible legal or regulatory action. Depending on the circumstances, failure to meet applicable regulatory requirements can result in criminal prosecution, fines or other penalties, injunctions, recall or seizure of products, total or partial suspension of production, denial or withdrawal of product approvals or refusal to allow a company to enter into supply contracts, including government contracts.

Changes in regulations, statutes or the interpretation of existing regulations could impact our business in the future by requiring, for example: (1) changes to our manufacturing arrangements; (2) additions or modifications to product labeling; (3) the recall or discontinuation of our products; (4) internal investigations into business practices; (5) the development, enhancement, and implementation of compliance policies and controls, or (6) additional record-keeping requirements. If any such changes were to be imposed, they could adversely affect the operation of our business.

Patient Protection and Affordable Care Act

In March 2010, the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act of 2010 (collectively, the "PPACA"), was enacted, which includes measures that have or will significantly change the way healthcare is financed by both governmental and private insurers. Among the provisions of the PPACA of greatest importance to the pharmaceutical industry are the following:

- The Medicaid Drug Rebate Program requires pharmaceutical manufacturers to enter into and have in effect a national rebate agreement with the Secretary of the Department of Health and Human Services as a condition for states to receive federal matching funds for the manufacturer's covered outpatient drugs furnished to Medicaid patients. Effective in 2010, the PPACA made several changes to the Medicaid Drug Rebate Program, including increasing pharmaceutical manufacturers' rebate liability by raising the minimum basic Medicaid rebate on most branded prescription drugs and biologic agents to 23.1% of the average manufacturer prices ("AMP") and adding a new rebate calculation for "line extensions" (i.e., new formulations, such as extended release formulations) of solid oral dosage forms of branded products, as well as potentially impacting their rebate liability by modifying the statutory definition of AMP. The PPACA also expanded the universe of Medicaid utilization subject to drug rebates by requiring pharmaceutical manufacturers to pay rebates on Medicaid managed care utilization and by expanding the population potentially eligible for Medicaid drug benefits. The CMS have proposed to expand Medicaid rebate liability to the territories of the United States as well. In addition, the PPACA provides for the public availability of retail survey prices and certain weighted average AMPs under the Medicaid program. The implementation of this requirement by the CMS may also provide for the public availability of pharmacy acquisition of cost data, which could negatively impact our sales.

- In order for a pharmaceutical product to receive federal reimbursement under the Medicare Part B and Medicaid programs or to be sold directly to U.S. government agencies, the manufacturer must extend discounts to entities eligible to participate in the 340B drug-pricing program. The required 340B discount on a given product is calculated based on the AMP and Medicaid rebate amounts reported by the manufacturer. The PPACA expanded the types of entities eligible to receive discounted 340B pricing, although, under the current state of the law, with the exception of children's hospitals, these newly-eligible entities will not be eligible to receive discounted 340B pricing on orphan drugs when used for the orphan indication. In addition, as 340B drug pricing is determined based on AMP and Medicaid rebate data, the revisions to the Medicaid rebate formula and AMP definition described above could cause the required 340B discount to increase.

- The PPACA imposes a requirement on manufacturers of branded drugs and biologic agents to provide a 50% discount off the negotiated price of branded drugs dispensed to Medicare Part D patients in the coverage gap (i.e., "donut hole").

- The PPACA imposes an annual, nondeductible fee on any entity that manufactures or imports certain branded prescription drugs and biologic agents, apportioned among these entities according to their market share in certain government healthcare programs, although this fee would not apply to sales of certain products approved exclusively for orphan indications.

- As noted above, the Physician Payments Sunshine Act section of the PPACA requires pharmaceutical manufacturers to track certain financial arrangements with physicians and teaching hospitals, including any "transfer of value" made or distributed to such entities, as well as any investment interests held by physicians and their immediate family members. Manufacturers are required to track this information and were required to make their first reports in March 2014. The information reported is publicly available on a searchable website.

- As of 2010, a new Patient-Centered Outcomes Research Institute was established pursuant to the PPACA to oversee, identify priorities in and conduct comparative clinical effectiveness research, along with funding for such research. The research conducted by the Patient-Centered Outcomes Research Institute may affect the market for certain pharmaceutical products.

- The PPACA created the Independent Payment Advisory Board, which has the authority to recommend certain changes to the Medicare program to reduce expenditures by the program that could result in reduced payments for prescription drugs. Under certain circumstances, these recommendations will become law unless Congress enacts legislation that will achieve the same or greater Medicare cost savings.

- The PPACA established the Center for Medicare and Medicaid Innovation within CMS to test innovative payment and service delivery models to lower Medicare and Medicaid spending, potentially including prescription drug spending. Funding has been allocated to support the mission of the Center for Medicare and Medicaid Innovation through 2020.

Full details regarding the implementation of the PPACA are yet to be determined, and, at this time, the full effect of the PPACA on our business remains unclear. Further, there have been recent public announcements by members of the U.S. Congress, President Trump and his administration regarding their plans to repeal and replace the PPACA. For example, on December 22, 2017, President Trump signed into law the Tax Cuts and Jobs Act of 2017, which, among other things, eliminated the individual mandate requiring most Americans (other than those who qualify for a hardship exemption) to carry a minimum level of health coverage, effective January 1, 2019. We cannot predict the ultimate form or timing of any repeal or replacement of the PPACA or the effect such a repeal or replacement would have on our business.

Pediatric Exclusivity and Pediatric Use

Under the Best Pharmaceuticals for Children Act (the "BPCA"), certain drugs may obtain an additional six months of exclusivity if the sponsor submits information requested in writing by the FDA (a "Written Request") relating to the use of the active moiety of the drug in children. Conditions for exclusivity include the FDA's determination that information relating to the use of a new drug in the pediatric population may produce health benefits in that population, the FDA making a written request for pediatric studies and the applicant agreeing to perform, and reporting on, the requested studies within the statutory timeframe. The FDA may issue a Written Request for studies on unapproved or approved indications where it determines that information relating to the use of a drug in a pediatric population, or part of the pediatric population, may produce health benefits in that population. A sponsor is not required to perform pediatric studies in response to a Written Request. To facilitate and expedite development for pediatric uses, applications under the BPCA are treated as priority applications, with all of the benefits that designation confers.

We have not received a Written Request for such pediatric studies, although we may ask the FDA to issue a Written Request for such studies in the future. To receive the six-month pediatric market exclusivity, we would need to receive a Written Request from the FDA, conduct the requested studies in accordance with a written agreement with the FDA or, if there is no written agreement, in accordance with commonly accepted scientific principles, and submit reports of the studies. A Written Request may include studies for indications that are not currently in the labeling if the FDA determines that such information will benefit the public health. The FDA will accept the reports upon its determination that the studies were conducted in accordance with, and are responsive to, the original Written Request or commonly accepted scientific principles, as appropriate, and that the reports comply with the FDA's filing requirements.

Under the Pediatric Research Equity Act of 2003 (the "PREA"), an NDA or supplement thereto must contain data that are adequate to assess the safety and effectiveness of the drug product for the claimed indications in all relevant pediatric subpopulations, and to

support dosing and administration for each pediatric subpopulation for which the product is safe and effective. The PREA also authorizes the FDA to require holders of approved NDAs for marketed drugs to conduct pediatric studies under certain circumstances. With the enactment of the Food and Drug Administration Safety and Innovation Act (the "FDASIA"), in 2012, sponsors must also submit pediatric study plans prior to the assessment data. Those plans must contain an outline of the proposed pediatric study or studies the applicant plans to conduct, including study objectives and design, any deferral or waiver requests, and other information required by regulation. The applicant, the FDA and the FDA's internal review committee must then review the information submitted, consult with each other and agree upon a final plan. The FDA or the applicant may request an amendment to the plan at any time.

The FDA may, on its own initiative or at the request of the applicant, grant deferrals for submission of some or all pediatric data until after approval of the product for use in adults, or full or partial waivers from the pediatric data requirements. Additional requirements and procedures relating to deferral requests and requests for extension of deferrals are contained in the FDASIA. Unless otherwise required by regulation, the pediatric data requirements do not apply to products with orphan designation.

Human Capital Resources

In order to achieve the goals and expectations of our Company, it is crucial that we continue to attract and retain top talent. To facilitate talent attraction and retention, we strive to make NeuBase a safe and rewarding workplace, with opportunities for our employees to grow and develop in their careers, supported by strong compensation, benefits and health and wellness programs, and by programs that build connections between our employees.

As of September 30, 2022, we had 37 full-time employees in the United States. As disclosed in our Current Report on Form 8-K filed October 14, 2022, in connection with a reprioritization of our clinical and research initiatives, we reduced our workforce by approximately 60% in October 2022. None of our employees are represented by a collective bargaining agreement. We believe that we have a good relationship with our employees.

The success of our business is fundamentally connected to the well-being of our employees. Accordingly, we are committed to their health, safety and wellness. We provide our employees and their families with access to a variety of innovative, flexible and convenient health and wellness programs, including benefits that provide protection and security so they can have peace of mind concerning events that may require time away from work or that impact their financial well-being; that support their physical and mental health by providing tools and resources to help them improve or maintain their health status and encourage engagement in healthy behaviors; and that offer choice where possible so they can customize their benefits to meet their needs and the needs of their families.

In response to the COVID-19 pandemic, we implemented significant changes that we determined were in the best interest of our employees, as well as the communities in which we operate, and which comply with government regulations.

We provide robust compensation and benefits programs to help meet the needs of our employees. In addition to salaries, these programs include potential annual discretionary bonuses, stock awards, a 401(k) Plan, healthcare and insurance benefits, health savings and flexible spending accounts, paid time off, family leave, and flexible work schedules, among others. In addition to our broad-based equity award programs, we have used targeted equity-based grants with vesting conditions to facilitate retention of personnel, particularly those with critical drug development skills and experience.

Corporate Information

We were incorporated under the laws of the State of Delaware on August 4, 2009, as successor to BBM Holdings, Inc. (formerly known as Prime Resource, Inc., which was organized March 29, 2002 as a Utah corporation) pursuant to a reincorporation merger. On August 4, 2009, we reincorporated in Delaware as "Ohr Pharmaceutical, Inc." On July 12, 2019, we completed the Merger with NeuBase Corporation (formerly known as NeuBase Therapeutics, Inc.), a Delaware corporation, and, upon completion of the Merger, we changed our name to "NeuBase Therapeutics, Inc." Shares of our common stock commenced trading on The Nasdaq Capital Market under the ticker symbol "NBSE" as of market open on July 15, 2019.

Address

Our principal executive offices are located at 350 Technology Drive, Pittsburgh, PA 15219, and our telephone number is (412) 763-3350. Our website is located at www.neubasetherapeutics.com. Any information contained on, or that can be accessed through, our website is not incorporated by reference into, nor is it in any way part of, this Form 10-K.

Recent Developments

Approval of Reverse Stock Split

On September 8, 2022, the Company held its 2022 annual meeting of stockholders, pursuant to which the stockholders of the Company voted in favor of a series of alternate amendments to the Company's amended and restated certificate of incorporation to effect, at the discretion of the Company's board of directors at any time prior to the Company's 2023 annual meeting of stockholders, a reverse split of the Company's common stock, whereby each outstanding 5, 6, 7, 8, 9, 10, 11, 12, 13, 14, 15, 16, 17, 18, 19 or 20 shares would be combined, converted or changed into one share of the Company's common stock.

Strategic Restructuring Focused on Advancing Platform in Gene Editing

On October 14, 2022, the Company announced a strategic restructuring to expand its focus to include the advancement of its platform into gene editing. As part of the cost-cutting strategy and development pipeline shift to gene editing, the Company will defer preclinical activities for its DM1, HD, and KRAS programs, hold plans to submit an IND application for DM1 to the FDA, and pursue collaborative initiatives, including partnerships, for these programs. The Company estimates that it will incur total expenses relating to the restructuring of approximately $0.5 million, consisting of severance and termination-related costs, and expects to record a significant portion of these charges in the fourth quarter of calendar year 2022.This restructuring plan is expected to extend the Company's cash runway into the second quarter of calendar year 2024 based on current operating plans and estimates.

On October 21, 2022, in line with our strategic restructuring to expand our focus to include the advancement of our platform into gene editing, we announced that we entered into a research agreement with a global healthcare company, pursuant to which such global healthcare company will evaluate our PATrOL™ platform for three monogenic genetic diseases and we will collaborate on the evaluation of drug candidates for three undisclosed indications.

Available Information

We file annual, quarterly and current reports, proxy statements and other information with the Securities and Exchange Commission (the "SEC"), and we have an Internet website address at www.neubasetherapeutics.com. We make available free of charge on our Internet website address 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 Sections 13(a) or 15(d) of the Securities Exchange Act of 1934, as amended (the "Exchange Act"), as well as our proxy statements as soon as reasonably practicable after we electronically file such material with, or furnish it to, the SEC. You may also obtain copies of such documents from the SEC's website at http://www.sec.gov.

ITEM 1A. RISK FACTORS

Summary of Risk Factors

Below is a summary of the principal factors that make an investment in our common stock speculative or risky. This summary does not address all of the risks that we face. Additional discussion of the risks summarized in this risk factor summary, and other risks that we face, can be found below under the heading "Risk Factors" and should be carefully considered, together with other information in this Form 10-K and our other filings with the SEC before making an investment decision regarding our common stock.

- We have a limited operating history and face significant challenges and expense as we build our capabilities.
- We have incurred net losses in every period since our inception and anticipate that we will incur substantial net losses in the future.

- The approach we are taking to discover and develop nucleic acid therapeutics is novel and may never lead to marketable products.
- Anti-gene technology is a relatively new technology, and our revenue opportunities will be materially limited if we are unable to use this technology in our intended product pipeline.
- We will need substantial additional financing to develop our products and implement our operating plan. If we fail to obtain additional financing, we will be unable to complete the development and commercialization of our product candidates.
- If we fail to establish and maintain proper and effective internal controls, our ability to produce accurate financial statements on a timely basis could be impaired, which would adversely affect our consolidated operating results, our ability to operate our business, our ability to raise capital, and our stock price.
- The COVID-19 pandemic has led to a national state of emergency in the United States. Despite the wide-spread availability of COVID-19 vaccines, given the prevalence of new COVID-19 variants it remains unclear what the overall impact of COVID-19 will be on our business.
- We will likely be heavily reliant on our partners for access to key resources for the manufacturing and development of our product candidates.
- Our product pipeline is based on novel technologies, which makes it difficult to predict the time and cost of product candidate development and obtaining regulatory approval.
- Our strategic restructuring focused on the prioritization of our gene editing platform, leadership transitions, and adjustments to our operating plans, including operating expense reductions, may not be successful, may not result in product revenues or anticipated savings, could result in total costs and expenses that are greater than expected, could result in our foregoing business opportunities and could disrupt our business.
- Our business is highly dependent on the success of our platform and lead product candidates. If we are unable to obtain approval for our lead product candidates and effectively commercialize our lead product candidates for the treatment of patients in approved indications, our business would be significantly harmed.
- The programs in our product pipeline may cause undesirable side effects or have other properties that could halt their preclinical or clinical development, prevent their regulatory approval, limit their commercial potential or result in significant negative consequences.
- Any potential clinical trials in the future may fail to demonstrate the safety and efficacy of any of our product candidates, which would prevent or delay regulatory approval and commercialization.
- We may encounter substantial delays in our preclinical testing and in future clinical trials (particularly given the effects of the COVID-19 global pandemic) or may not be able to conduct such efforts on the timelines we expect.
- If we encounter difficulties enrolling patients in our future clinical trials, our clinical development activities could be delayed or otherwise adversely affected.
- We face significant competition from other biotechnology and pharmaceutical companies, and our operating results will suffer if we fail to compete effectively.
- We are highly dependent on our key personnel, and if we are not successful in attracting and retaining highly qualified personnel, we may not be able to successfully implement our business strategy.

- We will rely on third parties to conduct our clinical trials and manufacture our product candidates in the future. If these third parties do not successfully carry out their contractual duties or meet expected deadlines, we may not be able to obtain regulatory approval of or commercialize our product candidates.

- We depend on intellectual property licensed from third parties and termination of any of these licenses could result in the loss of significant rights, which would harm our business.

- If we fail to adequately protect or enforce our intellectual property rights or secure rights to patents of others, the value of those rights would diminish. Legal proceedings to protect or enforce our patents, the patents of our partners, our licensed patents or our other intellectual property rights could be expensive, time consuming, and unsuccessful.

- Any claims or lawsuits relating to infringement of intellectual property rights brought by or against us will be costly and time consuming and may adversely affect our business, financial condition and results of operations.

- We have never paid dividends on our capital stock and we do not intend to pay dividends for the foreseeable future. Consequently, any gains from an investment in our common stock will likely depend on whether the price of our common stock increases.

- Our charter documents and Delaware law could prevent a takeover that stockholders consider favorable and could also reduce the market price of our stock.

- The market price of our common stock is likely to be volatile and could fluctuate or decline, resulting in a substantial loss of your investment.

- Our stock price could remain below $1.00 per share and cause us to become delisted from Nasdaq.

- Future sales and issuances of our common stock or rights to purchase common stock, including pursuant to our equity incentive plans, could cause you to incur dilution and could cause the market price of our common stock to fall.

Risk Factors

We operate in a dynamic and rapidly changing environment that involves numerous risks and uncertainties. Certain factors may have a material adverse effect on our business, prospects, financial condition and results of operations, and you should carefully consider them. Accordingly, in evaluating our business, we encourage you to consider the following discussion of risk factors in its entirety, in addition to other information contained in this Form 10-K and our other public filings with the SEC. Other events that we do not currently anticipate or that we currently deem immaterial may also affect our business, prospects, financial condition and results of operations.

Risks Related to the Company

We are a preclinical-stage company, have a very limited operating history, are not currently profitable, do not expect to become profitable in the near future and may never become profitable.

We are a preclinical-stage biotechnology company specializing in the discovery and development of a class of deoxy-ribonucleic acid and ribonucleic acid-targeting drugs called peptide nucleic acids or anti-genes, which did not change as a result of the merger between Ohr Pharmaceutical, Inc., a Delaware corporation and NeuBase Therapeutics, Inc., a Delaware corporation, in accordance with the terms of the Agreement and Plan of Merger Reorganization entered into on January 2, 2019. Since our incorporation, we have focused primarily on the development of our proprietary Peptide-nucleic acid AnTisense OLigo platform and preclinical-stage therapeutic candidates. Our platform technology and all of our therapeutic candidates are in the preclinical development stage, and we have not initiated clinical trials for any of our product candidates, nor have any products been approved for commercial sale and we have not generated any revenue. To date, we have not completed a clinical trial (including a pivotal clinical trial), obtained marketing approval for any product candidates, manufactured a commercial scale product or arranged for a third party to do so on our behalf, or conducted sales and marketing activities necessary for successful product commercialization. Drug development is also a highly uncertain undertaking and involves a substantial degree of risk.

As a result, we have no meaningful historical operations upon which to evaluate our business and prospects and have not yet demonstrated an ability to obtain marketing approval for any of our product candidates or successfully overcome the risks and uncertainties frequently encountered by companies in the pharmaceutical industry. We also have not generated any revenues from collaboration and licensing agreements or product sales to date and continue to incur research and development and other expenses. Our prior losses, combined with expected future losses, have had and will continue to have an adverse effect on our stockholders' equity and working capital, and our future success is subject to significant uncertainty.

For the foreseeable future, we expect to continue to incur losses, which we expect will increase significantly from recent historical levels as we advance our gene editing platform, expand our drug development activities, seek regulatory approvals for our product candidates and begin to commercialize them if they are approved by the U.S. Food and Drug Administration (the "FDA"), the European Medicines Agency (the "EMA") or comparable foreign authorities. Even if we succeed in developing and commercializing one or more product candidates, we may never become profitable.

Our strategic restructuring focused on the prioritization of our gene editing platform, leadership transitions, and adjustments to our operating plans, including operating expense reductions, may not be successful, may not result in product revenues or anticipated savings, could result in total costs and expenses that are greater than expected, could result in our forgoing business opportunities and could disrupt our business.

In October 2022, we announced a strategic restructuring to expand our focus to include the advancement of the differentiated gene editing capabilities of our platform. As part of the development pipeline shift to gene editing, we will defer preclinical activities for our DM1, HD, and KRAS programs, hold plans to submit an IND application for DM1 to the FDA, and pursue collaborative initiatives, including partnerships, for these programs. We estimate that we will incur total expenses relating to the restructuring of approximately $0.5 million, consisting of severance and termination-related costs and expect to record a significant portion of these charges in the fourth quarter of calendar year 2022. This restructuring plan is expected to extend our cash runway into the second quarter of calendar year 2024 based on current operating plans and estimates. As part of our cost reduction plan, we implemented a cross-functional reduction of approximately 60% of our then-current workforce. In October 2022, we also announced certain changes to our leadership team, including the resignations of our Head of Research and Development and Chief Medical Officer and President

and Chief Operating Officer, as well as the appointment of Dr. Dov A. Goldstein, a member of our board of directors since 2019, as Chairperson of the Board.

We may not realize, in full or in part, the anticipated benefits, savings and improvements from these changes or from any future changes we may decide to make. For instance, our aforementioned restructuring and the changes to our business strategy and organization may not result in advancement of our gene editing platform or product revenues. The reduction in the size of our organization may also limit the success of our strategy.

Our organizational changes, operating plan adjustments and operating expense reductions also may not be successful. If we are unable to realize the expected operational efficiencies and cost savings from these changes, our operating results and financial condition would be adversely affected. We also cannot ensure that we will not have to undertake additional workforce reductions or other cost-cutting measures in the future. Further, these changes as well as the leadership transitions, strategic reprioritization, and any future additional workforce reductions or other cost-cutting measures may be disruptive to our operations. For example, our workforce reductions, leadership changes and strategic reprioritization could yield unanticipated consequences, such as attrition beyond planned staff reductions and negative impact on employee morale or could make it more difficult to fulfill our day-to-day operations. Our workforce reductions, leadership changes and strategic reprioritization could also harm our ability to attract and retain qualified management, scientific, clinical, manufacturing and sales and marketing personnel who are critical to our business. In 2022, our annualized turnover rate was higher than in prior years and these changes could further or exacerbate that trend. Any failure to attract or retain qualified personnel could prevent us from successfully advancing our gene editing platform.

The approach we are taking to discover and develop nucleic acid therapeutics is novel and may never lead to marketable products.

We have concentrated our efforts and research and development activities on nucleic acid therapeutics and our synthetic chemistry drug discovery and development platform comprised of peptide nucleic acids with natural and engineered nucleotides and targeting technology. Our future success depends on the successful development and manufacturing of such therapeutics and the effectiveness of our platform. The scientific discoveries that form the basis for our efforts to discover and develop new drugs, including our discoveries about the relationships between oligonucleotide stereochemistry and pharmacology, are relatively new. The scientific evidence to support the feasibility of developing drugs based on these discoveries or PNAs in general is limited. Skepticism as to the feasibility of developing nucleic acid therapeutics and PNAs generally has been, and may continue to be, expressed in scientific literature. In addition, decisions by, and negative results of, other companies with respect to their oligonucleotide development efforts may increase skepticism in the marketplace regarding the potential for oligonucleotides and PNAs.

Relatively few nucleic acid therapeutic product candidates have been tested in humans, and a number of clinical trials for such therapeutics conducted by other companies have not been successful. Few nucleic acid therapeutics have received regulatory approval. The pharmacological properties ascribed to the investigational compounds we are testing in laboratory studies may not be positively demonstrated in clinical trials in patients, and they may interact with human biological systems in unforeseen, ineffective or harmful ways. If our nucleic acid product candidates prove to be ineffective, unsafe or commercially unviable, our entire platform and pipeline would have little, if any, value, which would substantially harm our business, financial condition, results of operations and prospects.

In addition, our approach, which focuses on using nucleic acid therapeutics for drug development, as opposed to multiple or other, more advanced proven technologies, may expose us to additional development and financial risks and make it more difficult to raise additional capital if we are not successful in developing a nucleic acid therapeutic that is timely and cost effective to manufacture and achieves proof of concept in animal models, desired tissue distribution, selectivity for the target, and/or regulatory approval. Because our programs are all in the preclinical stage, we have not yet been able to assess safety in humans, and there may be long-term effects from treatment with any product candidates that we develop using our platform that we cannot predict at this time. Any product candidates the Company may develop will act at the level of deoxyribonucleic acid (“DNA”) or ribonucleic acid (“RNA”), and because animal DNA and RNA often differs from human DNA or RNA at the sequence level, in its regulation and degradation, secondary and tertiary structural conformations and ultimately in being translated into proteins with varying amino acid sequences conformations and functions, testing of our product candidates in animal models may not be predictive of the results we observe in human clinical trials of our product candidates for either safety or efficacy. Also, animal models may not exist for some of the diseases we choose to pursue in our programs. As a result of these factors, it is more difficult for us to predict the time and cost of product candidate development, and we cannot predict whether the application of our gene silencing technology, or any similar or competitive gene silencing technologies, will result in the identification, development and regulatory approval of any products.

There can be no assurance that any development problems we experience in the future related to our gene silencing technology or any of our research programs will not cause significant delays or unanticipated costs, or that such development problems can be solved. Should we encounter development problems, including unfavorable preclinical or clinical trial results, the FDA and foreign regulatory authorities may refuse to approve our product candidates, or may require additional information, tests or trials, which could significantly delay product development and significantly increase our development costs. Moreover, even if we are able to provide the requested information or trials to the FDA, there would be no guarantee that the FDA would accept them or approve our product candidates. We may also experience delays in developing a sustainable, reproducible and scalable manufacturing process or developing or qualifying and validating product release assays, other testing and manufacturing methods, and our equipment and facilities in a timely manner, which may prevent us from completing our clinical trials or commercializing our product candidates on a timely or profitable basis, if at all. Any of these factors may prevent us from completing our preclinical studies or any clinical trials that we may initiate or from commercializing any product candidates we may develop on a timely or profitable basis, if at all.

We are highly dependent on the success of our initial product candidates targeting rare genetic diseases and our platform technology in general, and we cannot be certain that any of them will receive regulatory approval or be commercialized.

We have spent time, money and effort on the licensing and development of our core asset: our PATrOL™ platform. To date, we have not submitted an Investigational New Drug application ("IND") to the FDA, and no clinical trials have commenced for any of our product candidates. All of our product candidates will require additional development, including further preclinical studies and bioanalytic method development as well as clinical trials to evaluate their toxicology, carcinogenicity and pharmacokinetics, efficacy, and optimize their formulation, and receive regulatory clearances before they can be commercialized. Positive results obtained during early development do not necessarily mean later development will succeed or that regulatory clearances will be obtained. Our drug development efforts may not lead to commercial drugs, either because our product candidates or our PATrOL™ platform are not deemed safe and effective, because of competitive or market forces, intellectual property issues or because we have inadequate financial or other resources to advance our product candidates through the clinical development and approval processes. If any of our product candidates, or our PATrOL™ platform, fail to demonstrate safety or efficacy at any time or during any phase of development, we would experience potentially significant delays in, or be required to abandon, development of the product candidate.

We do not anticipate that any of our current product candidates will be eligible to receive regulatory approval from the FDA, the EMA or comparable foreign authorities and begin commercialization for a number of years, if ever. Even if we ultimately receive regulatory approval for any of these product candidates, we or our potential future partners, if any, may be unable to commercialize them successfully for a variety of reasons. These include, for example, the availability of alternative treatments, lack of cost-effectiveness, the cost of manufacturing the product on a commercial scale and competition with other drugs. The success of our product candidates and our PATrOL™ platform may also be limited by the prevalence and severity of any adverse side effects. If we fail to commercialize one or more of our current product candidates, we may be unable to generate sufficient revenues to attain or maintain profitability, and our financial condition may decline.

If development of our candidates does not produce favorable results, we and our collaborators, if any, may be unable to commercialize these products.

To receive regulatory approval for the commercialization of the PATrOL™ platform, or any product candidates that we may develop, adequate and well-controlled clinical trials must be conducted to demonstrate safety and efficacy in humans to the satisfaction of the FDA, the EMA and comparable foreign authorities. In order to support marketing approval, these agencies typically require successful results in one or more Phase III clinical trials, which our current product candidates have not yet reached and may never reach. The development process is expensive, can take many years and has an uncertain outcome. Failure can occur at any stage of the process. We may experience numerous unforeseen events during, or as a result of, the development process that could delay or prevent commercialization of our current or future product candidates, including the following:

- preclinical studies conducted with product candidates for potential clinical development to evaluate their toxicology, carcinogenicity and pharmacokinetics and optimize their formulation, among other things, may produce unfavorable results;
- patient recruitment and enrollment in clinical trials may be slower than we anticipate;
- clinical trials may produce negative or inconclusive results;

- costs of development may be greater than we anticipate;
- the potential advantages of the PATrOL™-enabled anti-gene drug candidates may not materialize and thus would confer no benefits to patients over other parties' products that may emerge;
- our product candidates or our PATrOL™ platform may cause undesirable side effects that delay or preclude regulatory approval or limit their commercial use or market acceptance, if approved;
- collaborators who may be responsible for the development of our product candidates may not devote sufficient resources to these clinical trials or other preclinical studies of these candidates or conduct them in a timely manner; or
- we may face delays in obtaining regulatory approvals to commence one or more clinical trials.

Additionally, because our technology potentially involves mutation silencing via genome binding and/or editing across multiple cell and tissue types, we are subject to many of the challenges and risks that advanced therapies, such as gene therapies, face, including:

- regulatory requirements governing gene and cell therapy products have changed frequently and may continue to change in the future;
- improper modification of a gene sequence in a patient's genome could lead to lymphoma, leukemia or other cancers, or other aberrantly functioning cells; and
- the FDA recommends a follow-up observation period of 15 years or longer for all patients who receive treatment using gene therapies, and we may need to adopt and support such an observation period for our product candidates.

Success in early development does not mean that later development will be successful because, for example, product candidates in later-stage clinical trials may fail to demonstrate sufficient safety and efficacy despite having progressed through initial clinical trials.

We may license or acquire intellectual property related to our product candidates from universities. Some of our preclinical studies and other analyses with respect to our product candidates may be performed by their original owners or collaborators. As a company, we have limited experience in conducting research on our platform technology and preclinical trials for our product candidates. Since our experience with our platform technology and product candidates is limited, we will need to train our existing personnel or hire additional personnel in order to successfully administer and manage our preclinical studies and clinical trials as anticipated, which may result in delays in completing such anticipated preclinical trials and clinical studies.

We currently do not have strategic collaborations in place for clinical development of our platform technology and any of our current product candidates. Therefore, in the future, we or any potential future collaborative partner will be responsible for establishing the targeted endpoints and goals for development of our product candidates. These targeted endpoints and goals may be inadequate to demonstrate the safety and efficacy levels required for regulatory approvals. Even if we believe data collected during the development of our product candidates are promising, such data may not be sufficient to support marketing approval by the FDA, the EMA or comparable foreign authorities.

Further, data generated during development can be interpreted in different ways, and the FDA, the EMA or comparable foreign authorities may interpret such data in different ways than we or our collaborators. Our failure to adequately demonstrate the safety and efficacy of our platform technology and any of our product candidates would prevent our receipt of regulatory approval, and such failure would ultimately prevent the potential commercialization of these product candidates.

Since we do not currently possess the resources necessary to independently develop and commercialize our product candidates or any other product candidates that we may develop, we may seek to enter into collaborative agreements to assist in the development and potential future commercialization of some or all of these assets as a component of our strategic plan. Our discussions with potential collaborators, however, may not lead to the establishment of collaborations on acceptable terms, if at all, or it may take longer than expected to establish new collaborations, leading to development and potential commercialization delays, which would adversely affect our business, financial condition and results of operations.

We expect to continue to incur significant research and development expenses, which may make it difficult for us to attain profitability.

We expect to expend substantial funds in research and development, including preclinical studies and clinical trials for our platform technology and product candidates, and to manufacture and market any product candidates in the event they are approved for commercial sale. We have spent significant time, money and effort on the development of our PATrOL™ platform. To date, we have not submitted an IND to the FDA, and no clinical trials have commenced for any of our product candidates. We expect to expend additional substantial funds into advancing our PATrOL™ platform before advancing several preclinical activities in connection with product candidates.

We will likely need additional funding to develop or acquire complementary companies, technologies and assets, as well as for working capital requirements and other operating and general corporate purposes. Moreover, an increase in our headcount would dramatically increase our costs in the near and long-term.

Such spending may not yield any commercially viable products. Due to our limited financial and managerial resources, we must focus on a limited number of research programs and product candidates and on specific indications. Our resource allocation decisions may cause us to fail to capitalize on viable commercial products or profitable market opportunities.

Because the successful development of our product candidates is uncertain, we are unable to precisely estimate the actual funds we will require to develop and potentially commercialize them. In addition, we may not be able to generate sufficient revenue, even if we are able to commercialize any of our product candidates, to become profitable.

We are significantly dependent on the success of our PATrOL™ platform and our product candidates based on this platform. A failure of any product candidate based on this platform in clinical development would adversely affect our business and may require us to discontinue development of other product candidates based on the same therapeutic approach.

We have invested, and we expect to continue to invest, significant efforts and financial resources in the development of product candidates based on our PATrOL™ platform. Our ability to generate meaningful revenue, which may not occur for the foreseeable future, if ever, will depend heavily on the successful development, regulatory approval and commercialization of one or more of these product candidates using our PATrOL™ platform. We will not be able to develop new product candidates if it is found that the PATrOL™ platform does not work or create product candidates that are not safe for use in humans. Since all of our product candidates in our current pipeline are based on our PATrOL™ platform, if any product candidate fails in development as a result of an underlying problem with our PATrOL™ platform, then we may be required to discontinue development of all product candidates that are based on our therapeutic approach. If we were required to discontinue the development of such product candidates based on the PATrOL™ platform, or if any of them were to fail to receive regulatory approval or achieve sufficient market acceptance, we could be prevented from or significantly delayed in achieving profitability. We can provide no assurance that we would be successful at developing other product candidates based on an alternative therapeutic approach from our PATrOL™ platform.

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.

Because we have limited financial and managerial resources, we will initially develop our lead product candidates for particular rare genetic diseases. As a result, we may forego or delay pursuit of opportunities in other types of diseases that may prove to have greater treatment potential. Likewise, we may forego or delay the pursuit of opportunities with other potential product candidates that may 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 product candidates. 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 similar arrangements in cases in which it would have been more advantageous for us to retain sole development and commercialization rights to the product candidate.

Management has determined that there are factors that raised substantial doubt about our ability to continue as a going concern.

The accompanying audited financial statements have been prepared assuming we will continue to operate as a going concern, which contemplates the realization of assets and settlement of liabilities in the normal course of business, and do not include any adjustments to reflect the possible future effects on the recoverability and classification of assets or the amounts and classifications of liabilities that may result from uncertainty related to our ability to continue as a going concern. We have had no revenues from product sales and have incurred operating losses since inception. As of September 30, 2022, we had $23.2 million in cash and cash equivalents and during the fiscal year ended September 30, 2022, we incurred a loss from operations of $33.3 million and used $29.0 million of cash in operating activities. These factors raised substantial doubt about our ability to continue as a going concern. In October 2022, the Company announced a restructuring plan to reduce its operating expenses and extend its cash runway into the second quarter of calendar year 2024 based on current operating plans and estimates. Management believes it is probable that the restructuring plan will be effectively implemented within the next twelve months and that the restructuring plan, when implemented, will mitigate the conditions that gave rise to substantial doubt about the Company's ability to continue as a going concern. Because the Company has sufficient resources on hand to fund operations through at least the next twelve months from the date these consolidated financial statements were available to be issued, the substantial doubt has been alleviated.

Our ability to continue as a going concern is dependent on our ability to raise the required additional equity or debt financing to meet short and long-term operating requirements. We may also encounter business endeavors that require significant cash commitments or unanticipated problems or expenses that could result in a requirement for additional cash, including as a result of COVID-19 and its impacts. If we raise additional funds through the issuance of equity or convertible debt securities in the future, the percentage ownership of our current stockholders could be reduced, and such securities might have rights, preferences or privileges senior to our common stock. Additional financing may not be available upon acceptable terms, or at all. If adequate funds are not available or are not available on acceptable terms, we may not be able to take advantage of prospective business endeavors or opportunities, which could significantly and materially restrict our operations.

Given our lack of current cash flow, we will need to raise additional capital to achieve our goals; however, it may be unavailable to us or, even if capital is obtained, may cause dilution or place significant restrictions on our ability to operate our business.

Since we will be unable to generate sufficient, if any, cash flow to fund our operations for the foreseeable future, we will need to seek additional equity or debt financing to provide the capital required to maintain or expand our operations.

Our existing balance of cash and cash equivalents may not be sufficient to enable us to fund our operations for at least the next twelve months from the date that this Form 10-K is filed with the SEC. In particular, we expect that we will need to obtain funding to allow us to achieve certain preclinical milestones for our NT-0100 program for HD and our NT-0200 program for DM1 and to obtain clinical data from the programs. We have based these estimates on assumptions that may prove to be wrong, and our use of our capital resources may be different than what we currently expect. Our operating plans and other demands on our cash resources may change as a result of many factors currently unknown to us, and we may need to seek additional funds sooner than planned, through public or private equity or debt financings or other capital sources, including potentially collaborations, licenses and other similar arrangements. In addition, we may seek additional capital due to favorable market conditions or strategic considerations even if we believe we have sufficient funds for our current or future operating plans.

There can be no assurance that we will be able to raise sufficient additional capital on acceptable terms or at all. If such additional financing is not available on satisfactory terms, or is not available in sufficient amounts, we may be required to delay, limit or eliminate the development of business opportunities, and our ability to achieve our business objectives, our competitiveness, and our business, financial condition and results of operations may be materially adversely affected. In addition, we may be required to grant rights to develop and market product candidates that we would otherwise prefer to develop and market ourselves. Our inability to fund our business could lead to the loss of your investment

Our future capital requirements will depend on many factors, including, but not limited to:

- the scope, rate of progress, results and cost of further validating our PATrOL™ platform's capabilities, our preclinical studies, clinical trials and other related activities;

- our ability to establish and maintain strategic collaborations, licensing or other arrangements and the financial terms of such arrangements;
- the timing of, and the costs involved in, obtaining regulatory approvals for any of our current or future product candidates;
- the number and characteristics of the product candidates it seeks to develop or commercialize;
- the cost of manufacturing clinical supplies, and establishing commercial supplies, of our product candidates;
- the cost of commercialization activities if any of our current or future product candidates are approved for sale, including marketing, sales and distribution costs;
- the expenses needed to attract and retain skilled personnel;
- the costs associated with being a public company;
- the costs incurred in maintaining appropriate facilities to be able to perform the necessary work to develop our products;
- the amount of revenue, if any, received from commercial sales of our product candidates, should any of our product candidates receive marketing approval; and
- the costs involved in preparing, filing, prosecuting, maintaining, defending and enforcing possible patent claims, including litigation costs and the outcome of any such litigation.

Any additional capital efforts may divert our management from their day-to-day activities, which may adversely affect our ability to develop and commercialize our product candidates. Moreover, if we raise additional capital by issuing equity securities, the percentage ownership of our existing stockholders may be reduced, and accordingly these stockholders may experience substantial dilution. We may also issue equity securities that provide for rights, preferences and privileges senior to those of our common stock.

Given our need for cash and that equity issuances are the most common type of fundraising for similarly situated companies, the risk of dilution is particularly significant for our stockholders. Furthermore, the incurrence of indebtedness would result in increased fixed payment obligations and we may be required to agree to certain restrictive covenants, such as limitations on our ability to incur additional debt and other operating restrictions that could adversely impact our ability to conduct our business. We could also be required to seek funds through arrangements with collaborators or otherwise at an earlier stage than otherwise would be desirable and we may be required to relinquish rights to some of our product candidates or otherwise agree to terms unfavorable to us, any of which may have a material adverse effect on our business, operating results and prospects.

Our efforts to discover product candidates beyond our current product candidates may not succeed, and any product candidates we recommend for clinical development may not actually begin clinical trials.

We intend to use our technology, including our licensed technology, knowledge and expertise, to develop novel drug candidates to address some of the world's most devastating and costly central nervous system, muscular, and other disorders, including orphan genetic and oncology indications. We intend to expand our existing pipeline of core assets by advancing drug candidate compounds from discovery programs into preclinical and clinical development. However, the process of researching and discovering drug candidate compounds is expensive, time-consuming and unpredictable. Data from our current preclinical programs may not support the clinical development of our lead compounds or other compounds from these programs, and we may not identify any additional drug compounds suitable for recommendation for clinical development. Moreover, any drug compounds we recommend for clinical development may not demonstrate, through preclinical studies, indications of safety and potential efficacy that would support advancement into clinical trials. Such findings would potentially impede our ability to maintain or expand our clinical development pipeline. Our ability to identify new drug compounds and advance them into clinical development also depends upon our ability to fund our research and development operations, and we cannot be certain that additional funding will be available on acceptable terms, or at all.

The pharmaceutical market and biotechnology industry are intensely competitive and involve a high degree of risk. If we are unable to compete effectively with existing drugs, new treatment methods and new technologies, we may be unable to commercialize successfully any drug candidates that we develop.

The pharmaceutical market and biotechnology industry are intensely competitive and rapidly changing. Many large pharmaceutical and biotechnology companies, academic institutions, governmental agencies and other public and private research organizations, both in the U.S. and worldwide, are pursuing the development of novel drugs for the same diseases that we are targeting or expect to target. Many of our competitors have, either alone or with strategic partners:

- much greater financial, research, technical and human resources than we have at every stage of the discovery, development, manufacture and commercialization of products and product candidates;
- more extensive experience in designing and conducting preclinical studies and clinical trials, obtaining regulatory approvals, and in manufacturing, marketing and selling pharmaceutical products and product candidates;
- product candidates that are based on previously tested or accepted technologies;
- products and product candidates that have been approved or are in late stages of development; and
- collaborative arrangements in our target markets with leading companies and research institutions.

We will face intense competition from drugs that have already been approved and accepted by the medical community for the treatment of the conditions for which we may develop drug candidates. We also expect to face competition from new drugs that enter the market. We believe there are a significant number of drugs currently under development that may become commercially available in the future, for the treatment of conditions for which we may try to develop drugs. These drugs may be more effective, safer, less expensive, introduced to market earlier, or marketed and sold more effectively or on a more cost-effective basis, than any product candidates we develop. It is possible that the potential advantages of PATrOL™-derived therapeutic candidates (including, among other potential advantages, the ability to systemically deliver drugs and get broad tissue distribution and penetration across the blood-brain barrier, minimal to no innate or adaptive immune responses after single dose or multiple-dose administration, preferential selectivity to mutant targets, and dose schedules to address the disease appropriately or that is viable in the marketplace) do not materialize.

Our competitors may develop or commercialize products with significant advantages over any product candidates we are able to develop and commercialize based on many different factors, including:

- the safety and effectiveness of our product candidates relative to alternative therapies, if any;
- the ease with which our product candidates can be administered and the extent to which patients accept relatively new routes of administration;
- the timing and scope of regulatory approvals for these product candidates;
- the availability and cost of manufacturing, marketing and sales capabilities;
- price;
- reimbursement coverage from governments and other third-party payors; and
- patent position and intellectual property protection.

Our commercial opportunity could be reduced or eliminated if our competitors develop and commercialize products that are viewed as safer, more effective, more convenient or less expensive than any products that we may develop. Our competitors may also obtain FDA or other regulatory approval for their competing products more rapidly than we may obtain approval for any of our product candidates, which could result in our competitors establishing a strong market position before we are able to enter the market. Further, we expect that we will also compete with others when recruiting clinical trial sites and subjects for our clinical trials and when recruiting and retaining qualified scientific and management personnel.

While there are currently no approved treatments available to slow the progression of Huntington's Disease or Myotonic Dystrophy Type 1, publicly available information shows that a number of companies are pursuing product candidates seeking to address the root cause of these indications. These include an investigational drug in Phase III clinical development for HD, and several ongoing clinical and preclinical programs targeting the underlying disease and symptoms in HD and DM1. The success of any of these competitors could reduce or eliminate our commercial opportunity.

Any collaboration arrangement that we may enter into in the future may not be successful, which could adversely affect our ability to develop and commercialize our current and potential future product candidates.

We may seek collaboration arrangements with pharmaceutical companies for the development or commercialization of our current and potential future product candidates. To the extent that we decide to enter into collaboration agreements, we will face significant competition in seeking appropriate collaborators. Moreover, collaboration arrangements are complex and time consuming to negotiate, execute and implement. We may not be successful in our efforts to establish and implement collaborations or other alternative arrangements should we choose to enter into such arrangements, and the terms of the arrangements may not be favorable to us. If and when we collaborate with a third party for development and commercialization of a product candidate, we can expect to relinquish some or all of the control over the future success of that product candidate to the third party. The success of our collaboration arrangements will depend heavily on the efforts and activities of our collaborators. Collaborators generally have significant discretion in determining the efforts and resources that they will apply to these collaborations. As such, our inability to control our collaborators, and the potentially adverse results of our collaborators, may materially and adversely affect our product candidates and, more generally, our PATrOL™ platform, and we may not be able to conduct our program in the manner or on the time schedule it currently contemplates, which could negatively impact our business.

If our potential future collaborations do not result in the successful discovery, development and commercialization of products or if one of our collaborators terminates our 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, our development of our platform technology and product candidates could be delayed and we may need additional resources to develop product candidates and our technology.

Finally, disagreements between parties to a collaboration arrangement can lead to delays in developing or commercializing the applicable product candidate and can be difficult to resolve in a mutually beneficial manner. In some cases, collaborations with biopharmaceutical companies and other third parties are terminated or allowed to expire by the other party. Any such termination or expiration could adversely affect our business, financial condition and results of operations.

We, or any future collaborators, may not be able to obtain orphan drug designation or orphan drug exclusivity for our product candidates.

Regulatory authorities in some jurisdictions, including the U.S. and Europe, 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 annually in the U.S. In the U.S. and Europe, obtaining orphan drug approval may allow us to obtain financial incentives, such as an extended period of exclusivity during which only we are allowed to market the orphan drug for the orphan indications that we are developing. While we may seek orphan drug designation from the FDA for any of our product candidates, we, or any future collaborators, may not be granted orphan drug designations for our product candidates in the U.S. or in other jurisdictions.

Even if we or any future collaborators obtain orphan drug designation for a product candidate, we or such collaborators may not be able to obtain orphan drug exclusivity for that product candidate. Generally, a product with orphan drug designation only becomes entitled to orphan drug exclusivity if it receives the first marketing approval for the indication for which it has such designation, in

which case the FDA or the EMA will be precluded from approving another marketing application for the same drug for that indication for the applicable exclusivity period. The applicable exclusivity period is seven years in the U.S. and ten years in Europe. 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.

Even if we or any future collaborators obtain orphan drug exclusivity for a product, that exclusivity may not effectively protect the product from competition because FDA has taken the position that, under certain circumstances, another drug with the same active chemical and pharmacological characteristics, or moiety, can be approved for the same condition. Specifically, the FDA's regulations provide that it can approve another drug with the same active moiety 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.

Our operations have been adversely affected by the coronavirus outbreak, and we face risks that could impact our business.

A novel strain of coronavirus, COVID-19, originated in Wuhan, China, in December 2019. The virus has spread globally and includes a significant number of cases in the U.S., Europe and Asia. On March 13, 2020, the United States declared a national emergency. As of September 30, 2022, the national state of emergency is still in effect, however states have reopened their economies at various levels, COVID-19 vaccinations are being distributed in mass quantities. However, with new variants of COVID-19 being detected across multiple countries, it still remains unclear how the current trends of states reopening their economies will be impacted and what the overall impact of COVID-19 will be on our business.

We have relationships with contract research organizations to conduct certain pre-clinical programs and testing and other services in Europe and certain of those business operations have been impacted by the COVID-19 pandemic and are further subject to potential business interruptions arising from new protective measures that may be taken by the governmental or other agencies or governing bodies. We also conduct limited operations within Asia through third-party contract manufacturing organizations whose operations have been and may continue to be negatively affected by the coronavirus outbreak. Shipping and importation of contracted manufactured goods could also be negatively affected. In addition, certain of our collaborative relationships with academic research institutions in the United States have been and may continue to be materially and adversely impacted by protective measures taken by those institutions or federal and state agencies and governing bodies to restrict access to, or suspend operations at, such facilities. Such protective measures, including quarantines, travel restrictions and business shutdowns, have impacted and may continue to negatively affect our core operations. We have taken precautionary measures aligned with Centers for Disease Control and Prevention, state and local guidelines that are intended to help minimize the risk of the virus to our employees, including the provision of personal protective equipment, suspension of non-essential travel worldwide for our employees, and we discourage employee attendance at other gatherings. Several of our employees work remotely. Business disruptions elsewhere in the world could also negatively affect the sources and availability of components and materials that are essential to the operation of our business.

Extended periods of interruption to our U.S. operations or those of our contract research and manufacturing organizations due to the coronavirus outbreak could adversely impact the growth of our business and could cause us to cease or delay operations. For example, one of our external contract research providers, was forced to shut down its vivarium where the *in vivo* testing for the NT0100 program was ongoing, and this disruption in operations contributed to a delay and may also incur additional future delays in that program. In such instances, we may need to locate an appropriate replacement third-party facility and establish a contractual relationship in connection with such facilities, which may not be readily available or on acceptable terms that would cause additional delay and increased expense, including as a result of additional required FDA approvals, and may have a material adverse effect on our business.

The extent to which the coronavirus impacts our business and results of operations will depend on future developments, which are highly uncertain and cannot be predicted. This includes new information that may emerge concerning the severity of the coronavirus, the spread and proliferation of the coronavirus around the world, and the actions taken to contain the coronavirus or treat its impact, among others.

We are subject to a multitude of manufacturing risks, any of which could substantially increase our costs and limit supply of our product candidates.

The process of manufacturing our product candidates is complex, highly regulated, and subject to several risks. For example, the process of manufacturing our product candidates is extremely susceptible to product loss due to contamination, equipment failure or improper installation or operation of equipment, low yielding processes, or vendor or operator error. Even minor deviations from normal manufacturing processes for any of our product candidates could result in reduced production yields, product defects, and other supply disruptions. If microbial, viral, or other contaminations are discovered in our product candidates or in the manufacturing facilities in which our product candidates are made, such manufacturing facilities may need to be closed for an extended period of time to investigate and remedy the contamination. In addition, the manufacturing facilities in which our product candidates are made could be adversely affected by equipment failures, labor shortages, natural disasters, public health crises, pandemics and epidemics, such as a novel strain of coronavirus (COVID-19), power failures and numerous other factors. For instance, our therapeutic molecules are complex and comprised of both peptides and nucleic acids, and it may be difficult or impossible to find GLP and cGMP grade manufacturers, manufacturing may be cost prohibitive, we or our third-party manufacturers may not be able to manufacture product candidates in a timely manner or within specification, or manufacturing may not be available to fulfill regulatory requirements. In addition, we or our third-party manufacturers may not be able to manufacture our product candidates at the necessary scale to meet our development and commercialization requirements.

In addition, any adverse developments affecting manufacturing operations for our product candidates may result in shipment delays, inventory shortages, lot failures, withdrawals or recalls, or other interruptions in the supply of our product candidates. We also may need to take inventory write-offs and incur other charges and expenses for product candidates that fail to meet specifications, undertake costly remediation efforts or seek costlier manufacturing alternatives.

We rely, and will continue to rely, predominantly, on third parties to manufacture our preclinical and clinical drug supplies and our business, financial condition and results of operations could be harmed if those third parties fail to provide us with sufficient quantities of drug product, or fail to do so at acceptable quality levels, prices, or timelines.

We have the capability internally to manufacture small quantities of our drugs for preclinical studies. However, we do not currently have, nor do we plan to acquire, the infrastructure or capability internally to manufacture our clinical drug supplies for use in our clinical trials, and we lack the resources and the capability to manufacture any of our product candidates on a clinical or commercial scale. We rely on our manufacturers to purchase from third-party suppliers the materials necessary to produce our product candidates for our clinical trials. There are a limited number of suppliers for raw materials that we use to manufacture our product candidates, and there may be a need to identify alternate suppliers to prevent a possible disruption of the manufacture of the materials necessary to produce our product candidates for our clinical trials, and, if approved, ultimately for commercial sale. We do not have any control over the process or timing of the acquisition of these raw materials by our manufacturers. Any significant delay or discontinuity in the supply of a product candidate, or the raw material components thereof, for an ongoing clinical trial due to the need to replace a third-party manufacturer could considerably delay completion of our clinical trials, product testing and potential regulatory approval of our product candidates, which could harm our business, financial condition and results of operations.

If we are unable to develop our own commercial organization or enter into agreements with third parties to sell and market our product candidates, we may be unable to generate significant revenues.

We do not have a sales and marketing organization, and we have no experience as a company in the sales, marketing and distribution of pharmaceutical products. If any of our product candidates are approved for commercialization, we may be required to develop our own sales, marketing and distribution capabilities, or make arrangements with a third party to perform sales and marketing services. Developing a sales force for any resulting product or any product resulting from any of our other product candidates is expensive and time consuming and could delay any product launch. We may be unable to establish and manage an effective sales force in a timely or cost-effective manner, if at all, and any sales force we do establish may not be capable of generating sufficient demand for our product candidates. To the extent that we enter into arrangements with collaborators or other third parties to perform sales and marketing services, our product revenues are likely to be lower than if we marketed and sold our product candidates independently. If we are unable to establish adequate sales and marketing capabilities, independently or with others, we may not be able to generate significant revenues and may not become profitable.

The commercial success of our product candidates depends upon their market acceptance among physicians, patients, healthcare payors and the medical community.

Even if our product candidates obtain regulatory approval, our products, if any, may not gain market acceptance among physicians, patients, healthcare payors and the medical community. The degree of market acceptance of any of our approved product candidates will depend on a number of factors, including:

- the effectiveness of our approved product candidates as compared to currently available products;
- patient willingness to adopt our approved product candidates in place of current therapies;
- our ability to provide acceptable evidence of safety and efficacy;
- relative convenience and ease of administration;
- the prevalence and severity of any adverse side effects;
- restrictions on use in combination with other products;
- availability of alternative treatments;
- pricing and cost-effectiveness assuming either competitive or potential premium pricing requirements, based on the profile of our product candidates and target markets;
- effectiveness of our or our partners' sales and marketing strategy;
- our ability to obtain sufficient third-party coverage or reimbursement; and
- potential product liability claims.

In addition, the potential market opportunity for our product candidates is difficult to precisely estimate. Our estimates of the potential market opportunity for our product candidates include several key assumptions based on our industry knowledge, industry publications, third-party research reports and other surveys. Independent sources have not verified all of our assumptions. If any of these assumptions proves to be inaccurate, then the actual market for our product candidates could be smaller than our estimates of our potential market opportunity. If the actual market for our product candidates is smaller than we expect, our product revenue may be limited, it may be harder than expected to raise funds and it may be more difficult for us to achieve or maintain profitability. If we fail to achieve market acceptance of our product candidates in the U.S. and abroad, our revenue will be limited, and it will be more difficult to achieve profitability.

If we fail to obtain and sustain an adequate level of reimbursement for our potential products by third-party payors, potential future sales would be materially adversely affected.

There will be no viable commercial market for our product candidates, if approved, without reimbursement from third-party payors. Reimbursement policies may be affected by future healthcare reform measures. We cannot be certain that reimbursement will be available for our current product candidates or any other product candidate we may develop. Additionally, even if there is a viable commercial market, if the level of reimbursement is below our expectations, our anticipated revenue and gross margins will be adversely affected.

Third-party payors, such as government or private healthcare insurers, carefully review and increasingly question and challenge the coverage of and the prices charged for drugs. Reimbursement rates from private health insurance companies vary depending on the company, the insurance plan and other factors. Reimbursement rates may be based on reimbursement levels already set for lower cost drugs and may be incorporated into existing payments for other services. There is a current trend in the U.S. healthcare industry toward cost containment.

Large public and private payors, managed care organizations, group purchasing organizations and similar organizations are exerting increasing influence on decisions regarding the use of, and reimbursement levels for, particular treatments. Such third-party payors, including Medicare, may question the coverage of, and challenge the prices charged for, medical products and services, and many third-party payors limit coverage of or reimbursement for newly approved healthcare products. In particular, third-party payors may limit the covered indications. Cost-control initiatives could decrease the price we might establish for products, which could result in product revenues being lower than anticipated. We believe our drugs will be priced significantly higher than existing generic drugs and consistent with current branded drugs. If we are unable to show a significant benefit relative to existing generic drugs, Medicare, Medicaid and private payors may not be willing to provide reimbursement for our drugs, which would significantly reduce the likelihood of our products gaining market acceptance.

We expect that private insurers will consider the efficacy, cost-effectiveness, safety and tolerability of our product candidates in determining whether to approve reimbursement for such product candidates and at what level. Obtaining these approvals can be a time consuming and expensive process. Our business, financial condition and results of operations would be materially adversely affected if we do not receive approval for reimbursement of our product candidates from private insurers on a timely or satisfactory basis. Limitations on coverage could also be imposed at the local Medicare carrier level or by fiscal intermediaries. Medicare Part B, which covers medical insurance to Medicare patients as discussed below, does not require participating insurance plans to cover all drugs that have been approved by the FDA. Our business, financial condition and results of operations could be materially adversely affected if Part B medical insurance were to limit access to, or deny or limit reimbursement of, our product candidates.

Reimbursement systems in international markets vary significantly by country and by region, and reimbursement approvals must be obtained on a country-by-country basis. In many countries, the product candidate cannot be commercially launched until reimbursement is approved. In some foreign markets, prescription pharmaceutical pricing remains subject to continuing governmental control even after initial approval is granted. The negotiation process in some countries can exceed 12 months. To obtain reimbursement or pricing approval in some countries, we may be required to conduct a clinical trial that compares the cost-effectiveness of our product candidates to other available therapies.

If the prices for our product candidates are reduced or if governmental and other third-party payors do not provide adequate coverage and reimbursement of our drugs, our future revenue, cash flows and prospects for profitability will suffer.

We are exposed to product liability, nonclinical and clinical liability risks which could place a substantial financial burden upon us, should lawsuits be filed against us.

Our business exposes us to potential product liability and other liability risks that are inherent in the testing, manufacturing and marketing of pharmaceutical formulations and products. In addition, the use in our anticipated clinical trials of pharmaceutical products and the subsequent sale of product candidates by us, if approved, or our potential collaborators may cause us to bear a portion of or all product liability risks. A successful liability claim or series of claims brought against us could have a material adverse effect on our business, financial condition and results of operations.

Because we do not currently have any clinical trials ongoing, we do not currently carry product liability insurance. We anticipate obtaining such insurance upon initiation of our clinical development activities; however, we may be unable to obtain product liability insurance on commercially reasonable terms or in adequate amounts. On occasion, large judgments have been awarded in class action lawsuits based on drugs that had unanticipated adverse effects. A successful product liability claim or series of claims brought against us could adversely affect our results of operations and business if judgments therewith exceed our insurance coverage.

If we fail to retain current members of our management, or to attract and keep additional key personnel, we may be unable to successfully develop or commercialize our product candidates.

Our success depends on our continued ability to attract, retain and motivate highly qualified management and scientific personnel. As of September 30, 2022, we had 37 full-time employees. In October 2022, in connection with the Company's reprioritization of clinical and research initiatives and restructuring of operations, we reduced our workforce by approximately 60%, resulting in 15 full-time employees. This reduction in workforce was designed to reduce costs and reallocate resources while maintaining the personnel needed to support the Company's key programs, including the PATrOL™ platform, and refocused pipeline. Further, since September 30, 2022, the Company's President and Chief Operating Officer, as well as its Head of Research and Development and Chief Medical Officer, have resigned. The Company has entered into retention arrangements with certain employees to remain at the Company. We

will rely primarily on outsourcing research, development and clinical trial activities, and manufacturing operations, as well as other functions critical to our business. We believe this approach enhances our ability to focus on our core product opportunities, allocate resources efficiently to different projects and allocate internal resources more effectively. Competition for qualified personnel is intense. We may not be successful in attracting qualified personnel to fulfill our current or future needs. In the event we are unable to fill critical open employment positions, we may need to delay our operational activities and goals, including the development of our product candidates, and may have difficulty in meeting our obligations as a public company. We do not maintain "key person" insurance on any of our employees.

In addition, competitors and others are likely in the future to attempt to recruit our employees. The loss of the services of any of our key personnel, the inability to attract or retain highly qualified personnel in the future or delays in hiring such personnel, particularly senior management and other technical personnel, could materially and adversely affect our business, financial condition and results of operations. In addition, the replacement of key personnel likely would involve significant time and costs and may significantly delay or prevent the achievement of our business objectives.

From time to time, our management seeks the advice and guidance of certain scientific advisors and consultants regarding clinical and regulatory development programs and other customary matters. These scientific advisors and consultants are not our employees and may have commitments to, or consulting or advisory contracts with, other entities that may limit their availability to us. In addition, our scientific advisors may have arrangements with other companies to assist those companies in developing products or technologies that may compete with us.

We may need to increase the size of our organization and may not successfully manage our growth.

We are a preclinical-stage pharmaceutical company with a small number of employees, and our management systems currently in place are not likely to be adequate to support our future growth plans. Our ability to grow and to manage our growth effectively will require us to hire, train, retain, manage and motivate additional employees and to implement and improve our operational, financial and management systems. These demands also may require the hiring of additional senior management personnel or the development of additional expertise by our senior management personnel. Hiring a significant number of additional employees, particularly those at the management level, would increase our expenses significantly. Moreover, if we fail to expand and enhance our operational, financial and management systems in conjunction with our potential future growth, such failure could have a material adverse effect on our business, financial condition and results of operations.

Because our Chief Executive Officer is involved with several unaffiliated privately held companies, he may experience conflicts of interest and competing demands for his time and attention.

Dietrich A. Stephan, Ph.D., our Chief Executive Officer, is a member of the governing bodies of several unaffiliated privately held companies, as well as a general partner of Cyto Ventures. Although Dr. Stephan expects to devote substantially all of his time to us, he expects to continue in each of these positions for the foreseeable future. Conflicts of interest could arise with respect to business opportunities that could be advantageous to third party organizations affiliated with Dr. Stephan, on the one hand, and us, on the other hand.

We rely significantly on information technology and any failure, inadequacy, interruption or security lapse of that technology, including any cybersecurity incidents, could harm our ability to operate our business effectively.

Despite the implementation of security measures, our internal computer systems and those of third parties with which we contract are vulnerable to damage from cyber-attacks, computer viruses, unauthorized access, natural disasters, terrorism, war and telecommunication and electrical failures. System failures, accidents or security breaches could cause interruptions in our operations and could result in a material disruption of our drug development and preclinical and clinical activities and business operations, in addition to possibly requiring substantial expenditures of resources to remedy. To the extent that any disruption or security breach was to result in a loss of or damage to our data or applications, or inappropriate disclosure of confidential or proprietary information, we could incur material legal claims and liability, damage to our reputation, suffer loss or harm to our intellectual property rights and the further research, development and commercial efforts of our products and product candidates could be delayed. The loss of drug development or clinical trial data could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data. To the extent that any disruption or security breach were to result in a loss of, or damage to, our data or applications, or inappropriate disclosure of confidential or proprietary information, we could incur liability and our development

programs and the development of our product candidates could be delayed. In addition, if we are held liable for a claim against which we are not insured or for damages exceeding the limits of our insurance coverage, whether arising out of cybersecurity matters, or from some other matter, that claim could have a material adverse effect on our results of operations.

Our employees, consultants, third-party vendors and collaborators may engage in misconduct or other improper activities, including noncompliance with regulatory standards and requirements.

We are exposed to the risk of employee, consultant, third-party vendor or collaborator fraud or other misconduct. Misconduct by our employees, consultants, third-party vendors or collaborators could include, among other things, intentional failures to comply with FDA regulations, provide accurate information to the FDA, comply with manufacturing standards, comply with federal and state healthcare fraud and abuse laws and regulations, report financial information or data accurately or disclose unauthorized activities to us. In particular, sales, marketing and business arrangements in the healthcare industry are subject to extensive laws and regulations intended to prevent fraud, 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 commissions, customer incentive programs and other business arrangements. Employee, consultant, vendor or collaborator misconduct also could involve the improper use of information obtained in the course of preclinical or clinical trials, which could result in regulatory sanctions and serious harm to our reputation. It is not always possible to identify and deter such misconduct, and the precautions we take to detect and prevent this activity may not be effective in controlling unknown or unmanaged risks or losses or in protecting us from governmental investigations or other actions or lawsuits stemming from a failure to be in compliance with such laws or regulations. If any such actions are instituted against us, and we are not successful in defending ourselves or asserting our rights, those actions could have a material adverse effect on our business, financial condition and results of operations, and result in the imposition of significant fines or other sanctions against us.

Business disruptions such as natural disasters could seriously harm our future revenues and financial condition and increase our costs and expenses.

We and our suppliers may experience a disruption in our and their business as a result of natural disasters. A significant natural disaster, such as an earthquake, hurricane, flood or fire, could severely damage or destroy our headquarters or facilities or the facilities of our manufacturers or suppliers, which could have a material and adverse effect on our business, financial condition and results of operations. In addition, terrorist acts or acts of war targeted at the U.S., and specifically the Pittsburgh, PA, Boston/Cambridge, MA, and greater New York, NY regions, could cause damage or disruption to us, our employees, facilities, partners and suppliers, which could have a material adverse effect on our business, financial condition and results of operations.

The impact of the Russian invasion of Ukraine on the global economy, energy supplies and raw materials is uncertain, but may prove to negatively impact our business and operations.

The short and long-term implications of Russia's invasion of Ukraine are difficult to predict at this time. We continue to monitor any adverse impact that the outbreak of war in Ukraine and the subsequent institution of sanctions against Russia by the United States and several European and Asian countries may have on the global economy in general, on our business and operations and on the businesses and operations of our suppliers and customers. For example, a prolonged conflict may result in challenges associated with timely receipt of customer payments and banking transactions, increased inflation, escalating energy prices and constrained availability, and thus increasing costs, of raw materials. We will continue to monitor this fluid situation and develop contingency plans as necessary to address any disruptions to our business operations as they develop. To the extent the war in Ukraine may adversely affect our business as discussed herein, it may also have the effect of heightening many of the other risks described herein. Such risks include, but are not limited to, adverse effects on macroeconomic conditions, including inflation; disruptions to our global technology infrastructure, including through cyberattack, ransom attack, or cyber-intrusion; adverse changes in international trade policies and relations; our ability to maintain or increase our product prices; disruptions in global supply chains; our exposure to foreign currency fluctuations; and constraints, volatility, or disruption in the capital markets, any of which could negatively affect our business and financial condition.

We may engage in strategic transactions that could impact our liquidity, increase our expenses and present significant distractions to our management.

From time to time, we may consider strategic transactions, such as spin-offs, strategic partnerships, joint ventures, restructurings, divestitures, business combinations and investments. Additional potential transactions that we may consider include a variety of

different business arrangements, including acquisitions of companies, asset purchases and out-licensing or in-licensing of products, product candidates or technologies. Any such transaction may require us to incur non-recurring or other charges, may increase our near- and long-term expenditures and may pose significant integration challenges or disrupt our management or business, which could adversely affect our business, financial condition and results of operations. For example, these transactions may entail numerous operational and financial risks, including:

- exposure to unknown liabilities;
- disruption of our business and diversion of our management's time and attention in order to develop acquired products, product candidates or technologies;
- incurrence of substantial debt or dilutive issuances of equity securities to pay for any of these transactions;
- higher-than-expected transaction and integration costs;
- write-downs of assets or goodwill or impairment charges;
- increased amortization expenses;
- difficulty and cost in combining the operations and personnel of any acquired businesses or product lines with our operations and personnel;
- impairment of relationships with key suppliers or customers of any acquired businesses or product lines due to changes in management and ownership; and
- inability to retain key employees of any acquired businesses.

For example, in late April 2021, we completed our acquisition of infrastructure, materials and intellectual property for peptide-nucleic acid scaffolds from Vera Therapeutics, Inc. ("Vera"). We are currently in the process of integrating the acquired assets in our operations and may experience delays and challenges as part of the integration process. We also may not realize the benefits we expect to achieve through the acquisition of Vera's assets.

In addition, although there can be no assurance that we will undertake or successfully complete any other transactions of the nature described above, any transactions that we do complete may be subject to the foregoing or other risks, and could have a material adverse effect on our business, financial condition and results of operations.

The estimates and judgments we make, or the assumptions on which we rely, in preparing our financial statements could prove inaccurate.

Our financial statements have been prepared in accordance with United States generally accepted accounting principles. The preparation of these financial statements requires us to make estimates and judgments that affect the reported amounts of our assets, liabilities, revenues and expenses, the amounts of charges accrued by us and related disclosure of contingent assets and liabilities. We base our estimates on historical experience and on various other assumptions that we believe to be reasonable under the circumstances. We cannot assure, however, that our estimates, or the assumptions underlying them, will not change over time or otherwise prove inaccurate. For example, our estimates as they relate to anticipated timelines and milestones for our preclinical development or clinical trials may prove to be inaccurate. If this is the case, we may be required to restate our financial statements, which could, in turn, subject us to securities class action litigation or regulatory investigation or action. Defending against such potential litigation or regulatory action relating to a restatement of our financial statements would be expensive and would require significant attention and resources of our management. Moreover, our insurance to cover our obligations with respect to the ultimate resolution of any such litigation or regulatory action may be inadequate. As a result of these factors, any such potential litigation or regulatory action could have a material adverse effect on our financial results or harm our business.

Risks Related to Our Intellectual Property

Our success depends in part on our ability to protect our intellectual property. It is difficult and costly to protect our proprietary rights and technology, and we may not be able to protect our intellectual property rights throughout the world.

Our commercial success will depend in large part on obtaining and maintaining patent, trademark and trade secret protection of our proprietary technologies and our product candidates, their respective components, formulations, methods used to manufacture them and methods of treatment, as well as successfully defending these patents against third-party challenges. Our ability to stop unauthorized third parties from making, using, selling, offering to sell or importing our product candidates is dependent upon the extent to which we have rights under valid and enforceable patents or trade secrets that cover these activities.

The patenting process is expensive and time-consuming, and we may not be able to file and prosecute all necessary or desirable patent applications at a reasonable cost or in a timely manner. In addition, we may not pursue or obtain patent protection in all relevant markets. It is also possible that we will fail to identify patentable aspects of our research and development output before it is too late to obtain patent protection. Our pending and future patent applications may not result in issued patents that protect our technology or products, in whole or in part. In addition, our existing patents and any future patents we obtain may not be sufficiently broad to prevent others from using our technology or from developing competing products and technologies.

In the future we may, and presently do, in-license intellectual property from licensors. We may rely on these licensors to file and prosecute patent applications and maintain patents and otherwise protect the intellectual property we license from them. We may have limited control over these activities or any other intellectual property that may be in-licensed. For example, we cannot be certain that such activities by licensors have been or will be conducted in compliance with applicable laws and regulations or will result in valid and enforceable patents and other intellectual property rights. We may have limited control over the manner in which our licensors initiate an infringement proceeding against a third-party infringer of the intellectual property rights, or defend certain of the intellectual property that is licensed to us. It is possible that the licensors' infringement proceeding or defense activities may be less vigorous than had we conducted them ourselves.

The growth of our business may depend in part on our ability to acquire or in-license additional proprietary rights. For example, our programs may involve additional product candidates that may require the use of additional proprietary rights held by third parties. Our product candidates may also require specific formulations to work effectively and efficiently. These formulations may be covered by intellectual property rights held by others. We may develop products containing our compounds and pre-existing pharmaceutical compounds. These pharmaceutical compounds may be covered by intellectual property rights held by others. We may be required by the FDA or comparable foreign regulatory authorities to provide a companion diagnostic test or tests with our product candidates. These diagnostic test or tests may be covered by intellectual property rights held by others. We may be unable to acquire or in-license any relevant third-party intellectual property rights that we identify as necessary or important to our business operations. We may fail to obtain any of these licenses at a reasonable cost or on reasonable terms, if at all, which would harm our business. We may need to cease use of the compositions or methods covered by such third-party intellectual property rights, and may need to seek to develop alternative approaches that do not infringe on such intellectual property rights which may entail additional costs and development delays, even if we were able to develop such alternatives, which may not be feasible. Even if we are able to obtain a license under such intellectual property rights, any such license may be non-exclusive, which may allow our competitors access to the same technologies licensed to us. If we are unable to successfully obtain rights to required third-party intellectual property or to maintain the existing intellectual property rights we have, we may have to abandon development of such program and our business and financial condition could suffer.

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

Because several of our programs currently require the use of proprietary rights held by third parties, the growth of our business will likely depend in part on our ability to maintain and exploit these proprietary rights. In addition, we may need to acquire or in-license additional intellectual property in the future. We may be unable to acquire or in-license any compositions, methods of use, processes or other intellectual property rights from third parties that we identify as necessary for our product candidates. We face competition with regard to acquiring and in-licensing third-party intellectual property rights, including from a number of more established companies. These established companies may have a competitive advantage over us due to their size, cash 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 intellectual property rights to us. We also may be unable to acquire or in-license third-party intellectual property rights on terms that would allow it to make an appropriate return on our investment, and we may not be able to market products or perform research and development or other activities covered by these patents.

We may enter into collaboration agreements with U.S. and foreign academic institutions to accelerate development of our current or future preclinical product candidates. Typically, these agreements include an option for the company to negotiate a license to the institution's intellectual property rights resulting from the collaboration. Even with such an option, we may be unable to negotiate a license within the specified timeframe or under terms that are acceptable to us. If we are unable to license rights from a collaborating institution, the institution may offer the intellectual property rights to other parties, potentially blocking our ability to pursue our desired program.

If we are unable to successfully obtain required third-party intellectual property rights or maintain our existing intellectual property rights, we may need to abandon development of the related program and our business, financial condition and results of operations could be materially and adversely affected.

If we are unable to obtain and maintain sufficient patent and other intellectual property protection for our product candidates and technology, our competitors could develop and commercialize products and technology similar or identical to ours, and we may not be able to compete effectively in our market or successfully commercialize any product candidates we may develop.

We rely upon a combination of patents, trade secret protection and confidentiality agreements to protect the intellectual property related to our technologies. We will only be able to protect our product candidates, proprietary technologies and their uses from unauthorized use by third parties to the extent that valid and enforceable patents or trade secrets cover them. Any disclosure to or misappropriation by third parties of our confidential proprietary information could enable competitors to quickly duplicate or surpass our technological achievements, thus eroding our competitive position in our market.

Composition-of-matter patents on the active pharmaceutical ingredient are generally considered to be the strongest form of intellectual property protection for pharmaceutical products, as such patents provide protection without regard to any method of use. Method-of-use patents protect the use of a product for the specified method. This type of patent does not prevent a competitor from making and marketing a product that is identical to our products for an indication that is outside the scope of the patented method. Moreover, even if competitors do not actively promote their product for our targeted indications, physicians may prescribe these products "off-label." Although off-label prescriptions may infringe or contribute to the infringement of method-of use patents, the practice is common and such infringement is difficult to prevent or prosecute.

The strength of patents in the biotechnology and pharmaceutical field involves complex legal and scientific questions and can be uncertain. The patent applications that we own may fail to result in issued patents in the United States or in other foreign countries. Even if the patents do successfully issue, third parties may challenge the validity, enforceability, inventorship, or scope thereof, which may result in such patents being narrowed, invalidated or held unenforceable. Furthermore, even if they are unchallenged, our patents and patent applications may not adequately protect our intellectual property or prevent others from designing around our claims. If the breadth or strength of protection provided by the patents and patent applications we hold with respect to our product candidates is threatened, it could dissuade companies from collaborating with us to develop, and threaten our ability to commercialize, our product candidates. Further, if we encounter delays in our clinical trials, the period of time during which we could market our product candidates under patent protection would be reduced. Since patent applications in the United States and most other countries are confidential for a period of time after filing, we cannot be certain that we were the first to file any patent application related to our product candidates. Furthermore, for applications in which all claims are entitled to a priority date before March 16, 2013, an interference proceeding can be provoked by a third-party or instituted by the United States Patent and Trademark Office or the USPTO, to determine who was the first to invent any of the subject matter covered by the patent claims of our applications.

In addition to the protection afforded by patents, we seek to rely on trade secret protection and confidentiality agreements to protect proprietary know-how that is not patentable, processes for which patents are difficult to enforce and any other elements of our drug discovery and development processes that involve proprietary know-how, information or technology that is not covered by patents. Although we require all of our employees to assign their inventions to us, and require all of our employees, consultants, advisors and any third parties who have access to our proprietary know-how, information or technology to enter into confidentiality agreements, we cannot be certain that our trade secrets and other confidential proprietary information will not be disclosed or that competitors will not otherwise gain access to our trade secrets or independently develop substantially equivalent information and techniques. Furthermore,

the laws of some foreign countries do not protect proprietary rights to the same extent or in the same manner as the laws of the United States. As a result, we may encounter significant problems in protecting and defending our intellectual property both in the United States and abroad. If we are unable to prevent unauthorized material disclosure of our intellectual property to third parties, we will not be able to establish or maintain a competitive advantage in our market, which could materially adversely affect our business, operating results and financial condition.

If we fail to comply with our obligations in the agreements under which we in-license intellectual property and other rights from third parties or otherwise experiences disruptions to our business relationships with our licensors, we could lose intellectual property rights that are important to our business.

We may enter into license agreements with universities. A license agreement may impose various royalties, sublicensing fees and other obligations on us. If we fail to comply with our obligations under these agreements, or if we file for bankruptcy, we may be required to make certain payments to the licensor, we may lose the exclusivity of our license, or the licensor may have the right to terminate the license, in which event we would not be able to develop or market products covered by the license. Additionally, the royalties and other payments associated with these licenses could materially and adversely affect our business, financial condition and results of operations.

Pursuant to the terms of our license agreement with CMU (as amended, the "CMU License Agreement"), CMU has the right to terminate the CMU License Agreement with respect to the program licensed under certain circumstances, including, but not limited to: (i) if we do not pay amounts when due and within the applicable cure periods or (ii) if we file or have filed against us a petition in bankruptcy or make an assignment for the benefit of creditors. In the event the CMU License Agreement is terminated by CMU, all licenses (or, in the determination of CMU, the exclusivity of such licenses) granted to us by CMU will terminate immediately.

In some cases, patent prosecution of our licensed technology may be controlled solely by the licensor. If our licensor fails to obtain and maintain patent or other protection for the proprietary intellectual property we in-license, then we could lose our rights to the intellectual property or our exclusivity with respect to those rights, and our competitors could market competing products using the intellectual property. In certain cases, we may control the prosecution of patents resulting from licensed technology. In the event we breach any of our obligations related to such prosecution, we may incur significant liability to our licensing partners. Licensing of intellectual property is of critical importance to our business and involves complex legal, business and scientific issues. Disputes may arise regarding intellectual property subject to a licensing agreement, including, but not limited to:

- the scope of rights granted under the license agreement and other interpretation-related issues;
- the extent to which our technology and processes infringe on intellectual property of the licensor that is not subject to the licensing agreement;
- the sublicensing of patent and other rights;
- our diligence obligations under the license agreement 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 collaborators; and
- the priority of invention of patented technology.

If disputes over intellectual property and other rights that we have in-licensed prevent or impair our ability to maintain our current licensing arrangements on acceptable terms, we may be unable to successfully develop and commercialize the affected product candidates. If we fail to comply with any such obligations to our licensor, such licensor may terminate their licenses to us, in which case we would not be able to market products covered by these licenses. The loss of our licenses would have a material adverse effect on our business, financial condition and results of operations.

We may be required to pay royalties and sublicensing fees pursuant to university licensing agreements, which could adversely affect the overall profitability for us of any product candidates that we may seek to commercialize.

If our sales are covered by a licensing agreement with a university, then we may be required to pay royalties on future worldwide net product sales and a percentage of sublicensing fees that we may earn. Pursuant to the CMU License Agreement, we must achieve certain milestones to demonstrate certain developments of the licensed product, and, subject to certain conditions, we will pay to CMU royalties at a low single-digit percentage of aggregate annual net sales of licensed products and a percentage at the higher range of the bottom third of sublicensing fees. These royalty payments and sublicensing fees could adversely affect the overall profitability for us of any product candidates that we may seek to commercialize.

We may not be able to protect our proprietary or licensed technology in the marketplace.

We depend on our ability to protect our proprietary or licensed technology. We rely on trade secret, patent, copyright and trademark laws, and confidentiality, licensing and other agreements with employees and third parties, all of which offer only limited protection. Our success depends in large part on our ability and any licensor's or licensee's ability to obtain and maintain patent protection in the U.S. and other countries with respect to our proprietary or licensed technology and product candidates. We currently in-license some of our intellectual property rights to develop our product candidates and may in-license additional intellectual property rights in the future. We cannot be certain that patent enforcement activities by our current or future licensors have been or will be conducted in compliance with applicable laws and regulations or will result in valid and enforceable patents or other intellectual property rights. We also cannot be certain that our current or future licensors will allocate sufficient resources or prioritize their or our enforcement of such patents. Even if we are not a party to these legal actions, an adverse outcome could prevent us from continuing to license intellectual property that we may need to operate our business, which would have a material adverse effect on our business, financial condition and results of operations.

If we are compelled to spend significant time and money protecting or enforcing our licensed patents and future patents we may own, designing around patents held by others or licensing or acquiring, potentially for large fees, patents or other proprietary rights held by others, our business, financial condition and results of operations may be materially and adversely affected. If we are unable to effectively protect the intellectual property that we own or in-license, other companies may be able to offer the same or similar products for sale, which could materially adversely affect our business, financial condition and results of operations. The patents of others from whom we may license technology, and any future patents we may own, may be challenged, narrowed, invalidated or circumvented, which could limit our ability to stop competitors from marketing the same or similar products or limit the length of term of patent protection that we may have for our products.

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

Periodic maintenance fees, renewal fees, annuity fees and various other governmental fees on patents and/or patent applications will be due to be paid to the U.S. Patent and Trademark Office ("USPTO") and various governmental patent agencies outside of the U.S. in several stages over the lifetime of the applicable patent and/or patent application. The USPTO and various non-U.S. governmental patent agencies require compliance with a number of procedural, documentary, fee payment and other similar provisions during the patent application process. In many cases, an inadvertent lapse can be cured by payment of a late fee or by other means in accordance with the applicable rules. However, there are situations in which noncompliance can result in abandonment or lapse of the patent or patent application, resulting in partial or complete loss of patent rights in the relevant jurisdiction. If this occurs with respect to our in-licensed patents or patent applications we may file in the future, our competitors might be able to use our technologies, which would have a material adverse effect on our business, financial condition and results of operations.

The patent position of pharmaceutical and biotechnology companies generally is highly uncertain and involves complex legal and factual questions for which many legal principles remain unresolved. In recent years patent rights have been the subject of significant litigation. As a result, the issuance, scope, validity, enforceability and commercial value of our patent rights are highly uncertain. Our pending and future patent applications may not result in patents being issued in the United States or in other jurisdictions which protect our technology or products or which effectively prevent others from commercializing competitive technologies and products. Changes in either the patent laws or interpretation of the patent laws in the United States and other countries may diminish the value

of our patents or narrow the scope of our patent protection. In addition, the laws of foreign countries may not protect our rights to the same extent as the laws of the United States. Publications of discoveries in the scientific literature often lag behind the actual discoveries, and patent applications in the United States and other jurisdictions are typically not published until 18 months after filing, or in some cases not at all. Therefore, we cannot be certain that we were the first to make the inventions claimed in our patents or pending patent applications, or that we were the first to file for patent protection of such inventions. In addition, the U.S. Patent and Trademark Office, or USPTO, might require that the term of a patent issuing from a pending patent application be disclaimed and limited to the term of another patent that is commonly owned or names a common inventor. As a result, the issuance, scope, validity, enforceability and commercial value of our patent rights are highly uncertain.

Even if our patent applications issue as patents, they may not issue in a form that will provide us with any meaningful protection, prevent competitors from competing with us or otherwise provide us with any competitive advantage. Our competitors may be able to circumvent our owned or licensed patents by developing similar or alternative technologies or products in a non-infringing manner. The issuance of a patent is not conclusive as to its scope, validity or enforceability, and our owned and in-licensed patents may be challenged in the courts or patent offices in the United States and abroad. Such challenges may result in the patent claims of our owned or in-licensed patents being narrowed, invalidated or held unenforceable, in whole or in part. This result could limit our ability to stop or prevent us from stopping others from using or commercializing similar or identical technology and products, or limit the duration of the patent protection of our technology and products. Such challenges may result in loss of exclusivity or freedom to operate. Given the amount of time required for the development, testing and regulatory review of new product candidates, patents protecting such candidates might expire before or shortly after such candidates are commercialized. As a result, our patent portfolio may not provide us with sufficient rights to exclude others from commercializing products similar or identical to ours or otherwise provide us with a competitive advantage.

The degree of future protection for our proprietary rights is uncertain because legal means afford only limited protection and may not adequately protect our rights or permit us to gain or keep our competitive advantage. For example:

- others may be able to make or use compounds that are similar to the pharmaceutical compounds used in our product candidates but that are not covered by the claims of our patents;
- the active pharmaceutical ingredients in our current product candidates will eventually become commercially available in generic drug products, and no patent protection may be available with regard to formulation or method of use;
- we or our licensors, as the case may be, may fail to meet our obligations to the U.S. government in regards to any in-licensed patents and patent applications funded by U.S. government grants, leading to the loss of patent rights;
- we might not have been the first to file patent applications for these inventions;
- others may independently develop similar or alternative technologies or duplicate any of our technologies;
- it is possible that our pending patent applications will not result in issued patents;
- it is possible that there are prior public disclosures that could invalidate our patents, as the case may be, or parts of our or their patents;
- it is possible that others may circumvent our patents;
- it is possible that there are unpublished patent applications maintained in secrecy that may later issue with claims covering our products or technology similar to ours;
- the laws of foreign countries may not protect our proprietary rights to the same extent as the laws of the United States;
- the claims of our issued patents or patent applications, if and when issued, may not cover our product candidates;

- our patents may not provide us with any competitive advantages, may be narrowed in scope, or be held invalid or unenforceable as a result of legal challenges by third parties;
- the inventors of our patents or patent applications may become involved with competitors, develop products or processes which design around our patents, or become hostile to us or the patents or patent applications on which they are named as inventors;
- collaborators may develop adjacent or competing products to ours that are outside the scope of our patents;
- we may not develop additional proprietary technologies for which we can obtain patent protection;
- it is possible that product candidates or diagnostic tests we develop may be covered by third parties' patents or other exclusive rights; or
- the patents of others may have an adverse effect on our business.

Patents that we currently license and patents that we may own or license in the future do not necessarily ensure the protection of our licensed or owned intellectual property for a number of reasons, including, without limitation, the following:

- the patents may not be broad or strong enough to prevent competition from other products that are identical or similar to our product candidates;
- there can be no assurance that the term of a patent can be extended under the provisions of patent term extensions afforded by U.S. law or similar provisions in foreign countries, where available;
- the issued patents and patents that we may obtain or license in the future may not prevent generic entry into the market for our product candidates;
- we, or third parties from whom we in-license or may license patents, may be required to disclaim part of the term of one or more patents;
- there may be prior art of which we are not aware that may affect the validity or enforceability of a patent claim;
- there may be prior art of which we are aware, which we do not believe affects the validity or enforceability of a patent claim, but which, nonetheless, ultimately may be found to affect the validity or enforceability of a patent claim;
- there may be other patents issued to others that will affect our freedom to operate;
- if the patents are challenged, a court could determine that they are invalid or unenforceable;
- there might be a significant change in the law that governs patentability, validity and infringement of our licensed patents or any future patents we may own that adversely affects the scope of our patent rights;
- a court could determine that a competitor's technology or product does not infringe our licensed patents or any future patents we may own; and
- the patents could irretrievably lapse due to failure to pay fees or otherwise comply with regulations or could be subject to compulsory licensing.

If we encounter delays in our development or clinical trials, the period of time during which we could market our potential products under patent protection would be reduced.

Our competitors may be able to circumvent our licensed patents or future patents we may own by developing similar or alternative technologies or products in a non-infringing manner. Our competitors may seek to market generic versions of any approved products by submitting abbreviated new drug applications to the FDA in which our competitors claim that our licensed patents or any future patents we may own are invalid, unenforceable or not infringed. Alternatively, our competitors may seek approval to market their own products similar to or otherwise competitive with our product candidates. In these circumstances, we may need to defend or assert our licensed patents or any future patents we may own, including by filing lawsuits alleging patent infringement. In any of these types of proceedings, a court or other agency with jurisdiction may find our licensed patents or any future patents we may own invalid or unenforceable. We may also fail to identify patentable aspects of our research and development before it is too late to obtain patent protection. Even if we own or in-license valid and enforceable patents, these patents still may not provide protection against competing products or processes sufficient to achieve our business objectives.

We also may rely on trade secrets to protect our technology, especially where we do not believe patent protection is appropriate or obtainable. However, trade secrets are difficult to protect, and we have limited control over the protection of trade secrets used by our collaborators and suppliers. Although we use reasonable efforts to protect our trade secrets, our employees, consultants, contractors, outside scientific collaborators and other advisors may unintentionally or willfully disclose our information to competitors or use such information to compete with us. Moreover, our competitors may independently develop equivalent knowledge, methods and know-how. If our confidential or proprietary information is divulged to or acquired by third parties, including our competitors, our competitive position in the marketplace will be harmed and this would have a material adverse effect on our business.

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

Many companies have encountered significant problems in protecting and defending intellectual property rights in certain countries. The legal systems of certain countries, particularly certain developing countries, do not favor the enforcement of patents, trade secrets and other intellectual property, particularly those relating to pharmaceutical and 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 generally. Proceedings to enforce our patent rights in foreign countries could result in substantial costs and divert our efforts and attention from other aspects of our business, could put our patents at risk of being invalidated or interpreted narrowly and our patent applications at risk of not issuing and could provoke third parties to assert claims against us. We may not prevail in any lawsuits that we initiate and the damages or other remedies awarded, if any, may not be commercially meaningful. Accordingly, our efforts to enforce our intellectual property rights around the world may be inadequate to obtain a significant commercial advantage from the intellectual property that we develop or license.

We may infringe the intellectual property rights of others, which may prevent or delay our drug development efforts and prevent us from commercializing or increase the costs of commercializing our product candidates.

Our commercial success depends significantly on our ability to operate without infringing the patents and other intellectual property rights of third parties. For example, there could be issued patents of which we are not aware that our current or potential future product candidates infringe. There also could be patents that we believe we do not infringe, but that we may ultimately be found to infringe. We have licensed intellectual property from CMU under the CMU License Agreement, and prior generation intellectual property was licensed to other entities. Such intellectual property, in conjunction with further developed technologies for gene editing therapies using intellectual property, may overlap with our own intellectual property.

As the biotechnology and pharmaceutical industries expand and more patents are issued, the risk increases that others may assert our product candidates infringe the patent rights of others. Moreover, it is not always clear to industry participants, including us, which patents cover various types of drugs, products or their methods of use or manufacture. Thus, because of the large number of patents issued and patent applications filed in our fields, there may be a risk that third parties may allege they have patent rights encompassing

our product candidates, technologies or methods. Furthermore, because the nucleic acid therapeutics intellectual property landscape is still evolving and our product candidates have not been through clinical trials or commercialized, it is difficult to conclusively assess our freedom to operate without infringing third party rights. There are numerous companies that have pending patent applications and issued patents directed to certain aspects of nucleic acid therapeutics. We are aware of third-party competitors in the oligonucleotide therapeutics space, whose patent filings and/or issued patents may include claims directed to targets and/or products related to some of our programs. It is possible that at the time that we commercialize our products these third-party patent portfolios may include issued patent claims that cover our product candidates or critical features of their production or use. Our competitive position may suffer if patents issued to third parties or other third-party intellectual property rights cover, or may be alleged to cover, our product candidates or elements thereof, or methods of manufacture or use relevant to our development plans. In such cases, we may not be in a position to develop or commercialize product candidates unless we successfully pursue litigation to nullify or invalidate the third-party intellectual property right concerned or enter into a license agreement with the intellectual property right holder, if available on commercially reasonable terms.

Moreover, patent applications are in some cases maintained in secrecy until patents are issued. The publication of discoveries in the scientific or patent literature frequently occurs substantially later than the date on which the underlying discoveries were made and patent applications were filed. Because patents can take many years to issue, there may be currently pending applications of which we are unaware that may later result in issued patents that our product candidates or potential products infringe. For example, pending applications may exist that claim or can be amended to claim subject matter that our product candidates or potential products infringe. Competitors may file continuing patent applications claiming priority to already issued patents in the form of continuation, divisional, or continuation-in-part applications, in order to maintain the pendency of a patent family and attempt to cover our product candidates. We cannot be certain that others have not filed patent applications for technology covered by our issued patents or our pending applications, or that we were the first to invent the technology. Our competitors may have filed, and may in the future file, patent applications covering our products or technology similar to ours. Any such patent application may have priority over our patent applications or patents, which could require us to obtain rights to issued patents covering such technologies.

Third parties may assert that we are employing their proprietary technology without authorization and may sue us for patent or other intellectual property infringement. These lawsuits are costly and could adversely affect our business, financial condition and results of operations and divert the attention of managerial and scientific personnel. If we are sued for patent infringement, we would need to demonstrate that our product candidates, potential products or methods either do not infringe the claims of the relevant patent or that the patent claims are invalid, and we may not be able to do this. Proving invalidity is difficult. For example, in the U.S., proving invalidity requires a showing of clear and convincing evidence to overcome the presumption of validity enjoyed by issued patents. Even if we are successful in these proceedings, we may incur substantial costs and the time and attention of our management and scientific personnel could be diverted in pursuing these proceedings, which could have a material adverse effect on us. In addition, we may not have sufficient resources to bring these actions to a successful conclusion. If a court holds that any third-party patents are valid, enforceable and cover our products or their use, the holders of any of these patents may be able to block our ability to commercialize our products unless it acquires or obtains a license under the applicable patents or until the patents expire.

If a third party claims that we infringe its intellectual property rights, we may face a number of issues, including, but not limited to: infringement and other intellectual property claims which, regardless of merit, may be expensive and time-consuming to litigate and may divert our management's attention from our core business; substantial damages for infringement, which we may have to pay if a court decides that the product candidate or technology at issue infringes on or violates the third party's rights, and, if the court finds that the infringement was willful, we could be ordered to pay treble damages and the patent owner's attorneys' fees; a court prohibiting us from developing, manufacturing, marketing or selling our product candidates, or from using our proprietary technologies, unless the third party licenses its product rights to us, which it is not required to do; if a license is available from a third party, we may have to pay substantial royalties, upfront fees and other amounts, and/or grant cross-licenses to intellectual property rights for our products; and redesigning our product candidates or processes so they do not infringe, which may not be possible or may require substantial monetary expenditures and time.

We may choose to challenge the patentability of claims in a third party's U.S. patent by requesting that the USPTO review the patent claims in an *ex-parte* re-exam, *inter partes* review or post-grant review proceedings. These proceedings are expensive and may consume our time or other resources. We may choose to challenge a third party's patent in patent opposition proceedings in the EPO, or other foreign patent office. The costs of these opposition proceedings could be substantial and may consume our time or other resources. If we fail to obtain a favorable result at the USPTO, EPO or other patent office then we may be exposed to litigation by a third party alleging that the patent may be infringed by our product candidates or proprietary technologies.

We may not be able to enter into licensing arrangements or make other arrangements at a reasonable cost or on reasonable terms. Any inability to secure licenses or alternative technology could result in delays in the introduction of our product candidates or lead to prohibition of the manufacture or sale of products by us. 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. We could be forced, including by court order, to cease commercializing the infringing technology or product candidate. In addition, in any such proceeding or litigation, we could be found liable for monetary damages, including treble damages and attorneys' fees, if we are found to have willfully infringed a patent. A finding of infringement could prevent us from commercializing our product candidates or force us to cease some of our business operations, which could materially and adversely affect our business, financial condition and results of operations. Any claims by third parties that we have misappropriated their confidential information or trade secrets could have a similar material and adverse effect on our business, financial condition and results of operations. In addition, any uncertainties resulting from the initiation and continuation of any litigation could have a material adverse effect on our ability to raise the funds necessary to continue our operations.

Our product candidates may also require specific formulations to work effectively and efficiently. These formulations may be covered by intellectual property rights held by others. We may develop products containing our compounds and pre-existing pharmaceutical compounds. These pharmaceutical compounds may be covered by intellectual property rights held by others. We may be required by the FDA or comparable foreign regulatory authorities to provide a companion diagnostic test or tests with our product candidates. These diagnostic test or tests may be covered by intellectual property rights held by others. We may be unable to acquire or in-license any relevant third-party intellectual property rights that we identify as necessary or important to our business operations.

We, or our licensors, may not be able to detect infringement against our owned or in-licensed patents, as the case may be, which may be especially difficult for manufacturing processes or formulation patents. Even if we or our licensors detect infringement by a third party of our owned or in-licensed patents, we or our licensors, as the case may be, may choose not to pursue litigation against or settlement with the third party. If we, or our licensors, later sue such third party for patent infringement, the third party may have certain legal defenses available to it, which otherwise would not be available except for the delay between when the infringement was first detected and when the suit was brought. Such legal defenses may make it impossible for us or our licensors to enforce our owned or in-licensed patents, as the case may be, against such third party.

Any claims or lawsuits relating to infringement of intellectual property rights brought by or against us will be costly and time consuming and may adversely affect our business, financial condition and results of operations.

Competitors may infringe or otherwise violate our patents, trademarks, copyrights or other intellectual property. To counter infringement or other violations, we may be required to initiate litigation to enforce or defend our licensed and owned intellectual property. Lawsuits to protect our intellectual property rights can be very time consuming and costly. There is a substantial amount of litigation involving patent and other intellectual property rights in the pharmaceutical industry generally. Such litigation or proceedings could substantially increase our operating expenses and reduce the resources available for development activities or any future sales, marketing or distribution activities.

In any infringement litigation, any award of monetary damages we receive may not be commercially valuable. 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 litigation. Moreover, there can be no assurance that we will have sufficient financial or other resources to file and pursue such infringement claims, which typically last for years before they are resolved. Further, any claims we assert against a perceived infringer could provoke these parties to assert counterclaims against us alleging that we have infringed their patents. Some of our competitors may be able to sustain the costs of such litigation or proceedings more effectively than we can because of their greater financial resources. Uncertainties resulting from the initiation and continuation of patent litigation or other proceedings could have a material adverse effect on our ability to compete in the marketplace. In addition, in a patent infringement proceeding, a court may decide that one or more of the patents we assert is invalid or unenforceable, in whole or in part, construe the patent's claims narrowly or refuse to prevent the other party from using the technology at issue on the grounds that our patents do not cover the technology. Similarly, if we assert trademark infringement claims, a court may determine that the marks we have asserted are invalid or unenforceable or that the party against whom we have asserted trademark infringement has superior rights to the marks in question. In such a case, we could ultimately be forced to cease use of such marks.

In addition, our licensed patents and patent applications, and patents and patent applications that we may apply for, own or license in the future, could face other challenges, such as interference proceedings, opposition proceedings, re-examination proceedings and

other forms of post-grant review. Any of these challenges, if successful, could result in the invalidation of, or in a narrowing of the scope of, any of our licensed patents and patent applications and patents and patent applications that we may apply for, own or license in the future subject to challenge. Any of these challenges, regardless of their success, would likely be time-consuming and expensive to defend and resolve and would divert our management and scientific personnel's time and attention.

Any issued patents we may own covering our product candidates could be narrowed or found invalid or unenforceable if challenged in court or before administrative bodies in the United States or abroad, including the USPTO.

Any of our intellectual property rights could be challenged or invalidated despite measures we take to obtain patent and other intellectual property protection with respect to our product candidates and proprietary technology. For example, if we were to initiate legal proceedings against a third party to enforce a patent covering one of our product candidates, the defendant could counterclaim that our patent is invalid and/or unenforceable. In patent litigation in the U.S. and in some other jurisdictions, defendant counterclaims alleging invalidity and/or unenforceability are commonplace. Grounds for a validity challenge could be an alleged failure to meet any of several statutory requirements, for example, lack of novelty, obviousness or enablement. Grounds for an unenforceability assertion could be an inequitable conduct allegation that someone connected with prosecution of the patent withheld material information from the USPTO or the applicable foreign counterpart, or made a misleading statement, during prosecution. A litigant or the USPTO itself could challenge our patents on this basis even if we believe that we have conducted our patent prosecution in accordance with the duty of candor and in good faith. The outcome following such a challenge is unpredictable.

With respect to challenges to the validity of our patents, for example, there might be invalidating prior art, of which we and the patent examiner were unaware during prosecution. If a defendant were to prevail on a legal assertion of invalidity and/or unenforceability, we would lose at least part, and perhaps all, of the patent protection on a product candidate. Even if a defendant does not prevail on a legal assertion of invalidity and/or unenforceability, our patent claims may be construed in a manner that would limit our ability to enforce such claims against the defendant and others. The cost of defending such a challenge, particularly in a foreign jurisdiction, and any resulting loss of patent protection could have a material adverse impact on one or more of our product candidates and our business. Enforcing our intellectual property rights against third parties may also cause such third parties to file other counterclaims against us, which could be costly to defend, particularly in a foreign jurisdiction, and could require us to pay substantial damages, cease the sale of certain products or enter into a license agreement and pay royalties (which may not be possible on commercially reasonable terms or at all). Any efforts to enforce our intellectual property rights are also likely to be costly and may divert the efforts of our scientific and management personnel.

We may be subject to claims challenging the inventorship of our patents and other intellectual property.

We may be subject to claims that former employees, collaborators or other third parties have an interest in our patents, trade secrets, or other intellectual property as an inventor or co-inventor. For example, we may have inventorship disputes arise from conflicting obligations of employees, consultants or others who are involved in developing our product candidates. While it is our policy to require our employees and contractors who may be involved in the development of intellectual property to execute agreements assigning such intellectual property to us, we may be unsuccessful in executing such an agreement with each party who in fact develops intellectual property that we regard as our own. Our and their assignment agreements may not be self-executing or may be breached, and litigation may be necessary to defend against these and other claims challenging inventorship or our ownership of our patents, trade secrets or other intellectual property. If we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights, such as exclusive ownership of, or right to use, intellectual property that is important to our product candidates. Even if we are successful in defending against such claims, litigation could result in substantial costs and be a distraction to management and other employees. Any of the foregoing could have a material adverse effect on our business, financial condition, results of operations and prospects.

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

Filing, prosecuting and defending patents on product candidates throughout the world would be prohibitively expensive. Competitors may use our licensed and owned technologies in jurisdictions where we have not licensed or obtained patent protection to develop their own products and, further, may export otherwise infringing products to territories where we may obtain or license patent protection, but where patent enforcement is not as strong as that in the U.S. These products may compete with our product candidates in jurisdictions where we do not have any issued or licensed patents, and any future patent claims or other intellectual property rights may not be effective or sufficient to prevent them from so competing.

Many companies have encountered significant problems in protecting and defending intellectual property rights in foreign jurisdictions. The legal systems of certain countries, particularly certain developing countries, do not favor the enforcement of patents and other intellectual property protection, particularly those relating to pharmaceuticals, which could make it difficult for us to stop the infringement of our licensed patents and future patents we may own, or marketing of competing products in violation of our proprietary rights generally. Further, the laws of some foreign countries do not protect proprietary rights to the same extent or in the same manner as the laws of the U.S. As a result, we may encounter significant problems in protecting and defending our licensed and owned intellectual property both in the U.S. and abroad. For example, China currently affords less protection to a company's intellectual property than some other jurisdictions. As such, the lack of strong patent and other intellectual property protection in China may significantly increase our vulnerability regarding unauthorized disclosure or use of our intellectual property and undermine our competitive position. Proceedings to enforce our future patent rights, if any, in foreign jurisdictions could result in substantial cost and divert our efforts and attention from other aspects of our business.

We may be unable to adequately prevent disclosure of trade secrets and other proprietary information.

In order to protect our proprietary and licensed technology and processes, we rely in part on confidentiality agreements with our corporate partners, employees, consultants, manufacturers, outside scientific collaborators and sponsored researchers and other advisors. These agreements may not effectively prevent disclosure of our confidential information and may not provide an adequate remedy in the event of unauthorized disclosure of confidential information. In addition, others may independently discover our trade secrets and proprietary information. Failure to obtain or maintain trade secret protection could adversely affect our competitive business position.

We may be subject to claims that our employees, consultants or independent contractors have wrongfully used or disclosed confidential information of third parties.

We expect to employ individuals who were previously employed at other pharmaceutical companies. Although we have no knowledge of any such claims against us, we may be subject to claims that us or our employees, consultants or independent contractors have inadvertently or otherwise used or disclosed confidential information of our employees' former employers or other third parties. Litigation may be necessary to defend against these claims. There is no guarantee of success in defending these claims, and even if we are successful, litigation could result in substantial cost and be a distraction to our management and other employees.

We may become involved in disputes with our employees, consultants, or independent contractors regarding the ownership of intellectual property.

We will rely on our employees, consultants, and independent contractors to develop intellectual property that we will own and commercialize. These persons may dispute the terms of their agreements with us, for example, their obligation to assign intellectual property, work product, and know how to us. In case of such a dispute, the person may assert that he owns the work that he performed on our behalf, and that all corresponding intellectual property rights vest in him. The person may assert ownership of the intellectual property, refuse to disclose the intellectual property to us, and fail to execute documents essential to document our ownership. If the person withholds the disclosure of new technology, we may not even know what technology has been withheld from us, or that the technology even exists. In this case, we may never be able to identify and perfect title to the technology. Such a person would pose a significant risk of disclosure of our confidential intellectual property. If the person chose to reveal the technology to a third party, we may have no means or opportunity to prevent the disclosure. Our confidential intellectual property would then become known to third parties, possibly even without us knowing about the disclosure. We would suffer material adverse effects from the disclosure and misuse of our intellectual property. To enforce our rights would require a complex dispute of state and federal intellectual property law to take place in a state court. The outcome of such a dispute in a state court, especially in a jury trial, is highly uncertain.

Patent terms may be inadequate to protect our competitive position on our product candidates for an adequate amount of time.

Patents have a limited lifespan. In the United States, if all maintenance fees are timely paid, the natural expiration of a patent is generally 20 years from its earliest U.S. non-provisional filing date. Various extensions may be available, but the life of a patent, and the protection it affords, is limited. Even if patents covering our product candidates are obtained, once the patent life has expired for a product candidate, we may be open to competition from competitive medications, including generic medications. Given the amount of time required for the development, testing and regulatory review of new product candidates, patents protecting such product candidates might expire before or shortly after such product candidates are commercialized. As a result, our patent portfolio may not

provide us with sufficient rights to exclude others from commercializing product candidates similar or identical to ours for a meaningful amount of time, or at all.

Depending upon the timing, duration and conditions of any FDA marketing approval of our product candidates, one or more of our U.S. patents may be eligible for limited patent term extension under the Drug Price Competition and Patent Term Restoration Act of 1984, referred to as the Hatch-Waxman Amendments, and similar legislation in the European Union and certain other countries. 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. However, we may not receive an extension if we fail to exercise due diligence during the testing phase or regulatory review process, fail to apply within applicable deadlines, fail to apply prior to expiration of relevant patents or otherwise fail to satisfy applicable requirements. Moreover, the length of the extension could be less than we request. Only one patent per approved product can be extended, the extension cannot extend the total patent term beyond 14 years from approval and only those claims covering the approved drug, a method for using it or a method for manufacturing it may be extended. If we are unable to obtain patent term extension or the term of any such extension is less than we request, the period during which we can enforce our patent rights for the applicable product candidate 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. Further, if this occurs, our competitors may take advantage of our investment in development and trials by referencing our clinical and preclinical data and launch their product earlier than might otherwise be the case, and our competitive position, business, financial condition, results of operations and prospects could be materially harmed.

Also, there are detailed rules and requirements regarding the patents that may be submitted to the FDA for listing in the Approved Drug Products with Therapeutic Equivalence Evaluations, or the Orange Book. We may be unable to obtain patents covering our product candidates that contain one or more claims that satisfy the requirements for listing in the Orange Book. Even if we submit a patent for listing in the Orange Book, the FDA may decline to list the patent, or a manufacturer of generic drugs may challenge the listing. If one of our product candidates is approved and a patent covering that product candidate is not listed in the Orange Book, a manufacturer of generic drugs would not have to provide advance notice to us of any abbreviated new drug application filed with the FDA to obtain permission to sell a generic version of such product candidate. Any of the foregoing could harm our competitive position, business, financial condition, results of operations and prospects.

Risks Related to Government Regulation

We are very early in our development efforts. All of our product candidates are still in preclinical development. If we are unable to advance our product candidates to clinical development, obtain regulatory approval and ultimately commercialize our product candidates or experience significant delays in doing so, our business will be materially harmed.

We are very early in our development efforts, and all of our product candidates are still in preclinical development. We have invested substantially all of our efforts and financial resources in the identification and preclinical development of PATrOL-enabled therapies, including the development programs for the treatment of Huntington's Disease and Myotonic Dystrophy Type 1. Our ability to generate product revenues, which we do not expect will occur for many years, if ever, will depend on the successful development and eventual commercialization of our product candidates, which may never occur. We currently generate no revenue from sales of any products, and we may never be able to develop or commercialize a marketable product. In addition, certain of our product candidate development programs contemplate the development of companion diagnostics, which are assays or tests to identify an appropriate patient population. Companion diagnostics are subject to regulation as medical devices and must themselves be approved for marketing by the FDA or certain other foreign regulatory agencies before we may commercialize our product candidates. The success of our product candidates will depend on several factors, including the following:

- successful completion of preclinical studies;
- approval of INDs for our planned clinical trials or future clinical trials;
- successful enrollment in, and completion of, clinical trials;
- successful development of companion diagnostics for use with certain of our product candidates;
- receipt of regulatory approvals from applicable regulatory authorities;

- establishing commercial manufacturing capabilities or making arrangements with third-party manufacturers for clinical supply and commercial manufacturing;
- obtaining and maintaining patent and trade secret protection or regulatory exclusivity for our product candidates;
- launching commercial sales of our product candidates, if and when approved, whether alone or in collaboration with others;
- acceptance of the product candidates, if and when approved, by patients, the medical community and third-party payors;
- effectively competing with other therapies;
- obtaining and maintaining third-party insurance coverage and adequate reimbursement;
- enforcing and defending intellectual property rights and claims; and
- maintaining a continued acceptable safety profile of the product candidates following approval, if approved.

If we do not achieve one or more of these factors in a timely manner or at all, we could experience significant delays or an inability to successfully commercialize our product candidates, which would materially harm our business. If we do not receive regulatory approvals for our product candidates, we may not be able to continue our operations.

Furthermore, the FDA has relatively limited experience with nucleic acid therapeutics, particularly PNAs, which may increase the complexity, uncertainty and length of the regulatory review process for our product candidates. To date, the FDA has approved few nucleic acid therapeutics for marketing and commercialization, and the FDA and our foreign counterparts have not yet established any definitive policies, practices or guidelines specifically in relation to these drugs. The lack of policies, practices or guidelines specific to nucleic acid therapeutics may hinder or slow review by the FDA of any regulatory filings that we may submit. Moreover, the FDA may respond to these submissions by defining requirements we may not have anticipated. Such responses could lead to significant delays in the development of our product candidates. In addition, because there may be approved treatments for some of the diseases for which we may seek approval, in order to receive regulatory approval, we may need to demonstrate through clinical trials that the product candidates we develop to treat these diseases, if any, are not only safe and effective, but safer or more effective than existing products. Furthermore, in recent years, there has been increased public and political pressure on the FDA with respect to the approval process for new drugs, and the FDA's standards, especially regarding drug safety, appear to have become more stringent. As a result of the foregoing factors, we may never receive regulatory approval to market and commercialize any product candidate.

Preclinical and clinical trials are 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.

All of our product candidates are still in the preclinical stage, and their risk of failure is high. Before we can commence clinical trials for a product candidate, we must complete extensive preclinical testing and studies that support our planned INDs in the U.S., or similar applications in other jurisdictions. We cannot be certain of the timely completion or outcome of our preclinical testing and studies and cannot predict if the FDA or other regulatory authorities will accept our proposed clinical programs or if the outcome of our preclinical testing and studies will ultimately support the further development of our programs. It is also possible that the FDA may require changes to our proposed clinical development programs. For example, our nonclinical studies for our DM1 program are using a proposed formulation of the NT-0200 development candidate that contains an excipient in quantities in excess of current FDA guidance. We believe that no additional nonclinical studies or modifications to the planned nonclinical toxicology studies related to the concentration of the excipient in the formulation will be needed to support clinical development. We cannot assure you that this will be the case, however. It is also impossible to predict when or if any of our product candidates will complete clinical trials evaluating their safety and effectiveness in humans or will receive regulatory approval. To obtain the requisite regulatory approvals to market and sell any of our product candidates, we must demonstrate through extensive preclinical studies and clinical trials that our PATrOL™ platform and product candidates are safe and effective in humans for use in each target indication. To date, we have never advanced a product candidate into a clinical trial. Preclinical and clinical testing is expensive and can take many years to complete, and the outcome is inherently uncertain. Failure can occur at any time during the preclinical or clinical trial process. Our preclinical programs may experience delays or may never advance to clinical trials, which would adversely affect our ability to obtain regulatory

approvals or commercialize these programs on a timely basis or at all, which would have an adverse effect on our business, financial condition and results of operations.

Additionally, the results of preclinical studies and future clinical trials of product candidates may not be predictive of the results of later-stage clinical trials. Product candidates in later stages of clinical trials may fail to show the desired safety and efficacy results despite having progressed through preclinical studies and initial clinical trials. Many companies in the pharmaceutical industry have suffered significant setbacks in advanced clinical trials due to adverse safety profiles or lack of efficacy, notwithstanding promising results in earlier studies. Similarly, our future clinical trial results may not be successful for these or other reasons.

This product candidate development risk is heightened by any changes in the anticipated clinical trials compared to the completed clinical trials. As product candidates are developed from preclinical through early to late-stage clinical trials towards approval and commercialization, it is customary that various aspects of the development program, such as manufacturing and methods of administration, are altered along the way in an effort to optimize processes and results. While these types of changes are common and are intended to optimize the product candidates for late-stage clinical trials, approval and commercialization, such changes carry the risk that they will not achieve these intended objectives.

Any of these changes could make the results of our anticipated clinical trials or other future clinical trials we may initiate less predictable and could cause our product candidates to perform differently, including causing toxicities, which could delay completion of our clinical trials, delay approval of our product candidates, and/or jeopardize our ability to commence product sales and generate revenues.

We may rely on third parties to conduct investigator-sponsored clinical trials of our product candidates. Any failure by a third party to meet our obligations with respect to the clinical development of our product candidates may delay or impair our ability to obtain regulatory approval for other product candidates.

We may rely on academic and private non-academic institutions to conduct and sponsor preclinical and clinical trials relating to our product candidates. We will not control the design or conduct of the investigator-sponsored trials, and it is possible that the FDA or non-U.S. regulatory authorities will not view these investigator-sponsored trials as providing adequate support for future preclinical and clinical trials, whether controlled by us or third parties, for any one or more reasons, including elements of the design or execution of the trials or safety concerns or other trial results. For example, we collaborate with, and rely on, academic centers to conduct preclinical and non-investigator-sponsored research and it is possible that the interests of such academic centers may not be aligned with our interests.

Such arrangements will likely provide us certain information rights with respect to the investigator-sponsored trials, including access to and the ability to use and reference the data, including for our own regulatory filings, resulting from the investigator-sponsored trials. However, we would not have control over the timing and reporting of the data from investigator-sponsored trials, nor would we own the data from the investigator-sponsored trials. If we are unable to confirm or replicate the results from the investigator-sponsored trials or if negative results are obtained, we would likely be further delayed or prevented from advancing further clinical development of our product candidates. Further, if investigators or institutions breach their obligations with respect to the clinical development of our product candidates, or if the data proves to be inadequate compared to the first-hand knowledge we might have gained had the investigator-sponsored trials been sponsored and conducted by us, then our ability to design and conduct any future preclinical or clinical trials ourselves may be adversely affected.

Additionally, the FDA or non-U.S. regulatory authorities may disagree with the sufficiency of our right of reference to the preclinical, manufacturing or clinical data generated by these investigator-sponsored trials, or our interpretation of preclinical, manufacturing or clinical data from these investigator-sponsored trials. If so, the FDA or other non-U.S. regulatory authorities may require us to obtain and submit additional preclinical, manufacturing, or clinical data before we may initiate our anticipated trials and/or may not accept such additional data as adequate to initiate our anticipated trials.

Our product candidates may cause undesirable side effects that could delay or prevent their regulatory approval or commercialization or have other significant adverse implications on our business, financial condition and results of operations.

Undesirable side effects observed in preclinical studies or in clinical trials with our product candidates could interrupt, delay or halt their development and could result in the denial of regulatory approval by the FDA, the EMA or comparable foreign authorities for

any or all targeted indications or adversely affect the marketability of any product candidates developed using our PATrOL™ platform that receive regulatory approval. In turn, this could eliminate or limit our ability to commercialize our product candidates.

Our product candidates may exhibit adverse effects in preclinical toxicology studies and adverse interactions with other drugs. There are also risks associated with additional requirements the FDA, the EMA or comparable foreign authorities may impose for marketing approval with regard to a particular disease.

Our product candidates may require a risk management program that could include patient and healthcare provider education, usage guidelines, appropriate promotional activities, a post-marketing observational study and ongoing safety and reporting mechanisms, among other requirements. Prescribing could be limited to physician specialists or physicians trained in the use of the drug, or could be limited to a more restricted patient population. Any risk management program required for approval of our product candidates could potentially have an adverse effect on our business, financial condition and results of operations.

Undesirable side effects involving our product candidates may have other significant adverse implications on our business, financial condition and results of operations. For example:

- we may be unable to obtain additional financing on acceptable terms, if at all;
- our collaborators may terminate any development agreements covering these product candidates;
- if any development agreements are terminated, we may determine not to further develop the affected product candidates due to resource constraints and may not be able to establish additional collaborations for their further development on acceptable terms, if at all;
- if we were to later continue the development of these product candidates and receive regulatory approval, earlier findings may significantly limit their marketability and thus significantly lower our potential future revenues from their commercialization;
- we may be subject to product liability or stockholder litigation; and
- we may be unable to attract and retain key employees.

In addition, if any of our product candidates receive marketing approval and we or others later identify undesirable side effects caused by the product:

- regulatory authorities may withdraw their approval of the PATrOL™-enabled product, or we or our partners may decide to cease marketing and sale of the product voluntarily;
- we may be required to change the way the product is administered, conduct additional preclinical studies or additional clinical trials after initial clinical trials regarding the product, change the labeling of the product, or change the product's manufacturing facilities; and
- our reputation may suffer.

Any of these events could prevent us from achieving or maintaining market acceptance of the affected product and could substantially increase the costs and expenses of commercializing the product, which in turn could delay or prevent us from generating significant revenues from the sale of the product.

Delays in the commencement or completion of clinical trials could result in increased costs to us and delay our ability to establish strategic collaborations.

Delays in the commencement or completion of clinical trials could significantly impact our drug development costs. We do not know whether anticipated clinical trials will begin on time or be completed on schedule, if at all. The commencement of clinical trials can be delayed for a variety of reasons, including, but not limited to, delays related to:

- obtaining regulatory approval to commence one or more clinical trials;
- reaching agreement on acceptable terms with prospective third-party contract research organizations ("CROs") and clinical trial sites;
- manufacturing sufficient quantities of a product candidate or other materials necessary to conduct clinical trials;
- changes in regulations as part of a response to the COVID-19 pandemic, which may require us to change the ways in which future clinical trials would otherwise be conducted;
- obtaining institutional review board approval to conduct one or more clinical trials at a prospective site;
- recruiting and enrolling patients to participate in one or more clinical trials; and
- the failure of our collaborators to adequately resource our product candidates due to their focus on other programs or as a result of general market conditions.

In addition, once a clinical trial has begun, it may be suspended or terminated by us, our collaborators, the institutional review boards or data safety monitoring boards charged with overseeing our clinical trials, the FDA, the EMA or comparable foreign authorities due to a number of factors, including:

- failure to conduct the clinical trial in accordance with regulatory requirements or clinical protocols;
- inspection of the clinical trial operations or clinical trial site by the FDA, the EMA or comparable foreign authorities resulting in the imposition of a clinical hold;
- unforeseen safety issues;
- lack of adequate funding to continue the clinical trials; and
- lack of patient enrollment in clinical studies.

If we experience delays in the completion or termination of any clinical trial of our product candidates, the commercial prospects of our product candidates will be harmed, and our ability to commence product sales and generate product revenues from any of our product candidates will be delayed. In addition, any delays in completing our clinical trials will increase our costs and slow down our product candidate development and approval process. Delays in completing our clinical trials could also allow our competitors to obtain marketing approval before we do or shorten the patent protection period during which we may have the exclusive right to commercialize our product candidates. Any of these occurrences may harm our business, financial condition and prospects significantly. 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.

If we experience delays in the enrollment of patients in our clinical trials, our receipt of necessary regulatory approvals could be delayed or prevented.

We may not be able to initiate or continue clinical trials for our product candidates if we are unable to locate and enroll a sufficient number of eligible patients to participate in these trials as required by the FDA or other regulatory authorities. Patient enrollment, a

significant factor in the timing of clinical trials, is affected by many factors, including the size and nature of the patient population, the proximity of patients to clinical sites, the eligibility criteria for the trial, the design of the clinical trial, competing clinical trials and clinicians' and patients' perceptions as to the potential advantages of the drug being studied in relation to other available therapies, including any new drugs that may be approved for the indications we are investigating. In addition, the COVID-19 pandemic may negatively impact our ability to recruit and enroll patients for our clinical trials because they may be reluctant or unable to visit clinical sites, or may delay seeking treatment for chronic conditions.

If we fail to enroll and maintain the number of patients for which the clinical trial was designed, the statistical power of that clinical trial may be reduced, which would make it harder to demonstrate that the product candidate being tested in such clinical trial is safe and effective. Additionally, enrollment delays in our clinical trials may result in increased development costs for our product candidates, which would cause our value to decline and limit our ability to obtain additional financing. Our inability to enroll a sufficient number of patients for any of our future clinical trials would result in significant delays or may require us to abandon one or more clinical trials altogether.

We intend to rely on third parties to conduct our preclinical studies and clinical trials and perform other tasks. If these third parties do not successfully carry out their contractual duties, meet expected deadlines, or comply with regulatory requirements, we may not be able to obtain regulatory approval for or commercialize our product candidates and our business, financial condition and results of operations could be substantially harmed.

We intend to rely upon third-party CROs, medical institutions, clinical investigators and contract laboratories to monitor and manage data for our ongoing preclinical and anticipated clinical programs. Nevertheless, we maintain responsibility for ensuring that each of our preclinical studies are, and anticipated clinical studies will be, conducted in accordance with the applicable protocol, legal, regulatory, and scientific standards and our reliance on these third parties does not relieve us of our regulatory responsibilities. We and our CROs and other vendors are required to comply with current requirements on cGMP, good clinical practices ("GCP") and GLP, which are a collection of laws and regulations enforced by the FDA, the EMA and comparable foreign authorities for all of our product candidates in clinical development. Regulatory authorities enforce these regulations through periodic inspections of preclinical study and clinical trial sponsors, principal investigators, preclinical study and clinical trial sites, and other contractors. If we or any of our CROs or vendors fails 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 comparable foreign authorities 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 authority, such regulatory authority will determine that any of our clinical trials comply with GCP regulations. In addition, our clinical trials must be conducted with products produced consistent with cGMP regulations. Our failure to comply with these regulations may require it to repeat clinical trials, which would delay the development and regulatory approval processes.

We may also not be able to enter into arrangements with CROs on commercially reasonable terms, or at all. In addition, our CROs will not be our employees, and except for remedies available to us under our agreements with such CROs, we will not be able to control whether or not they devote sufficient time and resources to our ongoing preclinical and clinical programs. If CROs do not successfully carry out their contractual duties or obligations or meet expected deadlines, if they need to be replaced or if the quality or accuracy of the data they obtain is compromised due to the failure to adhere to our protocols, regulatory requirements, or for other reasons, our clinical trials may be extended, delayed or terminated and we may not be able to obtain regulatory approval for or successfully commercialize our product candidates. CROs may also generate higher costs than anticipated. As a result, our business, financial condition and results of operations and the commercial prospects for our product candidates could be materially and adversely affected, our costs could increase, and our ability to generate revenue could be delayed.

Switching or adding additional CROs, medical institutions, clinical investigators or contract laboratories involves additional cost and requires management time and focus. In addition, there is a natural transition period when a new CRO commences work replacing a previous CRO. As a result, delays occur, which can materially impact our ability to meet our desired development timelines. There can be no assurance that we will not encounter similar challenges or delays in the future or that these delays or challenges will not have a material adverse effect on our business, financial condition or results of operations.

Our product candidates are subject to extensive regulation under the FDA, the EMA or comparable foreign authorities, which can be costly and time consuming, cause unanticipated delays or prevent the receipt of the required approvals to commercialize our product candidates.

The clinical development, manufacturing, labeling, storage, record-keeping, advertising, promotion, export, marketing and distribution of our product candidates are subject to extensive regulation by the FDA and other U.S. regulatory agencies, the EMA or comparable authorities in foreign markets. In the U.S., neither us nor our collaborators are permitted to market our product candidates until we or our collaborators receive approval of an NDA from the FDA or receive similar approvals abroad. The process of obtaining these approvals is expensive, often takes many years, and can vary substantially based upon the type, complexity and novelty of the product candidates involved. Approval policies or regulations may change and may be influenced by the results of other similar or competitive products, making it more difficult for us to achieve such approval in a timely manner or at all. Any guidance that may result from recent FDA advisory panel discussions may make it more expensive to develop and commercialize such product candidates. In addition, as a company, we have not previously filed NDAs with the FDA or filed similar applications with other foreign regulatory agencies. This lack of experience may impede our ability to obtain FDA or other foreign regulatory agency approval in a timely manner, if at all, for our product candidates for which development and commercialization is our responsibility. Even if we believe the data collected from clinical trials of our drug products is promising, data are susceptible to varying interpretations, and such data may not be sufficient to support approval by the FDA or any other U.S. or comparable foreign regulatory approval. Nonclinical and clinical data can be interpreted in different ways.

Despite the time and expense invested, regulatory approval is never guaranteed. The FDA, the EMA or comparable foreign authorities can delay, limit or deny approval of a product candidate for many reasons, including:

- a product candidate may not be deemed safe or effective;
- agency officials of the FDA, the EMA or comparable foreign authorities may not find the data from nonclinical or preclinical studies and clinical trials generated during development to be sufficient;
- the FDA, the EMA or comparable foreign authorities may not approve our third-party manufacturers' processes, systems, controls or facilities; or
- the FDA, the EMA or a comparable foreign authority may change our approval policies or adopt new regulations.

Our inability to obtain these approvals would prevent us from commercializing our product candidates.

Moreover, the commencement and completion of clinical trials may be delayed by many factors that are beyond our control, including:

- delays obtaining regulatory approval to commence a trial;
- delays in reaching agreement on acceptable terms with contract research organizations ("CROs"), and clinical trial sites;
- delays in obtaining institutional review board, or IRB, approval at each site;
- slower than anticipated patient enrollment or our inability to recruit and enroll patients to participate in clinical trials for various reasons;
- our inability to retain patients who have initiated a clinical trial;
- scheduling conflicts with participating clinicians and clinical institutions;
- lack of funding to start or continue the clinical trial, including as a result of unforeseen costs due to enrollment delays, requirements to conduct additional trials and studies and increased expenses associated with our CROs and other third parties;

- negative or inconclusive results;
- deficiencies in the conduct of the clinical trial, including failure to conduct the clinical trial in accordance with regulatory requirements, good clinical practice, or clinical protocols;
- deficiencies in the clinical trial operations or trial sites resulting in the imposition of a clinical hold;
- patient noncompliance with the protocol;
- adverse medical events or side effects experienced by patients during the clinical trials as a result of or resulting from the clinical trial treatments;
- fatalities or other adverse events arising during a clinical trial due to medical problems that may not be related to clinical trial treatments;
- our ability to sustain the quality or stability of the applicable product candidate in compliance with acceptable standards; or
- our inability to produce or obtain sufficient quantities of the applicable product candidate to complete the clinical trials.

We may rely on CROs and other third parties to conduct clinical trials and, in such cases, we are unable to directly control the timing, conduct and expense of our clinical trials.

We may rely, in full or in part, on third parties to conduct our clinical trials. In such situations, we have less control over the conduct of our clinical trials, the timing and completion of the trials, the required reporting of adverse events and the management of data developed through the trial than would be the case if we were relying entirely upon our own staff. Communicating with outside parties can also be challenging, potentially leading to errors as well as difficulties in coordinating activities. Outside parties may have staffing difficulties, may undergo changes in priorities or may become financially distressed, adversely affecting their willingness or ability to conduct our trials. We may experience unexpected cost increases that are beyond our control. Problems with the timeliness or quality of the work of a CRO may lead us to seek to terminate the relationship and use an alternative service provider. However, making this change may be costly and may delay our trials, and contractual restrictions may make such a change difficult or impossible. Additionally, it may be challenging or impossible to find a replacement organization that can conduct our trials in an acceptable manner and at an acceptable cost.

The FDA, the NIH and the EMA have demonstrated caution in their regulation of gene therapy treatments, and ethical and legal concerns about gene therapy and genetic testing may result in additional regulations or restrictions on the development and commercialization of our product candidates, which may be difficult to predict.

The FDA, National Institutes of Health ("NIH") and the EMA have each expressed interest in further regulating biotechnology, including gene therapy and genetic testing. For example, the EMA advocates a risk-based approach to the development of a gene therapy product. Agencies at both the federal and state level in the United States, as well as U.S. congressional committees and other governments or governing agencies, have also expressed interest in further regulating the biotechnology industry. Such action may delay or prevent commercialization of some or all of our product candidates.

Regulatory requirements in the U.S. and in other jurisdictions governing gene therapy products have changed frequently and may continue to change in the future. Within the broader genetic medicine field, few therapeutic products have received marketing authorization from the FDA. The FDA reviews gene therapies and certain related products through its Office of Tissues and Advanced Therapies within its Center for Biologics Evaluation and Research, and established the Cellular, Tissue and Gene Therapies Advisory Committee to advise this review. In addition to FDA oversight and oversight by IRBs, under guidelines promulgated by the National Institutes of Health, or NIH, gene therapy clinical trials are also subject to review and oversight by an Institutional Biosafety Committee ("IBC"), a local institutional committee that reviews and oversees research utilizing recombinant or synthetic nucleic acid molecules at that institution. The IBC assesses the safety of the research and identifies any potential risk to public health or the environment. While the NIH guidelines are not mandatory unless the research in question is being conducted at or sponsored by institutions receiving NIH funding of recombinant or synthetic nucleic acid molecule research, many companies and other institutions

not otherwise subject to the NIH Guidelines voluntarily follow them. Even if the FDA has reviewed the trial and approved its initiation, the review process and determinations of the IBC or other reviewing bodies can impede or delay the initiation of a clinical trial.

In addition, adverse developments in clinical trials of gene therapy products conducted by others may cause the FDA or other oversight bodies to change the information or data required for approval of any of our product candidates. Similarly, the EMA governs the development of gene therapies in the European Union and may issue new guidelines concerning the development and marketing authorization for gene therapy products and require that we comply with these new guidelines. These regulatory review agencies and committees and the new requirements or guidelines they promulgate may lengthen the regulatory review process, require us to perform additional studies or trials, increase our development costs, lead to changes in regulatory positions and interpretations, delay or prevent approval and commercialization of our product candidates or lead to significant post-approval limitations or restrictions. As we advance our product candidates, we will be required to consult with these regulatory agencies and committees and comply with applicable requirements and guidelines. If we fail to do so, we may be required to delay or discontinue development of such product candidates. These additional processes may result in a review and approval process that is longer than we otherwise would have expected. Delays as a result of an increased or lengthier regulatory approval process or further restrictions on the development of our product candidates can be costly and could negatively impact our or our collaborators' ability to complete clinical trials and commercialize our current and future product candidates in a timely manner, if at all.

Even if our product candidates receive regulatory approval in the U.S., it may never receive approval or commercialize our products outside of the U.S.

In order to market any products outside of the U.S., we must establish and comply with numerous and varying regulatory requirements of other countries regarding safety and efficacy. Approval procedures vary among countries and can involve additional product testing and additional administrative review periods. The time required to obtain approval in other countries might differ from that required to obtain FDA approval. The regulatory approval process in other countries may include all of the risks detailed above regarding FDA approval in the U.S. as well as other risks. Regulatory approval in one country does not ensure regulatory approval in another, but a failure or delay in obtaining regulatory approval in one country may have a negative effect on the regulatory process in others. Failure to obtain regulatory approval in other countries or any delay seeking or obtaining such approval would impair our ability to develop foreign markets for our product candidates.

Even if any of our product candidates receive regulatory approval, our product candidates may still face future development and regulatory difficulties.

If any of our product candidates receive regulatory approval, the FDA, the EMA or comparable foreign authorities may still impose significant restrictions on the indicated uses or marketing of the product candidates or impose ongoing requirements for potentially costly post-approval studies and trials. In addition, regulatory agencies subject a product, our manufacturer and the manufacturer's facilities to continual review and periodic inspections. If a regulatory agency discovers previously unknown problems with a product, including adverse events of unanticipated severity or frequency, or problems with the facility where the product is manufactured, a regulatory agency may impose restrictions on that product, our collaborators or us, including requiring withdrawal of the product from the market. Our product candidates will also be subject to ongoing FDA, EMA or comparable foreign authorities' requirements for the labeling, packaging, storage, advertising, promotion, record-keeping and submission of safety and other post-market information on the drug. If our product candidates fail to comply with applicable regulatory requirements, a regulatory agency may:

- issue warning letters or other notices of possible violations;
- impose civil or criminal penalties or fines or seek disgorgement of revenue or profits;
- suspend any ongoing clinical trials;
- refuse to approve pending applications or supplements to approved applications filed by us or our collaborators;
- withdraw any regulatory approvals;

- impose restrictions on operations, including costly new manufacturing requirements, or shut down our manufacturing operations; or
- seize or detain products or require a product recall.

The FDA, the EMA and comparable foreign authorities actively enforce the laws and regulations prohibiting the promotion of off-label uses.

The FDA, the EMA and comparable foreign authorities strictly regulate the promotional claims that may be made about prescription products, such as our product candidates, if approved. In particular, a product may not be promoted for uses that are not approved by the FDA, the EMA or comparable foreign authorities as reflected in the product's approved labeling. If we receive marketing approval for our product candidates for our proposed indications, physicians may nevertheless use our products for their patients in a manner that is inconsistent with the approved label, if the physicians believe in their professional medical judgment that our products could be used in such manner. However, if we are found to have promoted our product candidates, if approved, for any off-label uses, the federal government could levy civil, criminal or administrative penalties, and seek fines against us. Such enforcement has become more common in the industry. The FDA, the EMA or comparable foreign authorities could also request that we enter into a consent decree or a corporate integrity agreement, or seek a permanent injunction against us under which specified promotional conduct is monitored, changed or curtailed. If we cannot successfully manage the promotion of our product candidates, if approved, we could become subject to significant liability, which would materially adversely affect our business, financial condition and results of operations.

We and our potential contract manufacturers are subject to significant regulation with respect to manufacturing our product candidates. The manufacturing facilities on which we will rely may not continue to meet regulatory requirements.

All entities involved in the preparation of therapeutics for clinical trials or commercial sale, including our potential contract manufacturers for our product candidates, are subject to extensive regulation. Components of a finished therapeutic product approved for commercial sale or used in Phase 2 or 3 clinical trials must be manufactured in accordance with cGMP. These regulations govern manufacturing processes and procedures and the implementation and operation of quality systems to control and assure the quality of investigational products and products approved for sale. Poor control of production processes can lead to the introduction of contaminants or to inadvertent changes in the properties or stability of our product candidates that may not be detectable in final product testing. We or our potential contract manufacturers must supply all necessary documentation in support of an NDA or marketing authorization application ("MAA") on a timely basis and must adhere to GLP and cGMP regulations enforced by the FDA, the EMA or comparable foreign authorities through their facilities inspection program. Some of our potential contract manufacturers may not have produced a commercially approved pharmaceutical product and therefore may not have obtained the requisite regulatory authority approvals to do so. The facilities and quality systems of some or all of our potential third-party contractors must pass a pre-approval inspection for compliance with the applicable regulations as a condition of regulatory approval of our product candidates or any of our other potential product candidates. In addition, the regulatory authorities may, at any time, audit or inspect a manufacturing facility involved with the preparation of our product candidates or the associated quality systems for compliance with the regulations applicable to the activities being conducted. Although we plan to oversee the contract manufacturers, we cannot control the manufacturing process of, and will be completely dependent on, our contract manufacturing partners for compliance with applicable regulatory requirements and to be able to manufacture our product according to specification. If these facilities do not pass a pre-approval plant inspection, regulatory approval of the product candidates may not be granted or may be substantially delayed until any violations are corrected to the satisfaction of the regulatory authority, if ever.

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

If we or any of our potential third-party manufacturers fail to maintain regulatory compliance, the FDA, the EMA or comparable foreign authorities can impose regulatory sanctions including, among other things, fines, injunctions, civil penalties, refusal to approve

a pending application for a product candidate, withdrawal of an approval, or suspension of production, seizures or recalls of products, or criminal prosecution. As a result, our business, financial condition and results of operations may be materially and adversely affected.

Additionally, if supply from one manufacturer is interrupted, an alternative manufacturer would need to be qualified through an NDA supplement or MAA variation, or equivalent foreign regulatory filing, which could result in further delay. The regulatory agencies may also require additional studies or trials if a new manufacturer is relied upon for commercial production. Switching manufacturers may involve substantial costs and is likely to result in a delay in our desired clinical and commercial timelines.

These factors could cause us to incur higher costs and could cause the delay or termination of clinical trials, regulatory submissions, required approvals, or commercialization of our product candidates. Furthermore, if our suppliers fail to meet contractual requirements and we are unable to secure one or more replacement suppliers capable of production at a substantially equivalent cost, our clinical trials may be delayed or we could lose potential revenue.

Current and future legislation may increase the difficulty and cost of commercializing our product candidates and may affect the prices we may obtain if our product candidates are approved for commercialization.

In the U.S. and some foreign jurisdictions, there have been a number of adopted and proposed legislative and regulatory changes regarding the healthcare system that could prevent or delay regulatory approval of our product candidates, restrict or regulate post-marketing activities and affect our ability to profitably sell any of our product candidates for which we obtain regulatory approval.

In the U.S., the Medicare Prescription Drug, Improvement, and Modernization Act of 2003 (“MMA”) changed the way Medicare covers and pays for pharmaceutical products. Cost reduction initiatives and other provisions of this legislation could limit the coverage and reimbursement rate that we receive for any of our approved product candidates. While the MMA only applies to drug benefits for Medicare beneficiaries, private payors often follow Medicare coverage policy and payment limitations in setting their own reimbursement rates. Therefore, any reduction in reimbursement that results from the MMA may result in a similar reduction in payments from private payors.

In March 2010, the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act of 2010 (collectively the “ACA”), was enacted. The ACA was intended to broaden access to health insurance, reduce or constrain the growth of healthcare spending, enhance remedies against healthcare fraud and abuse, add new transparency requirements for healthcare and health insurance industries, impose new taxes and fees on the health industry and impose additional health policy reforms. The ACA increased manufacturers’ rebate liability under the Medicaid Drug Rebate Program by increasing the minimum rebate amount for both branded and generic drugs and revised the definition of “average manufacturer price” (“AMP”), which may also increase the amount of Medicaid drug rebates manufacturers are required to pay to states. The legislation also expanded Medicaid drug rebates and created an alternative rebate formula for certain new formulations of certain existing products that is intended to increase the rebates due on those drugs. The Centers for Medicare & Medicaid Services, which administers the Medicaid Drug Rebate Program, also has proposed to expand Medicaid rebates to the utilization that occurs in the territories of the U.S., such as Puerto Rico and the Virgin Islands. Further, beginning in 2011, the ACA imposed a significant annual fee on companies that manufacture or import branded prescription drug products. Legislative and regulatory proposals have been introduced at both the state and federal level to expand post-approval requirements and restrict sales and promotional activities for pharmaceutical products.

We are not sure whether additional legislative changes will be enacted, or whether the FDA regulations, guidance or interpretations will be changed, or what the impact of such changes on the marketing approvals of our product candidates, if any, may be. In addition, increased scrutiny by the U.S. Congress of the FDA’s approval process may significantly delay or prevent marketing approval, as well as subject us to more stringent product labeling and post-marketing approval testing and other requirements.

Additionally, there has been heightened governmental scrutiny in the U.S. of pharmaceutical pricing practices in light of the rising cost of prescription drugs and biologics. Such scrutiny has resulted in several recent congressional inquiries and proposed and enacted 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 both the federal and 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. One significant example of recent legislative action is the Inflation Reduction Act of 2022 (the "IRA"), which has been considered a scaled-back version of the Build Back Better Act. The IRA was signed into law on August 16, 2022. While the IRA is still subject to rulemaking (with more information to come via guidance documents from the responsible federal agencies), the IRA, as written, will, among other changes, give HHS the ability and authority to directly negotiate with manufacturers the price that Medicare will pay for certain high-priced drugs. The IRA will also require manufacturers of certain Part B and Part D drugs to issue to HHS rebates based on certain calculations and triggers (i.e., when drug prices increase and outpace the rate of inflation). At this time, we cannot predict the implications the IRA provisions will have on our business.

We expect that these and other healthcare reform measures that may be adopted in the future may result in more rigorous coverage criteria and in additional downward pressure on the price that we receive for any approved drug. Any reduction in reimbursement from Medicare or other government programs may result in a similar reduction in payments from private payors. The implementation of cost containment measures or other healthcare reforms may prevent us from being able to generate revenue, attain profitability or commercialize our drugs.

In June 2016, the United Kingdom ("UK") held a referendum pursuant to which voters elected to leave the European Union ("EU"), commonly referred to as Brexit. The UK formally left the EU on January 31, 2020 and began a transition period that ended on December 31, 2020. Although the long-term effects of Brexit will depend on any agreements the UK makes to retain access to the EU markets, Brexit has created additional uncertainties that may ultimately result in new regulatory costs and challenges for biotechnology companies. We cannot predict what consequences the withdrawal of the United Kingdom from the European Union, if it occurs, might have on the regulatory frameworks of the United Kingdom or the European Union, or on our future operations, if any, in these jurisdictions.

Environmental, social and governance matters may impact our business and reputation.

Increasingly, in addition to the importance of their financial performance, companies are being judged by their performance on a variety of environmental, social and governance ("ESG") matters, which are considered to contribute to the long-term sustainability of companies' performance.

A variety of organizations measure the performance of companies on such ESG topics, and the results of these assessments are widely publicized. In addition, investment in funds that specialize in companies that perform well in such assessments are increasingly popular, and major institutional investors have publicly emphasized the importance of such ESG measures to their investment decisions. Topics taken into account in such assessments include, among others, the company's efforts and impacts on climate change and human rights, ethics and compliance with law, and the role of the company's board of directors in supervising various sustainability issues. In addition to the topics typically considered in such assessments, in the healthcare industry, issues of the public's ability to access our medicines are of particular importance.

In light of investors' increased focus on ESG matters, there can be no certainty that we will manage such issues successfully, or that we will successfully meet society's expectations as to our proper role. Any failure or perceived failure by us in this regard could have a material adverse effect on our reputation and on our business, share price, financial condition, or results of operations, including the sustainability of our business over time.

Changes in government funding for the FDA and other government agencies could hinder their ability to hire and retain key leadership and other personnel, properly administer drug innovation, or prevent our product candidates from being developed or commercialized, which could negatively impact our business, financial condition and results of operations.

The ability of the FDA to review and approve new products can be affected by a variety of factors, including budget and funding levels, ability to hire and retain key personnel, and statutory, regulatory, policy changes, and pandemic conditions, such as COVID-19. In addition, government funding of other agencies that fund research and development activities is subject to the political process, which is inherently fluid and unpredictable.

In December 2016, the 21st Century Cures Act was signed into law. This new legislation is designed to advance medical innovation and empower the FDA with the authority to directly hire positions related to drug and device development and review. However, government proposals to reduce or eliminate budgetary deficits may include reduced allocations to the FDA and other related government agencies. These budgetary pressures may result in a reduced ability by the FDA to perform their respective roles;

including the related impact to academic institutions and research laboratories whose funding is fully or partially dependent on both the level and timing of funding from government sources.

Disruptions at the FDA and other agencies may also slow the time necessary for our product candidates to be reviewed or approved by necessary government agencies, which could adversely affect our business, financial condition and results of operations.

We are subject to "fraud and abuse" and similar laws and regulations, and a failure to comply with such regulations or prevail in any litigation related to noncompliance could harm our business, financial condition and results of operations.

We are subject to various federal and state healthcare "fraud and abuse" laws, including anti-kickback laws, false claims laws and other laws intended, among other things, to reduce fraud and abuse in federal and state healthcare programs. The federal Anti-Kickback Statute makes it illegal for any person, including a prescription drug manufacturer, or a party acting on its behalf, to knowingly and willfully solicit, receive, offer or pay any remuneration that is intended to induce the referral of business, including the purchase, order or prescription of a particular drug, or other good or service for which payment in whole or in part may be made under a federal healthcare program, such as Medicare or Medicaid. Although we seek to structure our business arrangements in compliance with all applicable requirements and safe harbors, these laws are broadly written, and it is often difficult to determine precisely how the law will be applied in specific circumstances. Accordingly, it is possible that our practices may be challenged under the federal Anti-Kickback Statute.

The federal False Claims Act prohibits anyone from, among other things, knowingly presenting or causing to be presented for payment to the government, including the federal healthcare programs, claims for reimbursed drugs or services that are false or fraudulent, claims for items or services that were not provided as claimed, or claims for medically unnecessary items or services. In addition, private individuals (whistleblowers)have the ability to bring actions on behalf of the government under the federal False Claims Act (i.e., qui tam actions) as well as under the false claims laws of several states.

Under the federal criminal statute on false statements (related to health care matters), we are prohibited from knowingly and willfully executing a scheme to defraud any healthcare benefit program, including private payors, or knowingly and willfully falsifying, concealing or covering up a material fact or making any materially false, fictitious or fraudulent statement in connection with the delivery of or payment for healthcare benefits, items or services to obtain money or property of any healthcare benefit program.

Many states have adopted laws similar to the federal Anti-Kickback Statute, some of which apply to the referral of patients for healthcare services reimbursed by any source, not just governmental payors. In addition, some states have passed laws that require pharmaceutical companies to comply with Pharmaceutical Research and Manufacturers of America's Code on Interactions with Healthcare Professionals. Several states also impose other marketing restrictions or require pharmaceutical companies to make marketing or price disclosures to the state. There are ambiguities as to what is required to comply with these state requirements and if we fail to comply with an applicable state law requirement, we could be subject to penalties.

Other related federal and state laws and regulations that may impact our operations include, without limitation, the federal False Statements Statue, the federal Civil Monetary Penalties Law, the federal Physician Payments Sunshine Act, and state and local disclosure requirements and marketing restrictions.

Violations of fraud and abuse laws may be punishable by criminal or civil sanctions, including penalties, fines or exclusion or suspension from federal and state healthcare programs such as Medicare and Medicaid and debarment from contracting with the U.S. Government.

While the government has issued certain material on the application of fraud and abuse laws to our business, the guidance is generally limited and not always directly applicable. Law enforcement authorities are increasingly focused on enforcing these laws, issuing new mandates and goals, and it is possible that some of our practices may be challenged under these laws. Efforts to ensure that our business arrangements and arrangements with third parties will comply with applicable healthcare laws and regulations will involve substantial costs. If we, or any agents acting on our behalf, are found in violation of one of these laws, we could be subject to significant civil, criminal and administrative penalties, damages, fines, exclusion from governmental funded federal or state healthcare programs and the curtailment or restructuring of our operations. If this occurs, our business, financial condition and results of operations may be materially adversely affected.

If we face allegations of noncompliance with the law and encounter sanctions, our reputation, revenues and liquidity may suffer, and any of our product candidates that are ultimately approved for commercialization could be subject to restrictions or withdrawal from the market.

Any government investigation of alleged violations of law could require us to expend significant time and resources in response, and could generate negative publicity. Any failure to comply with ongoing regulatory requirements may significantly and adversely affect our ability to generate revenues from any of our product candidates that are ultimately approved for commercialization. If regulatory sanctions are applied or if regulatory approval is withdrawn, our business, financial condition and results of operations will be adversely affected. Additionally, if we are unable to generate revenues from product sales, our potential for achieving profitability will be diminished and our need to raise capital to fund our operations will increase.

Risks Related to Our Common Stock

The market price of our common stock is expected to be volatile

The trading price of our stock is likely to be volatile. Our stock price could be subject to wide fluctuations in response to a variety of factors, including the following:

- our ability to conduct and achieve continued positive outcomes from our preclinical activities on the PATrOL™ platform and disease specific programs;
- public health crises, pandemics and epidemics, such as a novel strain of coronavirus (COVID-19) and their effects on our preclinical activities;
- results from, costs, and any delays in, anticipated preclinical and clinical studies;
- contracting with third parties such as academic institutions, and various CROs who will perform such studies, or the potential lack of performance of such organizations;
- acceptance of INDs by the FDA or similar regulatory filing by comparable foreign regulatory authorities for the conduct of clinical trials of our product candidates and our proposed design of future clinical trials;
- delays in publications of research findings;
- significant lawsuits, including patent or stockholder litigation;
- inability to obtain additional funding or funding on favorable terms;
- failure to successfully develop and commercialize our product candidates;
- changes in laws or regulations applicable to our product candidates;
- inability to obtain adequate product supply for our product candidates, or the inability to do so at acceptable prices or in an acceptable timeframe;
- unanticipated serious safety concerns related to our PATrOL™ platform or any of our product candidates;
- adverse regulatory decisions;
- introduction of new products or technologies by our competitors;
- adverse events or results for our competitors or our product candidate target areas that could generally adversely affect us or our industry;

- failure to meet or exceed drug development or financial projections we provide to the public;
- failure to meet or exceed the estimates, expectations and projections of the investment community and our stockholders;
- the perception of the pharmaceutical industry by the public, legislatures, regulators and the investment community;
- announcements of significant acquisitions, strategic partnerships, joint ventures or capital commitments by us or our competitors;
- disputes or other developments relating to proprietary rights, including patents, litigation matters and our ability to obtain patent protection for our licensed and owned technologies;
- additions or departures of key scientific or management personnel;
- changes in the market valuations of similar companies;
- general economic and market conditions and overall fluctuations in the U.S. equity market;
- sales of our common stock by us or our stockholders in the future;
- trading volume of our common stock;
- period-to-period fluctuations in our financial results;
- any identified material weakness in our internal control over financial reporting;
- changes in the structure of health care payments;
- changes in the Nasdaq listing of our stock; and
- recommendations of equity analysts covering our stock.

In addition, the stock market, and equity values of small pharmaceutical companies in particular, have experienced extreme price and volume fluctuations that have often been unrelated or disproportionate to the operating performance of these companies. Broad market and industry factors, including supply chain disruptions and inflationary impacts, may negatively affect the market price of our common stock, regardless of our actual operating performance. Further, a decline in the financial markets and related factors beyond our control may cause our stock price to decline rapidly and unexpectedly.

In the past, following periods of volatility in the market price of a company's securities, stockholders have often instituted class action securities litigation against those companies. Such litigation, if instituted, could result in substantial costs and diversion of management attention and resources, which could significantly harm our profitability and reputation.

If we are required to restate any of our financial statements in the future due to our inability to adequately remedy the issues that gave rise to these modifications or for any other reason, we may be subject to regulatory penalties and investors could lose confidence in the accuracy and completeness of our financial statements, which could cause our share price to decline.

Our common stock is subject to trading risks created by the influence of third party investor websites.

Our common stock is widely traded and held by retail investors, and these investors are subject to the influence of information provided by third party investor websites and independent authors distributing information on the internet. This information has become influential because it is widely distributed and links to it appear as top company headlines on commonly used stock quote and finance websites, or through services such as Google alerts. These emerging information distribution models are a consequence of the emergence of the internet. Some information and content distribution is by individuals through platforms that mainly serve as hosts

seeking advertising revenue. As such, we believe an incentive exists for these sites to increase advertising revenue by increasing page views, and for them to post or allow to be posted inflammatory information to achieve this end. It has been our experience that a significant portion of the information on these websites or distributed by independent authors about our company is false or misleading, and occasionally, we believe, purposefully misleading. These sites and internet distribution strategies also create opportunity for individuals to pursue both "pump and dump" and "short and distort" strategies. We believe that many of these websites have little or no requirements for authors to have professional qualifications. While these sites sometimes require disclosure of stock positions by authors, as far as we are aware these sites do not audit the accuracy of such conflict of interest disclosures. We believe that many of these websites have few or lax editorial standards, and thin or non-existent editorial staffs. Despite our best efforts, we have not and may not be able in the future to obtain corrections to information provided on these websites about our company, including both positive and negative information, and any corrections that are obtained may not be achieved prior to the majority of audience impressions being formed for a given article. These conditions create volatility and risk for holders of our common stock and should be considered by investors. We can make no guarantees that regulatory authorities will take action on these types of activities, and we cannot guarantee that legislators will act responsively, or ever act at all, to appropriately restrict the activities of these websites and authors.

Our management owns a significant percentage of our stock and is able to exert significant control over matters subject to stockholder approval.

Dr. Stephan, our Chief Executive Officer and a director of us, holds a significant number of shares of our outstanding common stock and an option to purchase additional shares of common stock. Accordingly, Dr. Stephan has the ability to influence us through his ownership position.

This significant concentration of stock ownership may adversely affect the trading price for our common stock because investors often perceive disadvantages in owning stock in companies with controlling stockholders. As a result, Dr. Stephan could significantly influence all matters requiring approval by our stockholders, including the election of directors and the approval of mergers or other business combination transactions. Dr. Stephan may be able to determine all matters requiring stockholder approval. The interests of these stockholders may not always coincide with our interests or the interests of other stockholders. This may also prevent or discourage unsolicited acquisition proposals or offers for our common stock that you may feel are in your best interests as one of our stockholders, and he may act in a manner that advances his best interests and not necessarily those of other stockholders, including seeking a premium value for his common stock, and might affect the prevailing market price for our common stock.

We previously identified a material weakness in our internal control over financial reporting, which has been remediated. If we identify additional material weaknesses in the future or otherwise fail to maintain an effective system of internal controls, we may not be able to accurately or timely report our financial condition or results of operations, which may adversely affect our business and stock price.

A material weakness is a deficiency, or a combination of deficiencies, in internal control over financial reporting, such that there is a reasonable possibility that a material misstatement of our annual or interim financial statements will not be prevented or detected in a timely basis.

We cannot assure you that the measures we have taken to date, and actions we may take in the future, will be sufficient to prevent or avoid potential future material weaknesses. In connection with the implementation of the necessary procedures and practices related to internal control over financial reporting, we may identify material weaknesses that we may not be able to remediate in time to meet the deadline imposed by the Sarbanes-Oxley Act for compliance with the requirements of Section 404. In addition, we may encounter problems or delays in completing the implementation of any improvements and receiving a favorable attestation by our independent registered public accounting firm, if and when required.

If we are unable to achieve and maintain an effective internal control environment in our disclosure controls or internal control over financial reporting, the accuracy and timing of our financial reporting may be adversely affected; our liquidity, our access to capital markets and our ability to complete acquisitions may be adversely affected; we may be unable to maintain or regain compliance with applicable securities laws, The Nasdaq Stock Market LLC ("Nasdaq") listing requirements, and the covenants under certain agreements regarding the timely filing of periodic reports; we may be subject to regulatory investigations and penalties; investors may lose confidence in our financial reporting; and our stock price may decline.

If we identify errors in our financial reporting in the future that require us to restate previously issued financial statements, such restatements may subject us to unanticipated costs or regulatory penalties and could cause investors to lose confidence in the accuracy and completeness of our financial statements, which could cause the price of our common stock to decline.

In the past we have had to restate previously issued financial statements. If we have to do so again, we may be subject to unanticipated costs and regulatory penalties and investors could lose confidence in the accuracy and completeness of our financial statements, which could cause our share price to decline, due to such restatement and if we are required to restate any of our other financial statements in the future.

Although we are currently eligible to file new short form registration statements on Form S-3, we cannot guarantee we will remain eligible to do so. If we were to lose such eligibility, it may impair our ability to raise capital on terms favorable to us, in a timely manner or at all.

Form S-3 permits eligible issuers to conduct registered offerings using a short form registration statement that allows the issuer to incorporate by reference its past and future filings and reports made under the Exchange Act. In addition, Form S-3 enables eligible issuers to conduct primary offerings "off the shelf" under Rule 415 of the Securities Act of 1933, as amended (the "Securities Act"). The shelf registration process, combined with the ability to forward incorporate information, allows issuers to avoid delays and interruptions in the offering process and to access the capital markets in a more expeditious and efficient manner than raising capital in a standard registered offering pursuant to a registration statement on Form S-1. The ability to newly register securities for resale may also be limited as a result of the loss of Form S-3 eligibility with respect to such registrations.

SEC regulations limit the amount of funds we may raise during any 12-month period pursuant to our shelf registration statement on Form S-3

Our public float was less than $75 million as of the date of filing of this Annual Report on Form 10-K. As a result, under General Instruction I.B.6 to Form S-3, the amount of funds we can raise through primary public offerings of securities, in any 12-month period using our registration statement on Form S-3 is limited to one-third of the aggregate market value of the shares of our common stock held by our non-affiliates. We are subject to this limitation until such time as our public float exceeds $75 million. If we are required to file a new registration statement on another form, we may incur additional costs and be subject to delays due to review by the SEC.

We may take advantage of specified reduced disclosure requirements applicable to a "smaller reporting company" under Regulation S-K, and the information that we provide to stockholders may be different than they might receive from other public companies.

We are a "smaller reporting company," as defined under Regulation S-K. As a smaller reporting company, we may take advantage of specified reduced disclosure and other requirements that are otherwise applicable generally to public companies. These provisions include, among other things, scaled disclosure requirements, including about our executive compensation arrangements.

We intend to continue to take advantage of certain of the scaled disclosure requirements of smaller reporting companies. We may continue to take advantage of these allowances until we are no longer a smaller reporting company. We will cease to be a smaller reporting company if we have (i) more than $250 million in market value of our shares held by non-affiliates as of the last business day of our second fiscal quarter or (ii) more than $100 million of annual revenues in our most recent fiscal year completed before the last business day of our second fiscal quarter and a market value of our shares held by non-affiliates more than $700 million as of the last business day of our second fiscal quarter. We may choose to take advantage of some but not all of these scaled disclosure requirements. Therefore, the information that we provide stockholders may be different than one might get from other public companies. Further, if some investors find our shares of common stock less attractive as a result, there may be a less active trading market for our shares of common stock and the market price of such shares of common stock may be more volatile.

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

The trading market for our common stock depends, in part, on the research and reports that securities or industry analysts publish about us or our business. If one or more of the analysts who cover us downgrade our stock or publish inaccurate or unfavorable research about our business, our stock price would likely decline. In addition, if our operating results fail to meet the forecast of

analysts, our stock price would likely decline. If one or more of these analysts cease coverage of us or fail to publish reports on us regularly, demand for our common stock could decrease, which might cause our stock price and trading volume to decline.

Sales of a substantial number of shares of our common stock in the public market by our stockholders, future issuances of our common stock or rights to purchase our common stock, could cause our stock price to fall.

Sales of a substantial number of shares of our common stock by our existing stockholders in the public market, or the perception that these sales might occur, could depress the market price of our common stock and could impair our ability to raise capital through the sale of additional equity securities. We are unable to predict the effect that such sales may have on the prevailing market price of our common stock.

Our amended and restated certificate of incorporation provides that the Court of Chancery of the State of Delaware and the federal district courts of the United States are the exclusive forums for substantially all disputes between us and our stockholders other than actions arising under the Securities Act of 1933, as amended, or the Securities Exchange Act of 1934, as amended, which could limit our stockholders' ability to obtain a favorable judicial forum for disputes with us or our directors, officers, or employees.

Our amended and restated certificate of incorporation provides that the Court of Chancery of the State of Delaware is the exclusive forum for:

- any derivative action or proceeding brought on our behalf;
- any action asserting a breach of fiduciary duty;
- any action asserting a claim against us arising under the General Corporation Law of the State of Delaware (the "DGCL"), our amended and restated certificate of incorporation or our amended and restated bylaws; and
- any action asserting a claim against us that is governed by the internal-affairs doctrine.

These exclusive-forum provisions do not apply to claims under the Securities Act, the Exchange Act or any other claims for which the federal courts have exclusive jurisdiction.

These exclusive forum provisions may limit a stockholder'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. If a court were to find either exclusive forum provision in our amended and restated certificate of incorporation to be inapplicable or unenforceable in an action, it may incur additional costs associated with resolving the dispute in other jurisdictions, which could harm our business.

Anti-takeover provisions in our charter documents and under Delaware law could make an acquisition of the Company more difficult and may prevent attempts by our stockholders to replace or remove our management.

Provisions in our amended and restated certificate of incorporation and amended and restated bylaws may significantly reduce the value of shares of our common stock to a potential acquirer or delay or prevent an acquisition or a change in management without the consent of our board of directors. The provisions in our amended and restated certificate of incorporation and amended and restated bylaws include the following:

- a classified board of directors with three-year staggered terms, which may delay the ability of stockholders to change the membership of a majority of our board of directors;
- no cumulative voting in the election of directors, which limits the ability of minority stockholders to elect director candidates;

- the exclusive rights of our board of directors to establish the authorized number of directors and to elect a director to fill a vacancy created by the expansion of our board of directors or the death, resignation, disqualification, retirement or removal of a director, which prevents stockholders from being able to fill vacancies on our board of directors;

- a provision that directors may be removed by our stockholders only for cause;

- the ability of our board of directors to authorize the issuance of shares of preferred stock and to determine the price and other terms of those shares, including preferences and voting rights, without stockholder approval, which could be used to significantly dilute the ownership of a hostile acquirer;

- the ability of our board of directors to make, alter or appeal our amended and restated bylaws without obtaining stockholder approval;

- the affirmative vote of the holders of at least 66 2/3% of the voting power of all of the then-outstanding shares of our capital stock entitled to vote generally in the election of directors is required to amend, alter, repeal or adopt any provision inconsistent with, several of the provisions of our amended and restated certificate of incorporation and amended and restated bylaws;

- a prohibition on stockholder action by written consent, which forces stockholder action to be taken at an annual or special meeting of our stockholders;

- the requirement that a special meeting of stockholders may be called only by our board of directors, chief executive officer or president, which may delay the ability of our stockholders to force consideration of a proposal or to take action, including the removal of directors;

- a restriction on the forum for certain litigation against us to Delaware; and

- advance notice procedures that stockholders must comply with in order to nominate candidates to our board of directors or to propose matters to be acted upon at a stockholders' meeting, which may discourage or deter a potential acquirer from conducting a solicitation of proxies to elect the acquirer's own slate of directors or otherwise attempting to obtain control of us.

Although we believe these provisions collectively will provide for an opportunity to receive higher bids by requiring potential acquirers to negotiate with our board of directors, they would apply even if the offer may be considered beneficial by some stockholders. In addition, these provisions may frustrate or prevent any attempts by our stockholders to replace or remove then current management by making it more difficult for stockholders to replace members of the board of directors, which is responsible for appointing the members of management.

Certain provisions of the DGCL deter hostile takeovers. Specifically, Section 203 of the DGCL prohibits a Delaware corporation from engaging in a business combination with an "interested stockholder" for a period of three years following the date the person first became an interested stockholder, unless (with certain exceptions) the business combination or the transaction by which the person became an interested stockholder is approved in a prescribed manner. Generally, a "business combination" includes a merger, asset or stock sale, or certain other transactions resulting in a financial benefit to the interested stockholder. Generally, an "interested stockholder" is a person who, together with affiliates and associates, beneficially owns or within three years prior to becoming an "interested stockholder" did own, 15% or more of a corporation's outstanding voting stock. While this statute permits a corporation to opt out of these protective provisions in its certificate of incorporation, our certificate of incorporation does not include any such opt-out provision.

Claims for indemnification by our directors and officers may reduce our available funds to satisfy successful third-party claims against us and may reduce the amount of money available to us.

Our amended and restated certificate of incorporation provides that we will indemnify our directors and officers, in each case to the fullest extent permitted by Delaware law.

In addition, as permitted by Section 145 of the General Corporation Law of the State of Delaware, or the DGCL, our amended and restated certificate of incorporation and our indemnification agreements that we have entered into with our directors and officers provide that:

- We will indemnify our directors and officers for serving us in those capacities or for serving other business enterprises at our request, to the fullest extent permitted by Delaware law. Delaware law provides that a corporation may indemnify such person if such person acted in good faith and in a manner such person reasonably believed to be in or not opposed to the best interests of the registrant and, with respect to any criminal proceeding, had no reasonable cause to believe such person's conduct was unlawful.

- We may, in our discretion, indemnify employees and agents in those circumstances where indemnification is permitted by applicable law.

- We are required to advance expenses actually and reasonably incurred by our directors and officers in connection with any proceeding, except that such directors or officers shall undertake to repay such advances if it is ultimately determined by a court of competent jurisdiction that such person is not entitled to indemnification.

- We will not be obligated pursuant to our amended and restated certificate of incorporation to indemnify a person with respect to proceedings initiated by that person against us or our other indemnitees, except with respect to proceedings authorized by our board of directors or brought to enforce a right to indemnification.

- The rights to indemnification conferred in our amended and restated certificate of incorporation are not exclusive, and we are authorized to enter into indemnification agreements with our directors, officers, employees and agents and to obtain insurance to indemnify such persons.

- We may not retroactively amend our amended and restated certificate of incorporation provisions to reduce our indemnification obligations to current or former directors or officers.

Our indemnification obligations could result in substantial expenditures by us, which we will be unable to recover.

Our pre-Merger net operating loss carryforwards and certain other tax attributes will likely be subject to limitations. The pre-Merger net operating loss carryforwards and certain other tax attributes of us may also be subject to limitations as a result of ownership changes resulting from the Merger.

In general, a corporation that undergoes an "ownership change," as defined in Section 382 of the Internal Revenue Code of 1986, as amended, is subject to limitations on its ability to utilize its pre-change net operating losses ("NOLs") to offset future taxable income (the "Section 382 Limitation"). Such an ownership change occurs if the aggregate stock ownership of certain stockholders, generally stockholders beneficially owning five percent or more of a corporation's common stock, applying certain look-through and aggregation rules, increases by more than 50 percentage points over such stockholders' lowest percentage ownership during the testing period, generally three years. Due to the ownership change of the Company upon completion of the Merger, our NOLs and certain other tax attributes will be subject to the Section 382 Limitation. Consequently, even if we achieve profitability, we may not be able to utilize a material portion of our NOLs and certain other tax attributes because of the Section 382 Limitation, which could have a material adverse effect on cash flow and results of operations. As of September 30, 2022, we estimated that we had approximately $77.1 million in NOL carryforwards. We have not completed an analysis regarding the limitation of net operating loss carryforwards; however, it is likely that the Section 382 Limitation will cause a significant portion of our NOL carryforwards to never be utilized. In addition, if we are determined to have discontinued our historic business following the completion of the Merger, subject to certain exceptions, the Section 382 Limitation could eliminate all possibility of utilizing our NOL carryforwards.

We may never pay dividends on our common stock so any returns would be limited to the appreciation of our stock.

We currently anticipate that we will retain future earnings for the development, operation and expansion of our business and do not anticipate we will declare or pay any cash dividends for the foreseeable future. In addition, the terms of any future debt agreements may preclude us from paying dividends. Any return to stockholders will therefore be limited to the appreciation of their stock.

Our failure to meet the continued listing standards on The Nasdaq Capital Market could result in a delisting of our common stock, which could negatively impact the price of our common stock, liquidity and our ability to access the capital markets.

The listing standards of The Nasdaq Capital Market provide that a company, in order to qualify for continued listing, must maintain a minimum stock price of $1.00 and satisfy standards relative to minimum stockholders' equity, minimum market value of publicly held shares and various additional requirements. If we fail to comply with all listing standards applicable to issuers listed on The Nasdaq Capital Market, our common stock may be delisted.

As previously disclosed on October 3, 2022, on September 29, 2022 we received a letter (the "Notice") from Nasdaq, notifying us that, because the closing bid price for the Company's common stock has been below $1.00 per share for 30 consecutive business days, the Company no longer complies with the minimum bid price requirement for continued listing on The Nasdaq Capital Market. Nasdaq Listing Rule 5550(a)(2) requires listed securities to maintain a minimum bid price of $1.00 per share (the "Minimum Bid Price Requirement"), and Nasdaq Listing Rule 5810(c)(3)(A) provides that a failure to meet the Minimum Bid Price Requirement exists if the deficiency continues for a period of 30 consecutive business days.

The Notice has no immediate effect on the listing of the Company's common stock on The Nasdaq Capital Market. Pursuant to Nasdaq Listing Rule 5810(c)(3)(A), the Company has been provided an initial compliance period of 180 calendar days, or until March 28, 2023, to regain compliance with the Minimum Bid Price Requirement. During the compliance period, the Company's shares of common stock will continue to be listed and traded on The Nasdaq Capital Market. To regain compliance, the closing bid price of the Company's common stock must meet or exceed $1.00 per share for a minimum of ten consecutive business days during the 180 calendar day grace period.

In the event the Company is not in compliance with the Minimum Bid Price Requirement by March 28, 2023, the Company may be afforded a second 180 calendar day grace period. To qualify, the Company would be required to meet the continued listing requirements for market value of publicly held shares and all other initial listing standards for The Nasdaq Capital Market, with the exception of the Minimum Bid Price Requirement. In addition, the Company would be required to provide written notice of its intention to cure the minimum bid price deficiency during this second 180-day compliance period by effecting a reverse stock split, if necessary.

We intend to actively monitor the bid price for our common stock between now and March 28, 2023 and will consider available options to regain compliance with the Minimum Bid Price Requirement. There can be no assurance that we will be able to regain compliance with the Minimum Bid Price Requirement or that we will otherwise be in compliance with the other listing standards for The Nasdaq Capital Market. If our common stock is delisted, it could reduce the price of our common stock and the levels of liquidity available to our stockholders. In addition, the delisting of our common stock could materially adversely affect our access to the capital markets, and any limitation on liquidity or reduction in the price of our common stock could materially adversely affect our ability to raise capital. Delisting from The Nasdaq Capital Market could also result in other negative consequences, including the potential loss of confidence by suppliers, customers and employees, the loss of institutional investor interest and fewer business development opportunities.

Effecting a reverse stock split, if determined by the Company's board of directors in its discretion, may not achieve one or more of our objectives.

On September 8, 2022, the Company held its 2022 annual meeting of stockholders, pursuant to which the stockholders of the Company voted in favor of an amendment to the Company's amended and restated certificate of incorporation to effect, at the discretion of the Company's board of directors at any time prior to the Company's 2023 annual meeting of stockholders, a reverse stock split of the Company's common stock, whereby each outstanding 5, 6, 7, 8, 9, 10, 11, 12, 13, 14, 15, 16, 17, 18, 19 or 20 shares would be combined, converted or changed into one share of the Company's common stock. There can be no assurance that the market price per share of our common stock after a reverse stock split would remain unchanged or increase in proportion to the reduction in the number of shares of our common stock outstanding before such reverse stock split. The market price of our shares may fluctuate and potentially decline after a reverse stock split. Accordingly, the total market capitalization of our common stock after a reverse stock split may be lower than the total market capitalization before such reverse stock split. Moreover, the market price of our common stock following a reverse stock split may not exceed or remain higher than the market price prior to the reverse stock split.

Additionally, there can be no assurance that a reverse stock split would result in a per-share market price that would attract brokers and investors who do not trade in lower priced stock or that it would increase the Company's ability to attract and retain employees and other service providers. As a result, the trading liquidity of our common stock may not necessarily improve. Further, if a reverse stock split is effected and the market price of our common stock declines, the percentage decline may be greater than would occur in the absence of a reverse stock split.

ITEM 1B. UNRESOLVED STAFF COMMENTS

None.

ITEM 2. PROPERTIES

In October 2020, we entered into a ten-year operating lease agreement with annual escalating rental payments for approximately 14,189 square feet of office and laboratory space in Pittsburgh, PA. The leased premises serves as our headquarters. The first and second amendments to the lease agreement were executed in December 2020 and April 2021, respectively (collectively with the lease agreement, the "Lease"). In November 2020, we prepaid rent of $0.3 million and paid a security deposit of $0.3 million for the Lease. The Lease commenced on May 1, 2021, and we were obligated to begin making rental payments on such date. We continued to apply the prepaid amount toward the rental payments through December 2021. We have the right to extend the term of the Lease for an additional five-year term.

We continued to operate under our prior operating lease in Pittsburgh, PA until we moved into our new headquarters and laboratory space, which occurred in June 2021. Our prior office and operating space was leased under operating leases with original terms of less than 12 months that expired at various dates through November 2021; therefore, our previous operating leases are not recognized as right-of-use assets on the consolidated balance sheet as of September 30, 2021. The Company also maintained a short-term rental of office space in San Diego, CA and New York, NY, which expired in November 2021. In October 2021, we commenced a one-year lease for rental office space in Boston, MA, which was renewed and extends through April 2023.

In February 2022, we entered into an eighteen-month lease agreement for private and shared laboratory facilities for general laboratory and office space in Boston, MA. We paid monthly rent payments of approximately $9,800 over the term of the lease. The lease agreement was terminated effective October 2022.

ITEM 3. LEGAL PROCEEDINGS

We may become involved in certain legal proceedings and claims, which arise in the normal course of business. If an unfavorable ruling were to occur, there exists the possibility of a material adverse impact on our results of operations, prospects, cash flows, financial position and brand.

ITEM 4. MINE SAFETY DISCLOSURES

Not applicable.

PART II.

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

Our common stock is traded on The Nasdaq Capital Market under the symbol "NBSE." Before July 15, 2019, our common stock traded under the ticker symbol "OHRP". The daily market activity and closing prices of our common stock can be found at www.nasdaq.com.

On December 16, 2022, the last reported sales price for our common stock on The Nasdaq Capital Market was $0.19 per share, and we had 174 holders of record of our common stock. One of our shareholders is Cede & Co., a nominee for Depository Trust Company ("DTC"). Shares of common stock that are held by financial institutions as nominees for beneficial owners are deposited into participant accounts at DTC and are considered to be held of record by Cede & Co. as one stockholder.

Unregistered Sales of Equity Securities and Use of Proceeds

None.

ITEM 6. [Reserved].

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

Disclosures Regarding Forward-Looking Statements

This Form 10-K includes "forward-looking statements" within the meaning of Section 21E of the Exchange Act. Those statements include statements regarding the intent, belief or current expectations of the Company and its subsidiaries and our management team. Any such forward-looking statements are not guarantees of future performance and involve risks and uncertainties, and actual results may differ materially from those projected in the forward-looking statements. These risks and uncertainties include but are not limited to those risks and uncertainties set forth in Item 1A. of this Form 10-K. In light of the significant risks and uncertainties inherent in the forward-looking statements included in this Form 10-K, the inclusion of such statements should not be regarded as a representation by us or any other person that our objectives and plans will be achieved. Further, these forward-looking statements reflect our view only as of the date of this Form 10-K. Except as required by law, we undertake no obligations to update any forward-looking statements and we disclaim any intent to update forward-looking statements after the date of this Form 10-K to reflect subsequent developments. Accordingly, you should also carefully consider the factors set forth in other reports or documents that we file from time to time with the SEC.

Overview

We have designed, built, and validated a new technology platform (a peptide-nucleic acid antisense oligonucleobase platform, which we call PATrOL™) that can uniquely Drug the Genome™ to address the three disease-causing mechanisms (i.e., gain-of-function, change-of-function, or loss-of-function of a gene), without the limitations of early precision genetic medicines. The technology is predicated on synthetic peptide-nucleic acid ("PNA") chemistry and can directly engage the genome in a sequence-specific manner and potentially address root causality of diseases. These compounds operate by temporarily engaging the genome (or single and double-stranded RNA targets, if desired) and interacting with cellular machinery that processes mutant genes to halt their ability to manifest a disease.

We have repeatedly demonstrated in proof-of-concept preclinical animal studies the ability to address multiple disease-causing genes, and different causal mechanisms, to resolve the disease state without the limitations of early genetic medicine technologies. As further validation of our PATrOL™ platform's capabilities, in FY2021 and FY2022 we described data illustrating that our first-in-class platform technology can address various types of causal insults by Drugging the Genome™ in animal models of a variety of human diseases after patient-friendly routes of administration and does so in a well-tolerated manner.

We are developing precision genetic medicines targeting rare, monogenic diseases for which there are no approved therapies, as well as more common genetic disorders, including cancers that are resistant to current therapeutic approaches. Our disclosed pipeline includes therapeutic candidates for the treatment of DM1, HD, as well as cancer-driving point mutations in KRAS, G12V and G12D, which are involved in many tumor types and have historically been "undruggable". In October 2022, the Company announced plans to expand its focus to include the advancement of the differentiated gene editing capabilities of its platform. The Company is currently identifying and evaluating multiple indications for potential future development.

We were incorporated under the laws of the State of Delaware on August 4, 2009, as successor to BBM Holdings, Inc. (formerly known as Prime Resource, Inc., which was organized March 29, 2002 as a Utah corporation) pursuant to a reincorporation merger. On August 4, 2009, we reincorporated in Delaware as "Ohr Pharmaceutical, Inc." On July 12, 2019, we completed the merger with NeuBase Corporation (formerly known as NeuBaseTherapeutics, Inc.), a Delaware corporation (the "Merger"), and, upon completion of the Merger, we changed our name to "NeuBase Therapeutics, Inc." Since the Merger, we have focused primarily on the development of our proprietary peptide-nucleic acid antisense oligo platform and preclinical-stage therapeutic candidates. Our platform technology and all our therapeutic candidates are in the preclinical development stage. We have not initiated clinical trials for any of our product candidates, nor have any products been approved for commercial sale, and we have not generated any revenue. To date, we have not completed a clinical trial (including a pivotal clinical trial), obtained marketing approval for any product candidates, manufactured a commercial scale product or arranged for a third party to do so on our behalf, or conducted sales and marketing activities necessary for successful product commercialization. Drug development is also a highly uncertain undertaking and involves a substantial degree of risk. As a result, we have no meaningful historical operations upon which to evaluate our business and prospects and have not yet demonstrated an ability to obtain marketing approval for any of our product candidates or successfully overcome the risks and uncertainties frequently encountered by companies in the pharmaceutical industry. We also have not generated any revenues from collaboration and licensing agreements or product sales to date and continue to incur research and development and other expenses. Our prior losses, combined with expected future losses, have had and will continue to have an adverse effect on our stockholders' equity and working capital, and our future success is subject to significant uncertainty.

For the foreseeable future, we expect to continue to incur operating losses, which we expect will increase significantly from recent historical levels as we advance our gene editing platform, expand our drug development activities, seek regulatory approvals for our product candidates and begin to commercialize them if they are approved by the U.S. Food and Drug Administration (the "FDA"), the European Medicines Agency (the "EMA") or comparable foreign authorities. Even if we succeed in developing and commercializing one or more product candidates, we may never become profitable.

We expect to expend substantial funds in research and development, including preclinical studies and clinical trials for our platform technology and product candidates, and to manufacture and market any product candidates in the event they are approved for commercial sale. We will likely need additional funding to develop or acquire complementary companies, technologies, and assets, as well as for working capital requirements and other operating and general corporate purposes. Moreover, an increase in our headcount would dramatically increase our costs in the near and long-term.

Such spending may not yield any commercially viable products. Due to our limited financial and managerial resources, we must focus on a limited number of research programs and product candidates and on specific indications. Our resource allocation decisions may cause us to fail to capitalize on viable commercial products or profitable market opportunities.

Because the successful development of our product candidates is uncertain, we are unable to precisely estimate the actual funds we will require to develop and potentially commercialize them. In addition, we may not be able to generate sufficient revenue, even if we are able to commercialize any of our product candidates, to become profitable.

The Company expects to incur substantial operating losses and negative cash flows from operations for the foreseeable future. We will need to seek additional equity or debt financing to provide the capital required to maintain or expand our operations.

In particular, we expect that we will need to obtain additional funding to obtain clinical data from our current pipeline programs. We have based these estimates on assumptions that may prove to be wrong, and we could use our capital resources sooner than we currently expect. Our operating plans and other demands on our cash resources may change as a result of many factors currently unknown to us, and we may need to seek additional funds sooner than planned, through public or private equity or debt financings or other capital sources, including potentially collaborations, licenses and other similar arrangements. In addition, we may seek additional capital due to favorable market conditions or strategic considerations even if we believe we have sufficient funds for our current or future operating plans.

There can be no assurance that we will be able to raise sufficient additional capital on acceptable terms or at all. If such additional financing is not available on satisfactory terms, or is not available in sufficient amounts, we may be required to delay, limit or eliminate the development of business opportunities, and our ability to achieve our business objectives, our competitiveness, and our business, financial condition and results of operations may be materially adversely affected. In addition, we may be required to grant rights to develop and market product candidates that we would otherwise prefer to develop and market ourselves.

Results of Operations

Results of operations for the fiscal year ended September 30, 2022 reflect the following changes from the fiscal year ended September 30, 2021.

	Year Ended September 30,		
	2022	2021	Change
OPERATING EXPENSES			
General and administrative	$ 11,869,747	$ 12,202,217	$ (332,470)
Research and development	21,448,592	11,475,201	9,973,391
Research and development, Vera acquisition	—	2,888,029	(2,888,029)
TOTAL OPERATING EXPENSES	33,318,339	26,565,447	6,752,892
LOSS FROM OPERATIONS	(33,318,339)	(26,565,447)	(6,752,892)
OTHER INCOME (EXPENSE)			
Interest expense	(24,047)	(32,330)	8,283
Interest income	148,556	12,550	136,006
Change in fair value of warrant liabilities	—	950,151	(950,151)
Equity in losses on equity method investment	(415,747)	(224,534)	(191,213)
Other (expense) income, net	(166,873)	450,309	(617,182)
Total other (expense) income, net	(458,111)	1,156,146	(1,614,257)
NET LOSS	$ (33,776,450)	$ (25,409,301)	$ (8,367,149)

Until we are able to generate revenues, our management expects to continue to incur net losses.

General and Administrative Expenses

General and administrative expenses consist primarily of legal and professional fees, wages, and stock-based compensation. General and administrative expenses decreased by $0.3 million for the fiscal year ended September 30, 2022, as compared to the fiscal year ended September 30, 2021, primarily due to decreases in legal and professional fees and stock-based compensation expense, partially offset by an increase in wage expense.

Research and Development Expenses

Research and development expenses consist primarily of professional fees, research, development, manufacturing expenses, wages, and stock-based compensation. Research and development expenses increased by $10.0 million for the fiscal year ended September 30, 2022, as compared to the fiscal year ended September 30, 2021, primarily due to increases in manufacturing expenses, employee headcount, and the ramp up of research and development activities in support of our preclinical programs.

Research and Development, Vera Acquisition

Research and development, Vera Acquisition consists of the fair value of acquired Vera assets that were determined to represent in-process research and development assets with no future alternative use. The in-process research and development assets of $2.9 million were expensed upon acquisition during the fiscal year ended September 30, 2021 under the guidance of ASC 730, *Research and Development*. No such expenses were incurred during the fiscal year ended September 30, 2022.

Change in Fair Value of Warrant Liabilities

Change in fair value of warrant liabilities reflects the changes in the fair value of outstanding warrants measured at fair value on a recurring basis, which is primarily driven by changes in our stock price. The fair value of warrant liabilities was $0 at September 30, 2022 and September 30, 2021; therefore, no change in fair value was recognized during the fiscal year ended September 30, 2022. During the fiscal year ended September 30, 2022, warrants measured at fair value expired unexercised. We recognized a gain of $1.0 million from the change in fair value of warrant liabilities for the fiscal year ended September 30, 2021.

Equity in Losses on Equity Method Investment

We account for our investment in DepYmed common shares using the equity method of accounting and record our proportionate share of DepYmed's net income and losses. As of each of September 30, 2022 and September 30, 2021, the carrying value of our investment in DepYmed common shares was reduced to zero; therefore, during the fiscal year ended September 30, 2022, we recorded our share of equity losses to the extent of our investment in preferred shares of DepYmed. We will continue to monitor the operating results of DepYmed and will record equity in earnings when the equity in earnings exceeds our previously unrecognized losses. Equity in losses was $0.4 million for the fiscal year ended September 30, 2022, and $0.2 million for the fiscal year ended September 30, 2021.

Other (Expense) Income, net

We recognized other expense of $0.2 million during the fiscal year ended September 30, 2022 related to the correction of a payroll tax expense credit received in a prior period. We recognized other income of $0.5 million during the fiscal year ended September 30, 2021 related to the sale of certain intellectual property to DepYmed in exchange for shares of Series A-4 preferred stock.

Liquidity, Capital Resources and Financial Condition

We have no revenues from product sales and have incurred operating losses since inception. As of September 30, 2022, we had cash and cash equivalents of $23.2 million. We have historically funded our operations through the sale of common stock and the issuance of convertible notes and warrants. We expect to continue to incur significant operating losses for the foreseeable future and may never become profitable. As a result, we will likely need to raise additional capital through one or more of the following: the issuance of additional debt or equity or the completion of a licensing transaction for one or more of our pipeline assets. Accordingly, there are material risks and uncertainties that raised substantial doubt about the Company's ability to continue as a going concern. In October 2022, the Company announced a restructuring plan to reduce its operating expenses and extend its cash runway into the second quarter of calendar year 2024 based on current operating plans and estimates. Management believes it is probable that the restructuring plan will be effectively implemented within the next twelve months and that the restructuring plan, when implemented, will mitigate the conditions that gave rise to substantial doubt about the Company's ability to continue as a going concern.

Net working capital decreased from September 30, 2021 to September 30, 2022 by $30.2 million (from $50.7 million to $20.5 million). Our annual cash burn has increased compared to prior periods due to increased research and development activities, though we expect it will decrease in the fiscal year ending September 30, 2023. We believe our current cash balance will provide sufficient capital to continue operations into the second calendar quarter of 2024.

At present, we have no bank line of credit or other fixed source of capital reserves. Should we need additional capital in the future, we will be primarily reliant upon a private or public placement of our equity or debt securities, or a strategic transaction, for which there can be no warranty or assurance that we may be successful in such efforts. If we are unable to maintain sufficient financial resources, our business, financial condition and results of operations will be materially and adversely affected. This could affect future development and business activities and potential future clinical studies and/or other future ventures. Failure to obtain additional equity or debt financing will have a material, adverse impact on the Company's business operations. There can be no assurance that we will be able to obtain the financing needed to achieve our goals on acceptable terms or at all. Additionally, any equity financings would likely have a dilutive effect on the holdings of the Company's existing stockholders.

Contractual Obligations and Commitments

Leases

In October 2020, we entered into a ten-year operating lease agreement with annual escalating rental payments for approximately 14,189 square feet of office and laboratory space in Pittsburgh, PA. The leased premises serves as our headquarters. The first and second amendments to the lease agreement were executed in December 2020 and April 2021, respectively (collectively with the lease agreement, the "Lease"). The Lease commenced on May 1, 2021 and will last for a period of ten years from delivery of the leased premises to the Company, unless earlier terminated in accordance with the Lease. We have the right to extend the term of the Lease for an additional five-year term. We will pay an escalating base rent over the life of the Lease of approximately $71,000 to $78,000 per month, and we will pay our pro rata portion of property expenses and operating expenses for the property.

In February 2022, we entered into an eighteen-month lease agreement for private and shared laboratory facilities for general laboratory and office space in Boston, MA. We paid monthly rent payments of approximately $9,800 over the term of the lease. The lease agreement was terminated effective October 2022.

Cash Flow Summary

The following table summarizes selected items in our consolidated statements of cash flows:

	Year Ended September 30,	
	2022	**2021**
Net cash used in operating activities	$ (29,011,966)	$ (18,873,684)
Net cash used in investing activities	(471,741)	(2,563,467)
Net cash (used in) provided by financing activities	(257,017)	42,338,255
Net (decrease) increase in cash and cash equivalents	$ (29,740,724)	$ 20,901,104

Operating Activities

Net cash used in operating activities was approximately $29.0 million for the fiscal year ended September 30, 2022 as compared to $18.9 million for the fiscal year ended September 30, 2021. Net cash used in operating activities in the fiscal year ended September 30, 2022 was primarily the result of our net loss, a decrease in accrued expenses and other current liabilities, and operating lease liability, partially offset by stock-based compensation expense, depreciation and amortization expenses, loss on equity method investment and a decrease in prepaid insurance, other prepaid expenses and current assets. Net cash used in operating activities in the fiscal year ended September 30, 2021 was primarily as a result of our net loss as well as the change in the fair value of warrant liabilities, gain on sale of intellectual property and cash used for the security deposit and prepaid insurance, other prepaid expenses and current assets, including prepayment of rent under our new operating lease for office and laboratory space, partially offset by the research and development expenses for the Vera acquisition, stock-based compensation expense, increased accrued expenses and depreciation and amortization expenses.

Investing Activities

Net cash used in investing activities was approximately $0.5 million for the fiscal year ended September 30, 2022, as compared to $2.6 million for the fiscal year ended September 30, 2021. Net cash used in investing activities in the fiscal year ended September 30, 2022 was primarily due to the purchase of laboratory and office equipment, whereas for the fiscal year ended September 30, 2021, net cash used in investing activities was due to cash paid for the Vera acquisition and the purchase of laboratory and office equipment.

Financing Activities

Net cash used in financing activities was approximately $0.3 million for the fiscal year ended September 30, 2022, as compared to net cash provided by financing activities of $42.3 million for the fiscal year ended September 30, 2021. Net cash used in financing activities for the fiscal year ended September 30, 2022 primarily reflects the principal payments of financed insurance and a finance lease liability, partially offset by proceeds received from the exercise of stock options. Net cash provided by financing activities for

the fiscal year ended September 30, 2021 primarily reflects the proceeds from the issuance of common stock of $42.6 million, net of issuance costs, partially offset by the principal payments of financed insurance.

Recent Accounting Standards

See Note 2 to our consolidated financial statements for a discussion of recent accounting standards and their effect, if any, on us.

Critical Accounting Estimates

Our management's discussion and analysis of our financial condition and results of operations is based on our consolidated financial statements, which have been prepared in accordance with United States generally accepted accounting principles ("GAAP"). The preparation of these consolidated financial statements requires us to make estimates, judgments and assumptions that affect the reported amounts of assets and liabilities, disclosure of contingent assets and liabilities as of the date of the balance sheet and the reported amounts of expenses during the reporting period. In accordance with GAAP, we evaluate our estimates and judgments on an ongoing basis. The most significant estimates relate to the valuation of stock-based compensation, the valuation of licenses, the fair value of warrant liabilities and the valuation allowance of deferred tax assets resulting from net operating losses. We base our estimates and assumptions on current facts, our limited historical experience from operations and 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 may differ from these estimates under different assumptions or conditions.

We define our critical accounting policies as those accounting principles that require it to make subjective estimates and judgments about matters that are uncertain and are likely to have a material impact on our financial condition and results of operations, as well as the specific manner in which we apply those principles. While our significant accounting policies are more fully described in Note 2 to our consolidated financial statements appearing elsewhere in this Form 10-K, we believe the following are the critical accounting policies used in the preparation of our consolidated financial statements that require significant estimates and judgments:

Fair Value of Financial Instruments

In accordance with Accounting Standards Codification ("ASC") 820, the carrying value of cash and cash equivalents, accounts payable and notes payable approximates fair value due to the short-term maturity of these instruments. ASC 820 clarifies the definition of fair value, prescribes methods for measuring fair value, and establishes a fair value hierarchy to classify the inputs used in measuring fair value as follows:

- Level 1 - Quoted prices in active markets for identical assets or liabilities on the reporting date.
- Level 2 - Pricing inputs are based on quoted prices for similar instruments in active markets, quoted prices for identical or similar instruments in markets that are not active and model-based valuation techniques for which all significant assumptions are observable in the market or can be corroborated by observable market data for substantially the full term of the assets or liabilities.
- Level 3 - Pricing inputs are generally unobservable and include situations where there is little, if any, market activity for the investment. The inputs into the determination of fair value require management's judgment or estimation of assumptions that market participants would use in pricing the assets or liabilities. The fair values are therefore determined using factors that involve considerable judgment and interpretations, including but not limited to private and public comparable, third-party appraisals, discounted cash flow models and fund manager estimates.

Prior to their expiration during the year ended September 30, 2022, warrant liabilities were measured at fair value on a recurring basis. The warrant liabilities were valued using Level 3 valuation inputs. At September 30, 2021, the fair value of outstanding warrant liabilities measured at fair value on a recurring basis was $0 million.

As of September 30, 2022 and 2021, the recorded values of cash and cash equivalents, accounts payable and the insurance note payable, approximate the fair values due to the short-term nature of the instruments.

Research and Development

Research and development expenses are expensed in the statement of operations as incurred in accordance with the Financial Accounting Standards Board ("FASB") ASC 730, *Research and Development*. Research and development expenses consist of salaries and related benefits for personnel in research and development functions, including stock-based compensation and benefits and fees paid to consultants and contract research organizations for preclinical development work on our PATrOL™ platform and programs, as well as other costs. During the fiscal years ended September 30, 2022 and 2021, we incurred $21.2 million and $11.5 million, respectively, in research and development expenses.

Stock-Based Compensation

We expense stock-based compensation to employees, non-employees and board members over the requisite service period based on the estimated grant-date fair value of the awards and actual forfeitures. We account for forfeitures as they occur. Stock-based awards with graded vesting schedules are recognized on a straight-line basis over the requisite service period for each separately vesting portion of the award.

We estimate the fair value of stock option grants using the Black-Scholes option pricing model, and the assumptions used in calculating the fair value of stock-based awards represent management's best estimates and involve inherent uncertainties and the application of management's judgment. We were historically a private company and in certain instances lacked sufficient company-specific historical and implied volatility information. Therefore, in instances where we lacked sufficient company-specific historical information, we estimated our expected stock volatility based on the historical volatility of a publicly traded set of peer companies. The expected term assumption for employee grants is based on a permitted simplified method, which is based on the vesting period and contractual term for each tranche of awards. The mid-point between the weighted-average vesting term and the expiration date is used as the expected term under this method. The risk-free interest rate is determined by reference to the U.S. Treasury yield curve in effect for time periods approximately equal to the expected term of the award. Expected dividend yield is zero based on the fact that we have never paid cash dividends and do not expect to pay any cash dividends in the foreseeable future.

All stock-based compensation costs are recorded in general and administrative or research and development expenses in the consolidated statements of operations based upon the underlying individual's role at the Company.

Income Taxes

Income taxes are accounted for under the asset and liability method. Deferred income taxes are recorded for temporary differences between financial statement carrying amounts and the tax basis of assets and liabilities. Deferred tax assets and liabilities reflect the tax rates expected to be in effect for the years in which the differences are expected to reverse. A valuation allowance is provided if it is more likely than not that some or all of the deferred tax assets will not be realized.

We also follow the provisions of accounting for uncertainty in income taxes which prescribes a model for the recognition and measurement of a tax position taken or expected to be taken in a tax return, and provides guidance on derecognition, classification, interest and penalties, disclosure and transition. In accordance with this guidance, tax positions must meet a more likely than not recognition threshold and measurement attribute for the financial statement recognition and measurement of tax position.

Our policy is to account for income tax-related interest and penalties in income tax expense in the accompanying consolidated statements of operations.

ITEM 7A. QUANTITATIVE AND QUALITATIVE DISCLOSURES ABOUT MARKET RISK

Not applicable.

ITEM 8. FINANCIAL STATEMENTS AND SUPPLEMENTARY DATA

INDEX TO FINANCIAL STATEMENTS

REPORT OF INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM

To the Shareholders and Board of Directors of
NeuBase Therapeutics, Inc.

Opinion on the Financial Statements

We have audited the accompanying consolidated balance sheets of NeuBase Therapeutics, Inc. and subsidiaries (the "Company") as of September 30, 2022 and 2021, the related consolidated statements of operations, statements of changes in stockholders' equity and cash flows for each of the two years in the period ended September 30, 2022, and the related notes (collectively referred to as the "financial statements"). In our opinion, the financial statements present fairly, in all material respects, the financial position of the Company as of September 30, 2022 and 2021, and the results of its operations and its cash flows for each of the two years in the period ended September 30, 2022, in conformity with accounting principles generally accepted in the United States of America.

Basis for Opinion

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

We conducted our audits in accordance with the standards of the PCAOB. Those standards require that we plan and perform the audits to obtain reasonable assurance about whether the financial statements are free of material misstatement, whether due to error or fraud. The Company is not required to have, nor were we engaged to perform, an audit of its internal control over financial reporting. As part of our audits we are required to obtain an understanding of internal control over financial reporting but not for the purpose of expressing an opinion on the effectiveness of the Company's internal control over financial reporting. Accordingly, we express no such opinion.

Our audits included performing procedures to assess the risks of material misstatement of the financial statements, whether due to error or fraud, and performing procedures that respond to those risks. Such procedures included examining, on a test basis, evidence regarding the amounts and disclosures in the financial statements. Our audits also included evaluating the accounting principles used and significant estimates made by management, as well as evaluating the overall presentation of the financial statements. We believe that our audits provide a reasonable basis for our opinion.

Critical Audit Matters

Critical audit matters are matters arising from the current period audit of the financial statements that were communicated or required to be communicated to the audit committee and that: (1) relate to accounts or disclosures that are material to the financial statements and (2) involved our especially challenging, subjective, or complex judgments. We determined that there are no critical audit matters.

/s/ Marcum LLP

Marcum LLP

We have served as the Company's auditor since 2020.

New York, NY
December 21, 2022

NeuBase Therapeutics, Inc. and Subsidiaries
Consolidated Balance Sheets

	September 30, 2022	September 30, 2021
ASSETS		
CURRENT ASSETS		
Cash and cash equivalents	$ 23,152,663	$ 52,893,387
Prepaid insurance	319,699	499,061
Other prepaid expenses and current assets	1,176,303	1,536,186
Total current assets	24,648,665	54,928,634
EQUIPMENT, net	2,156,851	2,463,882
OTHER ASSETS		
Investment	—	415,744
Right-of-use asset, operating lease asset	5,614,698	5,945,295
Security deposit	273,215	253,615
Other long-term assets	—	160,423
Total other assets	5,887,913	6,775,077
TOTAL ASSETS	$ 32,693,429	$ 64,167,593
LIABILITIES AND STOCKHOLDERS' EQUITY		
CURRENT LIABILITIES		
Accounts payable	$ 1,843,027	$ 1,807,885
Accrued expenses and other current liabilities	1,662,660	1,747,746
Insurance note payable	—	148,385
Operating lease liabilities	553,066	382,576
Finance lease liabilities	107,632	107,632
Total current liabilities	4,166,385	4,194,224
Long-term operating lease liability	5,335,164	5,794,096
Long-term finance lease liability	—	109,500
TOTAL LIABILITIES	9,501,549	10,097,820
COMMITMENTS AND CONTINGENCIES		
STOCKHOLDERS' EQUITY		
Preferred stock, $0.0001 par value; 10,000,000 shares authorized; no shares issued and outstanding as of September 30, 2022 and 2021	—	—
Common stock, $0.0001 par value; 250,000,000 shares authorized; 33,008,657 and 32,721,493 shares issued and outstanding as of September 30, 2022 and 2021, respectively	3,300	3,272
Additional paid-in capital	125,932,933	123,034,404
Accumulated deficit	(102,744,353)	(68,967,903)
Total stockholders' equity	23,191,880	54,069,773
TOTAL LIABILITIES AND STOCKHOLDERS' EQUITY	$ 32,693,429	$ 64,167,593

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

NeuBase Therapeutics, Inc. and Subsidiaries
Consolidated Statements of Operations

	Year Ended September 30,	
	2022	2021
OPERATING EXPENSES		
General and administrative	$ 11,869,747	$ 12,202,217
Research and development	21,448,592	11,475,201
Research and development, Vera acquisition	—	2,888,029
TOTAL OPERATING EXPENSES	33,318,339	26,565,447
LOSS FROM OPERATIONS	(33,318,339)	(26,565,447)
OTHER INCOME (EXPENSE)		
Interest expense	(24,047)	(32,330)
Interest income	148,556	12,550
Change in fair value of warrant liabilities	—	950,151
Equity in losses on equity method investment	(415,747)	(224,534)
Other (expense) income, net	(166,873)	450,309
Total other (expense) income, net	(458,111)	1,156,146
NET LOSS	$ (33,776,450)	$ (25,409,301)
BASIC AND DILUTED LOSS PER SHARE	$ (1.04)	$ (0.93)
WEIGHTED AVERAGE SHARES OUTSTANDING:		
BASIC AND DILUTED	32,487,244	27,306,043

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

NeuBase Therapeutics, Inc. and Subsidiaries
Consolidated Statements of Changes in Stockholders' Equity

	Common Stock		Additional Paid-In	Accumulated	Total Stockholders'
	Shares	Amount	Capital	Deficit	Equity
Balance as of September 30, 2020	**23,154,084**	**$ 2,315**	**$ 74,850,935**	**$ (43,558,602)**	**$ 31,294,648**
Stock-based compensation expense	—	—	3,696,384	—	3,696,384
Issuance of restricted stock for services	11,722	1	(1)	—	—
Exercise of stock options	47,052	5	112,441	—	112,446
Issuance of common stock, net of issuance costs	9,200,000	920	42,615,456	—	42,616,376
Issuance of common stock, Vera acquisition	308,635	31	1,759,189	—	1,759,220
Net loss	—	—	—	(25,409,301)	(25,409,301)
Balance as of September 30, 2021	**32,721,493**	**$ 3,272**	**$ 123,034,404**	**$ (68,967,903)**	**$ 54,069,773**
Stock-based compensation expense	—	—	2,897,689	—	2,897,689
Issuance of restricted stock for services	4,441	—	—	—	—
Exercise of stock options	792,250	79	789	—	868
Forfeiture of common stock	(509,527)	(51)	51	—	—
Net loss	—	—	—	(33,776,450)	(33,776,450)
Balance as of September 30, 2022	**33,008,657**	**$ 3,300**	**$ 125,932,933**	**$ (102,744,353)**	**$ 23,191,880**

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

NeuBase Therapeutics, Inc. and Subsidiaries
Consolidated Statements of Cash Flows

	Year Ended September 30,	
	2022	**2021**
Cash flows from operating activities		
Net loss	$ (33,776,450)	$ (25,409,301)
Adjustments to reconcile net loss to net cash used in operating activities		
Stock-based compensation	2,897,689	3,696,384
Research and development, Vera acquisition	—	2,888,029
Change in fair value of warrant liabilities	—	(950,151)
Depreciation and amortization	767,302	403,043
Loss on marketable securities	30	25,012
Loss on disposal of fixed assets	11,439	31,853
Equity in losses on equity method investment	415,747	224,534
Gain on sale of intellectual property	—	(316,724)
Amortization of right-of-use assets	495,210	172,478
Changes in operating assets and liabilities		
Prepaid insurance, other prepaid expenses and current assets	539,245	(666,945)
Long-term prepaid insurance	—	145,250
Security deposit	(19,600)	(253,615)
Other long-term assets	160,423	(160,420)
Accounts payable	35,142	294,548
Accrued expenses and other current liabilities	(85,088)	943,442
Operating lease liability	(453,055)	58,899
Net cash used in operating activities	(29,011,966)	(18,873,684)
Cash flows from investing activities		
Purchase of laboratory and office equipment	(471,711)	(1,438,415)
Purchase of marketable securities	(14,986,818)	(59,988,511)
Sale of marketable securities	14,986,788	59,963,499
Cash paid for Vera acquisition	—	(1,100,040)
Net cash used in investing activities	(471,741)	(2,563,467)
Cash flows from financing activities		
Principal payment of financed insurance	(148,385)	(381,797)
Principal payment of finance lease liability	(109,500)	(8,770)
Proceeds from issuance of stock, net of issuance costs	—	42,616,376
Proceeds from exercise of stock options	868	112,446
Net cash (used in) provided by financing activities	(257,017)	42,338,255
Net (decrease) increase in cash and cash equivalents	(29,740,724)	20,901,104
Cash and cash equivalents, beginning of period	52,893,387	31,992,283
Cash and cash equivalents, end of period	**$ 23,152,663**	**$ 52,893,387**
Supplemental disclosure of cash flow information:		
Cash paid for interest	$ —	$ —
Cash paid for income taxes	$ —	$ —
Non-cash investing and financing activities:		
Issuance of common stock, Vera acquisition	$ —	$ 1,759,220
Purchases of laboratory and office equipment in accounts payable	$ —	$ 8,295
Preferred shares in DepYmed received as consideration for sale of intellectual property	$ —	$ 316,724
Insurance financed through note payable	$ —	$ 391,625
Right-of-use asset obtained in exchange for operating lease liabilities	$ 164,613	$ —
Deferred offering costs accrued	$ —	$ 160,420

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

1. Organization, Description of Business and Liquidity

NeuBase Therapeutics, Inc. and its subsidiaries (the "Company" or "NeuBase") is developing a modular peptide-nucleic acid ("PNA") antisense oligo ("PATrOL™") platform to address genetic diseases, with a single, cohesive approach. NeuBase plans to use its platform to address diseases which have a genetic source, with an initial focus on gene silencing in myotonic dystrophy type 1 ("DM1"), Huntington's disease ("HD"), and oncology, and in gene editing applications.

NeuBase is a preclinical-stage biopharmaceutical company and continues to develop its clinical and regulatory strategy with its internal research and development team, with a view toward prioritizing market introduction as quickly as possible. NeuBase's disclosed programs are NT-0100 in HD, NT-0200 in DM1 and NT-0300 in KRAS-driven cancers.

The NT-0100 program is a PATrOL™-enabled therapeutic program being developed to target the mutant expansion in the HD DNA or RNA. The NT-0100 program includes proprietary PNAs which have the potential to be highly selective for the mutant copy of the gene versus the wild-type allele, the expectation being that the resultant therapy will be applicable for all HD patients as it directly targets the expansion itself, and the potential to be delivered systemically and address the brain and whole-body manifestations of the disease. PATrOL™-enabled drugs also have the unique ability to open DNA and RNA secondary structures and bind to either the primary nucleotide sequences or the secondary and/or tertiary structures.

The NT-0200 program is a PATrOL™-enabled therapeutic program being developed to target the mutant expansion in the DM1 disease RNA. The NT-0200 program has the potential to be highly selective for the mutant transcript versus the wild-type transcribed allele and the expectation to be effective for nearly all DM1 patients as it directly targets the expansion itself.

The NT-0300 program is a PATrOL-enabled therapeutic program being developed to target the mutated KRAS gene. The program is comprised of candidate compounds that target two activating mutations in the KRAS gene at the DNA or RNA levels: G12D and G12V. NeuBase believes these candidate compounds, and subsequent further optimized compounds, have the potential to inhibit transcription and/or translation of the oncogenic mutations and slow or stop tumor growth.

In October 2022, the Company announced plans to expand its focus to include the advancement of the differentiated gene editing capabilities of its platform. The Company is currently identifying and evaluating multiple indications for potential future development.

Liquidity and Going Concern

The Company has had no revenues from product sales and has incurred operating losses since inception. As of September 30, 2022, the Company had $23.2 million in cash and cash equivalents and during the year ended September 30, 2022 incurred a loss from operations of $3.3 million and used $29.0 million of cash in operating activities.

The Company expects to continue to incur substantial operating losses and negative cash flows from operations for the foreseeable future and may never become profitable. Accordingly, there are material risks and uncertainties that raised substantial doubt about the Company's ability to continue as a going concern. In October 2022, as further discussed below, the Company announced a restructuring plan to reduce its operating expenses and extend its cash runway into the second quarter of calendar year 2024 based on current operating plans and estimates. Management believes it is probable that the restructuring plan will be effectively implemented within the next twelve months and that the restructuring plan, when implemented, will mitigate the conditions that gave rise to substantial doubt about the Company's ability to continue as a going concern. Because the Company has sufficient resources on hand to fund operations through at least the next twelve months from the date these consolidated financial statements were available to be issued, the substantial doubt has been alleviated. There can be no assurance that the Company will be successful in acquiring additional funding, that the Company's projections of its future working capital needs will prove accurate, or that any additional funding would be sufficient to continue operations in future years.

The Company's future liquidity and capital funding requirements will depend on numerous factors, including:

- its ability to raise additional funds to finance its operations;
- its ability to maintain compliance with the listing requirements of The Nasdaq Capital Market ("Nasdaq");
- the outcome, costs and timing of preclinical and clinical trial results for the Company's current or future product candidates;
- the extent and amount of any indemnification claims;

- litigation expenses and the extent and amount of any indemnification claims;
- the emergence and effect of competing or complementary products;
- its ability to maintain, expand and defend the scope of its intellectual property portfolio, including the amount and timing of any payments the Company may be required to make, or that it may receive, in connection with the licensing, filing, prosecution, defense and enforcement of any patents or other intellectual property rights;
- its ability to retain its current employees and the need and ability to hire additional management and scientific and medical personnel;
- the trading price of its common stock; and
- its ability to increase the number of authorized shares outstanding to facilitate future financing events.

The Company will likely need to raise substantial additional funds through issuance of equity or debt or completion of a licensing transaction for one or more of the Company's pipeline assets. If the Company is unable to maintain sufficient financial resources, its business, financial condition and results of operations will be materially and adversely affected. This could affect future development and business activities and potential future clinical studies and/or other future ventures. Failure to obtain additional equity or debt financing will have a material, adverse impact on the Company's business operations. There can be no assurance that the Company will be able to obtain the needed financing on acceptable terms or at all. Additionally, any equity financings would likely have a dilutive effect on the holdings of the Company's existing stockholders.

2. Significant Accounting Policies

Basis of Presentation

The Company's consolidated financial statements have been prepared in accordance with accounting principles generally accepted in the United States ("GAAP"). The consolidated financial statements include the accounts of the Company and its wholly-owned subsidiaries. All intercompany accounts and transactions have been eliminated during the consolidation process. The Company manages its operations as a single segment for the purposes of assessing performance and making operating decisions.

Use of Estimates

The preparation of financial statements in conformity with GAAP requires management to make estimates and assumptions that affect the reported amounts of assets and liabilities and disclosure of contingent assets and liabilities at the date of the financial statements and the reported amounts of expenses during the reporting period. The most significant estimates in the Company's consolidated financial statements relate to the valuation of stock-based compensation, the valuation of licenses, the fair value of warrant liabilities and the valuation allowance of deferred tax assets resulting from net operating losses. These estimates and assumptions are based on current facts, historical experience and various other factors believed to be reasonable under the circumstances, the results of which form the basis for making judgments about the carrying values of assets and liabilities and the recording of expenses that are not readily apparent from other sources. The Company assesses and updates estimates each period to reflect current information, such as the economic considerations related to the impact that the novel coronavirus disease (COVID-19) could have on our significant accounting estimates. Actual results may differ materially and adversely from these estimates. To the extent there are material differences between the estimates and actual results, the Company's future results of operations will be affected.

Concentration of Credit Risk

Financial instruments that potentially subject the Company to credit risk consist principally of cash and cash equivalents. Cash and cash equivalents are maintained in accounts with financial institutions which at times may exceed the Federal depository insurance coverage of $250,000. The Company has not experienced losses on these accounts and management believes, based upon the quality of the financial institutions, that the credit risk with regard to these deposits is not significant.

Fair Value Measurements

Fair value measurements are based on the premise that fair value is an exit price representing the amount that would be received to sell an asset or paid to transfer a liability in an orderly transaction between market participants. As such, fair value is a market-based measurement that should be determined based on assumptions that market participants would use in pricing an asset or liability. As a

basis for considering such assumptions, the following three-tier fair value hierarchy has been used in determining the inputs used in measuring fair value:

Level 1- Quoted prices in active markets for identical assets or liabilities on the reporting date.

Level 2- Pricing inputs are based on quoted prices for similar instruments in active markets, quoted prices for identical or similar instruments in markets that are not active and model-based valuation techniques for which all significant assumptions are observable in the market or can be corroborated by observable market data for substantially the full term of the assets or liabilities.

Level 3- Pricing inputs are generally unobservable and include situations where there is little, if any, market activity for the investment. The inputs into the determination of fair value require management's judgment or estimation of assumptions that market participants would use in pricing the assets or liabilities. The fair values are therefore determined using factors that involve considerable judgment and interpretations, including but not limited to private and public comparables, third-party appraisals, discounted cash flow models and fund manager estimates.

Financial instruments measured at fair value are classified in their entirety based on the lowest level of input that is significant to the fair value measurement. Management's assessment of the significance of a particular input to the fair value measurement in its entirety requires judgment and considers factors specific to the asset or liability. The use of different assumptions and/or estimation methodologies may have a material effect on estimated fair values. Accordingly, the fair value estimates disclosed or initial amounts recorded may not be indicative of the amount that the Company or holders of the instruments could realize in a current market exchange.

Marketable securities

Marketable securities are classified as trading and are carried at fair value. The Company's marketable securities consist of corporate bonds and highly liquid mutual funds, and exchange-traded and closed-end funds which are valued at quoted market prices. The Company had no marketable securities as of September 30, 2022 and 2021.

Equipment

Equipment is stated at cost less accumulated depreciation. Depreciation is provided on a straight-line basis over the estimated useful lives of the assets. The Company estimates useful lives as follows:

- Laboratory equipment: five years
- Office equipment: three years
- Leasehold improvements: lesser of useful life or lease term

Impairment of Long-Lived Assets

The Company reviews the carrying value of long-lived assets for indicators of possible impairment whenever events and circumstances indicate that the carrying value of an asset or asset group may not be recoverable from the estimated future net undiscounted cash flows expected to result from its use and eventual disposition. In cases where estimated future net undiscounted cash flows are less than the carrying value, an impairment loss is recognized equal to an amount by which the carrying value exceeds the fair value of the asset or asset group. The factors that would be considered by management in performing this assessment include current operating results, trends and prospects, the manner in which the property is used and the effects of obsolescence, demand, competition and other economic factors. Based on this assessment, there was no impairment at September 30, 2022 and 2021.

Investment

The Company's investment consists of common and preferred shares of DepYmed, Inc.

Investments that the Company has the ability to exercise significant influence over are accounted for as equity method investments. Equity method investments are recorded at cost plus the proportional share of the issuer's income or loss.

Investments that do not have a readily determinable fair value and qualify for the measurement alternative for equity investments provided in ASC 321 are accounted for at cost, less any impairment, plus or minus changes resulting from observable price changes in orderly transactions for an identical or similar investment of the same issuer.

Leases

The Company determines if an arrangement is a lease at inception. Operating leases are included in operating right-of-use ("ROU") assets and operating lease liabilities on the consolidated balance sheets. Equipment finance leases are included in equipment, net and finance lease liabilities on the consolidated balance sheets.

Lease ROU assets and lease liabilities are initially recognized based on the present value of the future minimum lease payments over the lease term at commencement date calculated using the Company's incremental borrowing rate applicable to the lease asset, unless the implicit rate is readily determinable. ROU assets also include any lease payments made at or before lease commencement and exclude any lease incentives received. 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. For operating leases, lease expense for minimum lease payments is recognized on a straight-line basis over the lease term. For finance leases, interest on the lease liability and the amortization of the right-of-use asset results in front-loaded expense over the lease term. The Company accounts for lease and non-lease components as a single lease component for all its leases.

The Company does not recognize ROU assets and lease liabilities that arise from leases with an original term of 12 months or less. Rather, the Company recognizes the lease expense on a straight-line basis over the term of the lease.

Research and Development

The Company expenses research and development costs as operating expenses as incurred. Research and development expenses consist primarily of:

- salaries and related benefits for personnel in research and development functions, including stock-based compensation and benefits;
- fees paid to consultants and contract research organizations for preclinical development work on our PATrOL™ platform and programs;
- allocation of facility lease and maintenance costs;
- depreciation of laboratory equipment and computers;
- costs related to purchasing raw materials for and producing our product candidates;
- costs related to compliance with regulatory requirements; and
- license fees related to in-licensed technologies.

Research and Development Expense- Licenses Acquired

The Company evaluates whether acquired intangible assets are a business under applicable accounting standards. Additionally, the Company evaluates whether the acquired assets have an alternative future use. Intangible assets that do not have alternative future use are considered acquired in-process research and development. When the acquired in-process research and development assets are not part of a business combination, the value of the consideration paid is expensed on the acquisition date. Future costs to develop these assets are recorded to research and development expense as they are incurred.

Stock-Based Compensation

The Company expenses stock-based compensation to employees, non-employees and board members over the requisite service period based on the estimated grant date fair value of the awards and actual forfeitures. The Company accounts for forfeitures as they occur. Stock-based awards with graded vesting schedules are recognized on a straight-line basis over the requisite service period for each separately vesting portion of the award.

The Company estimates the fair value of stock option grants using the Black-Scholes option pricing model, and the assumptions used in calculating the fair value of stock-based awards represent management's best estimates and involve inherent uncertainties and the application of management's judgment including:

- Volatility- The Company was historically a private company and in certain instances lacks sufficient company-specific historical and implied volatility information. Therefore, when insufficient company-specific information is available, it estimates its expected stock volatility based on the historical volatility of a publicly traded set of peer companies.

- Expected term- The expected term assumption for employee grants is based on a permitted simplified method, which is based on the vesting period and contractual term for each tranche of awards. The mid-point between the weighted-average vesting term and the expiration date is used as the expected term under this method.

- Risk-free rate- The risk-free interest rate is determined by reference to the U.S. Treasury yield curve in effect for time periods approximately equal to the expected term of the award.

- Expected dividend- The expected dividend yield is zero based on the fact that the Company has never paid cash dividends and does not expect to pay any cash dividends in the foreseeable future.

Income Taxes

Income taxes are accounted for under the asset and liability method. Deferred income taxes are recorded for temporary differences between financial statement carrying amounts and the tax basis of assets and liabilities. Deferred tax assets and liabilities reflect the tax rates expected to be in effect for the years in which the differences are expected to reverse. A valuation allowance is provided if it is more likely than not that some or all of the deferred tax assets will not be realized.

The Company also follows the provisions of accounting for uncertainty in income taxes which prescribes a model for the recognition and measurement of a tax position taken or expected to be taken in a tax return and provides guidance on derecognition, classification, interest and penalties, disclosure and transition. In accordance with this guidance, tax positions must meet a more likely than not recognition threshold and measurement attribute for the financial statement recognition and measurement of tax position.

The Company's policy is to account for income tax-related interest and penalties in income tax expense in the accompanying consolidated statements of operations.

Net Loss Per Share

Basic net loss per share is computed by dividing net loss applicable to common stockholders by the weighted average number of shares of common stock outstanding during each period. Diluted net loss per share includes the dilutive effect, if any, from the potential exercise or conversion of securities, such as convertible debt, warrants and stock options that would result in the issuance of incremental shares of common stock. In computing the basic and diluted net loss per share applicable to common stockholders, the weighted-average number of shares remains the same for both calculations due to the fact that when a net loss exists dilutive shares are not included in the calculation as the impact is anti-dilutive.

Recent Accounting Pronouncements

In December 2019, the Financial Accounting Standards Board ("FASB") issued ASU No. 2019-12,*"Income Taxes (Topic 740): Simplifying the Accounting for Income Taxes"* ("ASU 2019-12"), which is intended to simplify various aspects related to accounting

for income taxes. ASU 2019-12 removes certain exceptions to the general principles in Topic 740 and also clarifies and amends existing guidance to improve consistent application. The adoption of this standard as of October 1, 2021, did not impact the Company's consolidated financial statements and related disclosures.

In November 2021, the FASB issued ASU No. 2021-10, "Government Assistance (Topic 832): Disclosures by Business Entities about Government Assistance", which amends disclosures to increase transparency of government assistance, including (i) the types of assistance, (ii) accounting for the assistance and (iii) the effect of the assistance on an entity's financial statements. The standard is effective for all business entities for annual periods beginning after December 15, 2021. The Company is currently evaluating the impact of this standard on its consolidated financial statements and related disclosures.

In June 2022, the FASB issued ASU 2022-03, "ASC Subtopic 820 Fair Value Measurement of Equity Securities Subject to Contractual Sale Restrictions" ("ASU 2022-03"). ASU 2022-03 amends ASC 820 to clarify that a contractual sales restriction is not considered in measuring an equity security at fair value and to introduce new disclosure requirements for equity securities subject to contractual sale restrictions that are measured at fair value. ASU 2022-03 applies to both holders and issuers of equity and equity-linked securities measured at fair value. The amendments in this ASU are effective for the Company in fiscal years beginning after December 15, 2023, and interim periods within those fiscal years. Early adoption is permitted for both interim and annual financial statements that have not yet been issued or made available for issuance. The Company is currently evaluating the impact of this pronouncement on its consolidated financial statements and related disclosures.

In June 2016, the FASB issued ASU No. 2016-13, “Financial Instruments- Credit Losses (Topic 326): Measurement of Credit Losses on Financial Instruments” (“ASU 2016-13”). This guidance introduces a new model for recognizing credit losses on financial instruments based on an estimate of current expected credit losses. ASU 2016-13 also provides updated guidance regarding the impairment of available-for-sale debt securities and includes additional disclosure requirements. The new guidance is effective for public business entities that meet the definition of a Smaller Reporting Company as defined by the Securities and Exchange Commission for interim and annual periods beginning after December 15, 2022. Early adoption is permitted. The Company is currently evaluating the impact of this standard on its consolidated financial statements and related disclosures.

In May 2021, the FASB issued ASU No. 2021-04, “Earnings Per Share (Topic 260), Debt-Modifications and Extinguishments (Subtopic 470-50), Compensation-Stock Compensation (Topic 718), and Derivatives and Hedging-Contracts in Entity’s Own Equity (Subtopic 815-40)” (“ASU 2021-04”). This guidance reduces diversity in an issuer’s accounting for modifications or exchanges of freestanding equity-classified written call options (for example, warrants) that remain equity classified after modification or exchange. ASU 2021-04 provides guidance for a modification or an exchange of a freestanding equity-classified written call option that is not within the scope of another Topic. It specifically addresses: (1) how an entity should treat a modification of the terms or conditions or an exchange of a freestanding equity-classified written call option that remains equity classified after modification or exchange; (2) how an entity should measure the effect of a modification or an exchange of a freestanding equity-classified written call option that remains equity classified after modification or exchange; and (3) how an entity should recognize the effect of a modification or an exchange of a freestanding equity-classified written call option that remains equity classified after modification or exchange. ASU 2021-04 will be effective for all entities for fiscal years beginning after December 15, 2021. An entity should apply the amendments prospectively to modifications or exchanges occurring on or after the effective date of the amendments. Early adoption is permitted, including adoption in an interim period. The Company is currently evaluating the impact of this standard on its consolidated financial statements and related disclosures.

3. Asset Purchase Agreement

On January 27, 2021, the Company entered into an Asset Purchase Agreement by and among us, NeuBase Corporation, our wholly-owned subsidiary, and Vera Therapeutics, Inc. (“Vera”), as amended by the Amendment to Asset Purchase Agreement, dated as of April 20, 2021, by and between the Company and Vera (collectively, the “APA”), and the transaction closed on April 26, 2021 (the “Vera Acquisition”). Pursuant to the terms of the APA, the Company acquired infrastructure, materials, and intellectual property for PNA scaffolds from Vera for total consideration of approximately $0.8 million in cash and 308,635 shares of Common Stock (of which 146,375 were issued to Vera and 162,260 were held in escrow and released to Vera in accordance with the terms of an escrow agreement between NeuBase Corporation and Vera).

The Company accounted for the Vera Acquisition as an asset acquisition.

Total consideration for the Vera transaction consisted of the following:

Cash consideration	$ 796,124
Acquisition costs	303,916
Fair value of common stock (1)	1,759,220
Total consideration	$ 2,859,260

(1) The fair value of common stock represents the closing share price of308,635 shares of the Company's common stock on April 26, 2021.

The total consideration for the Vera transaction was allocated as follows:

Property, plant and equipment	$ 59,231
Other liabilities	(88,000)
Fair value of net assets acquired	(28,769)
Research and development expense	2,888,029
Total cost of Vera acquisition	$ 2,859,260

In-process research and development assets with no future alternative use acquired in the Vera Acquisition were expensed.

4. Equipment

The Company's equipment consisted of the following:

	As of September 30, 2022	As of September 30, 2021	Estimated useful life (in years)
Laboratory equipment	$ 3,175,019	$ 2,737,390	5
Office equipment	259,978	259,978	3
Leasehold improvements	17,958	—	5
Total	3,452,955	2,997,368	
Accumulated depreciation and amortization	(1,296,104)	(533,486)	
Equipment, net	$ 2,156,851	$ 2,463,882	

Depreciation and amortization expense for the years ended September 30, 2022 and 2021 was approximately $0.8 million and $0.4 million, respectively.

5. Other Prepaid Expenses and Other Current Assets

The Company's prepaid expenses and other current assets consisted of the following:

	As of September 30, 2022	As of September 30, 2021
Prepaid research and development expense	$ 805,542	$ 583,267
Prepaid rent	—	172,518
Franchise tax receivable	127,715	—
Other prepaid expenses and other current assets	243,046	780,401
Total	$ 1,176,303	$ 1,536,186

6. Investment

On February 26, 2014, Ohr entered into a Joint Venture Agreement and related agreements with Cold Spring Harbor Laboratory ("CSHL") pursuant to which a joint venture, DepYmed Inc. ("DepYmed"), was formed to further preclinical and clinical development of the Company's intellectual property for rare diseases and oncology. DepYmed licenses research from CSHL and intellectual property from the Company.

Following the 2019 merger with Ohr Pharmaceutical, Inc., (the "Ohr Acquisition"), the Company owns common and preferred shares of DepYmed. In addition, in February 2021, the Company sold certain intellectual property to DepYmed in exchange for shares of Series A-4 preferred stock. In aggregate, the Company's ownership represents approximately 15% ownership of DepYmed.

The Company accounts for its investment in DepYmed common shares using the equity method of accounting and records its proportionate share of DepYmed's net income and losses in the accompanying consolidated statements of operations.

The Company accounts for its investment in preferred shares of DepYmed at cost, less any impairment, as the Company determined the preferred stock did not have a readily determinable fair value.

The carrying value of the Company's investment in DepYmed common shares was reduced to zero; therefore, during the year ended September 30, 2022, the Company recorded its share of equity losses to the extent of its investment in preferred shares of DepYmed. The Company will continue to monitor the operating results of DepYmed and will record equity in earnings when the equity in earnings exceeds the previously unrecognized losses.

Equity in losses for the years ended September 30, 2022 and 2021 were approximately $0.4 million and $0.2 million, respectively.

The carrying value of the Company's total investment is DepYmed is as follows:

	As of September 30,	
	2022	2021
Carrying value of DepYmed common shares	$ —	$ —
Fair value of DepYmed preferred shares assumed in connection with acquisition of Ohr	—	99,020
DepYmed preferred shares received in sale of intellectual property	—	316,724
Total Investment	$ —	$ 415,744

The Company recognized a gain of $0.3 million related to the sale of IP to DepYmed, which was recorded in other (expense) income, net on the Company's consolidated statement of operations for the year ended September 30, 2021.

7. Accrued Expenses and Other Current Liabilities

The Company's accrued expenses and other current liabilities consisted of the following:

	As of September 30,	
	2022	2021
Accrued compensation and benefits	$ 768,324	$ 880,707
Accrued consulting settlement	150,000	200,000
Accrued professional fees	191,516	299,557
Accrued research and development	512,570	297,047
Accrued franchise tax	36,542	30,720
Other accrued expenses	3,708	39,715
Total	$ 1,662,660	$ 1,747,746

8. Notes Payable

Insurance Note Payable

As of September 30, 2022 and 2021, the Company had the following insurance note payable outstanding:

	Maturity Date	Stated Interest Rate	Original Principal	Balance at September 30, 2022	Balance at September 30, 2021
Insurance Note Payable					
2021 Insurance Note	January 2022	4.99 %	$ 391,625	$ —	$ 148,385

The Company paid off the insurance note as of September 30, 2022.

9. Leases

In October 2020, the Company entered into a ten-year operating lease agreement with annual escalating rental payments for approximately 14,189 square feet of office and laboratory space in Pittsburgh, PA. The leased premises serves as the Company's headquarters. The first and second amendments to the lease agreement were executed in December 2020 and April 2021, respectively (collectively with the lease agreement, referred to herein as the "Lease"). In November 2020, the Company prepaid rent of $0.3 million and paid a security deposit of $0.3 million for the Lease. The Lease commenced on May 1, 2021, and the Company was obligated to begin making rental payments on this date. The Company applied the prepaid amount toward the rental payments through December 2021. The Company measured and recognized an initial ROU asset and operating lease liability upon lease commencement. The Company has the right to extend the term of the Lease for an additional five-year term; however, this extension has not been included in the calculation of the lease liability and ROU asset at the lease inception as the exercise of the option was not reasonably certain.

The Company continued to operate under its operating lease in Pittsburgh until the Company moved into its new headquarters and laboratory space, which occurred in June 2021. The Company's prior office and operating space was leased under operating leases with original terms of less than 12 months, which expired at various dates through November 2021; therefore, the Company's previous operating leases were not recognized as ROU assets on the consolidated balance sheets. The Company also maintained a short-term rental of office space in San Diego, CA and New York, NY, which expired in November 2021. In October 2021, the Company commenced a one-year lease for the rental of office space in Boston, MA, which was subsequently extended through April 2023.

In August 2021, the Company entered into a two-year finance lease for certain laboratory equipment. The Company measured and recognized an initial ROU asset and finance lease liability upon lease commencement.

In February 2022, the Company entered into an eighteen-month lease agreement for private and shared laboratory facilities for general laboratory and office space in Boston, MA. The Company measured and recognized the ROU asset and operating lease liability upon the lease commencement.

At September 30, 2022, ROU assets and lease liabilities were as follows:

		As of September 30,	
		2022	2021
Assets:	**Classification**		
Operating lease right-of-use-asset	Operating lease asset	$ 5,614,698	$ 5,945,295
Financing lease right-of-use-asset	Equipment, net	103,538	216,490
		$ 5,718,236	$ 6,161,785
Liabilities:			
Current	**Classification**		
Operating	Operating lease liability	$ 553,066	$ 382,576
Financing	Financing lease liability	107,632	107,632
Long-term			
Operating	Long-term portion of operating leases liability	5,335,164	5,794,096
Financing	Long-term portion of financing leases liability	—	109,500
		$ 5,995,862	$ 6,393,804

The following tables summarize quantitative information about the Company's leases for the years ended September 30, 2022 and 2021:

	Year Ended September 30,	
	2022	2021
Operating cash flows - operating leases	$ 901,060	$ 128,963
Operating cash flows - financing leases	12,252	1,376
Financing cash flows - financing leases	109,500	8,770
Right-of-use asset obtained in exchange for operating lease liabilities	$ 164,613	$ 6,117,772
Finance lease assets obtained in exchange for finance lease liabilities	—	225,902

	As of September 30,	
	2022	2021
Weighted-average remaining lease term – operating leases (in years)	8.74	9.83
Weighted-average discount rate – operating leases	7.3 %	7.3 %
Weighted-average remaining lease term – financing leases (in years)	0.9	1.9
Weighted-average discount rate – financing leases	7.3 %	7.3 %

The components of lease expense were as follows:

	Year Ended September 30,	
	2022	2021
Operating leases		
Operating lease cost	$ 943,215	$ 360,340
Variable lease costs	2,400	18,216
Total operating lease cost	945,615	378,556
Short-term lease rent expense	40,719	113,158
Financing leases		
Amortization of leased assets	112,951	9,413
Interest on lease liabilities	12,252	1,376
Financing lease cost	125,203	10,789
Net lease cost	$ 1,111,537	$ 502,503

As of September 30, 2022, future minimum lease payments under the non-cancelable leases were as follows:

	Operating Leases	Financing Leases
Year Ending September 30, 2023	$ 965,367	$ 111,606
Year Ending September 30, 2024	874,320	—
Year Ending September 30, 2025	881,391	—
Year Ending September 30, 2026	888,627	—
Year Ending September 30, 2027	895,864	—
Thereafter	3,505,166	—
Total	8,010,735	111,606
Less present value discount	(2,122,505)	(3,974)
Operating lease liabilities	$ 5,888,230	$ 107,632

10. Fair Value

Prior to their expiration during the year ended September 30, 2022, warrant liabilities were measured at fair value on a recurring basis.

	Fair Value Measurements as of September 30, 2022			
	(Level 1)	(Level 2)	(Level 3)	Total
Liabilities				
Warrant liabilities	$ —	—	—	$ —

	Fair Value Measurements as of September 30, 2021			
	(Level 1)	(Level 2)	(Level 3)	Total
Liabilities				
Warrant liabilities	$ —	—	—	$ —

The following assumptions were used in determining the fair value of the warrant liabilities as of September 30, 2021:

	As of September 30, 2021
Remaining contractual term (years)	0.2 - 0.5
Common stock price volatility	60.6% - 62.5%
Risk-free interest rate	0.04%
Expected dividend yield	—

During the year ended September 30, 2021, the Company utilized its historical volatility in the valuation of warrant liabilities as it had sufficient trading activity.

The change in fair value of the warrant liabilities for the years ended September 30, 2022 and 2021 is as follows:

Fair value as of September 30, 2020	$ 950,151
Change in fair value	(950,151)
Fair value as of September 30, 2021	—
Change in fair value	—
Fair value as of September 30, 2022	$ —

As of September 30, 2022 and 2021, the recorded values of cash and cash equivalents, accounts payable and the insurance note payable approximate fair value due to the short-term nature of the instruments.

11. Stockholders' Equity

Preferred Stock

The Company is authorized to issue 10 million shares of preferred stock, par value $0.0001 as of September 30, 2022 and 2021. No shares of preferred stock were issued or outstanding as of September 30, 2022 or 2021.

Common Stock

The Company has authorized 250 million shares of common stock, $0.0001 par value per share as of September 30, 2022 and 2021. Each share of common stock is entitled to one voting right. Common stock owners are entitled to dividends when funds are legally available and declared by the Company's board of directors.

During the year ended September 30, 2022, a former employee forfeited 509,527 shares of common stock for no consideration.

Common Stock Offerings

On April 26, 2021, the Company closed an underwritten public offering of 9,200,000 shares of its common stock (inclusive of 1,200,000 shares that were sold pursuant to the underwriters' full exercise of their option to purchase additional shares of the Company's common stock), at a price to the public of $5.00 per share. The Company received net proceeds from the offering of approximately $42.6 million, after deducting the underwriting discounts and commissions and other estimated offering expenses payable by the Company.

Warrants

Below is a summary of the Company's issued and outstanding warrants as of September 30, 2022:

Expiration date	Exercise Price	Warrants Outstanding
July 6, 2023	$ 8.73	105,000
September 20, 2024	6.50	75,000
		180,000

	Warrants	Weighted-Average Exercise Price	Weighted-Average Remaining Contractual Life (in years)
Outstanding as of September 30, 2020	820,939	$ 19.44	
Issued	75,000	6.50	
Outstanding as of September 30, 2021	895,939	18.35	0.9
Expired	(715,939)	21.01	
Outstanding as of September 30, 2022	180,000	7.80	1.3
Exercisable as of September 30, 2022	180,000	7.80	1.3

During the year ended September 30, 2022, the Company did not issue warrants. During the year ended September 30, 2021, the Company issued 75,000 warrants in exchange for certain financial advisory services. The warrants vest over six-month periods, have exercise prices of $6.50 per share, and expire three years from issuance. The Company determined the warrants issued in exchange for financial advisory services met the scope exception in ASC 815 and are therefore classified as equity. The Company determined the initial fair value of the advisory warrants issued during the year ended September 30, 2021 to be $0.1 million, which was recognized as stock-based compensation expense over the vesting period of the warrants, see Note 13.

Key assumptions used to estimate the fair value of the advisory warrants granted during the year ended September 30, 2021 were as follows:

	As of September 30, 2021
Remaining contractual term (years)	3.0
Common stock price volatility	83.9 %
Risk-free interest rate	0.5 %
Expected dividend yield	—

As of September 30, 2022, the balance of unrecognized compensation expense associated with the advisory warrants was $0.

12. Net Loss Per Common Share

The following potentially dilutive securities outstanding for the years ended September 30, 2022 and 2021 have been excluded from the computation of diluted weighted-average shares outstanding, as they would be anti-dilutive:

	As of September 30,	
	2022	2021
Common stock purchase options	7,629,281	7,397,154
Restricted stock units	—	10,000
Common stock purchase warrants	180,000	895,939
	7,809,281	8,303,093

13. Stock-Based Compensation

The Company has a 2016 Consolidated Stock Incentive Plan (the "2016 Plan") and a 2019 Stock Incentive Plan (the "2019 Plan"), which provide for the issuance of incentive and non-incentive stock options, restricted and unrestricted stock awards, stock unit awards and stock appreciation rights. Options and restricted stock units granted generally vest over a period of one to four years and have a maximum term of ten years from the date of grant. Upon completion of the Ohr Acquisition, the Company assumed the awards outstanding under the NeuBase Therapeutics, Inc. 2018 Equity Incentive Plan.

As of September 30, 2022, an aggregate of6,018,136 shares of common stock were authorized under the 2019 Plan, subject to an "evergreen" provision that will automatically increase the maximum number of shares of Common Stock that may be issued under the terms of the 2019 Plan. As of September 30, 2022, 697,893 common shares were available for future grants under the 2019 Plan. As of September 30, 2022,291,667 shares of common stock were authorized under the 2016 Plan and 147,041 common shares were available for future grants under the 2016 Plan.

The Company recorded stock-based compensation expense in the following expense categories of its consolidated statements of operations for the years ended September 30, 2022 and 2021:

	Year Ended September 30,	
	2022	2021
General and administrative	$ 1,817,797	$ 2,369,287
Research and development	1,079,892	1,327,097
Total	$ 2,897,689	$ 3,696,384

Stock Options

Below is a table summarizing the options issued and outstanding as of and for the years ended September 30, 2022 and 2021:

	Stock Options	Weighted-Average Exercise Price	Weighted-Average Remaining Contractual Life (in years)	Total Aggregate Intrinsic Value
Outstanding at September 30, 2020	6,190,790	$ 2.76		
Granted	1,758,599	5.45		
Exercised	(47,052)	2.39		
Forfeited	(505,183)	6.80		
Outstanding at September 30, 2021	7,397,154	3.13		
Granted	1,768,365	2.14		
Exercised	(792,250)	0.00		
Forfeited	(743,988)	4.74		
Outstanding at September 30, 2022	7,629,281	3.08	6.8	$ 992,754
Exercisable as of September 30, 2022	5,017,592	$ 2.90	5.7	$ 992,754

As of September 30, 2022, the unrecognized compensation costs of $2.5 million will be recognized over an estimated weighted-average amortization period of 1.4 years.

The intrinsic value of stock options exercised during the years ended September 30, 2022 and 2021 was $0.4 million and $0.2 million, respectively.

The weighted average grant date fair value of options granted during the year ended September 30, 2022 and 2021 was $1.40 and $3.84, respectively.

Key assumptions used to estimate the fair value of the stock options granted during the years ended September 30, 2022 and 2021 included:

	Year Ended September 30,	
	2022	2021
Expected term of options (years)	5.1 - 6.1	6.1
Expected common stock price volatility	73.8% - 78.5%	82.7% - 83.7%
Risk-free interest rate	1.1% - 3.4%	0.6% - 1.3%
Expected dividend yield	—	—

During the fiscal year ended September 30, 2021, the Company granted a stock option to purchase 225,000 shares to a consultant, which was cancelled and reissued in June 2021, in recognition of future service to the Company as an employee. The exercisability and vesting of the stock options are subject to the consultant's effective date of employment with the Company, which had not yet occurred as of September 30, 2022, and as a result, the grant date of such option has not occurred under GAAP. Therefore, the number and fair value of the shares subject to this option are not reflected in the table summarizing the options issued and outstanding as of and for the year ended September 30, 2022 and did not have impact on unrecognized compensation costs or the estimated weighted-average amortization period above as of September 30, 2022.

Restricted Stock

A summary of the changes in the outstanding restricted stock during the years ended September 30, 2022 and 2021 is as follows:

	Unvested Restricted Stock	Weighted-Average Grant Date Fair Value Price
Unvested as of September 30, 2020	—	$ —
Granted	11,722	6.37
Vested	(11,722)	6.37
Unvested as of September 30, 2021	—	—
Granted	4,441	3.94
Vested	(4,441)	3.94
Unvested as of September 30, 2022	—	—
Total unrecognized expense remaining	$ —	
Weighted-average years expected to be recognized over	—	

The fair value of restricted stock that vested during the years ended September 30, 2022 and 2021 was $0.02 million and $0.1 million, respectively.

Restricted Stock Units

Below is a table summarizing the restricted stock units granted and outstanding as of and for the year ended September 30, 2022:

	Restricted Stock Units	Weighted-Average Grant Date Fair Value Price
Unvested as of September 30, 2021	10,000	$ 5.09
Forfeited	(10,000)	5.09
Unvested as of September 30, 2022	—	—
Total unrecognized expense remaining	$ —	
Weighted-average years expected to be recognized over	—	

14. Income Taxes

The Company has accumulated net losses since inception and has not recorded an income tax provision or benefit for the United States (U.S.) federal and state income taxes during the years ended September 30, 2022 and 2021. The components of the income tax benefits, net are as follows:

	For the Year Ended September 30,	
	2022	2021
Federal		
Current	$ —	$ —
Deferred	(6,780,606)	(7,919,279)
State and Local		
Current	—	—
Deferred	(2,746,540)	(3,015,187)
Change in valuation allowance	9,527,146	10,934,466
Income tax provision (benefit)	$ —	$ —

A reconciliation of income taxes at the statutory federal income tax rate to net income taxes included in the consolidated statements of operations is as follows:

	For the Year Ended September 30,	
	2022	2021
U.S. federal income tax expense at the statutory rate	(21.0)%	(21.0)%
State income taxes, net of federal taxes	(7.7)	(7.9)
Stock-based compensation	1.0	1.2
Return to provision adjustment	(0.5)	(14.2)
Other permanent items	—	(1.1)
Change in valuation allowance	28.2	43.0
Income tax provision (benefit)	— %	— %

The components of our deferred tax assets and liabilities are:

	September 30,	
	2022	2021
Deferred tax assets		
Net operating loss carryforwards	$ 22,461,508	$ 13,254,067
Stock-based compensation	4,072,264	3,779,555
Amortization	2,277,085	2,643,500
Service Warrant	180,276	147,168
Investment	263,073	141,098
Other	50,979	—
Lease liability	1,715,159	66,982
Total deferred tax assets	31,020,344	20,032,370
Deferred tax liabilities		
Depreciation	(158,317)	(127,449)
ROU assets	(1,635,483)	—
Prepaid expenses	—	(205,523)
Total deferred tax liabilities	(1,793,800)	(332,972)
Valuation allowance	(29,226,544)	(19,699,398)
Net deferred tax assets, net of allowances	$ —	$ —

As of each reporting date, the Company considers existing evidence, both positive and negative, that could impact its view with regard to future realization of deferred tax assets. The Company believes that it is more likely than not that the benefit for deferred tax assets

will not be realized. In recognition of this uncertainty, a full valuation allowance was applied to the deferred tax assets. The Company did not record a tax provision for the years ended September 30, 2022 and September 30, 2021 due to the Company's estimate that the effective tax rate for each year is 0%.

Future realization depends on the Company's future earnings, if any, the timing and amount of which are uncertain as of September 30, 2022. In the future, should management conclude that it is more likely than not that the deferred tax assets are partially or fully realizable, the valuation allowance would be reduced to the extent of such expected realization and the amount would be recognized as a deferred income tax benefit in the Company's consolidated statements of operations.

As of September 30, 2022, the Company had available federal and state total net operating loss carryforwards of approximately $77.1 million. Federal net operating loss carryforwards of approximately $77.1 million carryforward indefinitely. State operating loss carryforwards of approximately $77.1 million begin to expire in 2024.

Pursuant to Section 382 of the Internal Revenue Code of 1986, as amended, the Company's federal and state net operating loss carryforwards may be limited in the event a cumulative change in ownership of more than 50% occurs within a three-year period. The Company has not completed a Section 382 analysis regarding the limitation of net operating loss carryforwards. There is a risk that changes in ownership have occurred since the Company's formation. If a change in ownership were to have occurred, the NOL carryforwards could be limited or restricted. If limited, the related asset would be removed from the deferred tax asset schedule with a corresponding reduction in the valuation allowance. Due to the existence of the valuation allowance, limitations created by future ownership changes, if any, related to the Company's operations will not impact the Company's effective tax rate.

There are open statutes of limitations whereby our US Federal and state tax returns from inception of the Company are subject to audit by the respective taxing authorities in these jurisdictions for taxing authorities in federal and state jurisdictions to audit our tax returns from inception of the Company. There have been no material income tax-related interest or penalties assessed or recorded.

No liability for uncertain tax positions or related interest and penalties related to uncertain tax positions is reported in the Company's consolidated financial statements.

The Inflation Reduction Act of 2022 (the "IRA"), which was signed into law in August 2022, includes several provisions that are specifically applicable to corporations. Among other changes, it created a new corporate alternative minimum tax based on adjusted financial statement income and imposes a 1% excise tax on corporate stock repurchases. The effective date of these provisions is January 1, 2023. The Company does not expect the enactment of the IRA will have an impact on its consolidated financial statements.

15. Commitments and Contingencies

Employee Benefit Plans

The Company has a defined contribution savings and investment plan (the "Plan") as allowed under Sections 401(k) and 401(a) of the Internal Revenue Code. The Plan provides employees with tax deferred salary deductions and alternative investment options. Employees are eligible to participate upon employment and may apply for and secure loans from their account in the Plan. The Company contributed approximately $134,927 and $14,158, respectively, to the Plan during the years ended September 30, 2022 and 2021 as an employee match contribution.

Litigation

The Company may become involved in certain legal proceedings and claims which arise in the normal course of business. If an unfavorable ruling were to occur, there exists the possibility of a material adverse impact on the Company's results of operations, prospects, cash flows, financial position and brand. Costs associated with the Company's involvement in legal proceedings are expensed as incurred.

Securities Litigation

On February 14, 2018, plaintiff Jeevesh Khanna, commenced an action in the Southern District of New York, against Ohr, which entered into a merger agreement with Legacy NeuBase on January 2, 2019 and which merger closed on July 12, 2019, and several of

its current and former officers and directors, alleging that they violated federal securities laws between June 24, 2014 and January 4, 2018. On August 7, 2018, the lead plaintiffs, now George Lehman and Insured Benefit Plans, Inc., filed an amended complaint, alleging a putative class period of April 8, 2014 through January 4, 2018. The plaintiffs did not quantify any alleged damages in their complaint, but, in addition to attorneys' fees and costs, they seek to maintain the action as a class action and to recover damages on behalf of themselves and other persons who purchased or otherwise acquired Ohr common stock during the putative class period and purportedly suffered financial harm as a result. Ohr and the individuals dispute these claims and are defending the matter vigorously. On September 17, 2018, Ohr filed a motion to dismiss the complaint. On September 20, 2019, the district court issued an opinion and order granting the motion to dismiss. On October 23, 2019, the plaintiffs filed a notice of appeal of that order dismissing the action. After full briefing and oral argument, on October 9, 2020, the U.S. Court of Appeals for the Second Circuit issued a summary order affirming the district court's order granting the motion to dismiss and remanding the action to the district court to make a determination on the record related to plaintiffs' request for leave to file an amended complaint. On remand, the district court denied plaintiffs' subsequent request to amend and dismissed with prejudice plaintiffs' claims. On December 16, 2020, plaintiffs filed a notice of appeal of that order denying plaintiffs leave to amend. On December 16, 2021, the Second Circuit affirmed the decision and order of the district court denying plaintiffs' motion for leave to amend, thereby dismissing the appeal and action in its entirety. Plaintiffs have neither sought reconsideration of the Second Circuit's decision nor filed a writ of certiorari for review by the Supreme Court. This matter is now considered closed.

Derivative Lawsuit

On May 3, 2018, plaintiff Adele J. Barke, derivatively on behalf of Ohr, commenced an action against Michael Ferguson, Orin Hirschman, Thomas M. Riedhammer, June Almenoff and Jason Slakter in the Supreme Court, State of New York, alleging that the action was brought in the right and for the benefit of Ohr seeking to remedy their "breach of fiduciary duties, corporate waste and unjust enrichment that occurred between June 24, 2014 and the present." It does not quantify any alleged damages.

On March 30, 2022, plaintiff filed a notice of voluntary dismissal of the complaint in this action. This matter is now considered closed.

Joint Proxy Statement Lawsuit

Following the issuance of the preliminary joint proxy statement/prospectus related to the merger of the Company and Ohr, on March 18, 2019, the Gomez Action was filed by an individual shareholder in the United States District Court for the Southern District of New York against Ohr and its board of directors. The plaintiff in the Gomez Action alleges that the preliminary joint proxy/prospectus statement filed by Ohr with the SEC on March 8, 2019 contained false and misleading statements and omitted material information in violation of Section 14(a) of the Exchange Act and SEC Rule 14a-9 promulgated thereunder, and further that the individual defendants are liable for those alleged misstatements and omissions under Section 20(a) of the Exchange Act.

On March 19, 2019, the Barke Action was filed in the United States District Court for the Southern District of New York asserting similar Section 14(a) and Section 20(a) claims against Ohr's board of directors and additionally naming NeuBase and Ohr Acquisition Corp., but not Ohr, as defendants. On March 20, 2019, the Wheby Action was filed in the United States District Court for District of Delaware asserting similar claims under Section 14(a) and Section 20(a) and naming as defendants Ohr and its board of directors, NeuBase, and Ohr Acquisition Corp. On March 20, 2019, the Lowinger Action was filed in the Court of Chancery of the State of Delaware asserting a breach of fiduciary duty claim against Ohr's board of directors arising out of the same facts and circumstances regarding certain alleged omissions in the preliminary joint proxy/prospectus statement. On April 4, 2019, the Garaygordobil Action was filed in the United States District Court for the Southern District of New York asserting similar Section 14(a) and Section 20(a) claims against Ohr and its board of directors. Each of the Gomez, Barke, Garaygordobil, and Lowinger Actions have been dismissed, and on July 12, 2019, the Company and Ohr consummated the Merger. On March 23, 2022, plaintiffs in the Wheby Action filed a notice of voluntary dismissal of the complaint and this case was closed.

16. Subsequent Events

In October 2022, the Company announced a strategic restructuring to expand its focus to include the advancement of the differentiated gene editing capabilities of its platform. As part of the development pipeline shift to gene editing, the Company will defer preclinical activities for its DM1, HD, and KRAS programs, hold plans to submit an IND application for DM1 to the FDA, and pursue collaborative initiatives, including partnerships, for these programs. The Company estimates that it will incur total expenses relating to the restructuring of approximately $0.5 million, consisting of severance and termination-related costs, and expects to record a significant portion of these charges in the fourth quarter of calendar year 2022. This restructuring plan is expected to extend the Company's cash runway into the second quarter of calendar year 2024 based on current operating plans and estimates. As part of the cost reduction plan, the Company implemented a cross-functional reduction of approximately 60% of the then-current workforce. In October 2022, the Company also announced certain changes to its leadership team, including the resignations of the Head of Research and Development and Chief Medical Officer and President and Chief Operating Officer, as well as the appointment of Dr. Dov A. Goldstein, a member of the Company's board of directors since 2019, as Chairperson of the Board.

ITEM 9. CHANGES IN AND DISAGREEMENTS WITH ACCOUNTANTS ON ACCOUNTING AND FINANCIALDISCLOSURE

None.

ITEM 9A. CONTROLS AND PROCEDURES

Evaluation of Disclosure Controls and Procedures

Our disclosure controls and procedures are designed to ensure that information required to be disclosed by us in reports that we file or submit under the Exchange Act, is recorded, communicated to our management to allow timely decisions regarding required disclosure, summarized and reported within the time periods specified in the SEC's rules and forms.

Under the supervision and with the participation of our management, including our Chief Executive Officer ("CEO") and Chief Financial Officer ("CFO") who serve as the principal executive officer and as the principal financial officer, respectively, we conducted an evaluation of the effectiveness of our disclosure controls and procedures, as such term is defined under Rules 13a-15(e) and 15d-15(e) under the Exchange Act, as of September 30, 2022. Based on this evaluation, our CEO and CFO concluded that our disclosure controls and procedures were effective as of September 30, 2022.

Management's Report on Internal Control Over Financial Reporting

Management is responsible for establishing and maintaining adequate internal control over financial reporting, as such term is defined in Exchange Act Rule 13a-15(f). Our internal control over financial reporting is a process designed by, under the supervision and, with the participation of our CEO and CFO who serve as our principal executive officer and principal financial officer, respectively, overseen by our board of directors and implemented by our management and other personnel, to provide reasonable assurance regarding the reliability of our financial reporting and the preparation of our financial statements for external purposes in accordance with generally accepted accounting principles. Our internal control over financial reporting includes policies and procedures that:

(1) Pertain to the maintenance of records that in reasonable detail accurately and fairly reflect the transactions and dispositions of our assets;

(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 are being made only in accordance with authorizations of our management and directors; and

(3) Provide reasonable assurance regarding prevention or timely detection of unauthorized acquisition, use or disposition of our assets that could have a material effect on our financial statements.

Because of its inherent limitations, our 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 performed an assessment of the effectiveness of our internal control over financial reporting as of September 30, 2022 using criteria established in the *Internal Control-Integrated Framework (2013)* issued by the Committee of Sponsoring Organizations of the Treadway Commission ("COSO"). Based on this assessment, management determined that, as of September 30, 2022, our internal control over financial reporting was effective. We reviewed the results of management's assessment with the Audit Committee of our board of directors.

Inherent Limitations on Effectiveness of Controls

A control system, no matter how well conceived and operated, can provide only reasonable, not absolute, assurance that the objectives of the control system are met. Because of inherent limitations, internal control over financial reporting may not prevent or detect misstatements. Accordingly, our disclosure controls and procedures are designed to provide reasonable, not absolute, assurance that the objectives of our disclosure system are met. 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.

Because we are a non-accelerated filer and smaller reporting company, Marcum LLP, our independent registered public accounting firm, is not required to attest to or issue a report on the effectiveness of our internal control over financial reporting.

Changes in Internal Control over Financial Reporting

There have been no changes in our internal control over financial reporting that materially affected, or are reasonably likely to materially affect, our internal control over financial reporting during the quarterly period ended September 30, 2022.

ITEM 9B. OTHER INFORMATION

None.

ITEM 9C. DISCLOSURE REGARDING FOREIGN JURISDICTIONS THAT PREVENT INSPECTIONS

Not applicable.

PART III.

ITEM 10. DIRECTORS, EXECUTIVE OFFICERS AND CORPORATE GOVERNANCE

Name	Age[1]	Position
Non-Employee Directors		
Dov A. Goldstein, M.D. [2]	54	Director
Franklyn G. Prendergast, M.D., Ph.D. [2] [4]	77	Director
Gerald J. McDougall[3] [4]	55	Director
Eric I. Richman [2] [3] [4]	61	Director
Eric J. Ende, M.D.[3] [4]	54	Director
Executive Officers		
Dietrich A. Stephan, Ph.D.	53	Chief Executive Officer and Director
Todd P. Branning	52	Chief Financial Officer

(1) Age as of September 30, 2022.
(2) Member of the Audit Committee.
(3) Member of the Nominating and Corporate Governance Committee.
(4) Member of the Compensation Committee.

Board of Directors

Our Amended and Restated Certificate of Incorporation provides that the Board is to be divided into three classes as nearly equal in number as possible, with directors in each class serving staggered three-year terms. The total Board size is currently fixed at six directors. The Class I directors (whose terms expire at the 2024 annual meeting of stockholders) are Dov A. Goldstein, M.D. and Eric I. Richman, M.B.A. The Class II directors (whose terms expire at the 2025 annual meeting of stockholders) are Gerald J. McDougall and Dietrich A. Stephan, Ph.D. The Class III directors (whose terms expire at the 2023 annual meeting of stockholders) are Franklyn G. Prendergast, M.D., Ph.D and Eric J. Ende, M.D.

Dietrich A. Stephan, Ph.D., age 53, has been our Chief Executive Officer since July 2019, and he served as our President from July 2019 until June 2022 and as our Chairperson from July 2019 until October 2022. Dr. Stephan was also the founder and Chief Executive Officer of Legacy NeuBase. Before founding Legacy NeuBase, Dr. Stephan was founder and Chief Executive Officer of LifeX Holdings, a healthcare startup incubator, and a tenured full professor of Human Genetics at the University of Pittsburgh. He served as Chair of the Department of Human Genetics at the University of Pittsburgh from 2013 to 2018, and earlier, as the founding Director of the Neurogenomics Division at the Translational Genomics Research Institute (TGen) and Deputy Director of Discovery Research at TGen. Dr. Stephan is Chairman of Peptilogics, a privately held peptide therapeutics company; a director of Sharp Edge Labs, a privately held small-molecule genetic disease therapeutics company; a director of FarmaceuticalRx, a privately held pharmaceutical company developing cannabinoid-based therapies; and partner in Cyto Ventures, an early-stage investment fund. Dr. Stephan received his B.S. in Biology from Carnegie Mellon University and his Ph.D. in Human Genetics from the University of Pittsburgh. He also completed a fellowship at the National Human Genome Research Institute.

We believe that Dr. Stephan's role as CEO of our Company, experience as the founder of Legacy NeuBase and in the biopharmaceutical industry qualify him to serve as a member of our Board.

Dov A. Goldstein, M.D., age 54, has served as a member of our Board since July 2019 and as Chairperson of our Board since October 2022. Dr. Goldstein is currently the Chief Financial Officer of BioAge Labs, Inc., as well as serving as on the Board of Directors of Gain Therapeutics, Inc. (Nasdaq: GANX) and Coya Therapeutics, Inc. He was previously Chief Financial Officer, Chief Business Officer and a director of Indapta Therapeutics, Inc., and he served as Chief Executive Officer and a director of RIGImmune, Inc., as well as a private investor. Prior to that, he was the Chief Financial Officer at Schrödinger, LLC from the fourth quarter of 2017 to the second quarter of 2018. Dr. Goldstein served as a Managing Partner at Aisling Capital, a private investment firm, from 2014 to October 2017, Partner from 2008 to 2014 and a principal at Aisling Capital from 2006 to 2008. Dr. Goldstein served as the Chief Financial Officer of Loxo Oncology, Inc. between July 2014 and January 2015, and was its acting Chief Financial Officer from January 2015 to May 2015. From 2000 to 2005, Dr. Goldstein served as Chief Financial Officer of Vicuron Pharmaceuticals, Inc., which was acquired by Pfizer, Inc. in September 2005. Prior to joining Vicuron, Dr. Goldstein was Director of Venture Analysis at HealthCare Ventures. Dr. Goldstein also completed an internship in the Department of Medicine at Columbia-Presbyterian Hospital. He previously served as a director of ADMA Biologics, Inc. (Nasdaq: ADMA), Loxo Oncology, Inc. (Nasdaq: LOXO) (which was acquired by Eli Lilly), Esperion Therapeutics, Inc. (Nasdaq: ESPR), and Cempra, Inc. (Nasdaq: CEMP) (which was acquired by Melinta Therapeutics, Inc.). Dr. Goldstein received a B.S. from Stanford University, an M.B.A. from Columbia Business School and an M.D. from Yale School of Medicine.

We believe that Dr. Goldstein's medical training and his experience in the biopharmaceutical industry as a venture capital investor, as a biotechnology executive and a member of the boards of directors of other biopharmaceutical companies, as well as his experience in financial matters and his service on audit and compensation committees, qualify him to serve as a member of our Board.

Franklyn G. Prendergast, M.D., Ph.D., age 77, has served as a member of our Board since July 2019. Dr. Prendergast retired from the Mayo Clinic in 2014 and is currently the Emeritus Edmond and Marion Guggenheim Professor of Biochemistry and Molecular Biology and Emeritus Professor of Molecular Pharmacology and Experimental Therapeutics at Mayo Medical School. At the Mayo Clinic, he served in several capacities, most significantly, as the Director for Research 1989 – 1992, inclusive, Member of the Mayo Clinic Board of Governors and Executive Committee 1991 – 2007, and Member of the Mayo Clinic Board of Trustees from 1991-2009, inclusive. From 1994 to 2006, he served as a director of Mayo Clinic Cancer Center. He also previously held several other teaching positions at the Mayo Medical School from 1975 through 2014. Dr. Prendergast has served for the National Institute of Health on numerous study section review groups; as a charter member of the Board of Advisors for the Division of Research Grants, now the Center for Scientific Review; the National Advisory General Medical Sciences Council; and the Board of Scientific Advisors of the National Cancer Institute. He held a Presidential Commission for service on the National Cancer Advisory Board. Dr. Prendergast also has served in numerous other advisory roles for the National Institute of Health and the National Research Council of the National Academy of Sciences. He is a member of the board of directors of Immunome, Inc. (Nasdaq: IMNM) and its nominating and corporate governance committee, a member of the board of director of Cancer Genetics, Inc. (Nasdaq: CGIX) and its audit, compensation and nominating committees, a member of the board of directors of Lantern Pharma, Inc. (Nasdaq: LTRN) and its audit and nominating committees, and a member of the board of directors of the Infectious Disease Research Institute (IDRI). He previously served on the board of directors of Eli Lilly & Co. from 1995 to 2017 and was a member of Eli Lilly's science and technology committee and public policy and compliance committee, and he previously served on the board of directors of Medibio Limited (ASX: MEB) (OTCQB: MDBIF). Dr. Prendergast obtained his medical degree with honors from the University of West

Indies and attended Oxford University as a Rhodes Scholar, earning an M.A. degree in physiology. He obtained his Ph.D. in Biochemistry at the University of Minnesota.

We believe that Dr. Prendergast's extensive experience and expertise as a medical clinician, researcher and academician, particularly in the areas of oncology and personalized medicine, developed through his roles with Mayo Clinic, including serving as director of the Mayo Clinic Cancer Center and the Mayo Clinic Center for Individualized Medicine, qualify him to serve as a member on our Board.

Eric I. Richman, age 61, has served as a member of our Board since July 2019. Mr. Richman currently serves as a senior advisor and a member of the Board of Directors of Gain Therapeutics, Inc. (Nasdaq: GANX), a biotechnology company, where he previously served as its Chief Executive Officer from July 2020 to September 2022. Mr. Richman was previously a Venture Partner at Brace Pharma Capital, a life science venture capital firm, from January 2016 to September 2018 and is involved with several private and public biotechnology companies. He also served as Chief Executive Officer of Tyrogenex Inc., a biopharmaceutical company, from 2016 to 2018. Mr. Richman served as the President and Chief Executive Officer of PharmAthene, Inc. ("PharmAthene"), subsequently acquired by Altimmune, Inc., between October 2010 and March 2015. He also served on PharmAthene's board of directors, when the company was listed on The New York Stock Exchange, from 2010 to 2017. Prior to joining PharmAthene, Mr. Richman held various commercial and strategic positions of increasing responsibility over a 12-year period at MedImmune, Inc. from its inception and was Director, International Commercialization at that company. Mr. Richman served as a director of Lev Pharmaceuticals, Inc. (acquired by Viropharma) and as Chairman of its Commercialization Committee and served as a director of American Bank Incorporated (acquired by Congressional Bancshares). Mr. Richman currently serves as a member of the board of directors of NovelStem International Corp. (OTCMKTS: NSTM), is the co-founder and Chairman of InFuse Holdings and LabConnect, Inc. where he serves as the Chairman of the Board, and previously served as a director of ADMA Biologics, Inc. (Nasdaq: ADMA) (as well as a member of such board's audit, compensation and governance and nominating committees). Mr. Richman received a B.S. in Biomedical Science from the Sophie Davis School of Biomedical Education (CUNY Medical School) and a M.B.A. from the American Graduate School of International Management.

We believe that Mr. Richman's experience in the biotechnology industry, including his successful efforts in gaining FDA drug approvals, as well as his recent experience as an executive officer of Gain Therapeutics and PharmAthene and his service on numerous public and private company boards of directors and on the committees of such boards, provide him with the qualifications and skills to serve as a member of our Board.

Gerald J. McDougall, age 55, has served as a member of our Board since May 2021. Mr. McDougall is a retired Senior Partner in PricewaterhouseCoopers's Health Sciences Practice, where he provided services for over 25 years to academic medical centers, bioscience companies, pharmaceutical companies, research universities, colleges, health systems and other research organizations. In this role, he linked scientific breakthroughs to clinical applications for the benefit of patients and society in many parts of healthcare (especially cancer and precision medicine) and drew on deep, trusted, long-standing relationships with leading scientists, entrepreneurs, academics industry groups and philanthropists. Mr. McDougall's experience includes a broad range of research business and compliance services, including strategic and business planning, financial analysis, research compliance, clinical research operations improvement, and information systems implementation services. In addition, his experience includes support services to the entire research continuum, from grant-funded basic science research to translational research and clinical trials, including a dedicated group focusing on Clinical Research Consulting services and Global oncology. Mr. McDougall has been involved in numerous volunteer and trade organizations throughout his career, including as a board member of the Infectious Disease Research Institute (IDRI), as a board member of the Multiple Myeloma Research Foundation (MMRF) and most recently as a board member of the American Society of Clinical Oncology (ASCO). Mr. McDougall received a Bachelor's degree in business from Northeastern University.

We believe that Mr. McDougall's 25 years of experience as a driving force behind large-scale strategic alliances, joint ventures, and industry partnerships across the healthcare industry to advance innovations in precision medicine and cancer qualify him to serve as a member of our Board.

Eric J. Ende, M.D., age 54, has served as a member of our Board since January 2022. Dr. Ende is the President of Ende BioMedical Consulting Group, Inc., a privately held consulting company focused on the life sciences industry, a position he has held since 2009. Since May 2017, Dr. Ende has been a member of the board of directors of Matinas BioPharma, Inc. (NYSE: MTNB), a clinical-stage biopharmaceutical company, where he chairs the Compensation Committee and serves on the Audit and Nominating & Corporate Governance Committees. Since December 2018, Dr. Ende has been a member of the board of directors of Avadel plc (Nasdaq:

AVDL), a clinical-stage biopharmaceutical company, where he chairs the Nominating & Corporate Governance Committee and serves on the Audit Committee. From November 2019 to June 2020, Dr. Ende served on the board of directors of Progenics Pharmaceuticals, Inc. (Nasdaq: PGNX), a biopharmaceutical company, where he also chaired the Compensation Committee and was a member of the Audit and Science Committees. From 2010 to 2011, Dr. Ende served on the board of directors and as a member of the Audit and Risk Management Committees of Genzyme Corp. (Nasdaq: GENZ), a biotechnology company, until it was acquired in 2011 by Sanofi S.A. From 2002 through 2008, Dr. Ende was the senior biotechnology analyst at Merrill Lynch; from 2000 through 2002, he was the senior biotechnology analyst at Bank of America Securities; and from 1997 to 2000, he was a biotechnology analyst at Lehman Brothers. Dr. Ende received an MBA in Finance and Accounting from NYU - Stern Business School in 1997, an MD from Mount Sinai School of Medicine in 1994, and a BS in Biology and Psychology from Emory University in 1990.

We believe that Dr. Ende's extensive experience and expertise as a biotechnology executive and a member of the boards of directors of other biopharmaceutical companies, as well as his experience in financial matters and his service on audit and compensation committees qualify him to serve as a member on our Board.

Executive Officers

Dietrich A. Stephan, Ph.D. For a brief biography of Dr. Stephan, please see "DIRECTORS, EXECUTIVE OFFICERS AND CORPORATE GOVERNANCE – Board of Directors" above.

Todd P. Branning, age 52, has served as our Chief Financial Officer since January of 2022. Mr. Branning previously served as Chief Financial Officer of Phathom Pharmaceuticals, Inc., a publicly traded late clinical-stage biopharmaceutical company, from July 2020 through June 2021. Before that, Mr. Branning served as Senior Vice President, Chief Financial Officer of Amneal Pharmaceuticals, Inc., a publicly traded pharmaceutical company, from January 2019 through March 2020. Prior to joining Amneal, he was Senior Vice President, Chief Financial Officer of the global generic medicines division at Teva Pharmaceutical Industries Ltd., a multinational generic pharmaceuticals company, from August 2016 to March 2018. Mr. Branning has also held financial leadership roles at Allergan plc, PricewaterhouseCoopers LLP, PPG Industries, Inc. and Merck & Co., Inc. He received his BBA from the University of Miami and MBA from Carnegie Mellon University. Mr. Branning is also a Certified Public Accountant and has completed a CFO certification program at The Wharton School at the University of Pennsylvania.

Family Relationships

There are no family relationships between any of our directors or executive officers.

Code of Ethics

We have adopted a Code of Conduct and Ethics, as amended, that applies to our Chief Executive Officer and to all of our other officers, directors and employees. The Code of Conduct and Ethics is available in the Governance section of the Investors page on our website at www.neubasetherapeutics.com. We will disclose future amendments to, or waivers from, certain provisions of our Code of Conduct and Ethics, if any, on the above website within four business days following the date of such amendment or waiver.

Audit Committee

We have a separately-designated standing audit committee established in accordance with Section 3(a)(58)(A) of the Exchange Act. Our Audit Committee currently consists of Dr. Dov A. Goldstein (Chair), Dr. Franklyn G. Prendergast, and Eric I. Richman. All are non-employee directors and are considered independent under the applicable independence standard promulgated by Nasdaq and the SEC. Our board of directors has currently designated each of Dr. Goldstein and Mr. Richman as an "audit committee financial expert" as defined in Item 407(d)(5)(ii) of Regulation S-K. We believe that the Audit Committee members are capable of analyzing and evaluating our financial statements and understanding internal control over financial reporting.

Compensation Committee

Our Compensation Committee is currently comprised of Eric I. Richman (Chair), Dr. Franklyn G. Prendergast, Gerald J. McDougall, and Dr. Eric Ende, each of whom is an independent director for purposes of the Nasdaq listing standards. The Compensation

Committee reviews and recommends executive compensation, including changes therein, and administers our equity compensation plans.

Compensation Committee Membership, Interlocks and Insider Participation

Each member of the Compensation Committee is a "non-employee" director within the meaning of Rule 16b-3 of the rules promulgated under the Exchange Act. None of Mr. Richman, Dr. Prendergast, Mr. McDougall or Dr. Ende is an officer or employee of ours, was formerly an officer of ours or had any relationship requiring disclosure by us under Item 404 of Regulation S-K. No interlocking relationship as described in Item 407(e)(4) of Regulation S-K exists between any of our executive officers or Compensation Committee members, on the one hand, and the executive officers or compensation committee members of any other entity, on the other hand, nor has any such interlocking relationship existed in the past.

Nominating and Corporate Governance Committee

Our Nominating and Corporate Governance Committee currently consists of Eric I. Richman (Chair), Gerald J. McDougall, and Dr. Eric Ende, each of whom is a non-employee director and is considered independent under the applicable independence standard promulgated by Nasdaq and the SEC.

Delinquent Section 16(a) Reports

Section 16(a) of the Exchange Act requires our directors, executive officers and holders of more than 10% of our Common Stock to file with the SEC initial reports of ownership and reports of changes in the ownership of Common Stock and other equity securities of the Company. Such persons are required to furnish us with copies of all Section 16(a) filings.

Based upon a review of the copies of the reports filed with the SEC we believe that our directors, officers and holders of more than 10% of our common stock complied with all applicable filing requirements during the 2022 fiscal year other than the following Form 4 filing: Mr. Richman on March 7, 2022. The Form 4 filing primarily relates to the purchase of Company common stock on the open market and was untimely filed.

ITEM 11. EXECUTIVE COMPENSATION

Summary Compensation Table

The following table sets forth the compensation paid by us during the years ended September 30, 2022 and 2021 to (1) our principal executive officer during the last completed fiscal year and (2) our two most highly compensated executive officers other than our principal executive officer who were serving as executive officers as of September 30, 2022 (collectively our "Named Executive Officers"):

Name and Position	Year	Salary	Bonus[1]	Stock Awards	Option Awards[2]	Non-Equity Incentive Plan Compensation	All Other Compensation[3]	Total
Dietrich A. Stephan, Ph.D., Chief Executive Officer	2022	$ 536,798	$ 247,000	$ —	$ 318,240	$ —	$ 52,782	$ 1,154,820
	2021	$ 469,149	$ 196,875	$ —	$ 799,886	$ —	$ 47,808	$ 1,513,718
Todd P. Branning, Chief Financial Officer	2022	$ 310,577	$ —	$ —	$ 495,000	$ —	$ 13,446	$ 819,023
	2021	$ —	$ —	$ —	$ —	$ —	$ —	$ —
William R. Mann, Ph.D., Former President and Chief Operating Officer[4]	2022	$ 422,221	$ 168,000	$ —	$ 62,587	$ —	$ 30,111	$ 682,919
	2021	$ 381,923	$ 62,500	$ —	$ 61,750	$ —	$ 25,975	$ 532,148

(1) In the fiscal year ended September 30, 2021, the Company awarded a cash bonus to Dr. Stephan in the amount of $196,875 for services rendered during the calendar year ended December 31, 2020, which was not committed during such fiscal year and was approved by the Compensation Committee of the Board during May 2021. Pursuant to the offer letter between the Company and Dr. Stephan, Dr. Stephan will be eligible for a discretionary annual bonus for service with the Company during calendar year 2022 equal to 35% of his base salary, as determined at the discretion of the Compensation Committee. Dr. Mann and Mr. Branning are both eligible for an annual performance bonus with a target amount equal to 40% of their

respective base salaries, provided that they remain employed by the Company on the date that such bonus would be paid, and subject to certain other conditions, as applicable, as determined at the discretion of the Compensation Committee.

(2) The amounts in this column reflect the aggregate grant date fair value of equity awards granted during the applicable fiscal year, calculated in accordance with FASB ASC Topic 718 and using a Black-Scholes valuation model. Assumptions used in the calculation of these amounts are included in Note 13 of the audited consolidated financial statements included in this Form 10-K.

(3) Consists of other compensation amounts for each named executive officer listed in the table entitled "All Other Compensation" below, including our contributions to such officers' 401(k), group term life insurance policy premiums, health benefits and paid time off buy back.

(4) Dr. Mann resigned from the Company effective October 31, 2022.

All Other Compensation

Name	Year	401(k) Company	Group Term	Health Benefits	Life Insurance	Paid Time Off Buy Back	Total Other Compensation
Dietrich A. Stephan, Ph.D.	2022	$ 10,694	$ —	$ 18,155	$ 23,933	—	$ 52,782
Chief Executive Officer	2021	$ —	$ —	$ 25,716	22,092	—	$ 47,808
Todd P. Branning	2022	$ 7,356	$ 439	$ 5,651	—	—	$ 13,446
Chief Financial Officer	2021	$ —	$ —	$ —	—	—	$ —
William R. Mann, Ph.D.	2022	$ 8,287	$ 684	$ 21,140	—	—	$ 30,111
Former President and Chief Operating Officer	2021	$ —	$ 454	25,222	—	—	$ 25,676

Narrative Disclosure to Summary Compensation Table

Base Salary

In general, base salaries for our Named Executive Officers are approved by the compensation committee of our board of directors (the "Compensation Committee") and are initially established through arm's length negotiation at the time the executive is hired, taking into account such executive's qualifications, experience, prior salary and market pay levels. Base salaries of our Named Executive Officers are approved and reviewed annually by our Compensation Committee and adjustments to base salaries are based on the scope of an executive's responsibilities, individual contribution, prior experience and sustained performance. Decisions regarding salary increases may take into account an executive officer's current salary, equity ownership, and the amounts paid to an executive officer's peers inside our company by conducting an internal analysis, which compares the pay of an executive officer to other members of the management team. Base salaries are also reviewed in the case of promotions or other significant changes in responsibility. Base salaries are not automatically increased if the Compensation Committee believes that other elements of the Named Executive Officer's compensation are more appropriate in light of our stated objectives. This strategy is consistent with our intent of offering compensation that is both cost-effective, competitive and contingent on the achievement of performance objectives.

With the exception of the increase of Dr. Stephan's annual salary to $540,000 in January 2022, our Named Executive Officers did not receive base salary increases in fiscal 2022 or 2021.

Equity Compensation

The Compensation Committee considers equity incentives to be important in aligning the interests of our executive officers with those of our stockholders. As part of our pay-for-performance philosophy, our compensation program tends to emphasize the long-term equity award component of total compensation packages paid to our executive officers.

Because vesting is based on continued employment, our equity-based incentives also encourage the retention of our Named Executive Officers through the vesting period of the awards. In determining the size of the long-term equity incentives to be awarded to our Named Executive Officers, we take into account a number of internal factors, such as the relative job scope, the value of existing long-

term incentive awards, individual performance history, prior contributions to us and the size of prior grants. Based upon these factors, the Compensation Committee determines the size of the long-term equity incentives at levels it considers appropriate to create a meaningful opportunity for reward predicated on the creation of long-term stockholder value.

To reward and retain our Named Executive Officers in a manner that best aligns employees' interests with stockholders' interests, we use stock options and restricted stock unit awards as the primary incentive vehicles for long-term compensation. We believe that stock options and restricted stock unit awards are effective tools for meeting our compensation goal of increasing long-term stockholder value by tying the value of the stock to our future performance. Because employees are able to profit from stock options only if our stock price increases relative to the stock option's exercise price, we believe stock options provide meaningful incentives to employees to achieve increases in the value of our stock over time.

We use stock options, and may also use restricted stock unit awards, to compensate our Named Executive Officers both in the form of initial grants in connection with the commencement of employment and annual refresher grants. While we intend that the majority of equity awards to our employees be made pursuant to initial grants or our annual grant program, the Compensation Committee retains discretion to grant equity awards to employees at other times, including in connection with the promotion of an employee, to reward an employee, for retention purposes or for other circumstances recommended by management or the Compensation Committee.

The exercise price of each stock option grant is the fair market value of our common stock on the grant date. Time-based stock option awards granted to Dr. Stephan provided for vesting over a four-year period in equal monthly installments over 48 months. Dr. Stephan's time-based stock option awards vested immediately and became exercisable upon the consummation of the Merger. Time-based stock option awards to Mr. Branning and Dr. Mann provided for vesting over a four-year period as follows: 25% of the shares underlying the options vest on the first anniversary of the date of the respective vesting commencement dates and the remainder of the shares underlying the options vest in equal monthly installments over the respective remaining 36 months thereafter. From time to time, our Compensation Committee may, however, determine that a different vesting schedule is appropriate. We do not have any stock ownership requirements for our Named Executive Officers.

Compensation Consultant

Our Compensation Committee has the authority to retain the services and obtain the advice of external advisors, including compensation consultants, legal counsel and other advisors to assist in the evaluation of executive officer compensation. In 2022, our Compensation Committee engaged Compensia, an independent executive compensation consulting firm, to review our executive compensation policies and practices and to conduct an executive compensation market analysis.

During fiscal 2022, Compensia reviewed and advised on principal aspects of our executive compensation program, including:

- Assisting in developing a peer group of publicly traded companies to be used to help assess executive compensation;
- Assisting in developing a competitive compensation strategy and consistent executive compensation assessment practices relevant to a public company, including review and recommendation of the annual performance-based cash incentive program as well as the equity strategy for the Company covering dilution, grant levels and type of equity; and
- Meeting with the Compensation Committee to review elements of executive compensation including the competitiveness of the executive compensation program.

Our Compensation Committee has assessed the independence of Compensia consistent with the Nasdaq listing requirements and has concluded that the engagement of Compensia does not raise any conflicts of interest.

Outstanding Equity Awards as of September 30, 2022

The following table shows information regarding our outstanding equity awards as of September 30, 2022 for the Named Executive Officers:

	Option Awards					Stock Awards			
Name	Number of Securities Underlying Unexercised Options Exercisable (#)	Number of Securities Underlying Unexercised Options Non-Exercisable (#)	Equity Incentive Plan Awards: Number of Securities Underlying Unexercised Unearned Options (#)	Option Exercise Price ($)	Option Expiration Date	Number of Shares or Units of Stock That Have Not Vested (#)	Market Value of Shares or Units of Stock That Have Not Vested ($)	Equity Incentive Plan Awards: Number of Unearned shares, Units or Other Rights That Have Not Vested (#)	Equity Incentive Plan Awards: Market or Payout Value of Unearned Shares, Units or Other Rights That Have Not Vested ($)
Dietrich A. Stephan, Ph.D.	2,519,680	—	—	$ 0.0011	12/31/2023 (1)	—	—	—	—
	72,154	92,771 (2)	—	$ 6.92	12/29/2030 (4)	—	—	—	—
	—	306,000 (3)	—	$ 1.58	01/24/2032 (4)	—	—	—	—
Todd P. Branning	—	300,000 (5)	—	$ 2.52	01/10/2032 (4)	—	—	—	—
William R. Mann, Ph.D.	94,791	80,209 (6)	—	$ 7.46	7/27/2030 (4)	—	—	—	—
	6,770	18,230 (7)	—	$ 3.49	8/16/2031 (4)	—	—	—	—
	—	60,180 (3)	—	$ 1.58	01/24/2032 (4)	—	—	—	—

(1) The options have a term of five years from the date of issuance.
(2) The stock options vest over four years from the date of grant on December 29, 2020, with 25% of the shares subject to the options vesting on the first anniversary of the date of grant and the remainder vesting in 36 monthly tranches thereafter.
(3) The stock options vest over four years from the date of grant on January 24, 2022, with 25% of the shares subject to the options vesting on the first anniversary of the date of grant and the remainder vesting in 36 monthly tranches thereafter.
(4) The options have a term of 10 years from the date of issuance.
(5) The stock options vest over four years from the date of grant on January 10, 2022, with 25% of the shares subject to the options vesting on the first anniversary of the date of grant and the remainder vesting in 36 monthly tranches thereafter.
(6) The stock options vest over four years from the date of grant of July 27, 2020, with 25% of the shares subject to the options vesting on the first anniversary of the date of grant and the remainder vesting in 36 monthly tranches thereafter.
(7) The stock options vest over four years from the date of grant of August 16, 2021, with 25% of the shares subject to the options vesting on the first anniversary of the date of grant and the remainder vesting in 36 monthly tranches thereafter.

Payments Upon Termination or Change In Control

We have entered into employment agreements with each of our Named Executive Officers. These agreements set forth the individual's base salary, annual incentive opportunities, equity compensation and other employee benefits, which are described in this Executive Compensation section. All employment agreements provide for "at-will" employment, meaning that either party can terminate the employment relationship at any time, although our agreements with our Named Executive Officers provide that they would be eligible for severance benefits in certain circumstances following a termination of employment without cause. Our Compensation Committee approved the severance benefits to mitigate certain risks associated with working in a biopharmaceutical company at our current stage of development and to help attract and retain qualified executives.

Dietrich A. Stephan, Ph.D.

Historically, Legacy NeuBase had one executive officer, Dietrich A. Stephan, Ph.D., President and Chief Executive Officer. Upon the formation of Legacy NeuBase and until the date that Legacy NeuBase and Dr. Stephan entered into an employment agreement, in recognition of our low levels of operating cash flow and Dr. Stephan's status as a stockholder of Legacy NeuBase, he forewent any cash compensation for his service as an executive officer.

Legacy NeuBase entered into an employment agreement as of December 22, 2018 with Dr. Stephan as its Chief Executive Officer, effective as of August 28, 2018 (the "Stephan Employment Agreement"). Beginning on December 22, 2018, Dr. Stephan's annual base salary was $75,000. If Legacy NeuBase issued and sold shares of its preferred or common stock in one or a series of transactions for aggregate proceeds of at least $4,000,000 (excluding all proceeds realized from the conversion or cancellation of debt in exchange for the issuance of such stock) ("Qualified Financing"), Dr. Stephan's annual base salary would be increased to $450,000, and Legacy NeuBase would pay Dr. Stephan an additional $2,000 per month for his supplemental life and disability insurance policies. Dr. Stephan's annual base salary is subject to increase or decrease by Legacy NeuBase's board of directors or a committee duly appointed by the board.

On or about December 28, 2018, Legacy NeuBase paid Dr. Stephan a bonus of $25,000. Upon the consummation of a Qualified Financing, Dr. Stephan would be eligible for a bonus of $150,000 (the "Bonus"), which may be modified from time to time in the discretion of Legacy NeuBase's board of directors, and would additionally be eligible for an annual bonus of $150,000 ("Annual Bonus") based on the attainment of individual and Legacy NeuBase performance objectives as may be set by Legacy NeuBase's board of directors.

Under the Stephan Employment Agreement, on December 31, 2018, Dr. Stephan was also granted a stock option to purchase 3,250,000 shares of Legacy NeuBase common stock with an exercise price of $0.0011 per share. Beginning on August 28, 2018, this stock option began to vest on an equal monthly basis over a 48-month period, subject to Dr. Stephan's continued employment with Legacy NeuBase. Upon completion of the Merger, however, this stock option vested in full, and Dr. Stephan was entitled to exercise his option to purchase 3,311,930 of the combined company's stock at an exercise price adjusted for the exchange ratio pursuant to the Merger Agreement.

Dr. Stephan's employment with Legacy NeuBase was at-will, meaning either Legacy NeuBase or Dr. Stephan could terminate the employment relationship at any time, with or without cause. If Legacy NeuBase terminated Dr. Stephan's employment without "cause" and not on account of his "disability" or Dr. Stephan resigns his employment for "good reason" (as such terms are defined in the Stephan Employment Agreement), then, so long as Dr. Stephan complies with certain obligations, including execution and delivery of a general release within a specified period of time, Legacy NeuBase would pay Dr. Stephan: (1) his base salary as of the termination date for 12 months following the termination date; and (2) subject to the discretion of Legacy NeuBase's board of directors, a pro-rata Bonus or Annual Bonus for the year in which the termination occurs, calculated based on the product of the Dr. Stephan's target Bonus or Annual Bonus times a fraction, the numerator of which is the number of days during the year of termination in which Dr. Stephan was employed and the denominator of which is 365. In addition, 100% of the unvested shares subject to his stock option vest.

Dr. Stephan was also a party to a confidential information, invention assignment and arbitration agreement with Legacy NeuBase, pursuant to which Dr. Stephan has made confidentiality, assignment of intellectual property, non-solicitation and noncompetition covenants in favor of Legacy NeuBase. Any severance payments that become payable under his employment agreement are conditioned on his compliance with these covenants.

On July 11, 2019, we entered into an Offer of Employment with Dr. Stephan (the "Offer of Employment") that became effective upon the consummation of the Merger on July 12, 2019 and replaced the Stephan Employment Agreement. Pursuant to the Offer of Employment, Dr. Stephan will serve as the Company's President, as applicable, and Chief Executive Officer, his initial annualized salary will be $450,000 and he will be eligible to receive a discretionary annual performance bonus of up to 35% of his base salary. In addition, consistent with the obligations of Legacy NeuBase under the Stephan Employment Agreement, we paid Dr. Stephan the Bonus on July 19, 2019 pursuant to and after the completion of the Merger. On January 7, 2022, the Company increased Dr. Stephan's base salary to $540,000 and determined he would be eligible to receive a discretionary annual performance bonus of up to 50% of his base salary in calendar year 2021.

Dr. Stephan's employment with us is at-will, meaning either we or Dr. Stephan could terminate the employment relationship at any time, with or without cause. Pursuant to the Offer of Employment, if Dr. Stephan is terminated by us without cause, we will be obligated to pay to Dr. Stephan (i) severance at a rate equal to 100% of his base salary for a period of 12 months from the date of such termination and (ii) subject to the discretion of our board of directors, a prorated discretionary bonus for the year in which such termination occurs.

In addition, pursuant to the Offer of Employment, Dr. Stephan's confidential information, invention assignment and arbitration agreement with Legacy NeuBase shall continue to apply and was assumed by us.

Todd P. Branning

On January 10, 2022, the Company entered into an offer letter with Mr. Branning. Pursuant to his offer letter, Mr. Branning's initial annual salary will be $425,000, and he will have an annual performance bonus with a target of 40% of his base salary. Mr. Branning's employment will be on an "at will" basis. Additionally, pursuant to the offer letter the Company granted Mr. Branning an option to purchase 300,000 shares of common stock under the Company's 2019 Stock Incentive Plan. Subject to Mr. Branning's continued employment with the Company, 1/4th of the shares underlying his option to purchase common stock will vest on the first anniversary of Mr. Branning's start date, and 1/36th of the remaining shares underlying such option will vest at the end of each calendar month thereafter.

Mr. Branning's employment with us is at-will, meaning either we or Mr. Branning could terminate the employment relationship at any time, with or without cause. If Mr. Branning is terminated by us without cause or Mr. Branning resigns for good reason (defined generally as a reduction in his salary amongst similarly-situated employees, relocation, or a material diminution in title, duties or responsibilities), in either case, within six months following a change in control (as defined in the 2019 Plan), then, subject to execution and delivery of a general release of all claims, his then outstanding, unvested options, if any, will vest and be exercisable as to all of the covered shares. If Mr. Branning is terminated by us without cause (whether or not in connection with a change in control), we will be obligated to pay Mr. Branning (1) severance pay at a rate equal to one hundred percent (100%) of his base salary for a period of twelve (12) months from the date of termination, (2) reimbursement of 12 months of health benefits (COBRA subsidization) in accordance with the Company's standard expense reimbursement procedures and (3) subject to the discretion of our board of directors, a prorated portion of his annual bonus target for the year of termination.

Mr. Branning also entered into the Company's standard indemnification agreement and standard confidentiality and invention assignment agreement with the Company.

William R. Mann, Ph.D.

On July 22, 2020, the Company entered into an offer letter with Dr. Mann. Pursuant to his offer letter, Dr. Mann's initial annual salary will be $375,000, and he will have an annual performance bonus with a target of 40% of his base salary. Dr. Mann's employment will be on an "at will" basis. Additionally, pursuant to the offer letter the Company granted Dr. Mann an option to purchase 175,000 shares of common stock under the Company's 2019 Stock Incentive Plan. Subject to Dr. Mann's continued employment with the Company, 1/4th of the shares underlying his option to purchase common stock will vest on the first anniversary of Dr. Mann's start date, and 1/36th of the remaining shares underlying such option will vest at the end of each calendar month thereafter. On July 28, 2021, the Company increased Dr. Mann's annual salary to $420,000, and effective August 16, 2021, granted him an additional option to purchase 25,000 shares of common stock under the Company's 2019 Stock Incentive Plan. Subject to Dr. Mann's continued employment with the Company, 1/4th of the shares underlying his option to purchase common stock, granted on July 28, 2021, will vest on July 28, 2022 and 1/36th of the remaining shares underlying such option will vest on a monthly basis thereafter. Additionally, on January 24, 2022, the Company granted Dr. Mann an additional option to purchase 60,180 shares of common stock under the Company's 2019 Stock Incentive Plan. Subject to Dr. Mann's continued employment with the Company, 1/4th of the shares underlying his option to purchase common stock, granted on January 24, 2022, will vest on January 24, 2023 and 1/36th of the remaining shares underlying such option will vest on a monthly basis thereafter.

Dr. Mann's employment with us is at-will, meaning either we or Dr. Mann could terminate the employment relationship at any time, with or without cause. If Dr. Mann is terminated by us without cause or Dr. Mann resigns for good reason (defined generally as a reduction in his salary amongst similarly-situated employees, relocation, or a material diminution in title, duties or responsibilities), in either case, within six months following a change in control (as defined in the 2019 Plan), then, subject to execution and delivery of a

general release of all claims, his then outstanding, unvested options, if any, will vest and be exercisable as to all of the covered shares. If Dr. Mann is terminated by us without cause (whether or not in connection with a change in control), we will be obligated to pay Dr. Mann (1) severance pay at a rate equal to one hundred percent (100%) of his base salary for a period of twelve (12) months from the date of termination, (2) reimbursement of 12 months of health benefits (COBRA subsidization) in accordance with the Company's standard expense reimbursement procedures and (3) subject to the discretion of our board of directors, a prorated portion of his annual bonus target for the year of termination.

Dr. Mann also entered into the Company's standard indemnification agreement and standard confidentiality and invention assignment agreement with the Company.

Dr. Mann resigned from the Company on October 31, 2022. Dr. Mann's resignation was not a result of any disagreement with the Company or any matter relating to its accounting or financial policies or procedures.

NeuBase Therapeutics, Inc. 2019 Stock Incentive Plan

On March 6, 2019, our board of directors adopted the 2019 Plan to assist us in recruiting and retaining individuals with ability and initiative by enabling them to receive awards and participate in our future success by associating their interests with those of the Company and our stockholders. Our stockholders approved the plan on July 10, 2019. The 2019 Plan is intended to permit the grant of stock options (both incentive stock options ("ISOs") and non-qualified stock options ("NQSOs")), stock appreciation rights ("SARs"), restricted stock ("Restricted Stock Awards"), restricted stock units ("RSUs") and other incentive awards ("Incentive Awards").

The 2019 Plan became effective on the day prior to the closing date of the Merger. No awards may be granted after March 6, 2029, the date which is 10 years after the adoption of the 2019 Plan by our board of directors.

The following is only a summary of the material terms of the 2019 Plan, is not a complete description of all provisions of the 2019 Plan and should be read in conjunction with the 2019 Plan, which is incorporated by reference as an exhibit to this Form 10-K.

Administration. We bear all expenses of administering the 2019 Plan. The Compensation Committee administers the 2019 Plan. The Compensation Committee has the authority to grant awards to such persons and upon such terms and conditions (not inconsistent with the provisions of the 2019 Plan), as it may consider appropriate. The Compensation Committee may delegate to one or more of our officers all or part of its authority and duties with respect to awards to individuals who are not subject to Section 16 of the Exchange Act.

Eligibility for Participation. Any of our employees or service providers, including any employees or service providers of our affiliates, and any non-employee member of our board of directors or the boards of directors of our affiliates, is eligible to receive an award under the 2019 Plan. However, ISOs may only be granted to our employees or employees of our affiliates.

Shares Subject to Plan. The maximum number of shares of common stock that may be issued under the life of the 2019 Plan will be 3,100,000 shares, subject to increase pursuant to an "evergreen" provision that will automatically increase the maximum number of shares of our common stock that may be issued under the life of the 2019 Plan on October 1st of each year beginning on October 1, 2019 and continuing through October 1, 2028 by a number of shares equal to 4.0% of the total number of shares of common stock outstanding as of September 30th of the preceding fiscal year, or a lesser number of shares to be determined by our board of directors. Notwithstanding the foregoing, the maximum number of shares of our common stock available for grants of ISOs under the 2019 Plan is 3,100,000 and will not increase.

A share of common stock issued in connection with any award under the 2019 Plan shall reduce the total number of shares of common stock available for issuance under the 2019 Plan by one; *provided*, *however*, that a share of our common stock covered under a stock-settled SAR shall reduce the total number of shares of common stock available for issuance under the 2019 Plan by one even though the shares of common stock are not actually issued in connection with settlement of the SAR. Except as otherwise provided in the 2019 Plan, any shares of common stock related to an award which terminates by expiration, forfeiture, cancellation or otherwise without issuance of shares of common stock, which is settled in cash in lieu of common stock or which is exchanged, with the Compensation Committee's permission, prior to the issuance of shares of common stock, for awards not involving shares of common stock, shall again be available for issuance under the 2019 Plan. The following shares of common stock, however, may not again be made available for issuance as awards under the 2019 Plan: (i) shares of common stock not issued or delivered as a result of a net

settlement of an outstanding award, (ii) shares of common stock tendered or held to pay the exercise price, purchase price or withholding taxes relating to an outstanding award, or (iii) shares of common stock repurchased on the open market with the proceeds of the exercise price of an award.

In any calendar year, no participant may be granted options, SARs, Restricted Stock Awards, RSUs, or any combination thereof that relate to more than 1,000,000 shares of our common stock (subject to adjustment as provided in the 2019 Plan). In any calendar year, no participant may be granted an Incentive Award (i) with reference to a specified dollar limit for more than $3,000,000 and (ii) with reference to a specified number of shares of our common stock for more than 1,000,000 shares of our common stock (subject to adjustment as provided in the 2019 Plan). In any calendar year, no participant who is a member of our board of directors but is not our employee of or an employee of our affiliate, may be granted options, SARs, Restricted Stock Awards, RSUs, or any combination thereof that relate to more than 300,000 shares of common stock (subject to adjustment as provided in the 2019 Plan). The maximum number of shares of our common stock that may be issued pursuant to awards, the per individual limits on awards and the terms of outstanding awards will be adjusted in a similar manner as the evergreen provisions that apply to the aggregate limits and as the Compensation Committee in its sole discretion determines is equitably required in the event of corporate transactions and other appropriate events.

Options. A stock option entitles the participant to purchase from us a stated number of shares of common stock. The Compensation Committee will determine whether the option is intended to be an ISO or a NQSO and specify the number of shares of common stock subject to the option. In the case of ISOs, the aggregate fair market value (determined as of the date of grant) of common stock with respect to which an ISO may become exercisable for the first time during any calendar year cannot exceed $100,000; and if this limitation is exceeded, the ISOs which cause the limitation to be exceeded will be treated as NQSOs. The exercise price per share of common stock may not be less than the fair market value of our common stock on the date the option is granted. With respect to an ISO granted to a participant who beneficially owns more than 10% of the combined voting power of the Company or any of our affiliates (determined by applying certain attribution rules), the exercise price per share may not be less than 110% of the fair market value of our common stock on the date the option is granted. The exercise price may be paid in cash or, if the agreement so provides, the Compensation Committee may allow a participant to pay all or part of the exercise price by tendering shares of our common stock the participant already owns, through a broker-assisted cashless exercise, by means of "net exercise" procedure, any other specified medium of payment or a combination.

Stock Appreciation Rights. A SAR entitles the participant to receive, upon exercise, the excess of the fair market value on that date of each share of common stock subject to the exercised portion of the SAR over the fair market value of each such share on the date of the grant of the SAR. A SAR can be granted alone or in tandem with an option. A SAR granted in tandem with an option is called a Corresponding SAR and entitles the participant to exercise the option or the SAR at which time the other tandem award expires. The Compensation Committee will specify the number of shares of common stock subject to a SAR and whether the SAR is a Corresponding SAR. No participant may be granted Corresponding SARs in tandem with ISOs which are first exercisable in any calendar year for shares of our common stock having an aggregate fair market value (determined as of the date of grant) that exceeds $100,000; and if this limitation is exceeded the tandem option will be treated as NQSOs. A Corresponding SAR may be exercised only to the extent that the related option is exercisable and the fair market value of our common stock on the date of exercise exceeds the exercise price of the related option. As set forth in the agreement, the amount payable as a result of the exercise of a SAR may be settled in cash, shares of common stock or a combination of each.

Restricted Stock Awards. A Restricted Stock Award is the grant or sale of shares of our common stock, which may be subject to forfeiture restrictions. The Compensation Committee will prescribe whether the Restricted Stock Award is forfeitable and the conditions to which it is subject. If the participant must pay for a Restricted Stock Award, payment for the award generally shall be made in cash or, if the agreement so provides, by surrendering shares of common stock the participant already owns or any other medium of payment. Prior to vesting or forfeiture, a participant will have all rights of a stockholder with respect to the shares of common stock underlying the Restricted Stock Award, including the right to receive dividends and vote the underlying shares of our common stock; provided, however, the participant may not transfer the shares. We may retain custody of the certificates evidencing the shares of our common stock until such shares are no longer forfeitable.

RSUs. An RSU entitles the participant to receive shares of common stock when certain conditions are met. The Compensation Committee will prescribe when the RSUs shall become payable. We will pay the participant one share of our common stock for each RSU that becomes earned and payable.

Incentive Awards. An Incentive Award entitles the participant to receive cash or common stock or a combination of each when certain conditions are met. The Compensation Committee will prescribe the terms and conditions of the Incentive Award. As set forth in the participant's agreement, an Incentive Award may be paid in cash, shares of common stock or a combination of each.

Change in Control. In the event of or in anticipation of a "Change in Control" (as defined in the 2019 Plan), the Compensation Committee in its discretion may terminate outstanding awards (i) by giving the participants an opportunity to exercise the awards that are then exercisable and then terminating, without any payment, all awards that have not been exercised (including those that were not then exercisable) or (ii) by paying the participant the value of the awards that are then vested, exercisable or payable without payment for any awards that are not then vested, exercisable or payable or that have no value. Alternatively, the Compensation Committee may take such other action as the Compensation Committee determines to be reasonable under the circumstances to permit the participant to realize the vested value of the award. The Compensation Committee may provide that a participant's outstanding awards become fully exercisable or payable on and after a Change in Control or immediately before the date the awards will be terminated in connection with a Change in Control. Awards will not be terminated to the extent they are to be continued after the Change in Control.

Stockholder Rights. No participant shall have any rights as a stockholder until the award is settled by the issuance of our common stock (other than a Restricted Stock Award or RSUs for which certain stockholder rights may be granted).

Transferability. An award is non-transferable except by will or the laws of descent and distribution, and during the lifetime of the participant to whom the award is granted, the award may only be exercised by, or payable to, the participant. The holder of the transferred award will be bound by the same terms and conditions that governed the award during the period that it was held by the participant.

Maximum Award Period. No award shall be exercisable or become vested or payable more than ten years after the date of grant. An ISO granted to a participant who beneficially owns more than 10% of the combined voting power of or any affiliate (determined by applying certain attribution rules) or a Corresponding SAR that relates to such an ISO may not be exercisable more than five years after the date of grant.

Compliance with Applicable Law. No award shall be exercisable, vested or payable except in compliance with all applicable federal and state laws and regulations (including, without limitation, tax and securities laws), any listing agreement with any stock exchange to which we are a party, and the rules of all domestic stock exchanges on which our securities may be listed.

Amendment and Termination of Plan. Our board of directors may amend or terminate the 2019 Plan at any time; provided, however, that no amendment may adversely impair the rights of a participant with respect to outstanding awards without the participant's consent. An amendment will be contingent on approval of our stockholders, to the extent required by law, by the rules of any stock exchange on which our securities are then traded or if the amendment would (i) increase the benefits accruing to participants under the 2019 Plan, including without limitation, any amendment to the 2019 Plan or any agreement to permit a repricing or decrease in the exercise price of any outstanding options or SARs, (ii) increase the aggregate number of shares of our common stock that may be issued under the 2019 Plan, or (iii) modify the requirements as to eligibility for participation in the 2019 Plan.

Forfeiture Provisions. Awards do not confer upon any individual any right to continue in the employ or service of the Company or any affiliate. All rights to any award that a participant has will be immediately forfeited if the participant is discharged from employment or service for "Cause" (as defined in the 2019 Plan).

Material U.S. Federal Income Tax Consequences

The following discussion summarizes the material United States federal income tax consequences associated with awards granted under the 2019 Plan to U.S. citizens. The discussion is based on laws, regulations, rulings and court decisions currently in effect, all of which are subject to change.

ISOs. A participant will not recognize taxable income on the grant or exercise of an ISO. A participant will recognize taxable income when he or she disposes of the shares of our common stock acquired under the ISO. If the disposition occurs more than two years after the grant of the ISO and more than one year after its exercise (the "ISO holding period"), the participant will recognize long-term capital gain (or loss) to the extent the amount realized from the disposition exceeds (or is less than) the participant's tax basis in the

shares of our common stock. A participant's tax basis in shares of our common stock generally will be the amount the participant paid for the shares.

If our common stock acquired under an ISO is disposed of before the expiration of the ISO holding period described above, the participant will recognize as ordinary income in the year of the disposition the excess of the fair market value of our common stock on the date of exercise of the ISO over the exercise price. Any additional gain will be treated as long-term or short-term capital gain, depending on the length of time the participant held the shares. A special rule applies to such a disposition where the amount realized is less than the fair market value of our common stock on the date of exercise of the ISO. In that case, the ordinary income the participant will recognize will not exceed the excess of the amount realized on the disposition over the exercise price. If the amount realized is less than the exercise price, the participant will recognize a capital loss (long-term if the stock was held more than one year and short-term if held one year or less). A participant will receive different tax treatment if the exercise price is paid by delivery of common stock the participant already owns.

Neither us nor any of our affiliates will be entitled to a federal income tax deduction with respect to the grant or exercise of an ISO. However, in the event a participant disposes of our common stock acquired under an ISO before the expiration of the ISO holding period described above, we or our affiliate will be entitled to a federal income tax deduction equal to the amount of ordinary income the participant recognizes.

NQSOs. A participant will not recognize any taxable income on the grant of a NQSO. On the exercise of a NQSO, the participant will recognize as ordinary income the excess of the fair market value of the common stock acquired over the exercise price. A participant's tax basis in our common stock is the amount paid plus any amounts included in income on exercise. The participant's holding period for the stock begins on acquisition of the shares. Any gain or loss that a participant realizes on a subsequent disposition of our common stock acquired on the exercise of a NQSO generally will be treated as long-term or short-term capital gain or loss, depending on the length of time the participant held such shares. The amount of the gain (or loss) will equal the amount by which the amount realized on the subsequent disposition exceeds (or is less than) the participant's tax basis in his or her shares. A participant will receive different tax treatment if the exercise price is paid by delivery of common stock the participant already owns.

The exercise of a NQSO will entitle us or our affiliate to claim a federal income tax deduction equal to the amount of ordinary income the participant recognizes. If the participant is an employee, that ordinary income will constitute wages subject to withholding and employment taxes.

SARs. A participant will not recognize any taxable income at the time the SARs are granted. The participant at the time of receipt will recognize as ordinary income the amount of cash and the fair market value of our common stock that he or she receives. We or our affiliate will be entitled to a federal income tax deduction equal to the amount of ordinary income the participant recognizes. If the participant is an employee, that ordinary income will constitute wages subject to withholding and employment taxes.

Restricted Stock Awards. A participant will recognize ordinary income on account of a Restricted Stock Award on the first day that the shares are either transferable or not subject to a substantial risk of forfeiture. The ordinary income recognized will equal the excess of the fair market value of our common stock on such date over the amount, if any, the participant paid for the Restricted Stock Award. However, even if the shares under a Restricted Stock Award are both nontransferable and subject to a substantial risk of forfeiture, the participant may make a special "83(b) election" within 30 days of the grant date to recognize income, and have his or her tax consequences determined, as of the date the Restricted Stock Award is made. The participant's tax basis in the shares received will equal the income recognized plus the price, if any, paid for the Restricted Stock Award. Any gain (or loss) that a participant realizes upon the sale of any of our common stock acquired pursuant to a Restricted Stock Award will be equal to the amount by which the amount realized on the disposition exceeds (or is less than) the participant's tax basis in the shares and will be treated as long-term (if the shares are held for more than one year) or short-term (if the shares are held for one year or less) capital gain or loss. The participant's holding period for the stock begins on the date the shares are either transferable or not subject to a substantial risk of forfeiture, except that the holding period will begin on the date of grant if the participant makes the special "83(b) election." We or our affiliate will be entitled to a federal income tax deduction equal to the ordinary income the participant recognizes. If the participant is an employee, that ordinary income will constitute wages subject to withholding and employment taxes.

RSUs. The participant will not recognize any taxable income at the time the RSUs are granted. When the terms and conditions to which the RSUs are subject have been satisfied and the RSUs are paid, the participant, at the time of receipt, will recognize as ordinary income the fair market value of our common stock he or she receives. The participant's holding period in our common stock

will begin on the date the stock is received. The participant's tax basis in our common stock will equal the amount he or she includes in ordinary income. Any gain or loss that a participant realizes on a subsequent disposition of the shares will be treated as long-term or short-term capital gain or loss, depending on the participant's holding period for the stock (long-term if the shares are held for more than one year; short-term if one year or less). The amount of the gain (or loss) will equal the amount by which the amount realized on the disposition exceeds (or is less than) the participant's tax basis in the common stock. We or our affiliate will be entitled to a federal income tax deduction equal to the ordinary income the participant recognizes. If the participant is an employee, that ordinary income will constitute wages subject to withholding and employment taxes.

Incentive Awards. A participant will not recognize any taxable income at the time an Incentive Award is granted. When the terms and conditions to which an Incentive Award is subject have been satisfied and the award is paid, the participant, at the time of receipt, will recognize as ordinary income the amount of cash and the fair market value of the common stock he or she receives. The participant's holding period in any of our common stock received will begin on the date of receipt. The participant's tax basis in our common stock will equal the amount he or she includes in ordinary income with respect to such shares. Any gain or loss that a participant realizes on a subsequent disposition of our common stock will be treated as long-term or short-term capital gain or loss, depending on the participant's holding period for the common stock (long-term if the shares are held for more than one year; short-term if one year or less). The amount of the gain (or loss) will equal the amount by which the amount realized on the disposition exceeds (or is less than) the participant's tax basis in our common stock. We or our affiliate will be entitled to a federal income tax deduction equal to the amount of ordinary income the participant recognizes. If the participant is an employee, that ordinary income will constitute wages subject to withholding and employment taxes.

Limitation on Deductions. The deduction for a publicly-held corporation for otherwise deductible compensation to a "covered employee" generally is limited to $1 million per year. An individual is a covered employee if he or she is the chief executive officer, chief financial officer, or one of the other three highest compensated officers for the year (other than the chief executive officer or chief financial officer) or ever was a covered employee after December 31, 2016.

Any grant, exercise, vesting or payment of an award may be postponed if we reasonably believes that our or any applicable affiliate's deduction with respect to such award would be limited or eliminated by application of Code Section 162(m) to the extent permitted by Section 409A of the Code; *provided*, *however*, such delay will last only until the earliest date at which we reasonably anticipates the deduction will not be limited or eliminated under Code Section 162(m).

Other Tax Rules. The 2019 Plan is designed to enable the Compensation Committee to structure awards that are intended to not be subject to Code Section 409A, which imposes certain restrictions and requirements on deferred compensation.

NeuBase Therapeutics, Inc. 2018 Equity Incentive Plan

On August 28, 2018, the board of directors of Legacy NeuBase adopted the 2018 Plan, and the Legacy NeuBase stockholders also approved the 2018 Plan on August 28, 2018. Pursuant to the Merger Agreement, at the effective time of the Merger, each outstanding and unexercised option to purchase shares of Legacy NeuBase common stock issued under the 2018 Plan was assumed by us, and became an option to purchase that number of shares of our common stock equal to the product obtained by multiplying (i) the number of shares of Legacy NeuBase common stock that were subject to such option immediately prior to the effective time of the Merger by (ii) the exchange ratio, rounded down to the nearest whole share. The per share exercise price for shares of our common stock issuable upon exercise of each Legacy NeuBase option assumed by us was determined by dividing (a) the per share exercise price of Legacy NeuBase common stock subject to such Legacy NeuBase option, as in effect immediately prior to the effective time of the Merger, by (b) the exchange ratio, rounded up to the nearest whole cent. No new equity awards will be issued under the 2018 Plan. However, the 2018 Plan will continue to govern outstanding awards granted thereunder.

The following is only a summary of the material terms of the 2018 Plan, is not a complete description of all provisions of the 2018 Plan and should be read in conjunction with the 2018 Plan, which is filed as an exhibit to this Form 10-K.

Stock Options and Stock Appreciation Rights. The exercise price of stock options and strike price of stock appreciation rights granted under the 2018 Plan must not be less than 100% of the fair market value of our common stock on the grant date, subject to certain exceptions as set forth in the 2018 Plan. The term of a stock option or stock appreciation rights may not exceed ten years. An ISO may only be granted to our employees or employees of certain of our affiliates, including officers who are employees. An ISO granted to an employee who owns more than 10% of the combined voting power of all of our classes of stock or that of our affiliates must have

an exercise price of at least 110% of the fair market value of our common stock on the grant date, and the term of the ISO may not exceed five years from the grant date. To the extent that the aggregate fair market value of shares of our common stock with respect to which ISOs first become exercisable by a participant in any calendar year exceeds $100,000, such excess stock options will be treated as Non-ISOs. The methods of payment of the exercise price of a stock option may include, among other things, cash, other shares (subject to certain conditions), "net exercise" (for Non-ISOs), cashless exercise, deferred payment or similar arrangements, as well as other forms of legal consideration that may be acceptable to our board of directors and specified in the applicable stock option award agreement. To exercise any outstanding stock appreciation right, the participant must provide written notice of exercise to us. The appreciation distribution payable on the exercise of a stock appreciation right may be paid in our common stock, cash, a combination of our common stock and cash or in any other form of consideration determined by our board of directors and contained in the award agreement. Our board of directors may establish and set forth in the applicable stock option award agreement or other agreement the terms and conditions on which a stock option or stock appreciation right will remain exercisable, if at all, following termination of a participant's service. Unless an award agreement provides otherwise: (1) if termination is due to death, the stock option or stock appreciation right will remain exercisable for six months after such termination of service; (2) if termination is due to disability, the stock option or stock appreciation right will remain exercisable for six months after such termination of service; and (3) if the termination is due to reasons other than for death or disability, the stock option or stock appreciation right generally will remain exercisable for thirty days following termination of service. If a participant is not entitled to exercise a stock option or stock appreciation right at the date of termination of service, or if the participant does not exercise the stock option or stock appreciation right to the extent so entitled within the time specified in the applicable stock option award agreement or other agreement or in the 2018 Plan, the stock option or stock appreciation right will terminate.

Restricted Stock Awards. Each restricted stock award agreement will be in the form and contain such terms and conditions as our board of directors deems appropriate. At our board of directors' election, shares of our common stock may be (1) held in book entry form until any restrictions relating to the restricted stock award lapse or (2) evidenced by a certificate that is held in a form and manner determined by our board of directors. The methods of payment of consideration for a restricted stock award may include any form of legal consideration that may be acceptable to our board of directors and permissible under applicable law including the provision of services. Shares of our common stock awarded under a restricted stock award agreement may be subject to forfeiture in accordance with a vesting schedule. Following termination of a participant's service, we may receive through a forfeiture condition or repurchase right, any or all of the shares of our common stock held by the participant as of the date of termination under the terms of the restricted stock award agreement. Dividends paid on restricted common stock may be subject to the same vesting and forfeiture restrictions that apply to the shares of our common stock under the restricted stock award.

Restricted Stock Unit Awards. Each restricted stock unit award agreement will be in the form and contain such terms and conditions as our board of directors deems appropriate. Payment for each share of our common stock subject to a restricted stock unit award may be in any form of legal consideration that may be acceptable to our board of directors and permissible under applicable law including the provision of services. Our board of directors may, in its sole discretion, impose restrictions on or conditions to the vesting of a restricted stock unit award. Each restricted stock unit award may be settled by delivery of our common stock, the cash value of our common stock, a combination of our common stock and cash or in any other form of consideration determined by our board of directors and contained in the award agreement. Our board of directors may, at the time of grant, impose restrictions or conditions on a restricted stock unit award that delay the delivery of our common stock subject to such restricted stock unit award to a time after such restricted stock unit award vests. Unless an award agreement provides otherwise, any unvested portion of a restricted stock unit award will be forfeited upon a participant's termination of service.

Taxes. Prior to the delivery of cash or shares in settlement or exercise of any award, we may withhold and/or require the holder to remit to us amount sufficient to satisfy all taxes required to be withheld under applicable laws.

Non-Transferability. Unless our board of directors provides otherwise in an award agreement, or unless transferred pursuant to a will or by the laws of descent and distribution, the 2018 Plan generally does not allow for the transfer of awards and only the participant who is granted an award may exercise an award during his or her lifetime.

Certain Adjustments. In the event of certain changes in our capitalization, such as any dividend or other distribution (whether in the form of cash, our common stock, other securities or other property), stock splits, reverse stock splits, combinations, recapitalizations or reorganizations with respect to our common stock, or mergers, consolidations, changes in organization form or other increases or decreases in the number of issued shares of our common stock effected without receipt or payment of consideration by us, our board of directors will proportionally adjust the number and price of shares covered by each outstanding award and the total number of

shares authorized for issuance under the 2018 Plan. Unless our board of directors provides otherwise in an award agreement, in the event of any proposed dissolution or liquidation of us, other than as part of a corporate transaction, we will notify each participant as soon as practicable and all awards will terminate immediately prior to the consummation of such proposed corporate transaction.

Corporate Transaction. In the event of a corporate transaction involving us, our board of directors has the discretion to take one or more of the following actions with respect to any or all awards: (1) arrange for the surviving company or acquiring company to assume or continue the stock awards or substitute a substantially equivalent stock award; (2) upon to notice to the holder, provide for the termination of such holder's awards upon or immediately prior to the transaction; (3) accelerate the vesting, in whole or in part, of the stock awards (and, if applicable, the time at which the stock awards may be exercised) to a date prior to the effective time of such corporate transaction as our board of directors will determine at or prior to the effective time of the corporate transaction; (4) arrange for the lapse, in whole or in part, of any reacquisition or repurchase rights held by us with respect to the stock awards; (5) cancel or arrange for the cancellation of the stock award in exchange for a payment cash and/or property equal to the excess, if any, of (A) the amount the participant would have received upon the exercise or settlement of the stock award immediately prior to the effective time of the corporate transaction, over (B) any exercise or strike price payable by such holder in connection with such exercise (and if as of the date of the occurrence of the corporate transaction our board of directors determines in good faith that no amount would have been attained upon the exercise or settlement of such award, then such award may be terminated without payment); (6) the replacement of such award with other rights or property selected by our board of directors in its sole discretion; or (7) any combination of the above. Our board of directors is not required to take the same action or actions with respect to all awards granted under the 2018 Plan, or portions thereof, or with respect to all participants, and may take any of the different actions described above with respect to the vested and unvested portions of any award. In the event that a successor corporation does not assume or substitute for the award, the award shall become fully vested (with any performance-based vesting deemed attained at 100% of target levels and other terms and conditions met). A corporate transaction means, (i) a merger, following which we are not the surviving company, (ii) any one person or more than one person acting as group acquires ownership of our stock that, together with any stock held by such person(s), constitutes more than 50% of the voting power of our stock, except that any change in the ownership of our stock as a result of a private financing that is approved by our board of directors; (iii) if we have a class of securities registered pursuant to Section 12 of the Exchange Act, a majority of members of our board of directors is replaced during any twelve month period by directors who appointment or election is not endorsed by a majority of members of our board of directors prior to the date of appointment or election; or (iv) any person acquires within any twelve month period our assets having a total gross fair market value of at least 50% of the total fair market value of our assets.

Amendment; Termination. The 2018 Plan may be amended or terminated by our board of directors as it deems advisable; however, stockholder approval is required for any change that that (1) materially increases the number of shares of our common stock available for issuance under the 2018 Plan, or (2) materially expands the class of individuals eligible to receive awards under the 2018 Plan. The 2018 Plan will terminate on July 8, 2026, if not sooner terminated by NeuBase's board of directors.

Ohr Pharmaceutical, Inc. 2016 Consolidated Stock Incentive Plan

On January 7, 2016, our board of directors adopted the 2016 Plan and our shareholders approved the plan on March 17, 2016 to assist us in recruiting and retaining individuals with ability and initiative by enabling them to receive awards and participate in our future success by associating their interests with those of us and our stockholders. The 2016 Plan is intended to permit the grant of stock options (both ISOs and NQSOs), SARs, Restricted Stock Awards, RSUs and Incentive Awards. The following is only a summary of the material terms of the 2016 Plan, is not a complete description of all provisions of the 2016 Plan and should be read in conjunction with the 2016 Plan, which is filed as an exhibit to this Form 10-K.

Prior Plans. We previously maintained each of the 2014 Plan and the 2009 Plan. The 2016 Plan is intended to consolidate the 2014 Plan and the 2009 Plan into a new plan, with an aggregate number of shares available for issuance under the 2016 Plan as set forth below under "- Shares Subject to Plan." For options and Restricted Stock Awards granted under the 2014 Plan and the 2009 Plan prior to January 7, 2016, the terms and conditions of the 2014 Plan and the 2009 Plan and the applicable award agreements will control, except that our Compensation Committee, in its discretion may allow a participant to pay all or part of the option price (i) by surrendering shares of common stock to us that the participant already owns and, if necessary to avoid adverse accounting consequences, has held for at least six months; (ii) by a cashless exercise through a broker; (iii) by means of a "net exercise" procedure, (iv) by such other medium of payment as the Compensation Committee in its discretion shall authorize or (v) by any combination of the aforementioned methods of payment. If shares of common stock are used to pay all or part of the option price, the

sum of the cash and cash equivalent and the fair market value (determined as of the day preceding the date of exercise) of the shares surrendered shall equal the option price of the shares for which the option is being exercised.

Written Agreements. All awards granted under the 2016 Plan will be governed by separate written agreements between us and the participants. The written agreements will specify when the award may become vested, exercisable or payable, as well as other terms and conditions that may apply to the award. No right or interest of a participant in any award will be subject to any lien, obligation or liability of the participant. The laws of the State of Delaware govern the 2016 Plan.

No awards may be granted after January 7, 2026, the date which is 10 years after the adoption of the 2016 Plan by the board of directors.

Tax Treatment. It is intended that awards granted under the 2016 Plan shall be exempt from treatment as "deferred compensation" subject to Section 409A of the Internal Revenue Code of 1986 (and any amendments thereto) (the "Code").

Administration. We bear all expenses of administering the 2016 Plan. The Compensation Committee administers the 2016 Plan. The Compensation Committee has the authority to grant awards to such persons and upon such terms and conditions (not inconsistent with the provisions of the 2016 Plan), as it may consider appropriate. The Compensation Committee may delegate to one or more of our officers all or part of its authority and duties with respect to awards to individuals who are not subject to Section 16 of the Exchange Act.

Eligibility for Participation. Any of our employees or service providers, including any employees or service providers of our affiliates, and any non-employee member of our board of directors or the boards of directors of our affiliates, is eligible to receive an award under the 2016 Plan. However, ISOs may only be granted to our employees or employees of our affiliates.

Shares Subject to Plan. The maximum number of shares of our common stock that may be issued under the life of the 2016 Plan pursuant to awards will be (a) 291,667 shares minus (b) the number of shares of our common stock that previously have been issued pursuant to the exercise of options under the 2009 Plan or 2014 Plan or the number of shares of restricted stock granted under the 2014 Plan and the 2009 Plan that, as of December 28, 2018 are no longer subject to a substantial risk of forfeiture. One hundred percent (100%) of such shares may be issued pursuant to options (including ISOs), SARs, Restricted Stock Awards, RSUs or Incentive Awards or any combination of awards. Of the 291,667 shares, 16,667 previously were authorized under the 2009 Plan and 137,500 previously were authorized under the 2014 Plan.

Shares of common stock covered by an award shall only be counted as issued to the extent they are actually issued. A share of common stock issued in connection with any award under the 2016 Plan shall reduce the total number of shares of our common stock available for issuance under the 2016 Plan by one; provided, however, that a share of our common stock covered under a stock-settled SAR shall reduce the total number of shares of common stock available for issuance under the 2016 Plan by one even though the shares of common stock are not actually issued in connection with settlement of the SAR. Except as otherwise provided in the 2016 Plan, any shares of common stock related to an award which terminates by expiration, forfeiture, cancellation or otherwise without issuance of shares of common stock, which is settled in cash in lieu of common stock or which is exchanged, with the Compensation Committee's permission, prior to the issuance of shares of common stock, for awards not involving shares of common stock, shall again be available for issuance under the 2016 Plan. The following shares of common stock, however, may not again be made available for issuance as awards under the 2016 Plan: (i) shares of common stock not issued or delivered as a result of a net settlement of an outstanding award, (ii) shares of common stock tendered or held to pay the exercise price, purchase price or withholding taxes relating to an outstanding award, or (iii) shares of common stock repurchased on the open market with the proceeds of the exercise price of an award.

In any calendar year, no participant may be granted options, SARs, Restricted Stock Awards, RSUs, or any combination thereof that relate to more than 500,000 shares of our common stock (subject to adjustment as provided in the 2016 Plan). In any calendar year, no participant may be granted an Incentive Award (i) with reference to a specified dollar limit for more than $3,000,000 million and (ii) with reference to a specified number of shares of common stock for more than 500,000 shares of common stock (subject to adjustment as provided in the 2016 Plan). The maximum number of shares of our common stock that may be issued pursuant to awards, the per individual limits on awards and the terms of outstanding awards will be adjusted as the Compensation Committee in its sole discretion determines is equitably required in the event of corporate transactions and other appropriate events.

Options. A stock option entitles the participant to purchase from us a stated number of shares of common stock. The Compensation Committee will determine whether the option is intended to be an ISO or a NQSO and specify the number of shares of common stock subject to the option. In the case of ISOs, the aggregate fair market value (determined as of the date of grant) of common stock with respect to which an ISO may become exercisable for the first time during any calendar year cannot exceed $100,000; and if this limitation is exceeded, the ISOs which cause the limitation to be exceeded will be treated as NQSOs. The exercise price per share of common stock may not be less than the fair market value of our common stock on the date the option is granted. With respect to an ISO granted to a participant who beneficially owns more than 10% of the combined voting power of the Company or any of our affiliate (determined by applying certain attribution rules), the exercise price per share may not be less than 110% of the fair market value of our common stock on the date the option is granted. The exercise price may be paid in cash or, if the agreement so provides, the Compensation Committee may allow a participant to pay all or part of the exercise price by tendering shares of our common stock the participant already owns, through a broker-assisted cashless exercise, by means of "net exercise" procedure, any other specified medium of payment or a combination.

Stock Appreciation Rights. A SAR entitles the participant to receive, upon exercise, the excess of the fair market value on that date of each share of common stock subject to the exercised portion of the SAR over the fair market value of each such share on the date of the grant of the SAR. A SAR can be granted alone or in tandem with an option. A SAR granted in tandem with an option is called a Corresponding SAR and entitles the participant to exercise the option or the SAR at which time the other tandem award expires. The Compensation Committee will specify the number of shares of common stock subject to a SAR and whether the SAR is a Corresponding SAR. No participant may be granted Corresponding SARs in tandem with ISOs which are first exercisable in any calendar year for shares of common stock having an aggregate fair market value (determined as of the date of grant) that exceeds $100,000; and if this limitation is exceeded the tandem option will be treated as NQSOs. A Corresponding SAR may be exercised only to the extent that the related option is exercisable and the fair market value of the common stock on the date of exercise exceeds the exercise price of the related option. As set forth in the agreement, the amount payable as a result of the exercise of a SAR may be settled in cash, shares of common stock or a combination of each.

Restricted Stock Awards. A Restricted Stock Award is the grant or sale of shares of our common stock, which may be subject to forfeiture restrictions. The Compensation Committee will prescribe whether the Restricted Stock Award is forfeitable and the conditions to which it is subject. If the participant must pay for a Restricted Stock Award, payment for the award generally shall be made in cash or, if the agreement so provides, by surrendering shares of common stock the participant already owns or any other medium of payment. Prior to vesting or forfeiture, a participant will have all rights of a shareholder with respect to the shares underlying the Restricted Stock Award, including the right to receive dividends and vote the underlying shares; provided, however, the participant may not transfer the shares. We may retain custody of the certificates evidencing the shares or our common stock until such shares are no longer forfeitable.

RSUs. An RSU entitles the participant to receive shares of common stock when certain conditions are met. The Compensation Committee will prescribe when the RSUs shall become payable. We will pay the participant one share of our common stock for each RSU that becomes earned and payable.

Incentive Awards. An Incentive Award entitles the participant to receive cash or common stock or a combination of each when certain conditions are met. The Compensation Committee will prescribe the terms and conditions of the Incentive Award. As set forth in the participant's agreement, an Incentive Award may be paid in cash, shares of common stock or a combination of each.

Performance Objectives. The Compensation Committee has discretion to establish objectively-determinable performance conditions for when awards will become vested, exercisable and payable. Objectively-determinable performance conditions are performance conditions (i) that are established in writing (a) at the time of grant (b) no later than the earlier of (x) 90 days after the beginning of the period of service to which they relate and (y) before the lapse of 25% of the period of service to which they relate; (ii) that are uncertain of achievement at the time they are established; and (iii) the achievement of which is determinable by a third party with knowledge of the relevant facts. These performance conditions may include any or any combination of the following: (a) gross, operating or net earnings before or after taxes; (b) return on equity; (c) return on capital; (d) return on sales; (e) return on investments; (f) return on assets or net assets; (g) earnings per share; (h) cash flow per share; (i) book value per share; (j) gross margin; (k) customers; (l) cash flow or cash flow from operations; (m) fair market value of us or any affiliate or shares of Common Stock; (n) share price or total shareholder return; (o) market share; (p) level of expenses or other costs; (q) gross, operating or net revenue; (r) earnings before interest and taxes; (s) adjusted earnings before interest and taxes; (t) profitability; (u) earnings before interest, taxes, depreciation and amortization; (v) adjusted earnings before interest, taxes, depreciation and amortization; (w) adjusted earnings

before interest, taxes, depreciation and amortization less capital expenditures; (x) research and development milestones; (y) business development objectives, partnerships and other collaborations; or (z) peer group comparisons of any of the aforementioned performance conditions. Performance conditions may be related to a specific customer or group of customers or geographic region. The form of the performance conditions also may be measured on a Company, affiliate, division, business unit, service line, segment or geographic basis or a combination thereof. Performance goals may reflect absolute entity performance or a relative comparison of entity performance to the performance of a peer group of entities or other external measure of the selected performance conditions. Profits, earnings and revenues used for any performance condition measurement may exclude any extraordinary or nonrecurring items. The performance conditions may, but need not, be based upon an increase or positive result under the aforementioned performance criteria and could include, for example and not by way of limitation, maintaining the status quo or limiting the economic losses (measured, in each case, by reference to the specific business criteria). An award that is intended to become exercisable, vested or payable on the achievement of performance conditions means that the award will not become exercisable, vested or payable solely on mere continued employment or service. However, such an award, in addition to performance conditions, may be subject to continued employment or service by the participant. Additionally, the vesting, exercise or payment of an award can be conditioned on mere continued employment or service if it is not intended to qualify as qualified performance-based compensation under Section 162(m) of the Code.

Change in Control. In the event of or in anticipation of a "Change in Control" (as defined in the 2016 Plan), the Compensation Committee in its discretion may terminate outstanding awards (i) by giving the participants an opportunity to exercise the awards that are then exercisable and then terminating, without any payment, all awards that have not been exercised (including those that were not then exercisable) or (ii) by paying the participant the value of the awards that are then vested, exercisable or payable without payment for any awards that are not then vested, exercisable or payable or that have no value. Alternatively, the Compensation Committee may take such other action as the Compensation Committee determines to be reasonable under the circumstances to permit the participant to realize the vested value of the award. The Compensation Committee may provide that a participant's outstanding awards become fully exercisable or payable on and after a Change in Control or immediately before the date the awards will be terminated in connection with a Change in Control. Awards will not be terminated to the extent they are to be continued after the Change in Control.

Stockholder Rights. No participant shall have any rights as a shareholder of us until the award is settled by the issuance of our common stock (other than a Restricted Stock Award or RSUs for which certain shareholder rights may be granted).

Transferability. An award is non-transferable except by will or the laws of descent and distribution, and during the lifetime of the participant to whom the award is granted, the award may only be exercised by, or payable to, the participant. The holder of the transferred award will be bound by the same terms and conditions that governed the award during the period that it was held by the participant.

Maximum Award Period. No award shall be exercisable or become vested or payable more than ten years after the date of grant. An ISO granted to a participant who beneficially owns more than 10% of the combined voting power of us or any affiliate (determined by applying certain attribution rules) or a Corresponding SAR that relates to such an ISO may not be exercisable more than five years after the date of grant.

Compliance With Applicable Law. No award shall be exercisable, vested or payable except in compliance with all applicable federal and state laws and regulations (including, without limitation, tax and securities laws), any listing agreement with any stock exchange to which we are a party, and the rules of all domestic stock exchanges on which our shares may be listed.

Amendment and Termination of Plan. Our board of directors may amend or terminate the 2016 Plan at any time; provided, however, that no amendment may adversely impair the rights of a participant with respect to outstanding awards without the participant's consent. An amendment will be contingent on approval of our shareholders, to the extent required by law, by the rules of any stock exchange on which our securities are then traded or if the amendment would (i) increase the benefits accruing to participants under the 2016 Plan, including without limitation, any amendment to the 2016 Plan or any agreement to permit a repricing or decrease in the exercise price of any outstanding options or SARs, (ii) increase the aggregate number of shares of our common stock that may be issued under the 2016 Plan, (iii) modify the requirements as to eligibility for participation in the 2016 Plan or (iv) change the stated performance conditions for qualified performance-based compensation under Section 162(m) of the Code. Additionally, to the extent the board of directors deems necessary for the 2016 Plan to continue to grant awards that are intended to comply with the performance-based exception to the deduction limits of Code Section 162(m), the board of directors will submit the material terms of

the stated performance conditions to our shareholders for approval no later than the first shareholder meeting that occurs in the fifth year following the year in which the shareholders previously approved the material terms of the performance goals.

Notwithstanding any other provision of the 2016 Plan, the Compensation Committee may amend any outstanding award without participant's consent if, as determined by the Compensation Committee in its sole discretion, such amendment is required either to (i) confirm exemption from Section 409A of the Code, (ii) comply with Section 409A of the Code or (iii) prevent the Participant from being subject to any tax or penalty under Section 409A of the Code.

Forfeiture Provisions. Awards do not confer upon any individual any right to continue in the employ or service of us or any affiliate. All rights to any award that a participant has will be immediately forfeited if the participant is discharged from employment or service for "Cause" (as defined in the 2016 Plan).

Material U.S. Federal Income Tax Consequences

The following discussion summarizes the material United States federal income tax consequences associated with awards granted under the 2016 Plan to U.S. citizens. The discussion is based on laws, regulations, rulings and court decisions currently in effect, all of which are subject to change.

ISOs. A participant will not recognize taxable income on the grant or exercise of an ISO. A participant will recognize taxable income when he or she disposes of the shares of our common stock acquired under the ISO. If the disposition occurs after the ISO holding period, the participant will recognize long-term capital gain (or loss) to the extent the amount realized from the disposition exceeds (or is less than) the participant's tax basis in the shares of our common stock. A participant's tax basis in shares of our common stock generally will be the amount the participant paid for the shares.

If our common stock acquired under an ISO is disposed of before the expiration of the ISO holding period described above, the participant will recognize as ordinary income in the year of the disposition the excess of the fair market value of our common stock on the date of exercise of the ISO over the exercise price. Any additional gain will be treated as long-term or short-term capital gain, depending on the length of time the participant held the shares. A special rule applies to such a disposition where the amount realized is less than the fair market value of our common stock on the date of exercise of the ISO. In that case, the ordinary income the participant will recognize will not exceed the excess of the amount realized on the disposition over the exercise price. If the amount realized is less than the exercise price, the participant will recognize a capital loss (long-term if the stock was held more than one year and short-term if held one year or less). A participant will receive different tax treatment if the exercise price is paid by delivery of our common stock the participant already owns.

Neither us nor any of our affiliates will be entitled to a federal income tax deduction with respect to the grant or exercise of an ISO. However, in the event a participant disposes of our common stock acquired under an ISO before the expiration of the ISO holding period described above, we or our affiliate will be entitled to a federal income tax deduction equal to the amount of ordinary income the participant recognizes.

NQSOs. A participant will not recognize any taxable income on the grant of a NQSO. On the exercise of a NQSO, the participant will recognize as ordinary income the excess of the fair market value of our common stock acquired over the exercise price. A participant's tax basis in our common stock is the amount paid plus any amounts included in income on exercise. The participant's holding period for the stock begins on acquisition of the shares. Any gain or loss that a participant realizes on a subsequent disposition of our common stock acquired on the exercise of a NQSO generally will be treated as long-term or short-term capital gain or loss, depending on the length of time the participant held such shares. The amount of the gain (or loss) will equal the amount by which the amount realized on the subsequent disposition exceeds (or is less than) the participant's tax basis in his or her shares. A participant will receive different tax treatment if the exercise price is paid by delivery of our common stock the participant already owns.

The exercise of a NQSO will entitle us or our affiliate to claim a federal income tax deduction equal to the amount of ordinary income the participant recognizes. If the participant is an employee, that ordinary income will constitute wages subject to withholding and employment taxes.

SARs. A participant will not recognize any taxable income at the time the SARs are granted. The participant at the time of receipt will recognize as ordinary income the amount of cash and the fair market value of our common stock that he or she receives. We or our

affiliate will be entitled to a federal income tax deduction equal to the amount of ordinary income the participant recognizes. If the participant is an employee, that ordinary income will constitute wages subject to withholding and employment taxes.

Restricted Stock Awards. A participant will recognize ordinary income on account of a Restricted Stock Award on the first day that the shares are either transferable or not subject to a substantial risk of forfeiture. The ordinary income recognized will equal the excess of the fair market value of our common stock on such date over the amount, if any, the participant paid for the Restricted Stock Award. However, even if the shares under a Restricted Stock Award are both nontransferable and subject to a substantial risk of forfeiture, the participant may make a special "83(b) election" within 30 days of the grant date to recognize income, and have his or her tax consequences determined, as of the date the Restricted Stock Award is made. The participant's tax basis in the shares received will equal the income recognized plus the price, if any, paid for the Restricted Stock Award. Any gain (or loss) that a participant realizes upon the sale of any common stock acquired pursuant to a Restricted Stock Award will be equal to the amount by which the amount realized on the disposition exceeds (or is less than) the participant's tax basis in the shares and will be treated as long-term (if the shares are held for more than one year) or short-term (if the shares are held for one year or less) capital gain or loss. The participant's holding period for the stock begins on the date the shares are either transferable or not subject to a substantial risk of forfeiture, except that the holding period will begin on the date of grant if the participant makes the special "83(b) election." We or our affiliate will be entitled to a federal income tax deduction equal to the ordinary income the participant recognizes. If the participant is an employee, that ordinary income will constitute wages subject to withholding and employment taxes.

RSUs. The participant will not recognize any taxable income at the time the RSUs are granted. When the terms and conditions to which the RSUs are subject have been satisfied and the RSUs are paid, the participant, at the time of receipt, will recognize as ordinary income the fair market value of our common stock he or she receives. The participant's holding period in our common stock will begin on the date the stock is received. The participant's tax basis in the common stock will equal the amount he or she includes in ordinary income. Any gain or loss that a participant realizes on a subsequent disposition of the shares will be treated as long-term or short-term capital gain or loss, depending on the participant's holding period for the stock (long-term if the shares are held for more than one year; short-term if one year or less). The amount of the gain (or loss) will equal the amount by which the amount realized on the disposition exceeds (or is less than) the participant's tax basis in our common stock. We or our affiliate will be entitled to a federal income tax deduction equal to the ordinary income the participant recognizes. If the participant is an employee, that ordinary income will constitute wages subject to withholding and employment taxes.

Incentive Awards. A participant will not recognize any taxable income at the time an Incentive Award is granted. When the terms and conditions to which an Incentive Award is subject have been satisfied and the award is paid, the participant, at the time of receipt, will recognize as ordinary income the amount of cash and the fair market value of our common stock he or she receives. The participant's holding period in any of our common stock received will begin on the date of receipt. The participant's tax basis in our common stock will equal the amount he or she includes in ordinary income with respect to such shares. Any gain or loss that a participant realizes on a subsequent disposition of our common stock will be treated as long-term or short-term capital gain or loss, depending on the participant's holding period for our common stock (long-term if the shares are held for more than one year; short-term if one year or less). The amount of the gain (or loss) will equal the amount by which the amount realized on the disposition exceeds (or is less than) the participant's tax basis in our common stock. We or our affiliate will be entitled to a federal income tax deduction equal to the amount of ordinary income the participant recognizes. If the participant is an employee, that ordinary income will constitute wages subject to withholding and employment taxes.

Limitation on Deductions. The deduction for a publicly-held corporation for otherwise deductible compensation to a "covered employee" generally is limited to $1 million per year. An individual is a covered employee if he or she is the chief executive officer or one of the other three highest compensated officers for the year (other than the chief executive officer or chief financial officer). The $1 million limit does not apply to compensation payable solely because of the attainment of performance conditions that meet the requirements set forth in Section 162(m) of the Code and the regulations thereunder. Compensation is considered performance-based only if (a) it is paid solely on the achievement of one or more performance conditions; (b) two or more "outside directors" set the performance conditions; (c) before payment, the material terms under which the compensation is to be paid, including the performance conditions, are disclosed to, and approved by, the shareholders; and (d) before payment, two or more "outside directors" certify in writing that the performance conditions have been met. The 2016 Plan has been designed to enable the Compensation Committee to structure awards that are intended to meet the requirements for qualified performance-based compensation that would not be subject to the $1 million per year deduction limit under Section 162(m) of the Code.

Any grant, exercise, vesting or payment of an award may be postponed if we reasonably believes that our or any applicable affiliate's deduction with respect to such award would be limited or eliminated by application of Section 162(m) of the Code to the extent permitted by Section 409A of the Code; provided, however, such delay will last only until the earliest date at which we reasonably anticipates the deduction will not be limited or eliminated under Section 162(m) of the Code.

Other Tax Rules. The 2016 Plan is designed to enable the Compensation Committee to structure awards that are intended to not be subject to Code Section 409A, which imposes certain restrictions and requirements on deferred compensation.

Director Compensation

On September 9, 2020, the Compensation Committee of the Board of the Company approved an amended Outside Director Compensation Policy (the "Prior Policy"). Further, on September 8, 2022, the Compensation Committee of the Board of the Company approved a further amended Outside Director Compensation Policy (the "Amended Policy"). The Company intended for the Outside Director Compensation Policy, as amended, to formalize the Company's policy regarding cash compensation, grants of equity and reimbursement of travel expenses to its Outside Directors.

Prior Policy

Cash Compensation of Directors

Under the Prior Policy, Outside Directors were entitled to an annual cash retainer of $35,000 for their service on the Board (exclusive of any participation on its Committees). Outside Directors serving on any of the Board's Audit, Compensation and Nominating and Corporate Governance Committees in a non-Chairperson capacity were entitled to an annual cash retainer of $7,500, $5,000 and $4,000, respectively, for services on such Committees, and the Chairpersons of such Committees were entitled to an annual cash retainer of $15,000, $10,000 and $8,000, respectively, for their collective service both as members of such Committees and as Chairpersons of such Committees. The Prior Policy did not provide for any per meeting attendance fees for any meeting of the Board or its Committees.

Equity Grants to Directors

Under the Prior Policy, subject to limitations on individual grants to Outside Directors under the Plan, upon an Outside Director's appointment to the Board, such Outside Director automatically was granted a nonstatutory stock option to purchase shares of common stock having a grant date fair value of $320,000, rounded down to the nearest whole share (the "Prior Policy NSO Appointment Award"). Subject to further adjustment provisions as described in the Prior Policy and the Plan, 25% of each Prior Policy NSO Appointment Award will vest on the one-year anniversary of the grant date, and the remaining portion of the Prior Policy NSO Appointment Award will vest on an equal monthly basis over the following 36 months, provided that the Outside Director was in continuous service with the Company or an affiliate of the Company through the applicable vesting date. Each Prior Policy NSO Appointment Award will vest fully upon a Change in Control (as defined in the Plan), in each case, provided that the Outside Director is in continuous service with the Company or an affiliate of the Company through the Change in Control.

In addition, subject to limitations on individual grants to Outside Directors under the Plan, on the first business day after each annual meeting of the Company's stockholders beginning with the 2021 annual meeting, each Outside Director automatically was granted a nonstatutory stock option to purchase shares of common stock having a grant date fair value of $90,000, rounded down to the nearest whole share (the "Prior Policy Annual NSO Award"); provided that the initial Prior Policy Annual NSO Award granted on or after the Prior Policy's effective date was made on September 9, 2020. Subject to further adjustment provisions as described in the Prior Policy and the Plan, 25% of each Prior Policy Annual NSO Award will vest on the one-year anniversary of the grant date, and the remaining portion of the Prior Policy Annual NSO Award will vest on an equal monthly basis over the following 36 months, provided that the Outside Director is in continuous service with the Company or an affiliate of the Company through the applicable vesting date. Each Prior Policy Annual NSO Award will vest fully upon a Change in Control (as defined in the Plan), in each case, provided that the Outside Director is in continuous service with the Company or an affiliate of the Company through the Change in Control.

With regard to any of the nonstatutory stock options granted under the Prior Policy described above, the per share exercise price for all such options will be 100% of the fair market value of the shares underlying the options on the grant date.

Furthermore, the Prior Policy provided that Outside Directors would be eligible to receive all types of awards (except incentive stock options) under the Company's 2019 Stock Incentive Plan, as amended (the "Plan") (or the applicable equity plan in place at the time of grant), including discretionary awards not covered under the Prior Policy.

Amended Policy

Cash Compensation of Directors

Under the Amended Policy, Outside Directors will be entitled to an annual cash retainer of $35,000 for their service on the Board (exclusive of any participation on its Committees) ("General Board Fee"). Outside Directors serving on any of the Board's Audit, Compensation and Nominating and Corporate Governance Committees in a non-Chairperson capacity will be entitled to an annual cash retainer of $7,500, $5,000 and $4,000, respectively, for services on such Committees, and the Chairpersons of such Committees will be entitled to an annual cash retainer of $15,000, $10,000 and $8,000, respectively, for their collective service both as members of such Committees and as Chairpersons of such Committees ("Committee Fee"). The Amended Policy does not provide for any per meeting attendance fees for any meeting of the Board or its Committees.

The Amended Policy provides that the Lead Independent Director of the Board shall be entitled to an annual cash retainer of $20,000 for their service as the Lead Independent Director, which amount is in addition to the General Board Fee and any Committee Fees.

Equity Grants to Directors

Under the Amended Policy, subject to limitations on individual grants to Outside Directors under the Plan, upon an Outside Director's appointment to the Board, such Outside Director automatically will be granted a nonstatutory stock option (i) to purchase that number of shares of Company common stock equal to 0.24% of the Company's outstanding shares of voting capital stock as of the close of business on the effective date of such Outside Director's appointment, rounded down to the nearest whole share, or (ii) with a grant date fair value of $180,000, rounded down to the nearest whole share, whichever of clause (i) and (ii) above results in the lowest number of shares subject to the stock option (the "Amended Policy NSO Appointment Award"). Subject to further adjustment provisions as described in the Amended Policy and the Plan, 25% of each Amended Policy NSO Appointment Award will vest on the one-year anniversary of the grant date, and the remaining portion of the Amended Policy NSO Appointment Award will vest on an equal monthly basis over the following 36 months, provided that the Outside Director is in continuous service with the Company or an affiliate of the Company through the applicable vesting date. Each Amended Policy NSO Appointment Award will vest fully upon a Change in Control (as defined in the Plan), in each case, provided that the Outside Director is in continuous service with the Company or an affiliate of the Company through the Change in Control.

In addition, subject to limitations on individual grants to Outside Directors under the Plan, on the first business day after each annual meeting of the Company's stockholders (the "Annual Meeting") beginning with the 2022 Annual Meeting, each Outside Director automatically will be granted a nonstatutory stock option (i) to purchase that number of shares equal to 0.12% of the Company's outstanding shares of voting capital stock as of the close of business on the date of such Annual Meeting, rounded down to the nearest whole share, or (ii) with grant date fair value of $90,000, rounded down to the nearest whole share, whichever of clause (i) and (ii) above results in the lowest number of shares subject to the stock option (the "Amended Policy Annual NSO Award"). Subject to further adjustment provisions as described in the Amended Policy and the Plan, 25% of each Amended Policy Annual NSO Award will vest on the one-year anniversary of the grant date, and the remaining portion of the Amended Policy Annual NSO Award will vest on an equal monthly basis over the following 36 months, provided that the Outside Director is in continuous service with the Company or an affiliate of the Company through the applicable vesting date. Each Amended Policy Annual NSO Award will vest fully upon a Change in Control (as defined in the Plan), in each case, provided that the Outside Director is in continuous service with the Company or an affiliate of the Company through the Change in Control.

With regard to any of the nonstatutory stock options granted under the Amended Policy described above, the per share exercise price for all such options will be 100% of the fair market value of the shares underlying the options on the grant date.

Non-Employee Director Compensation for Fiscal 2022

Below is a summary of the non-employee director compensation paid in the fiscal year ended September 30, 2022:

Name	Cash Compensation[1]	Option Grants [2]	Stock Awards [3]	Total
Dov A. Goldstein, M.D.	$ 58,625	$ 15,792	$ —	$ 74,417
Eric J. Ende, M.D. [4]	$ 28,125	$ 335,792	$ —	$ 363,917
Franklyn G. Prendergast, M.D., Ph.D.	$ 52,500	$ 15,792	$ —	$ 68,292
Eric I. Richman	$ 52,583	$ 15,792	$ —	$ 68,375
Gerald J. McDougall	$ 44,000	$ 15,792	$ —	$ 59,792

(1) Represents the value of the annual retainers payable to our non-employee directors during fiscal 2022.
(2) Pursuant to the Amended Policy, the Company granted each of our Outside Directors a stock option to purchase 37,600 shares of common stock with an exercise price of $0.60 on September 9, 2022. The amounts in this column reflect the aggregate grant date fair value of equity awards granted during the applicable fiscal year, calculated in accordance with FASB ASC Topic 718 and using a Black-Scholes valuation model. Assumptions used in the calculation of these amounts are included in Note 13 of the audited consolidated financial statements included in this Form 10-K.
(3) We did not grant any stock awards to our directors in fiscal 2022.
(4) Dr. Ende was appointed to our board of directors on January 1, 2022. In connection with the Prior Policy, Dr. Ende was granted stock options to purchase shares of Company common stock having a grant date fair value of $320,000.

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

Equity Compensation Plan Information

The following table gives information as of September 30, 2022 about shares of our common stock that may be issued upon the exercise of options under our existing equity compensation plans:

Plan Category	Number of securities to be issued upon exercise of outstanding options, warrants and rights (a)[1]	Weighted-average exercise price of outstanding options, warrants and rights (b)[2]	Number of securities remaining available for future issuance under equity compensation plans (excluding securities reflected in column (a)) (c)[3]
Equity compensation plans approved by security holders[4]	5,334,601	$ 4.62	844,934
Equity compensation plans not approved by security holders[5]	2,519,680	$ 0.0011	—
Total	7,854,281	$ 3.14	844,934

(1) Consists of options outstanding as of September 30, 2022 under the NeuBase Therapeutics, Inc. 2019 Stock Incentive Plan (the "2019 Plan"), the NeuBase Therapeutics, Inc. 2018 Equity Incentive Plan (the "2018 Plan"), the Ohr Pharmaceutical, Inc. 2016 Consolidated Stock Incentive Plan (the "2016 Plan").
(2) Consists of the weighted-average exercise price of outstanding options as of September 30, 2022.
(3) Consists entirely of shares of common stock that remain available for future issuance under the 2019 Plan and the 2016 Plan as of September 30, 2022.
(4) The number of shares of our common stock available for issuance under the 2019 Plan will automatically increase on October 1st of each year, for a period of not more than ten years, beginning October 1, 2019 and ending on (and including) October 1, 2028 by the lesser of (a) 4.0% of the total number of shares of our common stock outstanding as of September 30th of the immediately preceding fiscal year, and (b) such number of shares of common stock determined by our board of directors.
(5) Consists of the 2018 Plan.

Security Ownership of Certain Beneficial Owners and Management

The following table sets forth information with respect to the beneficial ownership, as of September 30, 2022, of our common stock by (a) each of our Named Executive Officers and current directors individually, (b) our current directors and executive officers as a group and (c) each holder of more than 5% of our outstanding common stock. This table is based upon information supplied by officers, directors and principal stockholders and a review of Schedules 13D and 13G, if any, filed with the SEC. Other than as set forth below, we are not aware of any other beneficial owner of more than five percent of our common stock as of September 30, 2022. Except as indicated by the footnotes below, we believe, based on the information furnished to us, that the persons and entities named in the table below have sole voting and investment power with respect to all shares of common stock that they beneficially own, subject to applicable community property laws.

Beneficial ownership and percentage ownership are determined in accordance with the Rule 13d-3 of the Exchange Act. Under these rules, shares of our common stock issuable under stock options that are exercisable within 60 days of September 30, 2022 are deemed outstanding for the purpose of computing the percentage ownership of the person holding the options, but are not deemed outstanding for the purpose of computing the percentage ownership of any other person.

Unless otherwise indicated and subject to applicable community property laws, to our knowledge, each stockholder named in the following table possesses sole voting and investment power over their shares of our common stock, except for those jointly owned with that person's spouse.

Name and Address of Beneficial Owner	Number of Shares Beneficially Owned	Percentage of Class (%)[1]
5% Stockholders		
Dietrich A. Stephan, Ph.D., Chief Executive Officer [2]	4,689,300	14.2 %
Greenlight Capital, Inc. [3]	2,727,027	8.3 %
Other Directors and Named Executive Officers [4]		
Todd P. Branning, Chief Financial Officer[5]	30,000	*
William R. Mann, Ph.D., Former President and Chief Operating Officer[6]	116,928	*
Dov A. Goldstein, M.D., Director [7]	277,173	*
Franklyn G. Prendergast, M.D., Ph.D., Director [8]	247,173	*
Eric I. Richman, Director[9]	292,133	*
Gerald J. McDougall, Director[10]	46,499	*
Eric J. Ende, M.D., Director	—	*
All current executive officers and directors as a group (seven persons) [11]	5,582,278	16.9 %

* Less than one percent.

(1) Percentage ownership is calculated based on a total of 33,008,657 shares of our common stock issued and outstanding as of September 30, 2022.

(2) Represents (i) 2,598,706 shares of common stock issuable pursuant to stock options exercisable within 60 days of September 30, 2022, (ii) 1,273,819 shares of our common stock held by family trusts, and (iii) 816,775 shares of our common stock held indirectly by Dietrich A. Stephan, Ph.D.

(3) Greenlight Capital, Inc. ("Greenlight Inc.") is the investment manager for Greenlight Capital Offshore Partners, Ltd. and as such has voting and dispositive power over 1,271,596 shares of our common stock. DME Capital Management, LP ("DME Management") (i) is the investment manager for Greenlight Capital Offshore Master, Ltd., and as such has voting and dispositive power over 783,631 shares of our common stock held by Greenlight Capital Offshore Master, Ltd. and (ii) manages a portfolio for a private investment fund, and as such has voting and dispositive power over 311,000 shares of our common stock held by such private investment fund. DME Advisors, LP ("DME Advisors") is the investment manager for Solasglas Investments, LP, and as such has voting and dispositive power over 360,800 shares of our common stock held by Solasglas Investments, LP. DME Advisors GP, LLC ("DME GP") is the general partner of DME Management and DME Advisors, and as such has voting and dispositive power over 1,455,431 shares of our common stock. David Einhorn is the principal of Greenlight Inc., DME Management and DME GP, and as such has voting and dispositive power over 2,727,027 shares of our common stock held by these affiliates of Greenlight, Inc. Mr. Einhorn disclaims beneficial ownership of these shares of our common stock, except to the extent of any pecuniary interest therein. The address of Greenlight Capital, Inc. is 2 Grand Central Tower, 140 East 45th Street, 24th Floor, New York, NY 10017.

(4) Unless otherwise indicated, the address for each of our executive officers and directors is c/o 350 Technology Drive, Pittsburgh, PA 15219.
(5) Represents 30,000 shares of common stock held directly by Todd P. Branning.
(6) Represents (i) 105,728 shares of common stock issuable pursuant to stock options exercisable within 60 days after September 30, 2022, and (ii) 11,200 shares of common stock held directly by William Mann, Ph.D.
(7) Represents (i) 247,173 shares of common stock issuable pursuant to stock options exercisable within 60 days after September 30, 2022 held directly by Dov A. Goldstein, M.D., and (ii) 30,000 shares of our common stock held directly by Dov A. Goldstein, M.D.
(8) Represents 247,173 shares of common stock issuable pursuant to stock options exercisable within 60 days after September 30, 2022 held directly by Franklyn G. Prendergast, M.D., Ph.D.
(9) Represents (i) 247,173 shares of common stock issuable pursuant to stock options exercisable within 60 days after September 30, 2022, (ii) 27,747 shares of our common stock held by Eric I. Richman jointly with his spouse, and (iii) 17,213 shares of our common stock held directly by Eric I. Richman.
(10) Represents 46,499 shares of common stock issuable pursuant to stock options exercisable within 60 days after September 30, 2022 held directly by Gerald J. McDougall.
(11) Comprised of shares included under "Other Directors and Named Executive Officers," as well as those of Dr. Stephan, our Chief Executive Officer, and excluding Dr. Mann, who resigned from the Company effective October 31, 2022.

ITEM 13. CERTAIN RELATIONSHIPS AND RELATED TRANSACTIONS AND DIRECTOR INDEPENDENCE

Described below are any transactions occurring since October 1, 2020 and any currently proposed transactions to which we were a party and in which:

- the amounts involved exceeded or will exceed $120,000; and
- a director, executive officer, holder of more than 5% of the outstanding capital stock of NeuBase, or any member of such person's immediate family had or will have a direct or indirect material interest.

Our board of directors has adopted a written related policy with respect to related person transactions. This policy governs the review, approval or ratification of covered related person transactions. The Audit Committee of our board of directors (the "Audit Committee") manages this policy.

For purposes of this policy, a "related person transaction" is a transaction, arrangement or relationship (or any series of similar transactions, arrangements or relationships) in which we (or any of our subsidiaries) were, are or will be a participant, and the amount involved exceeds $120,000 and in which any related person had, has or will have a direct or indirect interest. For purposes of determining whether a transaction is a related person transaction, the Audit Committee relies upon Item 404 of Regulation S-K, promulgated under the Exchange Act.

A "related person" is defined as:

- Any person who is, or at any time since the beginning of our last fiscal year was, one of our directors or executive officers or a nominee to become one of our directors;
- any person who is known to be the beneficial owner of more than five percent of any class of our voting securities;
- any immediate family member of any of the foregoing persons, which means any child, stepchild, parent, stepparent, spouse, sibling, mother-in-law, father-in-law, son-in-law, daughter-in-law, brother-in-law or sister-in-law of the director, executive officer, nominee or more than five percent beneficial owner, and any person (other than a tenant or employee) sharing the household of such director, executive officer, nominee or more than five percent beneficial owner; or
- any firm, corporation, or other entity in which any of the foregoing persons is employed or is a general partner or principal or in a similar position or in which such person has a ten percent or greater beneficial ownership interest.

The policy generally provides that we may enter into a related person transaction only if:

- the Audit Committee pre-approves such transaction in accordance with the guidelines set forth in the policy;
- the transaction is on terms comparable to those that could be obtained in arm's length dealings with an unrelated third party and the Audit Committee (or the chairperson of the Audit Committee) approves or ratifies such transaction in accordance with the guideline set forth in the policy;
- the transaction is approved by the disinterested members of our board of directors; or
- the transaction involves compensation approved by our Compensation Committee.

In the event a related person transaction is not pre-approved by the Audit Committee and our management determines to recommend such related person transaction to the Audit Committee, such transaction must be reviewed by the Audit Committee. After review, the Audit Committee will approve or disapprove such transaction. When our Chief Financial Officer in consultation with our Chief Executive Officer, determines that it is not practicable or desirable for us to wait until the next Audit Committee meeting, the chairperson of the Audit Committee possesses delegated authority to act on behalf of the Audit Committee. The Audit Committee (or the chairperson of the Audit Committee) may approve only those related person transactions that are in, or not inconsistent with, our best interests and the best interests of our stockholders, as the Audit Committee (or the chairperson of the Audit Committee) determines in good faith.

Our Audit Committee has determined that certain types of related person transactions are deemed to be pre-approved by our Audit Committee. Our related person transaction policy provides that the following transactions, even if the amount exceeds $120,000 in the aggregate, are considered to be pre-approved by our Audit Committee:

- any employment of certain named executive officers that would be publicly disclosed;
- director compensation that would be publicly disclosed;
- transactions with other companies where the related person's only relationship is as a director or owner of less than ten percent of said company (other than a general partnership), if the aggregate amount involved does not exceed the greater of $200,000 or five percent of that company's consolidated gross revenues;
- transactions where all stockholders receive proportional benefits;
- transactions involving competitive bids;
- transactions with a related person involving the rendering of services at rates or charges fixed inconformity with law or governmental authority; and
- transactions with a related person involving services as a bank depositary of funds, transfer agent, registrar, trustee under a trust indenture or similar services.

In addition, our Audit Committee will review the policy at least annually and recommend amendments to the policy to our board of directors from time to time.

The policy provides that all related person transactions will be disclosed to our Audit Committee, and all material related person transactions will be disclosed to our board of directors. Additionally, all related person transactions requiring public disclosure will be disclosed, as applicable, on our various public filings.

Our Audit Committee will review all relevant information available to it about the related person transaction. The policy provides that the Audit Committee may approve or ratify the related person transaction only if our Audit Committee determines that, under all of the circumstances, the transaction is in, or is not inconsistent with, our best interests. The policy provides that our Audit Committee

may, in its sole discretion, impose such conditions as it deems appropriate on us or the related person in connection with approval of the related person transaction.

Transactions with Related Persons

Participation in Our Public Offering

On April 26, 2021, the Company closed an underwritten public offering of 9,200,000 shares of its common stock (including shares of common stock purchased by the underwriters pursuant to the exercise of their option to cover over-allotments) at a price to the public of $5.00 per share. The Company received gross proceeds of $46.0 million from the offering before deducting the underwriting discounts and commissions and offering expenses payable by the Company. Entities affiliated with Greenlight Capital, Inc. ("Greenlight Inc.") purchased 700,000 shares of common stock in the public offering at the public offering price of $5.00 per share. Greenlight Inc. is the investment manager of Greenlight Capital Qualified, L.P., Greenlight Capital, L.P. and Greenlight Capital Offshore Partners and is affiliated with DME Capital Management, LP and DME Advisors, LP, each of whom are investment advisors to certain of our stockholders. These stockholders form a group representing one of our principal stockholders as set forth in "Item 12— Security Ownership of Certain Beneficial Owners and Management and Related Stockholder Matters."

Indemnification Agreements with Directors and Executive Officers

We have entered into indemnification agreements with our directors and executive officers under which we agreed to indemnify those individuals, under the circumstances and to the extent provided for in the agreements, for expenses, damages, judgments, fines, penalties, settlements and any other amounts they may be required to pay in actions, suits or proceedings which they are or may be made a party or threatened to be made a party by reason of their position as a director, officer or other agent of ours, and otherwise to the fullest extent permitted under Delaware law and our amended and restated certificate of incorporation and Restated Bylaws. We believe that these indemnification agreements are necessary to attract and retain qualified directors, officers and other key employees.

Director Independence

Our common stock is listed on The Nasdaq Capital Market. Under the Nasdaq rules (the "Nasdaq Rules"), independent directors must comprise a majority of a listed company's board of directors. In addition, the Nasdaq Rules require that, subject to specified exceptions, each member of a listed company's audit, compensation, and nominating and corporate governance committees be independent. Under the Nasdaq Rules, a director will only qualify as an "independent director" if, in the opinion of the listed company's board of directors, the director does not have a relationship that would interfere with the exercise of independent judgment in carrying out the responsibilities of a director.

Audit Committee members must also satisfy the independence criteria set forth in Rule 10A-3 under the Exchange Act and the Nasdaq Rules. In addition, Compensation Committee members must satisfy the independence criteria set forth in Rule 10C-1 under the Exchange Act and the Nasdaq Rules.

Our board of directors has determined that each of Drs. Goldstein, Prendergast and Ende and Messrs. Richman and McDougall meet the definitions of independence under the Nasdaq Marketplace Rules and Section 10A-3 of Exchange Act. Accordingly, all of our directors, other than our Chief Executive Officer, Dr. Stephan, are deemed to be independent.

Our board of directors has an independent Chairperson, Dr. Goldstein, and we believe that having independent leadership is an important component of our governance structure. Our independent Chairperson has authority, among other things, to preside over board of directors meetings, including meetings of the independent directors, and to call special meetings of our board of directors. Accordingly, the independent Chairperson has substantial ability to shape the work of our board of directors. We currently believe that having an independent Chairperson creates an environment that is more conducive to objective evaluation and oversight of management's performance, increasing management accountability and improving the ability of our board of directors to monitor whether management's actions are in the best interests of our company and our stockholders.

In addition, we believe that separation of the roles of Chairperson and Chief Executive Officer enhances the accountability of our Chief Executive Officer to our board of directors and encourages balanced decision making. While our Chief Executive Officer is

responsible for our day-to-day leadership and operations, our independent Chairperson provides guidance to our board of directors and sets the agenda for meetings of the board of directors.

However, no single leadership model is right for all companies and at all times. Our board of directors recognizes that, depending on the circumstances, other leadership models, such as combining the role of Chairperson with the role of Chief Executive Officer, might be appropriate. Accordingly, our board of directors periodically reviews its leadership structure and will continue to evaluate and implement the leadership structure that it concludes most effectively supports our board of directors in fulfilling its responsibilities.

ITEM 14. PRINCIPAL ACCOUNTANT FEES AND SERVICES

The following is a summary of the fees billed to us by Marcum LLP ("Marcum"), our current independent registered public accounting firm, for professional services rendered for the fiscal years ended September 30, 2022 and 2021, respectively:

	2022	2021
Audit Fees [(1)]:	$ 322,650	$ 272,950
Audit-Related Fees [(2)]:	—	—
Tax Fees [(3)]:	—	—
All Other Fees [(4)]:	—	—
Total All Fees:	$ 322,650	$ 272,950

(1) Audit fees for fiscal 2022 consist of fees for professional services performed by Marcum for the audit of our 2022 annual financial statements that are included in our Annual Report on Form 10-K for the year ended September 30, 2022, fees for the review of financial statements included in our Quarterly Reports on Form 10-Q filed in fiscal 2022, and reviews of registration statements and issuances of consents, comfort letters and services that are normally provided in connection with statutory and regulatory filings or engagements. Audit fees for fiscal 2021 consist of fees for professional services performed by Marcum for the audit of our 2021 annual financial statements that are included in our Annual Report on Form 10-K for the year ended September 30, 2021, fees for the review of financial statements included in our Quarterly Reports on Form 10-Q filed in fiscal 2021, and reviews of registration statements and issuances of consents, comfort letters and services that are normally provided in connection with statutory and regulatory filings or engagements.

(2) Audit-Related Fees consist of fees for other audit-related professional services.

(3) Consists of fees for tax compliance and consulting.

(4) No other fees were earned or paid for fiscal 2022 or fiscal 2021.

Pre-Approval Policies and Procedures

All audit and non-audit services previously provided by our independent registered public accounting firm must be pre-approved by the Audit Committee. Pre-approval may be given for a category of services, provided that (i) the category is reasonably narrow and detailed and (ii) the Audit Committee establishes a fee limit for such category. The Audit Committee may delegate to any other member of the Audit Committee the authority to grant pre-approval of permitted non-audit services to be provided by Marcum between Audit Committee meetings; provided, however, that any such pre-approval shall be presented to the full Audit Committee at its next scheduled meeting. The Audit Committee pre-approved all audit and permitted non-audit services provided by Marcum for professional services rendered for the fiscal years ended September 30, 2022 and 2021.

PART IV.

ITEM 15. EXHIBITS AND FINANCIAL STATEMENT SCHEDULES

(a) 1. Financial Statements
The information required by this item is included in Item 8 of Part II of this Form 10-K.

2. Financial Statement Schedules
The information required by this item is included in Item 8 of Part II of this Form 10-K.

3. Exhibits
The following exhibits are incorporated by reference or filed as part of this report:

		Incorporated by Reference			
Exhibit Number	**Description**	**Form**	**File Number**	**Filing Date**	**Exhibit**
2.1+	Agreement and Plan of Merger and Reorganization, dated as of January 2, 2019, by and among Ohr Pharmaceutical, Inc., Ohr Acquisition Com. and NeuBase Therapeutics, Inc.	8-K	001-35963	1/3/2019	2.1
2.2	First Amendment to the Agreement and Plan of Merger and Reorganization, dated as of June 27, 2019, by and among Ohr Pharmaceutical, Inc., Ohr Acquisition Corp. and NeuBase Therapeutics, Inc.	8-K	001-35963	7/3/2019	2.1
3.1	Amended and Restated Certificate of Incorporation of the Company.	8-K	001 35963	7/12/2019	3.1
3.2	Amended and Restated Bylaws of the Company.	8-K	001-35963	9/23/2019	3.1
4.1	Form of Consulting Warrants.	10-K	001-35963	8/15/2011	10.21
4.2	Form of Common Stock Certificate.	S-8	333-233346	8/16/2019	4.17
4.3	Description of Securities of NeuBase Therapeutics, Inc.	10-K	001-35963	1/10/2020	4.5
10.1#	Ohr Pharmaceutical, Inc. 2016 Consolidated Stock Incentive Plan.	8-K	001-35963	3/21/2016	10.1
10.2#	Form of Stock Option Agreement (2016 Consolidated Stock Incentive Plan).	10-K	001-35963	12/15/2017	10.11(b)
10.3#	Form of Restricted Stock Agreement (2016 Consolidated Stock Incentive Plan).	10-K	001-35963	12/15/2017	10.11(c)
10.4†	License Agreement, dated December 17, 2018, by and between NeuBase Therapeutics, Inc. and Carnegie Mellon University.	S-4	333-230168	3/8/2019	10.15
10.5++*	Amendment #1 to License Agreement, dated January 1, 2022, by and between NeuBase Therapeutics, Inc. and Carnegie Mellon University.				
10.6	Form of NeuBase Therapeutics, Inc. Warrant Certificate.	S-4	333-230168	3/8/2019	10.16
10.7#	NeuBase Therapeutics, Inc. 2018 Equity Incentive Plan.	S-4	333-230168	3/8/2019	10.19
10.8#	Restricted Stock Purchase Agreement, made as of September 6, 2018, by and between NeuBase Therapeutics, Inc. and Dietrich A. Stephan.	S-4	333-230168	3/8/2019	10.21
10.9#	Amendment to Restricted Stock Purchase Agreement, made as of December 26, 2018, by and between NeuBase Therapeutics, Inc. and Dietrich A. Stephan.	S-4	333-230168	3/8/2019	10.22
10.10#	Executive Employment Agreement, entered into as of December 22, 2018 and effective as of August 28, 2018, by and between NeuBase Therapeutics, Inc. and Dietrich A. Stephan.	S-4/A	333-230168	5/7/2019	10.23

Exhibit Number	Description	Incorporated by Reference			
		Form	File Number	Filing Date	Exhibit
10.11	At-Will Employment, Confidential Information, Invention Assignment and Arbitration Agreement, dated December 22, 2018, by and between NeuBase Therapeutics, Inc. and Dietrich A. Stephan.	S-4/A	333-230168	5/7/2019	10.24
10.12#	Offer of Employment, dated July 11, 2019, by and between NeuBase Therapeutics, Inc. and Dietrich A. Stephan.	8-K/A	001-35963	7/17/2019	10.1
10.13#	NeuBase Therapeutics, Inc. 2019 Stock Incentive Plan.	S-4	333-230168	3/8/2019	Annex E
10.14#	Form of Option Agreement under the NeuBase Therapeutics, Inc. 2019 Stock Incentive Plan.	S-8	333-233346	8/16/2019	4.6
10.15#	Form of Option Agreement under the NeuBase Therapeutics, Inc. 2018 Equity Incentive Plan.	S-8	333-233346	8/16/2019	4.8
10.16#	NeuBase Therapeutics, Inc. Outside Director Compensation Policy.	8-K	001-35963	09/12/2022	10.1
10.17	Sublease Agreement, dated as of March 12, 2019, by and between NeuBase Therapeutics, Inc. and StartUptown dba Avenu.	10-K	001-35963	1/10/2020	10.29
10.18	Amendment No. 1 to Sublease Agreement, dated as of May 21, 2019, by and between NeuBase Therapeutics, Inc. and StartUptown dba Avenu.	10-K	001-35963	1/10/2020	10.30
10.19	Amendment No. 2 to Sublease Agreement, dated as of July 29, 2019, by and between NeuBase Therapeutics, Inc. and StartUptown dba Avenu.	10-K	001-35963	1/10/2020	10.31
10.20	Lease Extension to Sublease Agreement, dated as of February 26, 2020, by and between NeuBase Therapeutics, Inc. and StartUptown dba Avenu.	10-Q	001-35963	3/26/2020	10.1
10.21	Amendment No. 4 to Sublease Agreement, dated as of August 20, 2020.	10-K	001-35963	12/23/2020	10.26
10.22	Amendment No. 5 to Sublease Agreement, dated as of September 25, 2020.	10-K	001-35963	12/23/2020	10.27
10.23#	Offer Letter of Employment, dated July 22, 2020, by and between NeuBase Therapeutics, Inc. and William Mann.	8-K	001-35963	7/28/2020	10.1
10.24#	Offer Letter of Employment, dated January 10, 2022, by and between NeuBase Therapeutics, Inc. and Todd Branning.	8-K	001-35963	1/10/2022	10.1
10.25	Lease Agreement, dated as of October 2, 2020, by and between NeuBase Therapeutics, Inc. and 350 Technology Drive Partners, LLC.	10-K	001-35963	12/23/2020	10.30
10.26	First Amendment to Lease Agreement, dated December 28, 2020.	10-Q	333-88480	2/11/2021	10.2
10.27	Second Amendment to Lease Agreement, dated April 21, 2021.	8-K	001-35963	4/27/2021	10.1
21.1*	Subsidiaries				
23.1*	Consent of Marcum LLP.				
31.1*	Certification of Chief Executive Officer Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.				
31.2*	Certification of Chief Financial Officer Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.				
32.1*	Certification of Chief Executive Officer and Chief Financial Officer Pursuant to 18 U.S.C Section 1350, As				

Exhibit Number	Description	Incorporated by Reference			
		Form	File Number	Filing Date	Exhibit
	Adopted Pursuant to Section 906 of the Sarbanes Oxley Act of 2002.				
101.INS*	XBRL Instance Document.				
101.SCH*	XBRL Taxonomy Extension Schema Document.				
101.CAL*	XBRL Taxonomy Extension Calculation Linkbase Document.				
101.DEF*	XBRL Taxonomy Extension Definition Linkbase Document.				
101.LAB*	XBRL Taxonomy Extension Label Linkbase Document				
101.PRE*	XBRL Taxonomy Extension Presentation Linkbase Document.				

* Filed herewith.

\+ All schedules and exhibits to the agreement have been omitted pursuant to Item 601(b)(2) of Regulation S-K. A copy of any omitted schedule and/or exhibit will be furnished to the SEC upon request.

++ Certain identified information has been omitted pursuant to Item 601(b)(10) of Regulation S-K because such information is both (i) not material and (ii) information that the registrant treats as private or confidential. The Registrant hereby undertakes to furnish supplemental copies of the unredacted exhibit upon request by the SEC.

\# Management compensatory plan or arrangement.

† The SEC has granted confidential treatment with respect to certain portions of this exhibit. Those portions have been omitted and filed separately with the SEC.

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.

Date: December 21, 2022

NeuBase Therapeutics, Inc.
/s/ Dietrich A. Stephan
Dietrich A. Stephan, Ph.D.
Chief Executive Officer

Signature	Title	Date
/s/ Dietrich A. Stephan Dietrich A. Stephan, Ph.D.	Chief Executive Officer and Director *(Principal Executive Officer)*	December 21, 2022
/s/ Todd P. Branning Todd P. Branning	Chief Financial Officer *(Principal Financial Officer and Accounting Officer)*	December 21, 2022
/s/ Dov A. Goldstein Dov A. Goldstein, M.D.	Director	December 21, 2022
/s/ Gerald J. McDougall Gerald J. McDougall	Director	December 21, 2022
/s/ Franklyn G. Prendergast Franklyn G. Prendergast, M.D., Ph.D.	Director	December 21, 2022
/s/ Eric I. Richman Eric I. Richman	Director	December 21, 2022
/s/ Eric J. Ende Eric J. Ende, M.D.	Director	December 21, 2022

***** Certain information in this document has been excluded pursuant to Regulation S-K, Item 601(b)(10). Such excluded information is not material and is the type the registrant treats as confidential. Such omitted information is indicated by brackets "[***]") in this exhibit. *****

AMENDMENT #1 TO LICENSE AGREEMENT

This Amendment #1 to the License Agreement (hereinafter this "Amendment") is made and entered into effective as of January 1, 2022 by and between Carnegie Mellon University (hereinafter "Carnegie Mellon") and NeuBase Therapeutics, Inc. (hereinafter "Licensee").

Witnesseth

Whereas, Carnegie Mellon and Licensee have previously entered into a License Agreement, effective December 17, 2018 (hereinafter the "License Agreement");

Whereas, Carnegie Mellon and Vera Therapeutics, Inc. f/k/a Trucode Gene Repair Inc. f/k/a PNA Innovations, Inc. ("Vera") entered into a License Agreement, effective June 7, 2012 as amended, for the license by Vera of certain Carnegie Mellon rights in certain technology relating to peptide nucleic acids (hereinafter the "Vera License"), and in connection with an asset purchase agreement between Licensee and Vera involving the peptide nucleic acid portfolio of Vera, effective as of January 27, 2021, Vera assigned the Vera License to Licensee and Licensee assumed and agreed to be bound to the terms and conditions of the Vera License, including all obligations to Carnegie Mellon under the Vera License;

Whereas, Licensee and Carnegie Mellon desire to amend the License Agreement to provide for, among other things, the license pursuant to License Agreement of Carnegie Mellon rights in certain technology relating to peptide nucleic acids that were previously licensed under the Vera License, termination of the Vera License and similar matters, all on and subject to the terms and conditions of this Amendment;

Now Therefore, in consideration of the mutual covenants contained herein and intending to be legally bound hereby, the parties agree as follows:

1. Section 1.1 of the License Agreement is amended by adding the following at the end of that section:

   "In addition to the foregoing, **"Patent(s)"** shall also mean any patent or patent application, including any continuation, continuation-in-part, divisional or modification filed in the U.S. or any other country and any patent claiming priority therefrom or reissue thereof, which issues to Carnegie Mellon and specifically claims any of the Amendment Licensed Technology in existence on the "as of" date specified in Exhibit A attached hereto.".

2. Section 1.2 of the License Agreement is amended by adding the following at the end of that section:

   "In addition to the foregoing, "**Licensed Technology**" or "**Technology**" shall also mean (a) the technology described in Exhibit A attached hereto on an "as is" basis on

the "as of" date specified in Exhibit A attached hereto (the "**Amendment Licensed Technology**"), and (b) the Patents, including the patents and patent applications specified in Exhibit A attached hereto.".

3. Section 1.11 of the License Agreement is amended and restated to read as follows:

"1.11 "**Sublicense Fees**" shall mean any and all consideration realized by Licensee from a sublicense in consideration of a sublicense for Licensed Technology, excluding Revenue realized by Licensee for the Disposition of Licensed Products to sublicensee, such Revenue being subject to Royalties, and further provided that 'Sublicense Fees' shall not include any payments for past, present or future research, development, manufacturing or commercial launch services or activities performed for or provided to a sublicensee (including any sponsored research performed by Licensee for a sublicensee); provided that such payments shall only be excluded to the extent that the amount paid represents fair market value for the applicable item or activity, and any payment made by or on behalf of a sublicensee in excess of such fair market value will be included in Sublicense Fees.".

4. Section 1 of the License Agreement is amended by adding a new Section 1.22 thereto, to read as follows:

"1.22 "**Phase II Clinical Trial**" shall mean a human clinical trial conducted to evaluate the efficacy of a drug for a particular indication in patients with a disease and to determine the common short-term side effects and risks associated with the drug as defined in 21 C.F.R. §312.21(b).".

5. Romanette (iii) of Section 5.2 of the License Agreement is deleted in its entirety and replaced with "Intentionally Omitted.".

6. Item b) of Section 5.2 (iv) of the License Agreement is hereby amended by replacing "2022" with "2023".

7. Section 6.2 of the License Agreement is amended and restated to read as follows:

"6.2. Licensee shall pay to Carnegie Mellon a percentage of Sublicense Fees realized according to the following schedule:

Where Sublicense Fees are payable:	Percent
Prior to initiation of the first Phase II Clinical Trial for a Licensed Product	[***]
After initiation of the first Phase II Clinical Trial for a Licensed Product	[***]

8. Section 7.4 of the License Agreement is amended by replacing the phrase "Within sixty (60) days after the end of each of Licensee's fiscal years," in that section with

"Within ninety (90) days after the end of each of Licensee's fiscal years, beginning in fiscal year 2022,".

9. Section 7.5 of the License Agreement is amended by replacing the phrase "Within sixty (60) days after the end of each of Licensee's fiscal years," in that section with "Within ninety (90) days after the end of each of Licensee's fiscal years, beginning in fiscal year 2022,".

10. Licensee's Address for Notices is amended and restated to read as follows:

"350 Technology Dr.
Pittsburgh, PA 15219".

11. The Vera License is hereby terminated. Termination of the Vera License does not terminate (a) the obligation of Licensee to pay any amounts which have been accrued or are otherwise required to be paid by Licensee under the terms of the Vera License through December 31, 2021, and (b) the obligations of Licensee that expressly or by their nature survive termination, including Licensee's indemnification obligations under the Vera License and obligations in respect of any dispute and resolution thereof under the Vera License.

12. In consideration of the entering into of this Amendment, within thirty (30) days of the effective date of this Amendment, Licensee shall pay Carnegie Mellon a non- refundable amendment fee of ten thousand U.S. Dollars ($U.S. 10,000). This amendment fee is not creditable against any other amounts due under the License Agreement or Vera License.

13. This Amendment and the License Agreement constitute the entire agreement of the parties with respect to the subject matter hereof and thereof and supersede all prior agreements and understandings (both written and oral) of the parties with respect to that subject matter and cannot be amended or otherwise modified except in a writing executed by the party against whom the amendment or other modification is sought to be charged. The License Agreement as amended by this Amendment shall continue in full force and effect, subject to the terms and provisions thereof and hereof. This Amendment shall be binding up and inure to the benefit of the parties hereto and their respective successors and permitted assigns.

(The balance of this page is intentionally left blank).

The parties hereto have caused this Amendment to be executed by their duly authorized representatives in duplicate counterparts, each of which shall be deemed to constitute an original, effective as of the date first above written.

Carnegie Mellon University	LICENSEE
By: /s/ Robert A. Woolridge	By: /s/ Dietrich Stephan
Robert A. Wooldridge	Dietrich Stephan
Associate Vice President for	Chief Executive Officer
Technology Transfer and Enterprise	
Creation	
Date: 5/2/2022	Date: 4/29/2022

Exhibit A

Licensed Technology (including Patents):

The invention entitled “A Method for Preparing Conformationally-Preorganized Polyethylene Glycol-Containing yPNA” and the related documentation, if any, disclosed on November 19, 2010 and bearing Carnegie Mellon File #2011-045, on an “as is” basis as of June 7, 2012.

Application Number(s): US 61/516,838
Title: Synthesis and Characterization of Conformationally-Preorganized, MiniPEG-Containing yPNAs with Superior Hybridization Properties and water Solubility
Inventors: Danith Ly, Srinivas Rapireddy, Bichismita Sahu

The invention entitled “A simplified and cost-effective method for preparing PNA monomers and oligomers using unprotected nucleobases” and the related documentation, if any, disclosed on March 10, 2011 and bearing Carnegie Mellon File #2011-080, on an “as is” basis as of June 7, 2012.

Patent Number(s): US 9,193,758, US 10,093,700, US 10,160,787, US 10,364,272, US 10,793,605, US 11,279,736, EP 2694683, ES 2694683, UK 2694683, IT 502019000080180, FR 2694683, DE 602012074955.5, EP 3428287, UK 3428287, FR 3428287
Application Number(s): US 61/516,812, PCT/US2012/032459, US 14/110,689, US 14/921,755, US 15/972,494, US 16/145,075, US 16/362,579, US 17/003,131, EP 12767656.7, EP 18187994.1, EP 21164008.1, CA 2,832,553
Title: Conformationally-preorganized, MiniPEG-Containing GAMMA-Peptide Nucleic Acids
Inventors: Danith Ly, Srinivas Rapireddy, Bichismita Sahu

The invention entitled “GammaPNA Probes for Fluorescent Telomere Labeling and Affinity Purification” and the related documentation, if any, disclosed on August 9, 2011 and bearing Carnegie Mellon File #2012-016, on an “as is” basis as of June 7, 2012.

Patent Number(s): US 9,926,592, EP 2780480, UK 2780480
Application Number(s): US 61/629,125, PCT/US2012/064976, US 14/357,874, EP 12849889.6, UK 12849889.6, CA 2854939
Title: Gamma-PNA Miniprobes for Fluorescent Labeling
Inventors: Bruce A. Armitage, Danith Ly, Patricia Opresko, Nathaniel Shank

The invention entitled “Safety-Clip Design for Improving the Recognition Specificity of y- Peptide Nucleic Acids (yPNAs)” and the related documentation, if any, disclosed on September 12, 2011 and bearing Carnegie Mellon File #2012-028, on an “as is” basis as of June 7, 2012.

Application Number(s): US 61/573,968
Title: Safety-Clip Design for Improving the Recognition Specificity of y-Peptide Nucleic Acids (yPNAs)
Inventors: Danith Ly, Srinivas Rapireddy, Arunava Manna

Exhibit 21.1

List of Subsidiaries of NeuBase Therapeutics, Inc.

1. NeuBase Corporation (incorporated in Delaware)
2. Ohr Opco, Inc. (incorporated in Delaware)
3. Ohr Pharma, LLC (organized in Delaware)

Exhibit 23.1

INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM'S CONSENT

We consent to the incorporation by reference in the Registration Statement of NeuBase Therapeutics, Inc. and subsidiaries (the "Company") on Form S-3 (File No.333-254980) of our report dated December 21, 2022, with respect to our audits of the consolidated financial statements of the Company as of September 30, 2022 and 2021 and for the years ended September 30, 2022 and 2021, which report is included in this Annual Report on Form 10-K of the Company for the fiscal year ended September 30, 2022.

/s/ Marcum LLP

Marcum LLP
New York, NY
December 21, 2022

Exhibit 31.1

CERTIFICATION PURSUANT TO
RULES 13a-14(a) AND 15d-14(a) UNDER THE SECURITIES EXCHANGE ACT OF 1934,
AS ADOPTED PURSUANT TO SECTION 302 OF THE SARBANES-OXLEY ACT OF 2002

I, Dietrich A. Stephan, Ph.D., certify that:

1. I have reviewed this Annual Report on Form 10-K of NeuBase Therapeutics, Inc.;

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:

   a. Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

   b. Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

   c. Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

   d. Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

   a. All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

   b. Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: December 21, 2022

By: /s/ Dietrich A. Stephan
Dietrich A. Stephan, Ph.D.
Chief Executive Officer
(Principal Executive Officer)

Exhibit 31.2

CERTIFICATION PURSUANT TO
RULES 13a-14(a) AND 15d-14(a) UNDER THE SECURITIES EXCHANGE ACT OF 1934,
AS ADOPTED PURSUANT TO SECTION 302 OF THE SARBANES-OXLEY ACT OF 2002

I, Todd P. Branning, certify that:

1. I have reviewed this Annual Report on Form 10-K of NeuBase Therapeutics, Inc.;

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:

   a. Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

   b. Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

   c. Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

   d. Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

   a. All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

   b. Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: December 21, 2022

By: /s/ Todd P. Branning
Todd P. Branning
Chief Financial Officer
(Principal Financial and Accounting Officer)

Exhibit 32.1

CERTIFICATION 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

In connection with the Annual Report on Form 10-K of NeuBase Therapeutics, Inc. (the "Company") for the period ended September 30, 2022 as filed with the Securities and Exchange Commission on the date hereof (the "Report"), the undersigned hereby certify, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, to their knowledge that:

(1) The Report fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934, as amended; and

(2) The information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

By: /s/ Dietrich A. Stephan, Ph.D.
Dietrich A. Stephan, Ph.D.
Chief Executive Officer
(Principal Executive Officer)
December 21, 2022

By: /s/ Todd P. Branning
Todd P. Branning
Chief Financial Officer
(Principal Financial and Accounting Officer)
December 21, 2022

A signed original of this written statement required by Section 906 has been provided to the Company and will be retained by the Company and furnished to the Securities and Exchange Commission or its staff upon request.

This certification accompanies the Report, is not deemed filed for purposes of Section 18 of the Securities Exchange Act of 1934, as amended (the "Exchange Act"), or otherwise subject to the liability of that section, nor shall it be deemed incorporated by reference into any filing under the Securities Act of 1933, as amended, or the Exchange Act (whether made before or after the date of the Report), irrespective of any general incorporation language contained in such filing.