Filed Pursuant to Rule 424(b)(5)
Registration No. 333-254980

PROSPECTUS SUPPLEMENT
(To Prospectus dated April 14, 2021)

neubase

Up to $50,000,000
Shares of Common Stock

We have entered into an Open Market Sale Agreement[SM], or the Sales Agreement, with Jefferies LLC, or Jefferies, dated August 27, 2021, relating to the sale of shares of our common stock, $0.0001 par value per share, offered by this prospectus supplement and the accompanying prospectus. In accordance with the terms of the Sales Agreement, under this prospectus supplement we may offer and sell shares of our common stock having an aggregate offering price of up to $50,000,000 from time to time through Jefferies, acting as our sales agent.

Our common stock is listed on the Nasdaq Capital Market under the symbol "NBSE." On August 25, 2021, the last reported sale price of our common stock on the Nasdaq Capital Market was $3.99 per share.

Sales of our common stock, if any, under this prospectus supplement and the accompanying prospectus may be made in sales deemed to be "at the market offerings" as defined in Rule 415(a)(4) promulgated under the Securities Act of 1933, as amended, or the Securities Act. Jefferies is not required to sell any specific number or dollar amount of shares of our common stock, but will act as our sales agent and use commercially reasonable efforts to sell on our behalf all of the shares of common stock requested to be sold by us, consistent with its normal trading and sales practices, on mutually agreed terms between Jefferies and us. There is no arrangement for funds to be received in any escrow, trust or similar arrangement.

Jefferies will be entitled to compensation under the terms of the Sales Agreement at a rate equal to 3.0% of the gross sales price per share sold under the Sales Agreement. In connection with the sale of shares of our common stock on our behalf, Jefferies will be deemed to be an "underwriter" within the meaning of the Securities Act and the compensation of Jefferies will be deemed to be underwriting commissions or discounts. We have also agreed to provide indemnification and contribution to Jefferies with respect to certain liabilities, including liabilities under the Securities Act. See "Plan of Distribution" beginning on page S-18 for additional information regarding Jefferies' compensation.

INVESTING IN OUR COMMON STOCK INVOLVES A HIGH DEGREE OF RISK. SEE THE "RISK FACTORS" ON PAGE S-10 OF THIS PROSPECTUS SUPPLEMENT, PAGE 4 OF THE ACCOMPANYING PROSPECTUS AND IN THE DOCUMENTS INCORPORATED BY REFERENCE IN THIS PROSPECTUS SUPPLEMENT AND THE ACCOMPANYING PROSPECTUS CONCERNING FACTORS YOU SHOULD CONSIDER BEFORE INVESTING IN OUR COMMON STOCK.

Neither the Securities and Exchange Commission nor any state securities commission has approved or disapproved of these securities, or determined if this prospectus supplement and the accompanying prospectus is truthful or complete. Any representation to the contrary is a criminal offense.

Jefferies

The date of this prospectus supplement is August 27, 2021

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PROSPECTUS SUPPLEMENT

PROSPECTUS

ABOUT THIS PROSPECTUS SUPPLEMENT

This prospectus supplement is part of a registration statement that we filed with the Securities and Exchange Commission, or the SEC, utilizing a "shelf" registration process and is in two parts. The first part is this prospectus supplement, which describes the specific terms of this offering and also adds to and updates information contained in the accompanying prospectus and the documents incorporated by reference herein. The second part, the accompanying prospectus dated April 14, 2021, provides more general information. Generally, when we refer to this prospectus, we are referring to both parts of this document combined. To the extent there is a conflict between the information contained in this prospectus supplement and the information contained in the accompanying prospectus or any document incorporated by reference therein filed prior to the date of this prospectus supplement, you should rely on the information in this prospectus supplement; provided that if any statement in one of these documents is inconsistent with a statement in another document having a later date — for example, a document incorporated by reference in the accompanying prospectus — the statement in the document having the later date modifies or supersedes the earlier statement.

We further note that the representations, warranties and covenants made by us in any agreement that is filed as an exhibit to any document that is incorporated by reference herein were made solely for the benefit of the parties to such agreement, including, in some cases, for the purpose of allocating risk among the parties to such agreement, and should not be deemed to be a representation, warranty or covenant to you. Moreover, such representations, warranties or covenants were accurate only as of the date when made. Accordingly, such representations, warranties and covenants should not be relied on as accurately representing the current state of our affairs.

Neither we nor Jefferies have authorized any person to provide any information or to make any representation other than those contained or incorporated by reference in this prospectus supplement or in the accompanying prospectus and any free writing prospectus prepared by or on behalf of us or to which we have referred you. We and Jefferies take no responsibility for, and can provide no assurance as to the reliability of, any other information that others may give you.

The information contained in this prospectus supplement or the accompanying prospectus, or incorporated by reference herein or therein, is accurate only as of the respective dates thereof, regardless of the time of delivery of this prospectus supplement and the accompanying prospectus or of any sale of our securities. It is important for you to read and consider all information contained in this prospectus supplement and the accompanying prospectus, including the documents incorporated by reference herein and therein, in making your investment decision. You should also read and consider the information in the documents to which we have referred you in the sections entitled "Where You Can Find More Information" and "Documents Incorporated by Reference" in this prospectus supplement.

We are not, and Jefferies is not, making an offer to sell our shares in any jurisdiction where the offer and sale is not permitted. The distribution of this prospectus supplement and the accompanying prospectus and this offering in certain jurisdictions may be restricted by law. Persons outside the United States who come into possession of this prospectus supplement and the accompanying prospectus must inform themselves about, and observe any restrictions relating to, this offering and the distribution of this prospectus supplement and the accompanying prospectus outside the United States. This prospectus supplement and the accompanying prospectus do not constitute, and may not be used in connection with, an offer to sell, or a solicitation of an offer to buy, any securities offered by this prospectus supplement and the accompanying prospectus by any person in any jurisdiction in which it is unlawful for such person to make such an offer or solicitation.

Unless otherwise indicated, we have based the information concerning our industry contained in this prospectus supplement and incorporated by reference herein on our general knowledge of and expectations concerning the industry, which involve risks and uncertainties and are subject to change based on various factors, including those discussed in the "Risk Factors" section of this prospectus supplement and in the other information contained or incorporated by reference in this prospectus supplement. These and other factors could cause the information concerning our industry to differ materially from those expressed in this prospectus supplement and incorporated by reference herein.

We use our unregistered trademark, PATrOL™, in this prospectus supplement. All other trademarks, trade names and service marks appearing in this prospectus supplement, the accompanying prospectus or the documents incorporated by reference herein or therein are the property of their respective owners. Use or display by us of other parties' trademarks, trade dress or products is not intended to and does not imply a relationship with, or endorsements or sponsorship of, us by the trademark or trade dress owner. Solely for convenience and, except for the use of PATrOL™ herein, trademarks and tradenames referred to in this prospectus appear without the ® and ™ symbols, but those references are not intended to indicate, in any way, that we will not assert, to the fullest extent under applicable law, our rights or that the applicable owner will not assert its rights, to these trademarks and tradenames.

Unless the context otherwise indicates, references in this prospectus supplement to "we," "our" and "us" refer, collectively, to NeuBase Therapeutics, Inc., a Delaware corporation, and its wholly owned subsidiaries.

FORWARD-LOOKING STATEMENTS

This prospectus and the documents incorporated by reference into this prospectus may contain forward-looking statements within the meaning of Section 27A of the Securities Act of 1933, as amended (the "Securities Act"), and Section 21E of the Securities Exchange Act of 1934, as amended (the "Exchange Act"), about NeuBase. These forward-looking statements are intended to be covered by the safe harbor for forward-looking statements provided by the Private Securities Litigation Reform Act of 1995. Forward-looking statements are not statements of historical fact, and can be identified by the use of forward-looking terminology such as "believes," "expects," "may," "will," "could," "should," "projects," "plans," "goal," "targets," "potential," "estimates," "pro forma," "seeks," "intends" or "anticipates" or the negative thereof or comparable terminology. Forward-looking statements include discussions of strategy, financial projections, guidance and estimates (including their underlying assumptions), statements regarding plans, objectives, expectations or consequences of various transactions, and statements about the future performance, operations, products and services of NeuBase. We caution our stockholders and other readers not to place undue reliance on such statements.

You should read this prospectus, any accompanying prospectus supplement and the documents incorporated by reference completely and with the understanding that our actual future results may be materially different from what we currently expect. Our business and operations are and will be subject to a variety of risks, uncertainties and other factors. Consequently, actual results and experience may materially differ from those contained in any forward-looking statements. Such risks, uncertainties and other factors that could cause actual results and experience to differ from those projected include, but are not limited to, the risk factors set forth in Part I - Item 1A, "Risk Factors," in our Annual Report on Form 10-K for the year ended September 30, 2020 filed with the SEC on December 23, 2020 and in ourQuarterly Report on Form 10-Q for the quarter ended June 30, 2021, filed with the SEC on August 13, 2021, and elsewhere in the other documents incorporated by reference into this prospectus.

You should assume that the information appearing in this prospectus, any related free writing prospectus and any document incorporated herein by reference is accurate as of its date only. Because the risk factors referred to above could cause actual results or outcomes to differ materially from those expressed in any forward-looking statements made by us or on our behalf, you should not place undue reliance on any forward-looking statements. Further, any forward-looking statement speaks only as of the date on which it is made. New factors emerge from time to time, and it is not possible for us to predict which factors will arise. In addition, we cannot assess the impact of each factor on our business or the extent to which any factor, or combination of factors, may cause actual results to differ materially from those contained in any forward-looking statements. All written or oral forward-looking statements attributable to us or any person acting on our behalf made after the date of this prospectus are expressly qualified in their entirety by the risk factors and cautionary statements contained in and incorporated by reference into this prospectus. Unless legally required, we do not undertake any obligation to release publicly any revisions to such forward-looking statements to reflect events or circumstances after the date of this prospectus or to reflect the occurrence of unanticipated events.

SUMMARY

This summary highlights certain information about us, this offering and selected information contained elsewhere in or incorporated by reference into this prospectus supplement or the accompanying prospectus. This summary is not complete and does not contain all of the information that you should consider before deciding whether to invest in our common stock. For a more complete understanding of our company and this offering, we encourage you to read and consider carefully the more detailed information in this prospectus supplement and the accompanying prospectus, including the information incorporated by reference herein, and any free writing prospectus prepared by us or on our behalf that we have authorized for use in connection with this offering, including the information in "Risk Factors" beginning on page S-10 of this prospectus supplement, along with our consolidated financial statements and notes to those consolidated financial statements incorporated by reference in this prospectus supplement and the accompanying prospectus.

Overview

We are a biotechnology company working towards accelerating the genetic revolution by developing a new class of synthetic medicines. Our modular peptide-nucleic acid antisense oligo ("PATrOL™") platform which outputs "anti-gene" candidate therapies is designed to combine the specificity of genetic sequence-based target recognition with a modularity that enables use of various *in vivo* delivery technologies to enable broad and also selective tissue distribution capabilities. Given that every human disease may have a genetic component, we believe that our differentiated platform technology has the potential for broad impact by increasing, decreasing or changing gene function at either the DNA or RNA levels to resolve the progression to disease, as appropriate, in a particular indication. We plan to use our platform to address diseases driven by genetic variation and mutation, and we are initially focused on myotonic dystrophy type 1 ("DM1"), Huntington's disease ("HD"), and oncology applications.

Globally, there are thousands of genetic diseases, most of which lack any therapeutic options. In addition, rare genetic diseases are often particularly severe, debilitating or fatal. Traditionally, therapeutic development for each rare genetic disorder has been approached with a unique strategy, which is inefficient, as there are thousands of diseases that need treatment solutions. The collective population of people with rare diseases stands to 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.

Mutated proteins resulting from errors in deoxyribonucleic acid ("DNA") sequences cause many rare genetic diseases and cancer. DNA in each cell of the body is transcribed into pre-RNA, which is then processed (spliced) into mRNA which is exported into the cytoplasm of the cell and translated into protein. This is termed the "central dogma" of biology. Therefore, when errors in a DNA sequence occur, they are propagated to RNAs and can become a damaging protein.

The field has learned that antisense oligonucleotides ("ASOs") can inactivate target RNAs before they can produce harmful proteins by binding them in a sequence-specific manner, which can delay disease progression or even eliminate genetic disease symptoms. ASOs designed by others to target known disease-related mutant RNA sequences have been shown to be able to degrade these transcripts and have a positive clinical impact. Similarly, applications in modifying splicing of pre-RNA in the nucleus of the cell have been developed by others to exclude damaging exons from the final mRNA product and have been approved by the Food and Drug Administration ("FDA"). We plan to extend upon these conceptual breakthroughs by utilizing our first-in-class technology which produces investigational therapies which are similar in structure to ASOs in that they are comprised of a backbone onto which are tethered nucleobases that engage a genetic sequence of interest using complementary base-pairing, but which we believe have significant benefits in certain application areas to better resolve clinical disorders.

We are developing "anti-gene" therapies. Anti-genes are similar to, but distinct from, antisense oligonucleotides (ASOs). ASOs are short single strands of nucleic acids (traditionally thought of as single-stranded DNA molecules) which bind to defective RNA targets in cells and inhibit their ability to form defective proteins. We believe we are a leader in the discovery and development of anti-gene therapies, a new class of investigational therapies derived from peptide-nucleic acids ("PNAs"). The key differentiator between ASOs and anti-genes is that the scaffold is not derived from a natural sugar-phosphate nucleic acid backbone, rather is a synthetic polyamide which is charge-neutral and characterized by high binding affinity to a nucleic acid target, high sequence specificity, high stability, and is relatively immunologically inert. These features provide potential advantages over ASOs and other genetic therapies for modulating disease-causing genes including increased unique disease target opportunities, improved target specificity and a reduction in both sequence-dependent and independent toxicities. In addition, as these anti-genes are manufactured via standard peptide synthesis methods, they efficiently leverage the advancements in the synthetic peptide industry to enable modulation of pharmacophore delivery, pharmacokinetics, sub-cellular placement and endosomal escape.

In addition to the scaffold, we have a kit of natural nucleobases, chemically modified nucleobases which add further precision to a nucleic acid target of interest, and

proprietary bi-specific nucleobases ("janus" nucleobases) which can be added to the scaffold to enable more precise target engagement. These bi-specific nucleobases, in particular, have been shown to enable accessing double stranded RNA targets comprised of secondary structures such as hairpins (double stranded RNA targets which are folded upon themselves). This allows us to potentially access regions of the target transcript which may be unique in secondary structure to allow enhanced selectivity for the target (mutant) RNA as compared to the normal RNA. Enhanced selectivity for mutant RNAs as compared to normal RNAs is often important as normal RNAs are often required for effective functioning of the cell.

A third component of the modular platform is the ability to add delivery technology to the anti-gene pharmacophores so as to reach a desired cell or tissue upon *in vivo* administration. There is flexibility to append various delivery technologies to the pharmacophore to allow either broad tissue distribution or narrow cell and/or tissue targeting if so desired based on targets. One such technology is a chemical moiety that can be used to decorate the scaffold directly and allows the anti-genes to penetrate cell membranes and into subcellular compartments where they act as well as to distribute throughout the body when administered systemically.

This toolkit of components forms the PATrOL™ platform and allows us to manufacture gene and transcript-specific anti-genes.

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 are developing several preclinical programs using our PATrOL™ platform, including the NT0100 program, targeted at Huntington's disease, a repeat expansion disorder, the NT0200 program, targeted at myotonic dystrophy, type 1, a second repeat expansion disorder, and a program targeting *KRAS* G12D and G12V mutations. Preclinical studies are being conducted to evaluate the PATrOL™ platform technology and program candidates in the areas of pharmacokinetics, pharmacodynamics and tolerability, and we reported results from certain of those studies in the first calendar quarter of 2020 and extended upon certain of those studies in the fourth calendar quarter of 2020 which illustrated that our anti-gene technology can be administered to human patient-derived cell lines and systemically (via intravenous (IV) administration) into animals with DM1 (a genetically modified model accepted as representative of the human disease in skeletal muscle) and can address the causal genetic defect. We also presented additional results from ongoing preclinical studies evaluating the PATrOL ™ platform and pipeline indications at an R&D day in June 2021. In addition, we believe that the emerging pipeline of other investigational therapies that target primary and secondary RNA structure and genomic DNA potentially allows a unique market advantage across a variety of rare diseases and oncology targets.

Overall, using our PATrOL™ platform, we believe we can create anti-gene therapies that may have distinct advantages over other chemical entities currently in the market or in development for genetic medicine applications to modulate mutant genes and improve a clinical trait or disorder. These potential advantages may differ by indication and can include, among others:

- increased unique target opportunities, improved target specificity and a reduction in both sequence-dependent and independent toxicities by virtue of a synthetic polyamide scaffold which is charge-neutral and characterized by high binding affinity to a nucleic acid target, high sequence specificity, high stability, and is relatively immunologically inert;
- potential long durability by nature of the relatively highly stable polyamide scaffold;

- our anti-genes are manufactured via standard peptide synthesis methods and thus they efficiently leverage advances in the synthetic peptide industry to enable facile addition of known moieties enabling modulating pharmacophore delivery, pharmacokinetics, sub-cellular placement and endosomal escape; and
- our anti-genes may be able to target double stranded structures in RNA, which may allow unique target opportunities that standard ASOs cannot access.

With these unique component parts and their advantages, we believe our PATrOL™ platform-enabled anti-gene therapies can potentially address a multitude of rare genetic diseases and cancer, among other indications.

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, with an initial emphasis on rare genetic diseases and cancers. A key component of this strategy is continuing to improve the scientific understanding and optimization of our platform technology and programs, including how various components of our platform technology perform, and how our investigational therapies impact the biological processes of the target diseases, so that we can utilize this information to reduce risk in our future programs and indications. In addition, with our expertise in discovering and characterizing novel anti-gene investigational therapies, we believe that our scientists can optimize the properties of our PATrOL™-enabled drug candidates for use with particular targets that we determine to be of high value.

We believe the depth of our knowledge and expertise with PNAs, engineered nucleotides, genetics and genomics and therapeutic development of first-in-class modalities provides potential flexibility to determine the optimal development and commercialization strategy to maximize the near and longer-term value of our therapeutic programs.

We plan to employ distinct 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.

We believe the breadth of the PATrOL™ platform gives us the ability to potentially address a multitude of inherited genetic diseases. The technology may allow us to target and inactivate gain-of-function and change-of-function mutations, and address targets in recessive disease and haploinsufficiencies by altering splicing to remove damaging exons/mutations or increasing expression of wild-type alleles by various means.

Modified scaffolds, optimized versions of traditional PNA scaffolds which we utilize, have demonstrated preclinical *in vivo* efficacy in several applications which we believe can be translated across many targets and into humans. For example, in oncology such scaffolds have reduced expression of an activated oncogene (the epidermal growth factor receptor of the EGFR gene) and have modified gene regulation by targeting microRNA to slow tumor growth. Such scaffolds have also demonstrated *in vivo* engagement with the double-stranded genome in studies done by others to perform *in vivo* single-base genome editing.

Product Pipeline

NT0100 Program - PATrOL™ Enabled Anti-Gene for Huntington's Disease

HD is a devastating rare neurodegenerative disorder. After onset, symptoms such as uncontrolled movements, cognitive impairments and emotional disturbances worsen over time. HD is caused by toxic aggregation of mutant huntingtin protein, leading to progressive neuron loss in the striatum and cortex of the brain. The wild-type huntingtin gene (*HTT*) 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. While the normal or wild-type function of HTT protein is largely uncharacterized, it is known to be essential for normal brain development. When the DNA sequence CAG is repeated 40 times or more in this region, the resulting protein becomes toxic and causes HD. 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. HD is one of many known repeat expansion disorders, which are a set of genetic disorders caused by a mutation that leads to a repeat of nucleotides exceeding the normal threshold. 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. There are approximately 30,000 symptomatic patients in the U.S. and more than 200,000 at-risk of inheriting the disease globally.

One especially important advantage of the PATrOL™ platform that we believe makes it promising for the treatment of repeat expansion disorders like HD is the ability of our anti-genes to potentially target the RNA hairpin. This allows our therapies to potentially inactivate mutant *HTT* mRNA before it can be translated into a harmful protein via selective binding to the expanded CAG repeats while leaving the normal *HTT* mRNA largely unbound to drug and producing functional protein. We believe achieving mutant allele selectivity would be a key advantage for any RNA-based approach aiming to treat HD. In March of 2020, we illustrated the ability of our anti-gene 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 anti-genes can inhibit ribosomal elongation via high-affinity binding to a target RNA. In June 2021, we presented new data demonstrating selective reduction of mutant huntingtin protein in the brain after subcutaneous dosing in the zQ175 Huntington's disease mouse model.

The PATrOL™-enabled NT0100 program is currently in preclinical development for the treatment of HD, with a target IND filing in calendar year 2023.

NT0200 Program - PATrOL™ Enabled Anti-Gene for Myotonic Dystrophy Type 1

Our pipeline also contains a second potentially transformative medicine, which we believe has significant potential for Myotonic dystrophy, type 1, a severe dominantly inherited genetic disease. DM1 is characterized clinically by myotonia (an inability to relax a muscle after contraction), muscle weakness and wasting, cardiac conduction defects and cognitive deficits. DM1 is caused by an expansion of a CUG trinucleotide repeat in the 3' untranslated region ("UTR"), a noncoding region of the myotonic dystrophy protein kinase gene (*DMPK*) transcript. Diagnosis is confirmed by molecular genetic testing of the length of a trinucleotide repeat expansion. Repeat length exceeding 34 repeats is abnormal and often patients have hundreds or thousands of repeat units. Molecular genetic testing detects pathogenic variants in nearly 100% of affected individuals. It is estimated that the global prevalence of DM1 is approximately 1 in 20,000 individuals.

The trinucleotide repeat expansion in the transcript causes disease by forming an aberrant hairpin structure in the nucleus of patient cells that captures and sequesters proteins that have critical functions in the nucleus related to appropriate splicing of hundreds of transcripts. These sequestered proteins cannot then fulfill their normal functions. In addition, it has been documented that sequestration of the mutant *DMPK* transcripts in the nucleus results in their inability to be translated and potentially results in haploinsufficiency, a situation where 50% of the protein in not enough to maintain normal function. Mice with both copies of their *Dmpk* gene knocked out manifest a cardiac conduction defect (Berul CI, Maguire CT, Aronovitz MJ, Greenwood J, Miller C, Gehrmann J, Housman D, Mendelsohn ME, Reddy S. *Dpmk* dosage alterations result in atrioventricular conduction abnormalities in a mouse myotonic dystrophy model. J Clin Invest. 1999 Feb;103(4):R1-7. doi: 10.1172/JCI5346. PMID: 10021468; PMCID: PMC408103.) and a CNS phenotype characterized by abnormal long-term potentiation (Schulz PE, McIntosh AD, Kasten MR, Wieringa B, Epstein HF. A role for myotonic dystrophy protein kinase in synaptic plasticity. J Neurophysiol. 2003 Mar;89(3):1177-86. doi: 10.1152/jn.00504.2002. Epub 2002 Nov 13. PMID: 12612014.) hypothesized to be due to inappropriate cytoskeletal remodeling. We propose that our mechanism of action is via direct engagement of our anti-gene with the expanded CUG repeat hairpin structure in the 3' UTR of the mutant transcript, invasion and opening of the hairpin structure, and release of the sequestered CUG-repeat binding proteins. This release of sequestered proteins which are normally involved in developmentally appropriate pre-mRNA splicing in the nucleus resolves the generalized splice defect and thus the major causal event. Our DM1 anti-gene is designed to not specifically degrade the mutant transcript, rather to release these RNA-protein aggregates through steric displacement, which could also resolve any potential haploinsufficiency and as a result may improve endophenotypes of the clinical condition, such as in the heart and brain (contingent on delivering effective concentrations of anti-gene to these tissues). Our recent data illustrates that we are able to systemically deliver our anti-genes in a DM1 genetic mouse model, engage the target in the skeletal muscles of the animals, and induce molecular rescue of the causal splice defects, and functional rescue of the phenotype.

The PATrOL™-enabled NT0200 program is currently in preclinical development for the treatment of DM1, with an IND filing planned in the fourth quarter of calendar year 2022.

Additional Indications

On June 8, 2021, we unveiled a program targeting two *KRAS* oncogenic driver mutations, G12D and G12V, and presented *in vitro* and *in vivo* data from the new program. In addition, we are in the process of building an early stage pipeline of other therapies that focus on the unique advantages of our technology across a variety of diseases with an underlying genetic driver.

For a complete description of our business, financial condition, results of operations and other important information, we refer you to our filings with the SEC that are incorporated by reference in this prospectus, including our Annual Report on Form 10-K for the year ended September 30, 2020 our Quarterly Report on Form 10-Q for the quarter ended June 30, 2021, our Quarterly Report on Form 10-Q for the quarter ended March 31, 2021, and our Quarterly Report on Form 10-Q for the quarter ended December 31, 2020. For instructions on how to find copies of these documents, see the section of this prospectus supplement entitled "Where You Can Find More Information."

See the section entitled "Risk Factors" in this prospectus for a discussion of some of the risks relating to the execution of our business strategy.

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 a reverse merger transaction (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.

Our principal executive offices are located at 350 Technology Drive, Pittsburgh, PA 15219, and our telephone number is (646) 450-1790. 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 prospectus and should not be relied upon in connection with making any decision with respect to an investment in our securities.

We are required to file annual, quarterly and current reports, proxy statements and other information with the SEC. You may obtain any of the documents filed by us with the SEC at no cost from the SEC's website at *www.sec.gov*.

We are a "smaller reporting company" as defined in Rule 12b-2 of the Exchange Act and have elected to take advantage of certain of the scaled disclosure available for smaller reporting companies in this prospectus as well as our filings under the Exchange Act.

THE OFFERING

Common stock offered by us:	Shares having an aggregate offering price of up to $50,000,000.
Common stock to be outstanding following the offering:	Up to 45,248,155 shares (as more fully described below in Outstanding Shares), assuming sales of 12,531,328 shares of our common stock in this offering at an offering price of $3.99 per share, which was the last reported sale price of our shares on the Nasdaq Capital Market on August 25, 2021. The actual number of shares issued will vary depending on the sales price under this offering.
Manner of offering:	"At the market offering" that may be made from time to time on the Nasdaq Capital Market or other existing trading markets for our common stock through our sales agent, Jefferies. See "Plan of Distribution" on page S-18 of this prospectus supplement.
Use of proceeds:	We currently intend to use the net proceeds from the sale of the securities offered hereby for operating costs, research and development and for general corporate purposes, including working capital. We may also use a portion of the net proceeds to invest in or acquire businesses or technologies that we believe are complementary to our own, although we have no current plans, commitments or agreements with respect to any acquisitions as of the date of this prospectus. See "Use of Proceeds" on page S-15 of this prospectus supplement.
Risk factors:	Investing in our common stock involves a high degree of risk. Please read the information contained in and incorporated by reference under the heading "Risk Factors" beginning on page S-10 of this prospectus supplement, the "Risk Factors" section beginning on page 4 of the accompanying prospectus and the documents incorporated by referenced into this prospectus supplement.
Nasdaq Capital Market symbol:	"NBSE"
Outstanding Shares	The number of shares of our common stock to be outstanding after this offering is based on32,716,827 shares of our common stock outstanding as of June 30, 2021, and excludes, as of June 30, 2021: · 7,227,421 shares of common stock issuable upon the vesting and exercise of stock options outstanding at a weighted average exercise price of approximately $3.20 per share, including 225,000 shares of common stock subject to stock options that were unrecognized as of such date because, under generally accepted accounting principles in the U.S. ("GAAP"), the vesting conditions were not estimable at such time; · 10,000 shares of common stock issuable upon the vesting of restricted stock units outstanding; ·147,041 shares of common stock available for issuance pursuant to the Company's 2016 Consolidated Stock Incentive Plan ("2016 Plan"); · 807,251 shares of common stock available for issuance pursuant to the Company's 2019 Stock Incentive Plan ("2019 Equity Incentive Plan"); and ·820,939 shares of common stock issuable pursuant to the exercise of outstanding warrants at a weighted average exercise price of $19.44 per share.

RISK FACTORS

An investment in our common stock involves a high degree of risk. You should carefully consider the risks described under "Risk Factors" in our most recent Annual Report on Form 10-K and Quarterly Reports on Form 10-Q, as updated by our subsequent filings under the Exchange Act, and all of the other information contained in this prospectus supplement and the accompanying prospectus, and incorporated by reference into this prospectus supplement and the accompanying prospectus, including our financial statements and related notes, before investing in our common stock. If any of the possible events described below or in those sections actually occur, our business, business prospects, cash flow, results of operations or financial condition could be harmed, the trading price of our common stock could decline, and you might lose all or part of your investment in our common stock. Additional risks and uncertainties not presently known to us or that we currently deem immaterial may also impair our operations and results.

Risks Related to this Offering of our Common Stock

Resales of our common stock in the public market during this offering by our stockholders may cause the market price of our common stock to fall.

We may issue common stock from time to time in connection with this offering. This issuance from time to time of these new shares of our common stock, or our ability to issue these shares of common stock in this offering, could result in resales of our common stock by our current stockholders concerned about the potential dilution of their holdings. In turn, these resales could have the effect of depressing the market price for our common stock. A substantial majority of the outstanding shares of our common stock are, and the shares of common stock sold in this offering upon issuance will be, freely tradable without restriction or further registration under the Securities Act.

Our common stock prices may be volatile which could cause the value of our common stock to decline.

The trading price of our common stock is likely to be highly volatile and could fluctuate in response to factors such as:

- 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 and data releases;
- 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;
- 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 our 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, management, or board personnel;
- changes in the market valuation 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.

The stock market is subject to significant price and volume fluctuations. In the past, following periods of volatility in the market price of a company's securities, securities class action litigation has often been initiated against such a company. Litigation initiated against us, whether or not successful, could result in substantial costs and diversion of our management's attention and resources, which could harm our business and financial condition.

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.

You will experience immediate and substantial dilution.

The offering price per share in this offering may exceed the net tangible book value per share of our common stock outstanding prior to this offering. For illustrative purposes only, assuming that an aggregate of $50 million of shares of our common stock are sold at the assumed offering price of $3.99 per share (the last reported sale price of our common stock on the Nasdaq Capital Market on August 25, 2021), and after deducting commissions and estimated aggregate offering expenses payable by us, you will experience immediate dilution of $1.59 per share, representing the difference between our as adjusted net tangible book value per share as of June 30, 2021 after giving effect to this offering and the assumed offering price. In addition, we are not restricted from issuing additional securities in the future, including shares of common stock, securities that are convertible into or exchangeable for, or that represent the right to receive, common stock or substantially similar securities. The issuance of these securities may cause further dilution to our stockholders. The exercise of outstanding stock options and warrants may also result in further dilution of your investment. See the section entitled "Dilution" on page S-17 below for a more detailed illustration of the dilution you may incur if you participate in this offering.

Our management will have broad discretion over the actual amounts and timing of the expenditures of the proceeds we receive in this offering and might not apply the proceeds in ways that enhance our operating results or increase the value of your investment.

Our management will have broad discretion as to the actual amounts and timing of the expenditures of the net proceeds from this offering, and you will be relying on the judgment of our management regarding the application of these proceeds. Our management might not apply the net proceeds of this offering in ways that enhance our operating results or increase the value of your investment. Additionally, until the net proceeds we receive are used, they may be placed in investments that do not produce income or that lose value.

The actual number of shares we will issue under the Sales Agreement with Jefferies, and the prices at which the shares are sold, at any one time or in total, is uncertain.

Subject to certain limitations in the Sales Agreement with Jefferies and compliance with applicable law, we have the discretion to deliver placement notices to Jefferies at any time throughout the term of the Sales Agreement. The number of shares that are sold by Jefferies after delivering a placement notice, and the prices at which shares are sold, will fluctuate based on the market price of the common stock during the sales period and limits we set with Jefferies.

The shares of common stock will be sold in "at the market" offerings, and investors who buy shares at different times will likely pay different prices.

Investors who purchase shares in this offering at different times will likely pay different prices, and so may experience different outcomes in their investment results. We will have discretion, subject to market demand, to vary the timing, prices, and numbers of shares sold, and there is no minimum or maximum sales price. Investors may experience a decline in the value of their shares as a result of share sales made at prices lower than the prices they paid.

Certain anti-takeover defenses and applicable law may limit the ability of a third party to acquire control of us.

Our certificate of incorporation and bylaws contain provisions that could delay or prevent a change of control of our company or changes in our Board of Directors that our stockholders might consider favorable. Some of these provisions:

- authorize the issuance of preferred stock which can be created and issued by the Board of Directors without prior stockholder approval, with rights senior to those of the common stock;
- provide for a classified Board of Directors, with each director serving a staggered three-year term;
- provide that each director may be removed by the stockholders only for cause;
- prohibit our stockholders from filling board vacancies, calling special stockholder meetings, or taking action by written consent; and
- require advance written notice of stockholder proposals and director nominations.

In addition, we have elected to be subject to Section 203 of the General Corporation Law of the State of Delaware (the "DGCL") by provision of our charter. In general, Section 203 of the DGCL prevents an "interested stockholder" (as defined in the DGCL) from engaging in a "business combination" (as defined in the DGCL) with us for three years following the date that person becomes an interested stockholder unless one or more of the following occurs:

- Before that person became an interested stockholder, our Board of Directors approved the transaction in which the interested stockholder became an interested stockholder or approved the business combination;
- Upon consummation of the transaction that resulted in the interested stockholder becoming an interested stockholder, the interested stockholder owned at least 85% of our voting stock outstanding at the time the transaction commenced, excluding for purposes of determining the voting stock outstanding (but not the outstanding voting stock owned by the interested stockholder) stock held by directors who are also officers of our Company and by employee stock plans that do not provide employees with the right to determine confidentially whether shares held under the plan will be tendered in a tender or exchange offer; or
- Following the transaction in which that person became an interested stockholder, the business combination is approved by our Board of Directors and authorized at a meeting of stockholders by the affirmative vote of the holders of at least 66 2/3% of our outstanding voting stock not owned by the interested stockholder.

The DGCL generally defines "interested stockholder" as any person who, together with affiliates and associates, is the owner of 15% or more of our outstanding voting stock or is our affiliate or associate and was the owner of 15% or more of our outstanding voting stock at any time within the three-year period immediately before the date of determination. As a result, our election to be subject to Section 203 of the DGCL could limit the ability of a third party to acquire control of us.

We do not intend to pay dividends on our common stock for the foreseeable future.

We have paid no dividends on our common stock to date and it is not anticipated that any dividends will be paid to holders of our common stock in the foreseeable future. While our future dividend policy will be based on the operating results and capital needs of our business, it is currently anticipated that any earnings will be retained to finance our future expansion and for the implementation of our business plan. As an investor, you should take note of the fact that a lack of a dividend can further affect the market value of our stock, and could significantly affect the value of any investment.

Future sales of our common stock, or preferred stock, or of other securities convertible into our common stock or preferred stock, could cause the market value of our common stock to decline and could result in dilution of your shares.

We are authorized to issue 250,000,000 shares of common stock and 10,000,000 shares of preferred stock. As of August 25, 2021, there were an aggregate of 41,719,060 shares of our common stock issued and outstanding on a fully diluted basis and no shares of preferred stock outstanding. That total for our common stock includes, as of August 25, 2021, 7,256,529 shares of our common stock that may be issued upon the exercise of outstanding stock options, 10,000 shares of common stock issuable upon vesting of restricted share units, 914,765 shares available for future awards issuable under our equity incentive plans, and a total of 820,939 warrants issued and outstanding.

In the future, we may issue additional authorized but previously unissued equity securities to raise funds to support our continued operations and to implement our business plan. We may also issue additional shares of our capital stock or other securities that are convertible into or exercisable for our capital stock in connection with hiring or retaining employees, future acquisitions, or for other business purposes. If we raise additional funds from the issuance of equity securities, substantial dilution to our existing stockholders may result. In addition, the future issuance of any such additional shares of capital stock may create downward pressure on the trading price of our common stock. There can be no assurance that we will not be required to issue additional shares, warrants or other convertible securities in the future in conjunction with any capital raising efforts, including at a price (or exercise prices) below the price at which shares of our common stock is currently traded on the Nasdaq Capital Market. Moreover, depending on market conditions, we cannot be sure that additional financing will be available when needed or that, if available, financing will be obtained on terms favorable to us or to our stockholders.

Additional funds may not be available when we need them on terms that are acceptable to us, or at all. If adequate funds are not available to us on a timely basis, we may be required to curtail or cease our operations.

Raising additional funding through debt or equity financing will be difficult or not successful at all, would be dilutive and may cause the market price of our common stock to decline further. Raising additional funding through debt or equity financing is likely to be difficult or unavailable altogether given the early stage of our therapeutic candidates. Furthermore, the issuance of additional securities, whether equity or debt, by us, or the possibility of such issuance, may cause the market price of our common stock to decline further and existing stockholders may not agree with our financing plans or the terms of such financings.

If securities analysts do not publish research or reports about our company, or if they issue unfavorable commentary about us or our industry or downgrade the outlook of our common stock, the market price of our common stock could decline.

The trading market for our common stock will depend in part on the research and reports that third-party securities analysts publish about our company and our industry. One or more analysts could downgrade the outlook for our common stock or issue other negative commentary about our company or our industry. Furthermore, if one or more of these analysts cease coverage of our company, we could lose visibility in the market. As a result of one or more of these factors, the market price of our common stock could decline and cause you to lose all or a portion of your investment.

USE OF PROCEEDS

We may issue and sell shares of our common stock having aggregate gross sales proceeds of up to $50 million from time to time (before deducting sales agent commissions and expenses). Because there is no minimum offering amount required as a condition to close this offering, the actual total public offering amount, commissions and proceeds to us, if any, are not determinable at this time. There can be no assurance that, in the future, we will sell any shares under or fully utilize the Sales Agreement with Jefferies as a source of financing.

We will retain broad discretion over the use of the net proceeds from the sale of our securities offered hereby. Except as described in any free writing prospectus that we may authorize to be provided to you, we currently intend to use the net proceeds from the sale of the securities offered hereby for operating costs, research and development and for general corporate purposes, including working capital. We may also use a portion of the net proceeds to invest in or acquire businesses or technologies that we believe are complementary to our own, although we have no current plans, commitments or agreements with respect to any acquisitions as of the date of this prospectus supplement.

The timing and amount of our actual expenditures will be based on many factors, including cash flows from operations and the anticipated growth of our business. As a result, our management will have broad discretion to allocate the net proceeds of the offerings. Pending their ultimate use, we intend to invest the net proceeds in short-term, investment-grade, interest-bearing instruments.

DIVIDEND POLICY

We have never declared or paid any dividends on our common stock and do not anticipate paying any in the foreseeable future. We currently intend to retain all of our future earnings, if any, to finance the operation and expansion of our business. Any future determination relating to our dividend policy will be made at the discretion of our Board of Directors and will depend on a number of factors, including future earnings, capital requirements, financial conditions, future prospects, contractual restrictions and covenants and other factors that our Board of Directors may deem relevant.

DILUTION

If you invest in this offering, your ownership interest will be diluted to the extent of the difference between the offering price per share and the as adjusted net tangible book value per share after giving effect to this offering. We calculate net tangible book value per share by dividing the net tangible book value, which is tangible assets less total liabilities, by the number of outstanding shares of our common stock. Dilution represents the difference between the portion of the amount per share paid by purchasers of shares in this offering and the as adjusted net tangible book value per share of our common stock immediately after giving effect to this offering. Our net tangible book value as of June 30, 2021 was approximately $60.3 million, or $1.84 per share.

For illustrative purposes, after giving effect to the sale of our common stock during the term of the Sales Agreement with Jefferies in the aggregate amount of $50 million at an assumed offering price of $3.99 per share, the last reported sale price of our common stock on the Nasdaq Capital Market on August 25, 2021, and after deducting commissions and estimated aggregate offering expenses payable by us, our net tangible book value as of June 30, 2021 would have been approximately $108.4 million, or approximately $2.40 per share of common stock. This represents an immediate increase in the net tangible book value of approximately $0.56 per share to our existing stockholders and an immediate dilution in net tangible book value of approximately $1.59 per share to new investors.

Assumed public offering price per share			$	3.99
Net tangible book value per share as June 30, 2021	$	1.84		
Increase per share attributable to investors purchasing our common stock in this offering	$	0.56		
As adjusted net tangible book value per share as of June 30, 2021, after giving effect to this offering			$	2.40
Dilution in net tangible book value per share to investors purchasing our common stock in this offering			$	1.59

* Per share numbers may not add due to rounding

The information and table above are based on 32,716,827 shares of our common stock outstanding as of June 30, 2021, and excludes, as of June 30, 2021:

- 7,227,421 shares of common stock issuable upon the vesting and exercise of stock options outstanding at a weighted average exercise price of approximately $3.20 per share, including 225,000 shares of common stock subject to stock options that were unrecognized as of such date because, under GAAP, the vesting conditions were not estimable at such time;
- 10,000 shares of common stock issuable upon the vesting of restricted stock units outstanding;
- 147,041 shares of common stock available for issuance pursuant to the Company's 2016 Plan;
- 807,251 shares of common stock available for issuance pursuant to the Company's 2019 Equity Incentive Plan; and
- 820,939 warrants issued and outstanding at a weighted average exercise price of $19.44 per warrant;

To the extent options or warrants outstanding as of June 30, 2021 are additionally exercised or settled or other shares are issued, there may be further dilution to new investors. In addition, we may choose to raise additional capital due to market conditions or strategic considerations even if we believe we have sufficient funds for our current or future operating plans. To the extent that additional capital is raised through the sale of equity or convertible debt securities, the issuance of these securities could result in further dilution to our stockholders.

PLAN OF DISTRIBUTION

We have entered into a Sales Agreement with Jefferies, under which we may offer and sell up to $50.0 million of our shares of common stock from time to time through Jefferies acting as sales agent. Sales of our shares of common stock, if any, under this prospectus supplement and the accompanying prospectus will be made by any method that is deemed to be an "at the market offering" as defined in Rule 415(a)(4) under the Securities Act.

Each time we wish to issue and sell our shares of common stock under the Sales Agreement, we will notify Jefferies of the number of shares to be issued, the dates on which such sales are anticipated to be made, any limitation on the number of shares to be sold in any one day and any minimum price below which sales may not be made. Once we have so instructed Jefferies, unless Jefferies declines to accept the terms of such notice, Jefferies has agreed to use its commercially reasonable efforts consistent with its normal trading and sales practices to sell such shares up to the amount specified on such terms. The obligations of Jefferies under the Sales Agreement to sell our shares of common stock are subject to a number of conditions that we must meet.

The settlement of sales of shares between us and Jefferies is generally anticipated to occur on the second trading day following the date on which the sale was made. Sales of our shares of common stock as contemplated in this prospectus supplement will be settled through the facilities of The Depository Trust Company or by such other means as we and Jefferies may agree upon. There is no arrangement for funds to be received in an escrow, trust or similar arrangement.

We will pay Jefferies a commission equal to 3% of the aggregate gross proceeds we receive from each sale of our shares of common stock. Because there is no minimum offering amount required as a condition to close this offering, the actual total public offering amount, commissions and proceeds to us, if any, are not determinable at this time. In addition, we have agreed in the Sales Agreement to reimburse Jefferies for the fees and disbursements of its counsel, payable upon the execution of the Sales Agreement, in an amount not to exceed $75,000, in addition to certain ongoing disbursements of its legal counsel. We estimate that the total expenses for the offering, excluding any commissions or expense reimbursement payable to Jefferies under the terms of the Sales Agreement, will be approximately $175,000. The remaining sale proceeds, after deducting any other transaction fees, will equal our net proceeds from the sale of such shares.

Jefferies will provide written confirmation to us before the open on The Nasdaq Capital Market on the day following each day on which our shares of common stock are sold under the Sales Agreement. Each confirmation will include the number of shares sold on that day, the aggregate gross proceeds of such sales and the proceeds to us.

In connection with the sale of our shares of common stock on our behalf, Jefferies may be deemed to be an "underwriter" within the meaning of the Securities Act, and the compensation of Jefferies will be deemed to be underwriting commissions or discounts. We have agreed to indemnify Jefferies against certain civil liabilities, including liabilities under the Securities Act. We have also agreed to contribute to payments Jefferies may be required to make in respect of such liabilities.

The offering of our shares of common stock pursuant to the Sales Agreement will terminate upon the earlier of (i) the sale of all shares of common stock subject to the Sales Agreement and (ii) the termination of the Sales Agreement as permitted therein. We and Jefferies may each terminate the Sales Agreement at any time upon ten days' prior notice.

This summary of the material provisions of the Sales Agreement does not purport to be a complete statement of its terms and conditions. A copy of the Sales Agreement is filed as an exhibit to a current report on Form 8-K filed under the Exchange Act, and incorporated by reference in this prospectus supplement.

Jefferies and its affiliates may in the future provide various investment banking, commercial banking, financial advisory and other financial services for us and our affiliates, for which services they may in the future receive customary fees. In the course of its business, Jefferies may actively trade our securities for its own account or for the accounts of customers, and, accordingly, Jefferies may at any time hold long or short positions in such securities.

A prospectus supplement and the accompanying prospectus in electronic format may be made available on a website maintained by Jefferies, and Jefferies may distribute the prospectus supplement and the accompanying prospectus electronically.

LEGAL MATTERS

The validity of the securities offered hereby is being passed upon for us by Paul Hastings LLP, Palo Alto, California. Wilmer Cutler Pickering Hale and Dorr LLP, New York, New York is acting as counsel for the sales agent, Jefferies LLC, in connection with this offering.

EXPERTS

The consolidated financial statements of NeuBase as of September 30, 2020 and 2019, and for each of the two years in the period ended September 30, 2020 incorporated by reference in this prospectus have been so incorporated in reliance on the report of Marcum LLP, an independent registered public accounting firm, incorporated herein by reference, given on the authority of said firm as experts in auditing and accounting

DOCUMENTS INCORPORATED BY REFERENCE

The SEC allows us to incorporate by reference information we file with the SEC into this prospectus supplement, which means that we can disclose important information to you by referring you to those publicly available documents. The information that we incorporate by reference in this prospectus supplement is considered to be part of this prospectus supplement. Because we are incorporating by reference future filings with the SEC, this prospectus supplement is continually updated and those future filings may modify or supersede some of the information included or incorporated in this prospectus supplement. This means that you must look at all of the SEC filings that we incorporate by reference to determine if any of the statements in this prospectus supplement or in any document previously incorporated by reference have been modified or superseded. This prospectus supplement incorporates by reference the documents listed below that have been previously filed with the SEC and any future filings we make with the SEC under Sections 13(a), 13(c), 14 or 15(d) of the Securities Exchange Act of 1934, as amended, or the Exchange Act, (in each case, other than those documents or the portions of those documents not deemed to be filed) until the offering of the securities under this prospectus supplement is terminated or completed:

1. The Registrant's Annual Report on Form 10-K for the fiscal year ended September 30, 2020, filed with the SEC on December 23, 2020

2. The Registrant's Quarterly Report on Form 10-Q for the quarter ended December 31, 2020 filed with the SEC on February 11, 2021, the Registrant's Quarterly Report on Form 10-Q for the quarter ended March 31, 2021 filed with the SEC on May 14, 2021, and the Registrant's Quarterly Report on Form 10-Q for the quarter ended June 30, 2021, filed with the SEC on August 13, 2021;

3. The information specifically incorporated by reference into the Registrant's Annual Report on Form 10-K from its Definitive Proxy Statement on Schedule 14A, filed with the SEC on July 2, 2021;

4. The Registrant's Current Reports on Form 8-K (other than portions thereof furnished under Item 2.02 or Item 7.01 of Form 8-K and exhibits accompanying such reports that relate to such items) filed with the SEC on (i) October 6, 2020, (ii) December 2, 2020, (iii) December 16, 2020, (iv) March 26, 2021, (v) April 22, 2021, (vi) April 26, 2021, (vii) April 27, 2021, (viii) April 30, 2021, (ix) May 12, 2021, (x) May 20, 2021 and (xi) May 25, 2021; (xii) August 12, 2021, and (xiii) August 19, 2021;

5. The description of the Registrant's common stock set forth in Exhibit 4.5 the Registrant's Annual Report on Form 10-K for the year ended September 30, 2019 (File No. 001-35963), filed with the SEC on January 10, 2020, including any amendments or reports filed for the purpose of updating such description.

You may request a copy of these filings, at no cost, by writing or telephoning us at the following address or telephone number:

NeuBase Therapeutics, Inc.
350 Technology Drive
Pittsburgh, PA 15219
(646) 450-1790

WHERE YOU CAN FIND MORE INFORMATION

We file annual, quarterly and current reports, proxy statements and other information with the SEC. Our SEC filings are available to the public over the Internet at the SEC's website at http://www.sec.gov. Copies of certain information filed by us with the SEC are also available on our website at https://www.neubasetherapeutics.com/. Our website is not a part of this prospectus supplement or the accompanying prospectus and is not incorporated by reference in this prospectus supplement or the accompanying prospectus.

This prospectus supplement and the accompanying prospectus are part of a registration statement we filed with the SEC. This prospectus supplement and the accompanying prospectus omit some information contained in the registration statement in accordance with SEC rules and regulations. You should review the information and exhibits in the registration statement for further information about us and our consolidated subsidiaries and the securities we are offering. Statements in this prospectus supplement or the accompanying prospectus concerning any document we filed as an exhibit to the registration statement or that we otherwise filed with the SEC are not intended to be comprehensive and are qualified by reference to these filings. You should review the complete document to evaluate these statements.

PROSPECTUS

neubase

$250,000,000

Common Stock
Preferred Stock
Debt Securities
Warrants
Units

We may offer and sell, from time to time in one or more offerings, up to $250,000,000 in the aggregate of any combination of the securities identified above from time to time in one or more offerings, either individually or in combination with other securities. We may also offer common stock or preferred stock upon conversion of debt securities, common stock upon conversion of preferred stock, or common stock, preferred stock or debt securities upon the exercise of warrants.

Each time we offer and sell securities, we will provide a supplement to this prospectus that contains specific information about the offering and the amounts, prices and terms of the securities. We may also authorize one or more free writing prospectuses to be provided to you in connection with these offerings. The prospectus supplement and any related free writing prospectuses may also add, update or change information contained in this prospectus with respect to that offering. You should carefully read this prospectus and the applicable prospectus supplement and any related free writing prospectus, as well as any documents incorporated by reference, before you invest in any of our securities.

We may offer and sell the securities described in this prospectus and any prospectus supplement to or through one or more underwriters, dealers and agents, or directly to purchasers, or through a combination of these methods. If any underwriters, dealers or agents are involved in the sale of any of the securities, their names and any applicable purchase price, fee, commission or discount arrangement between or among them will be set forth, or will be calculable from the information set forth, in the applicable prospectus supplement. See the sections of this prospectus entitled "About this Prospectus" and "Plan of Distribution" for more information. No securities may be sold without delivery of this prospectus and the applicable prospectus supplement describing the method and terms of the offering of such securities.

Investing in our securities involves a high degree of risk. You should review carefully the risks and uncertainties described under the heading "Risk Factors" on page 7 of this prospectus, the applicable prospectus supplement and in any applicable free writing prospectuses, and under similar headings in the documents that are incorporated by reference into this prospectus.

Our common stock is currently listed on the Nasdaq Capital Market under the symbol "NBSE." On March 31, 2021, the last reported sale price of our common stock was $7.39 per share. Our stock price is subject to fluctuation. There has been no change recently in our financial condition or results of operations that is consistent with a recent change in our stock price.

The applicable prospectus supplement will contain information, where applicable, as to any other listing on the Nasdaq Capital Market or any securities market or other exchange of the securities, if any, covered by the applicable prospectus supplement.

Neither the Securities and Exchange Commission nor any state securities commission has approved or disapproved of these securities, or passed upon the adequacy or accuracy of this prospectus. Any representation to the contrary is a criminal offense.

The date of this prospectus is April 14, 2021.

ABOUT THIS PROSPECTUS

This prospectus is part of a registration statement on Form S-3 that we filed with the Securities and Exchange Commission (the "SEC") utilizing a "shelf" registration process. Under this shelf registration process, we may offer and sell shares of our common stock and preferred stock, various series of debt securities, warrants to purchase any of such securities and/or units consisting of any combination of such securities, either individually or in combination with other securities, in one or more offerings, up to a total dollar amount of $250,000,000. This prospectus provides you with a general description of the securities we may offer.

Each time we offer securities under this prospectus, we will provide a prospectus supplement that will contain more specific information about the terms of that offering. We may also authorize one or more free writing prospectuses to be provided to you that may contain material information relating to these offerings. The prospectus supplement and any related free writing prospectus we have authorized for use in connection with a specific offering may also add, update or change any of the information contained in this prospectus or in the documents that we have incorporated by reference into this prospectus. We urge you to read carefully this prospectus, the applicable prospectus supplement and any free writing prospectuses we have authorized for use in connection with a specific offering, together with the information incorporated herein by reference as described under the section entitled "Incorporation of Documents by Reference," before buying any of the securities being offered.

THIS PROSPECTUS MAY NOT BE USED TO CONSUMMATE A SALE OF SECURITIES UNLESS IT IS ACCOMPANIED BY A PROSPECTUS SUPPLEMENT.

You should rely only on the information contained in, or incorporated by reference into, this prospectus, the applicable prospectus supplement and any free writing prospectuses, along with the information contained in any free writing prospectuses we have authorized for use in connection with a specific offering. We have not authorized anyone to provide you with different or additional information. This prospectus is an offer to sell only the securities offered hereby, but only under circumstances and in jurisdictions where it is lawful to do so.

The information appearing in this prospectus, the applicable prospectus supplement or any related free writing prospectus is accurate only as of the date on the front of the document and any information we have incorporated by reference is accurate only as of the date of the document incorporated by reference, regardless of the time of delivery of this prospectus, the applicable prospectus supplement or any related free writing prospectus, or any sale of a security. Our business, financial condition, results of operations and prospects may have changed since those dates.

This prospectus contains summaries of certain provisions contained in some of the documents described herein, but reference is made to the actual documents for complete information. All of the summaries are qualified in their entirety by the actual documents. Copies of some of the documents referred to herein have been filed, will be filed or will be incorporated by reference as exhibits to the registration statement of which this prospectus is a part, and you may obtain copies of those documents as described below under the heading "Where You Can Find More Information."

We own or have rights to use the trademarks and trade names that we use in conjunction with the operation of our business. Solely for convenience, our trademarks and trade names referred to in this prospectus may appear without the ® or ™ symbols, but those references are not intended to indicate, in any way, that we will not assert, to the fullest extent under applicable law, our rights or the right of the applicable licensor to these trademarks and trade names.

INDUSTRY AND MARKET DATA

Unless otherwise indicated, we have based the information concerning our industry contained in this prospectus and incorporated by reference herein on our general knowledge of and expectations concerning the industry, which involve risks and uncertainties and are subject to change based on various factors, including those discussed in the "Risk Factors" section of this prospectus and in the other information contained or incorporated by reference in this prospectus. These and other factors could cause the information concerning our industry to differ materially from those expressed in this prospectus and incorporated by reference herein.

SUMMARY

This summary highlights selected information that is presented in greater detail elsewhere in this prospectus or incorporated by reference in this prospectus.Because it is only a summary, it does not contain all of the information you should consider before investing in our common stock, preferred stock, debt securities, warrants or units, and it is qualified in its entirety by, and should be read in conjunction with, the more detailed information included elsewhere in this prospectus. Before you decide whether to purchase shares of our common stock or preferred stock, or our debt securities, warrants or units, you should read this entire prospectus, the applicable prospectus supplement and any related free writing prospectus carefully, including the risks of investing in our securities discussed under the heading "Risk Factors" contained in the applicable prospectus supplement and any related free writing prospectus, and under similar headings in the other documents that are incorporated by reference into this prospectus. You should also carefully read the information incorporated by reference into this prospectus, including our financial statements, and the exhibits to the registration statement of which this prospectus is a part. Unless the context otherwise requires, the terms "NeuBase," the "Company," "we," "us" and "our" in this prospectus refer to NeuBase Therapeutics, Inc. and its wholly owned subsidiaries.

NeuBase Therapeutics, Inc.

We are a biotechnology company working towards accelerating the genetic revolution by developing a new class of synthetic medicines. Our modular peptide-nucleic acid antisense oligo ("PATrOL™") platform which outputs "anti-gene" candidate therapies is designed to combine the specificity of genetic sequence-based target recognition with a modularity that enables use of various *in vivo* delivery technologies to enable broad and also selective tissue distribution capabilities. Given that every human disease may have a genetic component, we believe that our differentiated platform technology has the potential for broad impact by increasing, decreasing or changing gene function at either the DNA or RNA levels to resolve the progression to disease, as appropriate, in a particular indication. We plan to use our platform to address diseases driven by genetic abnormalities, and we are initially focused on Huntington's disease ("HD") and myotonic dystrophy type 1 ("DM1").

We are developing "anti-gene" therapies. Anti-genes are similar to, but distinct from, antisense oligonucleotides (ASOs). ASOs are short single strands of nucleic acids (traditionally thought of as single-stranded DNA molecules) which bind to defective RNA targets in cells and inhibit their ability to form defective proteins. We believe we are a leader in the discovery and development of anti-gene therapies, a new class of investigational therapies derived from peptide-nucleic acids ("PNAs"). The key differentiator between ASOs and anti-genes is that the scaffold is not derived from a natural sugar-phosphate nucleic acid backbone, rather is a synthetic polyamide which is charge-neutral and characterized by high binding affinity to a nucleic acid target, high sequence specificity, high stability, and is relatively immunologically inert. These features provide potential advantages over ASOs and other genetic therapies for modulating disease-causing genes including increased unique disease target opportunities, improved target specificity and a reduction in both sequence-dependent and independent toxicities. In addition, as these anti-genes are manufactured via standard peptide synthesis methods, they efficiently leverage the advancements in the synthetic peptide industry to enable modulation of pharmacophore delivery, pharmacokinetics, sub-cellular placement and endosomal escape.

In addition to the scaffold, the Company has a kit of natural nucleobases, chemically modified nucleobases which add further precision to a nucleic acid target of interest, and proprietary bi-specific nucleobases which can be added to the scaffold to allow more precise target engagement. These bi-specific nucleobases, in particular, have been shown to enable accessing double stranded RNA targets comprised of secondary structures such as hairpins (double stranded RNA targets which are folded upon themselves). This allows us to potentially access regions of the target transcript which may be unique in secondary structure to allow enhanced selectivity for the target (mutant)

RNA as compared to the normal RNA. Enhanced selectivity for mutant RNAs as compared to normal RNAs is often important as normal RNAs are often required for effective functioning of the cell.

A third component of the modular platform is the ability to add delivery technology to the anti-gene pharmacophores so as to reach a desired cell or tissue upon *in vivo* administration. There is flexibility to append various delivery technologies to the pharmacophore to allow either broad tissue distribution or narrow cell and/or tissue targeting if so desired based on targets. One such technology is a chemical moiety that can be used to decorate the scaffold directly and allows the anti-genes to penetrate cell membranes and into subcellular compartments where they act as well as to distribute throughout the body when administered systemically.

Finally, in addition to the anti-gene scaffold, modified nucleobases and delivery technology, the platform toolkit also includes linker technology which, when added to both ends of the anti-genes, has been shown in early pre-clinical studies to allow cooperative binding between individual drug molecules once they are engaged with the nucleic acid target to form longer and more tightly bound drugs.

This toolkit of components forms the PATrOL™ platform and allows us to manufacture gene and transcript-specific anti-genes.

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 are developing several preclinical programs using our PATrOL™ platform, including the NT0100 program, targeted at Huntington's disease ("HD"), a repeat expansion disorder, and the NT0200 program, targeted at myotonic dystrophy, type 1 ("DM1"), a second repeat expansion. Preclinical studies are being conducted to evaluate the PATrOL™ platform technology and program candidates in the areas of pharmacokinetics, pharmacodynamics and tolerability, and we reported results from certain of those studies in the first calendar quarter of 2020 and have extended upon certain of those studies in the fourth calendar quarter of 2020 which illustrated that our anti-gene technology can be administered to human patient-derived cell lines and systemically (via intravenous (IV) administration) into animals with DM1 (a genetically modified model accepted as representative of the human disease in skeletal muscle) and can address the causal genetic defect. We expect to present additional results from ongoing preclinical studies evaluating the PATrOL™ platform and pipeline indications in the second quarter of calendar 2021, begin IND enabling studies in one or more of our programs in calendar year 2021 and begin a clinical trial in one or more of our programs in calendar 2022. In addition, the emerging pipeline of other investigational therapies that target primary and secondary RNA structure and genomic DNA potentially allows a unique market advantage across a variety of rare diseases and oncology targets.

Overall, using our PATrOL™ platform, we believe we can create anti-gene therapies that have distinct advantages over other chemical entities currently in the market or in development for genetic medicine applications to modulate mutant genes and improve a clinical trait or disorder. These advantages may differ by indication and can include, among others:

- increased unique target opportunities, improved target specificity and a reduction in both sequence-dependent and independent toxicities by virtue of a synthetic polyamide scaffold which is charge-neutral and characterized by high binding affinity to a nucleic acid target, high sequence specificity, high stability, and is relatively immunologically inert;
- potential long durability by nature of the relatively highly stable polyamide scaffold;
- our anti-genes are manufactured via standard peptide synthesis methods and thus they efficiently leverage advances in the synthetic peptide industry to enable facile addition of known moieties enabling modulating pharmacophore delivery, pharmacokinetics, sub-cellular placement and endosomal escape; and
- our anti-genes can uniquely target double stranded structures in RNA, which allow unique target opportunities that standard ASOs cannot access.

With these unique component parts and their advantages, our PATrOL™ platform-enabled anti-gene therapies can potentially address a multitude of rare genetic diseases and cancer, among other indications.

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, with an initial emphasis on rare genetic diseases and cancers. A key component of this strategy is continuing to improve the scientific understanding and optimization of our platform technology and programs, including how various components of our platform technology perform, and how our investigational therapies impact the biological processes of the target diseases, so that we can utilize this information to reduce risk in our future programs and indications. In addition, with our expertise in discovering and characterizing novel anti-gene investigational therapies, we believe that our scientists can optimize the properties of our PATrOL™-enabled drug candidates for use with particular targets that we determine to be of high value.

The depth of our knowledge and expertise with PNAs, bi-facial and engineered nucleotides, genetics and genomics and therapeutic development of first-in-class modalities provides potential flexibility to determine the optimal development and commercialization strategy to maximize the near and longer-term value of our therapeutic programs.

We have distinct partnering strategies that we plan to employ 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.

Globally, there are thousands of genetic diseases, most of which lack any therapeutic options. In addition, rare genetic diseases are often particularly severe, debilitating or fatal. Traditionally, therapeutic development for each rare genetic disorder has been approached with a unique strategy, which is inefficient, as there are thousands of diseases that need treatment solutions. The collective population of people with rare diseases stands to benefit profoundly from the emergence of a scalable and modular treatment development platform that allows for a more efficient discovery of drug product candidates to address these conditions cohesively.

Mutated proteins resulting from errors in deoxyribonucleic acid ("DNA") sequences cause many rare genetic diseases and cancer. DNA in each cell of the body is transcribed into pre-RNA, which is then processed (spliced) into mRNA which is exported into the cytoplasm of the cell and translated into protein. This is termed the "central dogma" of biology. Therefore, when errors in a DNA sequence occur, they are propagated to RNAs and can become a damaging protein.

The field has learned that ASOs can inactivate target RNAs before they can produce harmful proteins by binding them in a sequence-specific manner, which can delay disease progression or even eliminate genetic disease symptoms. ASOs designed by others to target known disease-related mutant RNA sequences have been shown to be able to degrade these transcripts and have a positive clinical impact. Similarly, applications in modifying splicing of pre-RNA in the nucleus of the cell have been developed by others to exclude damaging exons from the final mRNA product and have been approved by the Food and Drug Administration ("FDA"). We plan to extend upon these conceptual breakthroughs by utilizing our first-in-class technology which we believe has significant benefits in certain application areas to better resolve clinical disorders with well tolerated therapies.

We believe the breadth of the PATrOL™ platform gives us the ability to potentially address a multitude of inherited genetic diseases. The technology may allow us to target and inactivate gain-of-function and change-of-function mutations, and address targets in recessive disease and haploinsufficiencies by altering splicing to remove damaging exons/mutations or increasing expression of wild-type alleles by various means.

Gamma-modified scaffolds, an optimized version of which we utilize, have demonstrated preclinical *in vivo* efficacy in several applications which we believe can be translated across many targets and into humans. For example, in oncology such scaffolds have reduced expression of an activated oncogene (the epidermal growth factor receptor of the *EGFR* gene) and have modified gene regulation by targeting microRNA to slow tumor growth. Such scaffolds have also demonstrated *in vivo* engagement with the double-stranded genome in studies done by others to perform *in vivo* single-base genome editing.

For a complete description of our business, financial condition, results of operations and other important information, we refer you to our filings with the SEC that are incorporated by reference in this prospectus, including our Annual Report on Form 10-K for the year ended September 30, 2020 and our Quarterly Report on Form 10-Q for the quarter ended December 31, 2020. For instructions on how to find copies of these documents, see the section of this prospectus entitled "Where You Can Find More Information."

See the section entitled "Risk Factors" in this prospectus for a discussion of some of the risks relating to the execution of our business strategy.

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 a reverse merger transaction (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. Our principal executive offices are located at 350 Technology Drive, Pittsburgh, PA 15219, and our telephone number is (646) 450-1790. 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 prospectus and should not be relied upon in connection with making any decision with respect to an investment in our securities. We are required to file annual, quarterly and current reports, proxy statements and other information with the SEC. You may obtain any of the documents filed by us with the SEC at no cost from the SEC's website at *www.sec.gov*.

We are a "smaller reporting company" as defined in Rule 12b-2 of the Securities Exchange Act of 1934, as amended (the "Exchange Act"), and have elected to take advantage of certain of the scaled disclosure available for smaller reporting companies in this prospectus as well as our filings under the Exchange Act.

RISK FACTORS

Risk Factor Summary

Below is a summary of the principal factors that make an investment in our securities 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 and should be carefully considered, together with all of the other information appearing in or incorporated by reference into this prospectus, the applicable prospectus supplement and any related free writing prospectus before making investment decisions regarding the offered securities.

- 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 global pandemic is adversely impacting our business, including our manufacturing and preclinical studies.
- 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 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, 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.

- 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

Investing in any securities offered pursuant to this prospectus, the applicable prospectus supplement and any related free writing prospectus involves a high degree of risk. Before making an investment decision, you should carefully consider the risks described below, under "Risk Factors" in the applicable prospectus supplement, any related free writing prospectus and in our most recent Annual Report on Form 10-K, or in any updates in our Quarterly Reports on Form 10-Q, together with all of the other information appearing in or incorporated by reference into this prospectus, the applicable prospectus supplement and any related free writing prospectus, before deciding whether to purchase any of the securities being offered. Our business, financial condition or results of operations could be materially adversely affected by any of these risks. The occurrence of any of these risks might cause you to lose all or part of your investment in the offered securities.

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 ("Ohr"), and NeuBase Therapeutics, Inc., a Delaware corporation ("Legacy NeuBase"), in accordance with the terms of the Agreement and Plan of Merger Reorganization entered into on January 2, 2019 (the "Merger Agreement"). Since our incorporation, we have focused primarily on the development of our proprietary Peptide-nucleic acid AnTisense OLigo ("PATrOL™") 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 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.

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 peptide nucleic acids ("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.

Furthermore, we have licensed or acquired virtually all of the intellectual property related to our product candidates from Carnegie Mellon University ("CMU"). Some of our preclinical studies and other analyses performed to date with respect to our product candidates have been conducted by their original owners or collaborators. Therefore, 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 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 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.

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.

We believe that our existing balance of cash and cash equivalents will enable us to fund our operations into the first calendar quarter of 2022. In particular, we expect that these funds will allow us to achieve certain preclinical milestones for our NT0100 program for HD and our NT0200 program for DM1, but we expect that we will need to obtain additional funding to obtain clinical data from the 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. 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 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.

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 creates 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.

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 investigational drugs in clinical development for HD, and several ongoing 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. 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. 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 CDC, 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 Good Laboratory Practice- ("GLP") and Current Good Manufacturing Practice- ("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, non-clinical 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 March 30, 2021, we had 21 full-time employees. 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. We have filled several key open positions and are currently recruiting for several other remaining positions. However, 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 will 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 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.

The majority of our current management lacks public company experience, which could put us at greater risk of incurring fines or regulatory actions for failure to comply with federal securities laws and could put us at a competitive disadvantage and require our management to devote additional time and resources to ensure compliance with applicable corporate governance requirements.

The majority of our current executive management do not have experience in managing and operating a public company, which could have an adverse effect on our ability to quickly respond to problems or adequately address issues and matters applicable to public companies. Any failure to comply with federal securities laws, rules or regulations could subject us to fines or regulatory actions, which may materially adversely affect our business, financial condition and results of operations. Further, since certain of our current executive officers do not have experience managing and operating a public company, we may need to dedicate additional time and resources to comply with legally mandated corporate governance policies relative to our competitors whose management teams have more public company experience.

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, Pennsylvania, greater New York, New York, and greater southern California 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.

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.

Accordingly, although there can be no assurance that we will undertake or successfully complete any 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.

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.

Our license agreement with CMU (the "CMU License Agreement"), as the licensor (the "Licensor"), is important to our business, and we expect to enter into additional license agreements in the future. The CMU License Agreement imposes, and we expect that future license agreements will 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 the CMU License Agreement, the Licensor 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 makes an assignment for the benefit of creditors. In the event the CMU License Agreement is terminated by the Licensor, all licenses (or, in the determination of the Licensor, the exclusivity of such licenses) granted to us by the Licensor 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 the CMU License Agreement, which could adversely affect the overall profitability for us of any product candidates that we may seek to commercialize.

Under the terms of the CMU License Agreement, we will be required to pay royalties on future worldwide net product sales and a percentage of sublicensing fees that we may earn. 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 such 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 non-enablement. Grounds for an unenforceability assertion could be an 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 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 the Company of our regulatory responsibilities. The Company 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.

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 non-clinical 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 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.

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. The FDA established the Office of Cellular, Tissue and Gene Therapies within its Center for Biologics Evaluation and Research to consolidate the review of gene therapy and related products, and established the Cellular, Tissue and Gene Therapies Advisory Committee to advise this review. Prior to submitting an IND, our human clinical trials will be subject to review by the NIH Office of Biotechnology Activities ("OBA") Recombinant DNA Advisory Committee (the "RAC"). Following an initial review, RAC members make a recommendation as to whether the protocol raises important scientific, safety, medical, ethical or social issues that warrant in-depth discussion at the RAC's quarterly meetings. Even though the FDA decides whether individual gene therapy protocols may proceed under an IND, the RAC's recommendations are shared with the FDA and the RAC public review process, if undertaken, can delay the initiation of a clinical trial, even if the FDA has reviewed the trial design and details and has not objected to its initiation or has notified the sponsor that the study may begin. Conversely, the FDA can put an IND on a clinical hold even if the RAC has provided a favorable review or has recommended against an in-depth, public review. Moreover, under guidelines published by the NIH, patient enrollment in our future gene silencing clinical trials cannot begin until the investigator for such clinical trial has received a letter from the OBA indicating that the RAC review process has been completed; and Institutional Biosafety Committee ("IBC") approval as well as all other applicable regulatory authorizations have been obtained. In addition to the government regulators, the IBC and IRB of each institution at which we will conduct clinical trials of our product candidates, or a central IRB if appropriate, would need to review the proposed clinical trial to assess the safety of the 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 requirements 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 personally 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 late-stage 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. 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, refusal to approve a pending application for a product candidate, withdrawal of an approval, or suspension of production. 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.

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 ACA and Medicare. 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 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 the federal level, the Trump administration's budget proposal for fiscal year 2019 contains further drug price control measures that could be enacted during the 2019 budget process or in other future legislation, including, for example, measures to permit Medicare Part D plans to negotiate the price of certain drugs under Medicare Part B, to allow some states to negotiate drug prices under Medicaid, and to eliminate cost sharing for generic drugs for low-income patients. Further, the Trump administration released a "Blueprint," or plan, to lower drug prices and reduce out of pocket costs of drugs that contains additional proposals to increase drug manufacturer competition, increase the negotiating power of certain federal healthcare programs, incentivize manufacturers to lower the list price of their products, and reduce the out of pocket costs of drug products paid by consumers. The U.S. Department of Health and Human Services has already started the process of soliciting feedback on some of these measures and, at the same, is immediately implementing others under its existing authority. While some proposed measures will require authorization through additional legislation to become effective, Congress and the Trump administration have each indicated that it will continue to seek new legislative and/or administrative measures to control drug costs. At the state level, legislatures are increasingly passing legislation and implementing regulations designed to control pharmaceutical and biological product pricing, including price or patient reimbursement constraints, discounts, restrictions on certain product access and marketing cost disclosure and transparency measures, and, in some cases, designed to encourage importation from other countries and bulk purchasing.

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 the UK remained subject to EU law during a "Brexit 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 UK from the EU, if it occurs, might have on the regulatory frameworks of the UK or the EU, or on our future operations, if any, in these jurisdictions.

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 and policy changes. Average review times at the agency have fluctuated in recent years as a result. 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.

In the U.S., 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, 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. Under the Health Insurance Portability and Accountability Act of 1996, 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. 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. In addition, private individuals have the ability to bring actions on behalf of the government under the federal False Claims Act as well as under the false claims laws of several states.

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 the April 2003 Office of Inspector General Compliance Program Guidance for Pharmaceutical Manufacturers or the 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.

Neither the government nor the courts have provided definitive guidance on the application of fraud and abuse laws to our business. Law enforcement authorities are increasingly focused on enforcing these laws, and it is possible that some of our practices may be challenged under these laws. Efforts to ensure that our business arrangements with third parties will comply with applicable healthcare laws and regulations will involve substantial costs. If we 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 and data releases;
- 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 our 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, management, or board 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 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.

As previously disclosed in our Annual Report on Form 10-K for the fiscal year ended September 30, 2019 (the "2019 Form 10-K"), in connection with the preparation of the Company's consolidated financial statements for the fiscal year ended September 30, 2019, but prior to the issuance of such financial statements, the Company determined the accounting treatment and valuations pertaining to the PATrOL™ technology license acquired during the first quarter of fiscal 2019 should be modified. The 2019 Form 10-K disclosed that the change in accounting treatment and valuations resulted in an increase in total operating expenses of approximately $0.9 million on the Company's consolidated statements of operations for the fiscal year ended September 30, 2019 and a decrease in intangible assets of approximately $1.5 million on the Company's consolidated balance sheet as of and for the fiscal year ended September 30, 2019, as well as a decrease in total operating expenses of approximately $0.3 million on the

Company's consolidated statements of operations in connection with the adjustment of the valuation of certain share-based awards for the fiscal year ended September 30, 2019.

In addition, the Company identified an error in one of the Black-Scholes option pricing model assumptions, utilized in calculating the fair value of a stock option award granted during the year ended September 30, 2019, which resulted in an overstatement of share-based compensation expense. The Company concluded that the error was not material to any prior annual or interim period. Nevertheless, the Company has revised its historical financial statements to properly reflect the fair value of options granted in the prior period.

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

Dr. Stephan, our President, 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.

We previously identified a material weakness in our internal control over financial reporting as of September 30, 2019. Although this material weakness was remediated as of September 30, 2020 as discussed in Item 9A of Part II of our Annual Report on Form 10-K for the fiscal year ended September 30, 2020, we cannot assure you that we will not identify another material weakness in the future. 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.

As previously disclosed in our 2019 Form 10-K, in connection with the preparation of our financial statements for such fiscal year, our management and the Audit Committee of our board of directors determined that our accounting treatment and valuations pertaining to the PATrOL™ technology license should be modified. In connection with such revisions, our management identified a material weakness in our internal control over financial reporting due to a lack of expertise in complex accounting transactions, which were not operating effectively to provide reasonable assurance that complex transactions were accounted for correctly. We subsequently restated our unaudited condensed consolidated statements of operations and our unaudited condensed consolidated balance sheets as of and for the three months ended December 31, 2018 and as of and for the three and six months ended March 31, 2019 upon the filing of our Quarterly Reports on Form 10-Q for such periods. As discussed in Item 9A of Part II of our Annual Report on Form 10-K for the fiscal year ended September 30, 2020, we took remedial measures during fiscal 2019 and throughout fiscal 2020 to remediate this material weakness.

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.

We restated our previously issued unaudited financial statements for the three months ended December 31, 2019 and the three and six months ended March 31, 2019. As a result, and if we identify errors in our financial reporting in the future that require us to restate other 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.

As discussed further in our Quarterly Reports on Form 10-Q for the quarters ended December 31, 2019 and March 31, 2020, we restated our unaudited condensed consolidated financial statements and related disclosures for the three months ended December 31, 2018 and the three and six months ended March 31, 2019 in a Form 8-K amendment filed on March 26, 2020. The misstatements were quantitatively material to the period presented in such prior financial statements, and we determined that it would be appropriate to correct the misstatements in such previously issued interim financial statements by restating such financial statements. 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.

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.

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.

We are subject to securities class action litigation and derivative shareholder litigation. If an unfavorable ruling were to occur, there exists the possibility of a material adverse impact on us.

On February 14, 2018, plaintiff Jeevesh Khanna, commenced an action in the Southern District of New York, against Ohr and several 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, stating the class period to be 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. We and the individuals dispute these claims and intend to defend the matter vigorously. On September 17, 2018, Ohr filed a motion to dismiss the complaint. On September 20, 2019, the district court entered an order granting the defendants' motion to dismiss. On October 23, 2019, the plaintiffs filed a notice of appeal of that order dismissing the action and other related orders by the district court. 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 October 16, 2020, the district court requested the parties' positions as to how they proposed to proceed in light of the Second Circuit's decision. After letter briefing on this issue and plaintiffs' alternative request for leave to file a second amended complaint, on November 16, 2020, the district court denied plaintiffs' 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, and the plaintiffs filed their appellate brief with respect to such matters with the Court on March 31, 2021. This litigation could result in substantial costs and a diversion of management's resources and attention, which could harm the Company's business and the value of the Company's common stock.

On May 3, 2018, plaintiff Adele J. Barke, derivatively on behalf of Ohr, commenced an action against certain former directors of Ohr, including Michael Ferguson, Orin Hirschman, Thomas M. Riedhammer, June Almenoff and Jason S. 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. We and the individuals dispute these claims and intend to defend the matter vigorously. Such litigation has been stayed pursuant to a stipulation by the parties, which has been so ordered by the court, pending a decision in the Southern District case on the motion to dismiss, but that status could change. This litigation could result in substantial costs and a diversion of management's resources and attention, which could harm our business and the value of our common stock.

On March 20, 2019, a putative class action lawsuit was filed in the United States District Court for District of Delaware naming as defendants Ohr and its board of directors, Legacy NeuBase and Ohr Acquisition Corp., captioned *Wheby v. Ohr Pharmaceutical, Inc., et al*, Case No. 1:19-cv-00541-UNA (the "Wheby Action"). The plaintiffs in the Wheby Action allege 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. The complaint in the Wheby Action has not been served on, nor was service waived by, any of the named defendants in that action. The action seeks, among other things, to rescind the Merger or an award of damages, and an award of attorneys' and experts' fees and expenses. The defendants dispute the claims raised in the Wheby Action. Management believes that the likelihood of an adverse decision from the sole remaining action is unlikely; however, the litigation could result in substantial costs and a diversion of management's resources and attention, which could harm our business and the value of our common stock.

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, 2020, we had approximately $20.0 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.

General Risk Factors

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.

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 U.S. GAAP. 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.

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.

DISCLOSURE REGARDING FORWARD-LOOKING STATEMENTS

This prospectus, any accompanying prospectus supplement and the documents incorporated by reference into this prospectus may contain forward-looking statements within the meaning of Section 27A of the Securities Act of 1933, as amended (the "Securities Act"), and Section 21E of the Exchange Act, about NeuBase. These forward-looking statements are intended to be covered by the safe harbor for forward-looking statements provided by the Private Securities Litigation Reform Act of 1995. Forward-looking statements are not statements of historical fact, and can be identified by the use of forward-looking terminology such as "believes," "expects," "may," "will," "could," "should," "projects," "plans," "goal," "targets," "potential," "estimates," "pro forma," "seeks," "intends" or "anticipates" or the negative thereof or comparable terminology. Forward-looking statements include discussions of strategy, financial projections, guidance and estimates (including their underlying assumptions), statements regarding plans, objectives, expectations or consequences of various transactions, and statements about the future performance, operations, products and services of NeuBase. We caution our stockholders and other readers not to place undue reliance on such statements.

You should read this prospectus, any accompanying prospectus supplement and the documents incorporated by reference completely and with the understanding that our actual future results may be materially different from what we currently expect. Our business and operations are and will be subject to a variety of risks, uncertainties and other factors. Consequently, actual results and experience may materially differ from those contained in any forward-looking statements. Such risks, uncertainties and other factors that could cause actual results and experience to differ from those projected include, but are not limited to, the risk factors set forth in Part I - Item 1A, "Risk Factors," in our Annual Report on Form 10-K for the year ended September 30, 2020 filed with the SEC on December 23, 2020 and inour Quarterly Report on Form 10-Q for the quarter ended December 31, 2020, filed with the SEC on February 11, 2021, and elsewhere in the other documents incorporated by reference into this prospectus.

You should assume that the information appearing in this prospectus, any accompanying prospectus supplement, any related free writing prospectus and any document incorporated herein by reference is accurate as of its date only. Because the risk factors referred to above could cause actual results or outcomes to differ materially from those expressed in any forward-looking statements made by us or on our behalf, you should not place undue reliance on any forward-looking statements. Further, any forward-looking statement speaks only as of the date on which it is made. New factors emerge from time to time, and it is not possible for us to predict which factors will arise. In addition, we cannot assess the impact of each factor on our business or the extent to which any factor, or combination of factors, may cause actual results to differ materially from those contained in any forward-looking statements. All written or oral forward-looking statements attributable to us or any person acting on our behalf made after the date of this prospectus are expressly qualified in their entirety by the risk factors and cautionary statements contained in and incorporated by reference into this prospectus. Unless legally required, we do not undertake any obligation to release publicly any revisions to such forward-looking statements to reflect events or circumstances after the date of this prospectus or to reflect the occurrence of unanticipated events.

USE OF PROCEEDS

Except as otherwise provided in the applicable prospectus supplement or in any free writing prospectuses we have authorized for use in connection with a specific offering, we currently intend to use the net proceeds from the sale of the securities offered by this prospectus, if any, for working capital and general corporate purposes, which may include capital expenditures, research and development expenditures, regulatory affairs expenditures, preclinical and clinical trial expenditures, pipeline expansion, legal expenditures, including intellectual property protection and maintenance expenditures, acquisitions of new technologies and investments, business combinations and the repurchase of capital stock.

The intended application of proceeds from the sale of any particular offering of securities using this prospectus will be described in the accompanying prospectus supplement relating to such offering. The precise amount and timing of the application of these proceeds will depend upon a number of factors, such as the timing and progress of our research and development efforts, the timing of any acquisition or business combination efforts, our funding requirements and the availability and costs of other funds. Pending application of the net proceeds as described above, we intend to temporarily invest the proceeds in short term, investment-grade, interest-bearing securities.

DESCRIPTION OF CAPITAL STOCK

General Matters

The following description summarizes the most important terms of our capital stock. Because it is only a summary of the provisions of our certificate of incorporation, as amended (the "Certificate of Incorporation"), and bylaws, as amended (the "Bylaws"), it does not contain all of the information that may be important to you. For a complete description of the matters set forth in this "Description of Capital Stock," you should refer to our Certificate of Incorporation and Bylaws, each of which are included as exhibits to the registration statement of which this prospectus is a part, and to the applicable provisions of Delaware law.

As of December 31, 2020, our authorized capital stock consisted of 250,000,000 shares of common stock, $0.0001 par value per share, and 10,000,000 shares of preferred stock, $0.0001 par value per share. Our Board may establish the rights and preferences of the preferred stock from time to time. As of March 31, 2021, there were 23,180,024 shares of our common stock issued and outstanding and no shares of preferred stock issued and outstanding.

Common Stock

Dividend Rights. We have never paid cash dividends on our Common Stock. Moreover, we do not anticipate paying periodic cash dividends on Common Stock for the foreseeable future. Any future determination about the payment of dividends will be made at the discretion of our board of directors and will depend upon its earnings, if any, capital requirements, operating and financial conditions and on such other factors as our board of directors deems relevant.

Preferred Stock

We currently have no outstanding shares of Preferred Stock. Under our Certificate of Incorporation, our board of directors has the authority, without further action by stockholders, to designate one or more series of Preferred Stock and to fix the voting powers, designations, preferences, limitations, restrictions and relative rights granted to or imposed upon the Preferred Stock, including dividend rights, conversion rights, voting rights, rights and terms of redemption, liquidation preference and sinking fund terms, any or all of which may be preferential to or greater than the rights of our Common Stock.

Our board of directors may authorize the issuance of Preferred Stock with voting or conversion rights that could adversely affect the voting power or other rights of the holders of Common Stock. The issuance of Preferred Stock, while providing flexibility in connection with possible acquisitions and other corporate purposes, could, among other things, have the effect of delaying, deferring or preventing a change in control and may adversely affect the market price of the Common Stock and the voting and other rights of the holders of Common Stock.

Our board of directors may specify the following characteristics of any Preferred Stock:

- the designation and stated value, if any, of the class or series of Preferred Stock;
- the number of shares of the class or series of Preferred Stock offered, and the liquidation preference, if any, per share;

- the dividend rate(s), period(s) or payment date(s) or method(s) of calculation, if any, applicable to the class or series of Preferred Stock;
- whether dividends, if any, are cumulative or non-cumulative and, if cumulative, the date from which dividends on the class or series of Preferred Stock will accumulate;
- the provisions for a sinking fund, if any, for the class or series of Preferred Stock;
- the provision for redemption, if applicable, of the class or series of Preferred Stock;
- the terms and conditions, if applicable, upon which the class or series of Preferred Stock will be convertible into Common Stock, including the conversion price or manner of calculation and conversion period;
- voting rights, if any, of the class or series of Preferred Stock;
- the relative ranking and preferences of the class or series of Preferred Stock as to dividend rights and rights, if any, upon the liquidation, dissolution or winding up of our affairs;
- any limitations on issuance of any class or series of Preferred Stock ranking senior to or on a parity with the class or series of Preferred Stock as to dividend rights and rights, if any, upon liquidation, dissolution or winding up of our affairs; and
- any other specific terms, preferences, rights, limitations or restrictions of the class or series of Preferred Stock.

Anti-Takeover Effects of Certain Provisions of our Certificate of Incorporation, Bylaws and the General Corporation Law of the State of Delaware

Certain provisions of Delaware law and the Company's Certificate of Incorporation contain provisions that could make the following transactions more difficult: acquisition of the Company by means of a tender offer; acquisition of the Company by means of a proxy contest or otherwise; or removal of the Company's incumbent officers and directors. It is possible that these provisions could make it more difficult to accomplish or could deter transactions that stockholders may otherwise consider to be in their best interest or in the Company's best interests, including transactions that might result in a premium over the market price for the Company's capital stock.

These provisions, summarized below, are expected to discourage coercive takeover practices and inadequate takeover bids. These provisions are also designed to encourage persons seeking to acquire control of the Company to first negotiate with the Company's board of directors. The Company believes that the benefits of increased protection of the Company's potential ability to negotiate with the proponent of an unfriendly or unsolicited proposal to acquire or restructure the Company outweigh the disadvantages of discouraging these proposals because negotiation of these proposals could result in an improvement of their terms.

Delaware Anti-Takeover Statute

The Company is subject to Section 203 of the General Corporation Law of the State of Delaware, which prohibits persons deemed to be "interested stockholders" from engaging in a "business combination" with a publicly-held Delaware corporation for three years following the date these persons become interested stockholders unless the business combination is, or the transaction in which the person became an interested stockholder was, approved in a prescribed manner or another prescribed exception applies. Generally, an "interested stockholder" is a person who, together with affiliates and associates, owns, or within three years prior to the determination of interested stockholder status did own, 15% or more of a corporation's voting stock. Generally, a "business combination" includes a merger, asset or stock sale, or other transaction resulting in a financial benefit to the interested stockholder. The existence of this provision may have an anti-takeover effect with respect to transactions not approved in advance by the board of directors, such as discouraging takeover attempts that might result in a premium over the market price of the Common Stock.

Undesignated Preferred Stock

The ability to authorize undesignated Preferred Stock will make it possible for the Company's board of directors to issue Preferred Stock with voting or other rights or preferences that could impede the success of any attempt to change control of the Company. These and other provisions may have the effect of deterring hostile takeovers or delaying changes in control or management of the Company.

Elimination of Stockholder Action by Written Consent

Our Certificate of Incorporation eliminates the right of stockholders to act by written consent without a meeting.

Classified Board; Election and Removal of Directors; Filling Vacancies

The Company's board of directors are divided into three classes. The directors in each class serve for a three-year term, one class being elected each year by the Company's stockholders, with staggered three-year terms. Only one class of directors will be elected at each annual meeting of the Company's stockholders, with the other classes continuing for the remainder of their respective three-year terms. At all meetings of stockholders for the election of directors, a plurality of the votes cast is sufficient to elect each director. The Company's Certificate of Incorporation provides for the removal of any of the Company's directors only for cause and requires a stockholder vote by the holders of at least 66 2/3% of the voting power of the then outstanding voting stock. Furthermore, any vacancy on the Company's board of directors, however occurring, including a vacancy resulting from an increase in the size of the board, may only be filled by a resolution of the board of directors unless the board of directors determines that such vacancies shall be filled by the stockholders. This system of electing and removing directors and filling vacancies may tend to discourage a third party from making a tender offer or otherwise attempting to obtain control of the Company, because it generally makes it more difficult for stockholders to replace a majority of the directors.

Choice of Forum

The Company's Certificate of Incorporation provides that, unless the Company consents in writing to the selection of an alternative forum, the Court of Chancery of the State of Delaware will be the exclusive forum for: any derivative action or proceeding brought on the Company's behalf; any action asserting a claim of breach of fiduciary duty; any action asserting a claim against the Company arising pursuant to the Delaware General Corporation Law, the Company's Certificate of Incorporation or the Amended and Restated Bylaws of the Company; or any action asserting a claim against the Company that is governed by the internal affairs doctrine. Such exclusive forum provision, however, does not apply to suits brought to enforce any liability or duty created by the Securities Act or the Exchange Act. Although the Company's Certificate of Incorporation contains the choice of forum provision described above, it is possible that a court could find that such a provision is inapplicable for a particular claim or action or that such provision is unenforceable.

Amendment of Charter Provisions

The amendment of any of the above provisions in the Company's Certificate of Incorporation, except for the provision making it possible for the Company's board of directors to issue undesignated Preferred Stock, would require approval by a stockholder vote by the holders of at least 66 2/3% of the voting power of the then outstanding voting stock.

The provisions of the Delaware General Corporation Law and the Company's Certificate of Incorporation could have the effect of discouraging others from attempting hostile takeovers and, as a consequence, they may also inhibit temporary fluctuations in the market price of the Common Stock that often result from actual or rumored hostile takeover attempts. These provisions may also have the effect of preventing changes in the Company's management. It is possible that these provisions could make it more difficult to accomplish transactions that stockholders may otherwise deem to be in their best interests.

Transfer Agent and Registrar

The transfer agent and registrar for our Common Stock is Standard Registrar and Transfer Company. The transfer agent and registrar's address is 440 East 400 South, Suite 200, Salt Lake City, UT 84111.

Listing

Our common stock is listed on the Nasdaq Capital Market under the symbol "NBSE."

DESCRIPTION OF DEBT SECURITIES

We may issue debt securities from time to time, in one or more series, as either senior or subordinated debt or as senior or subordinated convertible debt. While the terms we have summarized below will apply generally to any debt securities that we may offer under this prospectus, we will describe the particular terms of any debt securities that we may offer in more detail in the applicable prospectus supplement. The terms of any debt securities offered under a prospectus supplement may differ from the terms described below. Unless the context requires otherwise, whenever we refer to the indenture, we also are referring to any supplemental indentures that specify the terms of a particular series of debt securities.

We will issue the debt securities under the indenture that we will enter into with the trustee named in the indenture. The indenture will be qualified under the Trust Indenture Act of 1939, as amended (the "Trust Indenture Act"). We have filed the form of indenture as an exhibit to the registration statement of which this prospectus is a part, and supplemental indentures and forms of debt securities containing the terms of the debt securities being offered will be filed as exhibits to the registration statement of which this prospectus is a part or will be incorporated by reference from reports that we file with the SEC.

The following summary of material provisions of the debt securities and the indenture is subject to, and qualified in its entirety by reference to, all of the provisions of the indenture applicable to a particular series of debt securities. We urge you to read the applicable prospectus supplements and any related free writing prospectuses we authorize for use in connection with a specific offering of debt securities, as well as the complete indenture that contains the terms of the debt securities.

General Matters

The indenture does not limit the amount of debt securities that we may issue. It provides that we may issue debt securities up to the principal amount that we may authorize and in any currency or currency unit that we may designate. Except for the limitations on consolidation, merger and sale of all or substantially all of our assets contained in the indenture, the terms of the indenture do not contain any covenants or other provisions designed to give holders of any debt securities protection against changes in our operations or financial condition or transactions involving us.

We may issue the debt securities issued under the indenture as "discount securities," which means they may be sold at a discount below their stated principal amount. These debt securities, as well as other debt securities that are not issued at a discount, may be issued with "original issue discount" ("OID") for U.S. federal income tax purposes because of interest payment and other characteristics or terms of the debt securities. Material U.S. federal income tax considerations applicable to debt securities issued with OID will be described in more detail in the applicable prospectus supplement.

We will describe in the applicable prospectus supplement the terms of the series of debt securities being offered, including:

- the title of the series of debt securities;
- any limit upon the aggregate principal amount that may be issued;
- the maturity date or dates;
- the form of the debt securities of the series;
- the applicability of any guarantees;
- whether or not the debt securities will be secured or unsecured, and the terms of any secured debt;
- whether the debt securities rank as senior debt, senior subordinated debt, subordinated debt or any combination thereof, and the terms of any subordination;
- if the price (expressed as a percentage of the aggregate principal amount thereof) at which the debt securities will be issued is a price other than the principal amount thereof, the portion of the principal amount thereof payable upon declaration of acceleration of the maturity thereof, or if applicable, the portion of the principal amount of such debt securities that is convertible into another security or the method by which any such portion shall be determined;
- the interest rate or rates, which may be fixed or variable, or the method for determining the rate and the date interest will begin to accrue, the dates interest will be payable and the regular record dates for interest payment dates or the method for determining such dates;
- our right, if any, to defer payment of interest and the maximum length of any such deferral period;
- if applicable, the date or dates after which, or the period or periods during which, and the price or prices at which, we may, at our option, redeem the series of debt securities pursuant to any optional or provisional redemption provisions and the terms of those redemption provisions;

- the date or dates, if any, on which, and the price or prices at which we are obligated, pursuant to any mandatory sinking fund or analogous fund provisions or otherwise, to redeem, or at the holder's option to purchase, the series of debt securities and the currency or currency unit in which the debt securities are payable;
- the denominations in which we will issue the series of debt securities, if other than denominations of $1,000 and any integral multiple thereof;
- any and all terms, if applicable, relating to any auction or remarketing of the debt securities of that series and any security for our obligations with respect to such debt securities and any other terms which may be advisable in connection with the marketing of debt securities of that series;
- whether the debt securities of the series shall be issued in whole or in part in the form of a global security or securities; the terms and conditions, if any, upon which such global security or securities may be exchanged in whole or in part for other individual securities, and the depositary for such global security or securities;
- if applicable, the provisions relating to conversion or exchange of any debt securities of the series and the terms and conditions upon which such debt securities will be so convertible or exchangeable, including the conversion or exchange price, as applicable, or how it will be calculated and may be adjusted, any mandatory or optional (at our option or at the holders' option) conversion or exchange features, the applicable conversion or exchange period and the manner of settlement for any conversion or exchange;
- if other than the full principal amount thereof, the portion of the principal amount of debt securities of the series which shall be payable upon declaration of acceleration of the maturity thereof;
- additions to or changes in the covenants applicable to the particular debt securities being issued, including, among others, the consolidation, merger or sale covenant;
- additions to or changes in the events of default with respect to the securities and any change in the right of the trustee or the holders to declare the principal, premium, if any, and interest, if any, with respect to such securities to be due and payable;
- additions to or changes in or deletions of the provisions relating to covenant defeasance and legal defeasance;
- additions to or changes in the provisions relating to satisfaction and discharge of the indenture;

- additions to or changes in the provisions relating to the modification of the indenture both with and without the consent of the holders of the debt securities issued under the indenture;

- the currency of payment of the debt securities if other than U.S. dollars and the manner of determining the equivalent amount in U.S. dollars;

- whether interest will be payable in cash or additional debt securities at our or the holders' option and the terms and conditions upon which the election may be made;

- the terms and conditions, if any, upon which we will pay amounts in addition to the stated interest, premium, if any, and principal amounts of the debt securities of the series to any holder that is not a "United States person" for federal tax purposes;

- any restrictions on transfer, sale or assignment of the debt securities of the series; and

- any other specific terms, preferences, rights or limitations of, or restrictions on, the debt securities, any other additions or changes in the provisions of the indenture, and any terms that may be required by us or advisable under applicable laws or regulations.

Conversion or Exchange Rights

We will set forth in the applicable prospectus supplement the terms on which a series of debt securities may be convertible into or exchangeable for our common stock or our other securities. We will include provisions as to settlement upon conversion or exchange and whether conversion or exchange is mandatory, at the option of the holder or at our option. We may include provisions pursuant to which the number of shares of our common stock or our other securities that the holders of the series of debt securities receive would be subject to adjustment.

Consolidation, Merger or Sale

Unless we provide otherwise in the prospectus supplement applicable to a particular series of debt securities, the indenture will not contain any covenant that restricts our ability to merge or consolidate, or sell, convey, transfer or otherwise dispose of our assets as an entirety or substantially as an entirety. However, any successor to or acquirer of such assets (other than a subsidiary of ours) must assume all of our obligations under the indenture or the debt securities, as appropriate.

Events of Default under the Indenture

Unless we provide otherwise in the prospectus supplement applicable to a particular series of debt securities, the following are events of default under the indenture with respect to any series of debt securities that we may issue:

- if we fail to pay any installment of interest on any series of debt securities, as and when the same shall become due and payable, and such default continues for a period of 90 days; provided, however, that a valid extension of an interest payment period by us in accordance with the terms of any indenture supplemental thereto shall not constitute a default in the payment of interest for this purpose;

- if we fail to pay the principal of, or premium, if any, on any series of debt securities as and when the same shall become due and payable whether at maturity, upon redemption, by declaration or otherwise, or in any payment required by any sinking or analogous fund established with respect to such series; provided, however, that a valid extension of the maturity of such debt securities in accordance with the terms of any indenture supplemental thereto shall not constitute a default in the payment of principal or premium, if any;

- if we fail to observe or perform any other covenant or agreement contained in the debt securities or the indenture, other than a covenant specifically relating to another series of debt securities, and our failure continues for a period of 90 days after we receive written notice of such failure, requiring the same to be remedied and stating that such is a notice of default thereunder, from the trustee or holders of at least 25% of the aggregate principal amount of the outstanding debt securities of the applicable series; and

- if specified events of bankruptcy, insolvency or reorganization occur.

If an event of default with respect to debt securities of any series occurs and is continuing, other than certain specified events of bankruptcy, insolvency or reorganization, the trustee or the holders of at least 25% of the aggregate principal amount of the outstanding debt securities of that series, by notice to us in writing, and to the trustee if notice is given by such holders, may declare the unpaid principal, premium, if any, and accrued interest, if any, of such series of debt securities immediately due and payable. If certain specified events of bankruptcy, insolvency or reorganization occur with respect to us, the principal amount and accrued interest, if any, of each issue of debt securities then outstanding shall be due and payable without any notice or other action on the part of the trustee or any holder.

The holders of a majority of the principal amount of the outstanding debt securities of an affected series may waive any default or event of default with respect to the series and its consequences, except defaults or events of default regarding payment of principal, premium, if any, or interest, unless we have cured the default or event of default in accordance with the indenture. Any waiver shall cure the default or event of default.

Subject to the terms of the indenture, if an event of default under an indenture shall occur and be continuing, the trustee will be under no obligation to exercise any of its rights or powers under such indenture at the request or direction of any of the holders of the applicable series of debt securities, unless such holders have offered the trustee reasonable indemnity. The holders of a majority of the principal amount of the outstanding debt securities of any series will have the right to direct the time, method and place of conducting any proceeding for any remedy available to the trustee, or exercising any trust or power conferred on the trustee, with respect to the debt securities of that series, provided that:

- the direction so given by the holder is not in conflict with any law or the applicable indenture; and

- subject to its duties under the Trust Indenture Act, the trustee need not take any action that might involve it in personal liability or might be unduly prejudicial to the holders not involved in the proceeding.

A holder of the debt securities of any series will have the right to institute a proceeding under the indenture or to appoint a receiver or trustee, or to seek other remedies, only if:

- the holder has given written notice to the trustee of a continuing event of default with respect to that series;
- the holders of at least 25% of the aggregate principal amount of the outstanding debt securities of that series have made a written request;
- such holders have offered to the trustee indemnity satisfactory to it against the costs, expenses and liabilities to be incurred by the trustee in compliance with the request; and
- the trustee does not institute the proceeding, and does not receive from the holders of a majority of the aggregate principal amount of the outstanding debt securities of that series other conflicting directions within 90 days after the notice, request and offer.

These limitations do not apply to a suit instituted by a holder of debt securities if we default in the payment of the principal of, or the premium, if any, or interest on, the debt securities.

We will periodically file statements with the trustee regarding our compliance with specified covenants in the indenture.

Modification of Indenture; Waiver

Unless we provide otherwise in the prospectus supplement applicable to a particular series of debt securities, we and the trustee may change an indenture without the consent of any holders with respect to specific matters, including, but not limited to, the following:

- to cure any ambiguity, defect or inconsistency in the indenture or in the debt securities of any series;
- to comply with the provisions described above under "—Consolidation, Merger or Sale";
- to provide for uncertificated debt securities in addition to or in place of certificated debt securities;
- to add to our covenants, restrictions, conditions or provisions such new covenants, restrictions, conditions or provisions for the benefit of the holders of all or any series of debt securities, to make the occurrence, or the occurrence and the continuance, of a default in any such additional covenants, restrictions, conditions or provisions an event of default or to surrender any right or power conferred upon us in the indenture;
- to add to, delete from or revise the conditions, limitations and restrictions on the authorized amount, terms or purposes of issue, authentication and delivery of debt securities, as set forth in the indenture;
- to make any change that does not adversely affect the interests of any holder of debt securities of any series in any material respect;
- to provide for the issuance of, and to establish the form and terms and conditions of, the debt securities of any series as provided above under "—General Matters," to establish the form of any certifications required to be furnished pursuant to the terms of the indenture or any series of debt securities, or to add to the rights of the holders of any series of debt securities;
- to evidence and provide for the acceptance of appointment under any indenture by a successor trustee; or
- to comply with any requirements of the SEC in connection with the qualification of any indenture under the Trust Indenture Act.

In addition, under the indenture, the rights of holders of a series of debt securities may be changed by us and the trustee with the written consent of the holders of at least a majority of the aggregate principal amount of the outstanding debt securities of each series that is affected. However, unless we provide otherwise in the prospectus supplement applicable to a particular series of debt securities, we and the trustee may make the following changes only with the consent of each holder of any outstanding debt securities affected:

- extending the fixed maturity of any debt securities of any series;

- reducing the principal amount, reducing the rate of or extending the time of payment of interest, or reducing any premium payable upon the redemption of any series of debt securities; or
- reducing the percentage of debt securities, the holders of which are required to consent to any amendment, supplement, modification or waiver.

Discharge

The indenture provides that we can elect to be discharged from our obligations with respect to one or more series of debt securities, except for specified obligations, including, but not limited to, the following obligations to:

- provide for payment;
- register the transfer or exchange of debt securities of the series;
- replace stolen, lost or mutilated debt securities of the series;
- pay principal of and premium and interest on any debt securities of the series;
- maintain paying agencies;
- hold monies for payment in trust;
- recover excess money held by the trustee;
- compensate and indemnify the trustee; and
- appoint any successor trustee.

In order to exercise our rights to be discharged, we must deposit with the trustee money or government obligations sufficient to pay all the principal of, and any

premium, if any, and interest on, the debt securities of the series on the dates payments are due.

Form, Exchange and Transfer

We will issue the debt securities of each series only in fully registered form without coupons and, unless we provide otherwise in the applicable prospectus supplement, in denominations of $1,000 and any integral multiple thereof. The indenture provides that we may issue debt securities of a series in temporary or permanent global form and as book-entry securities that will be deposited with, or on behalf of, The Depository Trust Company, New York, New York, known as DTC, or another depositary named by us and identified in the applicable prospectus supplement with respect to that series. To the extent the debt securities of a series are issued in global form and as book-entry, a description of terms relating to any book-entry securities will be set forth in the applicable prospectus supplement.

At the option of the holder, subject to the terms of the indenture and the limitations applicable to global securities described in the applicable prospectus supplement, the holder of the debt securities of any series can exchange the debt securities for other debt securities of the same series, in any authorized denomination and of like tenor and aggregate principal amount.

Subject to the terms of the indenture and the limitations applicable to global securities set forth in the applicable prospectus supplement, holders of the debt securities may present the debt securities for exchange or for registration of transfer, duly endorsed or with the form of transfer endorsed thereon duly executed if so required by us or the security registrar, at the office of the security registrar or at the office of any transfer agent designated by us for this purpose. Unless otherwise provided in the debt securities that the holder presents for transfer or exchange, we will impose no service charge for any registration of transfer or exchange, but we may require payment of any taxes or other governmental charges.

We will name in the applicable prospectus supplement the security registrar, and any transfer agent in addition to the security registrar, that we initially designate for any debt securities. We may at any time designate additional transfer agents or rescind the designation of any transfer agent or approve a change in the office through which any transfer agent acts, except that we will be required to maintain a transfer agent in each place of payment for the debt securities of each series.

If we elect to redeem the debt securities of any series, we will not be required to:

- issue, register the transfer of, or exchange any debt securities of that series during a period beginning at the opening of business 15 days before the date of mailing of a notice of redemption of any debt securities that may be selected for redemption and ending at the close of business on the date of the mailing; or

- register the transfer of or exchange any debt securities so selected for redemption, in whole or in part, except for the unredeemed portion of any debt securities we are redeeming in part.

Information Concerning the Trustee

The trustee, other than during the occurrence and continuance of an event of default under an indenture, undertakes to perform only those duties as are specifically set forth in the applicable indenture. Upon an event of default under an indenture, the trustee must use the same degree of care as a prudent person would exercise or use in the conduct of his or her own affairs. Subject to this provision, the trustee is under no obligation to exercise any of the powers given to it by the indenture at the request of any holder of debt securities unless it is offered reasonable security and indemnity against the costs, expenses and liabilities that it might incur.

Payment and Paying Agents

Unless we otherwise indicate in the applicable prospectus supplement, we will make payment of the interest on any debt securities on any interest payment date to the person in whose name the debt securities, or one or more predecessor securities, are registered at the close of business on the regular record date for the interest.

We will pay principal of and any premium and interest on the debt securities of a particular series at the office of the paying agents designated by us, except that, unless we otherwise indicate in the applicable prospectus supplement, we will make interest payments by check that we will mail to the holder or by wire transfer to certain holders. Unless we otherwise indicate in the applicable prospectus supplement, we will designate the corporate trust office of the trustee as our sole paying agent for payments with respect to debt securities of each series. We will name in the applicable prospectus supplement any other paying agents that we initially designate for the debt securities of a particular series. We will maintain a paying agent in each place of payment for the debt securities of a particular series.

All money we pay to a paying agent or the trustee for the payment of the principal of, or any premium or interest on, any debt securities that remains unclaimed at the end of two years after such principal, premium or interest has become due and payable will be repaid to us, and the holder of the debt security thereafter may look only to us for payment thereof.

Governing Law

The indenture and the debt securities, and any claim, controversy or dispute arising under or related to the indenture or the debt securities, will be governed by and construed in accordance with the laws of the State of New York, except to the extent that the Trust Indenture Act is applicable.

DESCRIPTION OF WARRANTS

The following description, together with the additional information we may include in the applicable prospectus supplements and free writing prospectuses we have authorized for use in connection with a specific offering, summarizes the material terms and provisions of the warrants that we may offer under this prospectus, which may consist of warrants to purchase common stock, preferred stock or debt securities and may be issued in one or more series.

Warrants may be issued independently or together with common stock, preferred stock or debt securities offered by any prospectus supplement, and may be attached to or separate from those securities. While the terms we have summarized below will apply generally to any warrants that we may offer under this prospectus, we will describe the particular terms of any series of warrants that we may offer in more detail in the applicable prospectus supplement and any applicable free writing prospectus we authorize for use in connection with the specific offering. The terms of any warrants offered under a prospectus supplement may differ from the terms described below.

We will file as exhibits to the registration statement of which this prospectus is a part, or will incorporate by reference from reports that we file with the SEC, the form of warrant agreement, if any, including a form of warrant certificate, that describes the terms of the particular series of warrants we are offering. The following summaries of material provisions of the warrants and the warrant agreements are subject to, and qualified in their entirety by reference to, all the provisions of the warrant agreement and warrant certificate applicable to the particular series of warrants that we may offer under this prospectus. We urge you to read the applicable prospectus supplements related to the particular series of warrants that we may offer under this prospectus, as well as any related free writing prospectuses we have authorized for use in connection with a specific offering, and the complete warrant agreements and warrant certificates that contain the terms of the warrants.

General Matters

We will describe in the applicable prospectus supplement the terms relating to a series of warrants being offered, including:

- the title of such securities;
- the offering price or prices and aggregate number of warrants offered;
- the currency or currencies for which the warrants may be purchased;
- if applicable, the designation and terms of the securities with which the warrants are issued and the number of warrants issued with each such security or each principal amount of such security;
- if applicable, the date on and after which the warrants and the related securities will be separately transferable;
- if applicable, the minimum or maximum amount of such warrants which may be exercised at any one time;
- in the case of warrants to purchase debt securities, the principal amount of debt securities purchasable upon exercise of one warrant and the price at which, and currency in which, this principal amount of debt securities may be purchased upon such exercise;
- in the case of warrants to purchase common stock or preferred stock, the number of shares of common stock or preferred stock, as the case may be, purchasable upon the exercise of one warrant and the price at which, and the currency in which, these shares may be purchased upon such exercise;
- the effect of any merger, consolidation, sale or other disposition of our business on the warrant agreements and the warrants;
- the terms of any rights to redeem or call the warrants;
- the terms of any rights to force the exercise of the warrants;
- any provisions for changes to or adjustments in the exercise price or number of securities issuable upon exercise of the warrants;
- the dates on which the right to exercise the warrants will commence and expire;
- the manner in which the warrant agreements and warrants may be modified;
- a discussion of any material or special United States federal income tax consequences of holding or exercising the warrants;
- the terms of the securities issuable upon exercise of the warrants; and
- any other specific terms, preferences, rights or limitations of or restrictions on the warrants.

Before exercising their warrants, holders of warrants will not have any of the rights of holders of the securities purchasable upon such exercise, including:

- in the case of warrants to purchase debt securities, the right to receive payments of principal of, or premium, if any, or interest on, the debt securities purchasable upon exercise or to enforce covenants in the applicable indenture; or
- in the case of warrants to purchase common stock or preferred stock, the right to receive dividends, if any, or, payments upon our liquidation, dissolution or winding up or to exercise voting rights, if any.

Exercise of Warrants

Each warrant will entitle the holder to purchase the securities that we specify in the applicable prospectus supplement at the exercise price that we describe in the applicable prospectus supplement. Unless we otherwise specify in the applicable prospectus supplement, holders of the warrants may exercise the warrants at any time up to the specified time on the expiration date that we set forth in the applicable prospectus supplement. After the close of business on the expiration date, unexercised warrants will become void.

Unless we otherwise specify in the applicable prospectus supplement, holders of the warrants may exercise the warrants by delivering the warrant certificate representing the warrants to be exercised together with specified information, and paying the required amount to the warrant agent in immediately available funds, as provided in the applicable prospectus supplement. We will set forth on the reverse side of the warrant certificate and in the applicable prospectus supplement the information that the holder of the warrant will be required to deliver to the warrant agent in connection with the exercise of the warrant.

Upon receipt of the required payment and the warrant certificate properly completed and duly executed at the corporate trust office of the warrant agent or any other office indicated in the applicable prospectus supplement, we will issue and deliver the securities purchasable upon such exercise. If fewer than all of the warrants represented by the warrant certificate are exercised, then we will issue a new warrant certificate for the remaining amount of warrants. If we so indicate in the applicable prospectus supplement, holders of the warrants may surrender securities as all or part of the exercise price for warrants.

Governing Law

Unless we provide otherwise in the applicable prospectus supplement, the warrants and warrant agreements, and any claim, controversy or dispute arising under or related to the warrants or warrant agreements, will be governed by and construed in accordance with the laws of the State of New York.

Enforceability of Rights By Holders of Warrants

Each warrant agent will act solely as our agent under the applicable warrant agreement and will not assume any obligation or relationship of agency or trust with any holder of any warrant. A single bank or trust company may act as warrant agent for more than one issue of warrants. A warrant agent will have no duty or responsibility in case of any default by us under the applicable warrant agreement or warrant, including any duty or responsibility to initiate any proceedings at law or otherwise, or to make any

demand upon us. Any holder of a warrant may, without the consent of the related warrant agent or the holder of any other warrant, enforce by appropriate legal action its right to exercise, and receive the securities purchasable upon exercise of, its warrants.

Warrant Agreement Will Not Be Qualified Under Trust Indenture Act

No warrant agreement will be qualified as an indenture, and no warrant agent will be required to qualify as a trustee, under the Trust Indenture Act. Therefore, holders of warrants issued under a warrant agreement will not have the protection of the Trust Indenture Act with respect to their warrants.

Calculation Agent

Calculations relating to warrants may be made by a calculation agent, an institution that we appoint as our agent for this purpose. The prospectus supplement for a particular warrant will name the institution that we have appointed to act as the calculation agent for that warrant as of the original issue date for that warrant. We may appoint a different institution to serve as calculation agent from time to time after the original issue date without the consent or notification of the holders.

The calculation agent's determination of any amount of money payable or securities deliverable with respect to a warrant will be final and binding in the absence of manifest error.

DESCRIPTION OF UNITS

We may issue units consisting of any combination of the other types of securities offered under this prospectus in one or more series. We may evidence each series of units by unit certificates that we will issue under a separate agreement. We may enter into unit agreements with a unit agent. Each unit agent will be a bank or trust company that we select. We will indicate the name and address of the unit agent in the applicable prospectus supplement relating to a particular series of units.

The following description, together with the additional information included in the applicable prospectus supplement, summarizes the general features of the units that we may offer under this prospectus. You should read any prospectus supplement and any free writing prospectus we authorize for use in connection with a specific offering of units, as well as the complete unit agreements that contain the terms of the units. Specific unit agreements will contain additional important terms and provisions and we will file as an exhibit to the registration statement of which this prospectus is a part, or will incorporate by reference from another report that we file with the SEC, the form of each unit agreement relating to units offered under this prospectus.

If we offer any units, certain terms of that series of units will be described in the applicable prospectus supplement, including, without limitation, the following, as applicable:

- the title of the series of units;
- identification and description of the separate constituent securities comprising the units;
- the price or prices at which the units will be issued;
- the date, if any, on and after which the constituent securities comprising the units will be separately transferable;
- a discussion of certain U.S. federal income tax considerations applicable to the units; and
- any other terms of the units and their constituent securities.

LEGAL OWNERSHIP OF SECURITIES

We can issue securities in registered form or in the form of one or more global securities. We describe global securities in greater detail below. We refer to those persons who have securities registered in their own names on the books that we or any applicable trustee or depositary maintain for this purpose as the "holders" of those securities. These persons are the legal holders of the securities. We refer to those persons who, indirectly through others, own beneficial interests in securities that are not registered in their own names, as "indirect holders" of those securities. As we discuss below, indirect holders are not legal holders, and investors in securities issued in book-entry form or in street name will be indirect holders.

Book-Entry Holders

We may issue securities in book-entry form only, as we will specify in the applicable prospectus supplement. This means securities may be represented by one or more global securities registered in the name of a financial institution that holds them as depositary on behalf of other financial institutions that participate in the depositary's book-entry system. These participating institutions, which are referred to as participants, in turn, hold beneficial interests in the securities on behalf of themselves or their customers.

Only the person in whose name a security is registered is recognized as the holder of that security. Global securities will be registered in the name of the depositary or its participants. Consequently, for global securities, we will recognize only the depositary as the holder of the securities, and we will make all payments on the securities to the depositary. The depositary passes along the payments it receives to its participants, which in turn pass the payments along to their customers who are the beneficial owners. The depositary and its participants do so under agreements they have made with one another or with their customers; they are not obligated to do so under the terms of the securities.

As a result, investors in a global security will not own securities directly. Instead, they will own beneficial interests in a global security, through a bank, broker or other financial institution that participates in the depositary's book-entry system or holds an interest through a participant. As long as the securities are issued in global form, investors will be indirect holders, and not legal holders, of the securities.

Street Name Holders

We may terminate a global security in certain situations, as described under "—Special Situations When a Global Security Will Be Terminated," or issue securities that are not issued in global form. In these cases, investors may choose to hold their securities in their own names or in "street name." Securities held by an investor in street name would be registered in the name of a bank, broker or other financial institution that the investor chooses, and the investor would hold only a beneficial interest in those securities through an account he or she maintains at that institution.

For securities held in street name, we or any applicable trustee or depositary will recognize only the intermediary banks, brokers and other financial institutions in whose names the securities are registered as the holders of those securities, and we or any such trustee or depositary will make all payments on those securities to them. These institutions pass along the payments they receive to their customers who are the beneficial owners, but only because they agree to do so in their customer agreements or because they are legally required to do so. Investors who hold securities in street name will be indirect holders, not holders, of those securities.

Legal Holders

Our obligations, as well as the obligations of any applicable trustee or third party employed by us or a trustee, run only to the legal holders of the securities. We do not have obligations to investors who hold beneficial interests in global securities, in street name or by any other indirect means. This will be the case whether an investor chooses to be an indirect holder of a security or has no choice because we are issuing the securities only in global form.

For example, once we make a payment or give a notice to the legal holder, we have no further responsibility for the payment or notice even if that legal holder is required, under agreements with its participants or customers or by law, to pass the payment or notice along to the indirect holders but does not do so. Similarly, we may want to obtain the approval of the holders to amend an indenture, to relieve us of the consequences of a default or of our obligation to comply with a particular provision of an indenture, or for other purposes. In such an event, we would seek approval only from the legal holders, and not the indirect holders, of the securities. Whether and how the legal holders contact the indirect holders is up to the legal holders.

Special Considerations for Indirect Holders

If you hold securities through a bank, broker or other financial institution, either in book-entry form because the securities are represented by one or more global securities or in street name, you should check with your own institution to find out:

- how it handles securities payments and notices;
- whether it imposes fees or charges;
- how it would handle a request for the holders' consent, if ever required;
- whether and how you can instruct it to send you securities registered in your own name so you can be a holder, if that is permitted in the future;
- how it would exercise rights under the securities if there were a default or other event triggering the need for holders to act to protect their interests; and
- if the securities are in book-entry form, how the depositary's rules and procedures will affect these matters.

Global Securities

A global security is a security that represents one or any other number of individual securities held by a depositary. Generally, all securities represented by the same global securities will have the same terms.

Each security issued in book-entry form will be represented by a global security that we issue to, deposit with and register in the name of a financial institution or its nominee that we select. The financial institution that we select for this purpose is called the depositary. Unless we specify otherwise in the applicable prospectus supplement, The Depository Trust Company, New York, New York, known as DTC, will be the depositary for all securities issued in book-entry form.

A global security may not be transferred to or registered in the name of anyone other than the depositary, its nominee or a successor depositary, unless special termination situations arise. We describe those situations below under "—Special Situations When a Global Security Will Be Terminated." As a result of these arrangements, the depositary, or its nominee, will be the sole registered owner and legal holder of all securities represented by a global security, and investors will be permitted to own only beneficial interests in a global security. Beneficial interests must be held by means of an account with a broker, bank or other financial institution that in turn has an account with the depositary or with another institution that does. Thus, an investor whose security is represented by a global security will not be a legal holder of the security, but only an indirect holder of a beneficial interest in the global security.

If the prospectus supplement for a particular security indicates that the security will be issued as a global security, then the security will be represented by a global security at all times unless and until the global security is terminated. If termination occurs, we may issue the securities through another book-entry clearing system or decide that the securities may no longer be held through any book-entry clearing system.

Special Considerations for Global Securities

As an indirect holder, an investor's rights relating to a global security will be governed by the account rules of the investor's financial institution and of the depositary, as well as general laws relating to securities transfers. We do not recognize an indirect holder as a holder of securities and instead deal only with the depositary that holds the global security.

If securities are issued only as global securities, an investor should be aware of the following:

- an investor cannot cause the securities to be registered in his or her name, and cannot obtain non-global certificates for his or her interest in the securities, except in the special situations described below;
- an investor will be an indirect holder and must look to his or her own bank or broker for payments on the securities and protection of his or her legal rights relating to the securities, as described above;
- an investor may not be able to sell interests in the securities to some insurance companies and to other institutions that are required by law to own their securities in non-book-entry form;
- an investor may not be able to pledge his or her interest in the global security in circumstances where certificates representing the securities must be delivered to the lender or other beneficiary of the pledge in order for the pledge to be effective;
- the depositary's policies, which may change from time to time, will govern payments, transfers, exchanges and other matters relating to an investor's interest in the global security;

- we and any applicable trustee have no responsibility for any aspect of the depositary's actions or for its records of ownership interests in the global security, nor will we or any applicable trustee supervise the depositary in any way;

- the depositary may, and we understand that DTC will, require that those who purchase and sell interests in the global security within its book-entry system use immediately available funds, and your broker or bank may require you to do the same; and

- financial institutions that participate in the depositary's book-entry system, and through which an investor holds its interest in the global security, may also have their own policies affecting payments, notices and other matters relating to the securities.

There may be more than one financial intermediary in the chain of ownership for an investor. We do not monitor and are not responsible for the actions of any of those intermediaries.

Special Situations When a Global Security Will Be Terminated

In a few special situations described below, a global security will terminate and interests in it will be exchanged for physical certificates representing those interests. After that exchange, the choice of whether to hold securities directly or in street name will be up to the investor. Investors must consult their own banks or brokers to find out how to have their interests in securities transferred to their own names, so that they will be direct holders. The rights of holders and street name investors are described above.

A global security will terminate when the following special situations occur:

- if the depositary notifies us that it is unwilling, unable or no longer qualified to continue as depositary for that global security and we do not appoint another institution to act as depositary within 90 days;

- if we notify any applicable trustee that we wish to terminate that global security; or

- if an event of default has occurred with regard to securities represented by that global security and has not been cured or waived.

The applicable prospectus supplement may also list additional situations for terminating a global security that would apply only to the particular series of securities covered by the prospectus supplement. When a global security terminates, the depositary, and neither we nor any applicable trustee, is responsible for deciding the names of the institutions that will be the initial direct holders.

PLAN OF DISTRIBUTION

We may sell the securities from time to time pursuant to underwritten public offerings, direct sales to the public, "at the market" offerings, negotiated transactions, block trades or a combination of these methods. We may sell the securities to or through underwriters or dealers, through agents, or directly to one or more purchasers. We may distribute securities from time to time in one or more transactions:

- at a fixed price or prices, which may be changed;

- at market prices prevailing at the time of sale;

- at prices related to such prevailing market prices; or

- at negotiated prices.

A prospectus supplement or supplements (and any related free writing prospectus that we may have authorized for use in connection with a specific offering) will describe the terms of the offering of the securities, including, to the extent applicable:

- the name or names of the underwriters, if any;

- the purchase price of the securities or other consideration therefor, and the proceeds, if any, we will receive from the sale;

- any over-allotment options under which underwriters may purchase additional securities from us;

- any agency fees or underwriting discounts and other items constituting agents' or underwriters' compensation;

- any public offering price;

- any discounts or concessions allowed or re-allowed or paid to dealers; and

- any securities exchange or market on which the securities may be listed.

Only underwriters named in the prospectus supplement will be underwriters of the securities offered by the prospectus supplement.

If underwriters are used in the sale, they will acquire the securities for their own account and may resell the securities from time to time in one or more transactions at a fixed public offering price or at varying prices determined at the time of sale. The obligations of the underwriters to purchase the securities will be subject to the conditions set forth in the applicable underwriting agreement. We may offer the securities to the public through underwriting syndicates represented by managing underwriters or by underwriters without a syndicate. Subject to certain conditions, the underwriters will be obligated to purchase all of the securities offered by the prospectus supplement, other than securities covered by any over-allotment option. Any public offering price and any discounts or concessions allowed or re-allowed or paid to dealers may change from time to time. We may use underwriters with whom we have a material relationship. We will describe in the prospectus supplement, naming the underwriter, the nature of any such relationship.

We may sell securities directly or through agents we designate from time to time. We will name any agent involved in the offering and sale of securities and we will describe any commissions we will pay the agent in the prospectus supplement. Unless the prospectus supplement states otherwise, our agent will act on a best-efforts basis for the period of its appointment.

We may authorize agents or underwriters to solicit offers by certain types of institutional investors to purchase securities from us at the public offering price set forth in the prospectus supplement pursuant to delayed delivery contracts providing for payment and delivery on a specified date in the future. We will describe the conditions to these contracts and the commissions we must pay for solicitation of these contracts in the prospectus supplement.

We may provide agents and underwriters with indemnification against civil liabilities, including liabilities under the Securities Act, or contribution with respect to payments that the agents or underwriters may make with respect to these liabilities. Agents and underwriters may engage in transactions with, or perform services for, us in the ordinary course of business.

All securities we may offer, other than common stock, will be new issues of securities with no established trading market. Any underwriters may make a market in these securities, but will not be obligated to do so and may discontinue any market making at any time without notice. We cannot guarantee the liquidity of the trading markets for any securities.

Any underwriter may engage in over-allotment, stabilizing transactions, short-covering transactions and penalty bids in accordance with Regulation M under the Exchange Act. Over-allotment involves sales in excess of the offering size, which create a short position. Stabilizing transactions permit bids to purchase the underlying security so long as the stabilizing bids do not exceed a specified maximum price. Syndicate-covering or other short-covering transactions involve purchases of the securities, either through exercise of the over-allotment option or in the open market after the distribution is completed, to cover short positions. Penalty bids permit the underwriters to reclaim a selling concession from a dealer when the securities originally sold by the dealer are purchased in a stabilizing or covering transaction to cover short positions. Those activities may cause the price of the securities to be higher than it would otherwise be. If commenced, the underwriters may discontinue any of the activities at any time.

Any underwriters that are qualified market makers on the Nasdaq Capital Market may engage in passive market making transactions in the common stock on the Nasdaq Capital Market in accordance with Regulation M under the Exchange Act, during the business day prior to the pricing of the offering, before the commencement of offers or sales of the common stock. Passive market makers must comply with applicable volume and price limitations and must be identified as passive market makers. In general, a passive market maker must display its bid at a price not in excess of the highest independent bid for such security; if all independent bids are lowered below the passive market maker's bid, however, the passive market maker's bid must then be lowered when certain purchase limits are exceeded. Passive market making may stabilize the market price of the securities at a level above that which might otherwise prevail in the open market and, if commenced, may be discontinued at any time.

In compliance with guidelines of the Financial Industry Regulatory Authority ("FINRA"), the maximum consideration or discount to be received by any FINRA member or independent broker dealer may not exceed 8% of the aggregate amount of the securities offered pursuant to this prospectus and the applicable prospectus supplement.

LEGAL MATTERS

Unless otherwise indicated in the applicable prospectus supplement, the validity of the securities offered by this prospectus, and any supplement thereto, will be passed upon for us by Paul Hastings LLP, Palo Alto, California.

EXPERTS

The consolidated financial statements of NeuBase as of September 30, 2020 and 2019, and for each of the two years in the period ended September 30, 2020 incorporated by reference in this prospectus have been so incorporated in reliance on the report of Marcum LLP, independent registered public accounting firm, upon the authority of said firm as experts in auditing and accounting.

WHERE YOU CAN FIND MORE INFORMATION

We are a reporting company and file annual, quarterly and current reports, proxy statements and other information with the SEC. We have filed with the SEC a registration statement on Form S-3 under the Securities Act with respect to the securities being offered under this prospectus. This prospectus does not contain all of the information set forth in the registration statement and the exhibits to the registration statement. For further information with respect to us and the securities being offered under this prospectus, we refer you to the registration statement and the exhibits and schedules filed as a part of the registration statement. The SEC maintains an Internet site that contains reports, proxy and information statements, and other information regarding issuers that file electronically with the SEC, including NeuBase Therapeutics, Inc. The SEC's Internet site can be found at *http://www.sec.gov*.

INCORPORATION OF DOCUMENTS BY REFERENCE

The SEC allows us to "incorporate by reference" information into this prospectus, which means that we can disclose important information to you by referring you to another document filed separately with the SEC. The documents incorporated by reference into this prospectus contain important information that you should read about us.

The following documents are incorporated by reference into this prospectus:

a) The Registrant's Annual Report on Form 10-K for the fiscal year ended September 30, 2020, filed with the SEC on December 23, 2020;

b) The Registrant's Quarterly Report on Form 10-Q for the quarter ended December 31, 2020, filed with the SEC on February 11, 2021;

c) The Registrant's Current Reports on Form 8-K filed with the SEC on (i) October 6, 2020, (ii) December 2, 2020, (iii) December 16, 2020 (other than with respect to information furnished under Item 7.01 therein) and (iv) March 26, 2021; and

d) The description of the Registrant's common stock set forth in Exhibit 4.5 the Registrant's Annual Report on Form 10-K for the year ended September 30, 2019 (File No. 001-35963), filed with the SEC on January 10, 2020, including any amendments or reports filed for the purpose of updating such description.

We also incorporate by reference any future filings (other than current reports furnished under Item 2.02 or Item 7.01 of Form 8-K and exhibits filed on such form that are related to such items unless such Form 8-K expressly provides to the contrary) made with the SEC pursuant to Sections 13(a), 13(c), 14 or 15(d) of the Exchange Act, including those made after the date of the initial filing of the registration statement of which this prospectus is a part and prior to effectiveness of such registration statement, until we file a post-effective amendment that indicates the termination of the offering of the securities made by this prospectus and such future filings and will become a part of this prospectus from the respective dates that such documents are filed with the SEC. Any statement contained herein or in a document incorporated or deemed to be incorporated by reference herein shall be deemed to be modified or superseded for purposes hereof or of the related prospectus supplement to the extent that a statement contained herein or in any other subsequently filed document which is also incorporated or deemed to be incorporated by reference herein modifies or supersedes such statement. Any such statement so modified or superseded shall not be deemed, except as so modified or superseded, to constitute a part of this prospectus.

Documents incorporated by reference are available from us, without charge. You may obtain documents incorporated by reference in this prospectus by requesting them in writing or by telephone at the following address:

NeuBase Therapeutics, Inc.
350 Technology Drive
Pittsburgh, PA 15219
Phone: (646) 450-1790

Up to $50,000,000
Shares of Common Stock

PROSPECTUS SUPPLEMENT

Jefferies

August 27, 2021