Risk Factors Comparison 2025-03-13 to 2024-02-27 Form: 10-K

Legend: **New Text** ~~Removed Text~~ Unchanged Text Moved Text Section

You should carefully consider the risk factors set forth below as well as the other information contained in this Annual Report on Form 10- K and in our other public filings in evaluating our business. Any of the following risks could materially and adversely affect our business, financial condition or results of operations. In addition, these risks could cause actual results and developments to differ materially and adversely from those projected in the forward- looking statements contained in this Annual Report on Form 10- K (please read the Information Regarding Forward- Looking Statements appearing at the beginning of this Form 10- K). Additional risks and uncertainties not currently known to us or that we currently view to be immaterial may also **have a** ~~materially~~ -- **material** ~~adversely~~ -- **adverse** ~~affect~~ **effect on** our business, financial condition or results of operations. In these circumstances, the market price of our common stock would likely decline and you could lose all or part of your investment. Risk Factor Summary Risks Related to Our NAV Technology Platform and the Development of Our Product Candidates • It is difficult to predict the time and cost of development and of obtaining regulatory approval for our product candidates. • Our business depends substantially on the success of our lead product candidates. • We have limited clinical results for ~~most~~ **some** of our product candidates. • Regulatory authorities may not consider the endpoints of our clinical trials to provide clinically meaningful results. • The results from our preclinical studies or clinical trials for our product candidates may not support as broad a marketing approval as we seek, and we may be required to conduct additional clinical trials or evaluate subjects for a follow- up period. • We may encounter substantial delays in our planned clinical trials, or we may fail to demonstrate safety and efficacy to the satisfaction of applicable regulatory authorities. • We may be negatively impacted if the results of our planned clinical trials are inconclusive or if there are safety concerns or serious adverse events associated with our product candidates. • Undesirable side effects may delay or prevent our product candidates and those of our licensees or collaborators from obtaining regulatory approval, limit their commercial potential or result in significant negative consequences following approval. • We cannot predict when, or if, we will obtain regulatory approval to commercialize a product candidate. Risks Related to Our Financial Position • We face significant competition and there is a possibility that our competitors may achieve regulatory approval before us or develop products that are safer, less expensive or more convenient or effective than ours. • We expect to ~~normally~~ **regularly** incur losses for the foreseeable future and may never again achieve or maintain profitability. • Failure to obtain additional funding when needed may force us to delay, limit or terminate certain of our licensing activities, product development efforts or other operations. • We have never generated revenue from sales of our product candidates and may never do so in the future. Risks Related to Third Parties • If third parties do not meet our deadlines, our preclinical and clinical development programs could be delayed or unsuccessful. • If our licensing arrangements or collaborations are not successful, our business could be harmed. **Risks Related to Manufacturing** • Products intended for use in gene therapies are novel,complex and difficult to manufacture.• Delays in obtaining regulatory approval of our manufacturing process or disruptions in our manufacturing process may delay or disrupt our commercialization efforts.• Third parties we rely upon to conduct our product manufacturing may not perform satisfactorily.• We are required to comply with ongoing manufacturing regulatory requirements.Risks Related to the Commercialization of Our Product Candidates • If we are unable to establish sales and marketing capabilities or enter into agreements with third parties to market and sell our product candidates,if approved,we may be unable to generate any product revenue.• We may not achieve our projected **timelines that** ~~development goals in the timeframes~~ we ~~announce~~ **announced** ~~and expect~~ .• Even if we receive regulatory approval,we still may not be able to successfully commercialize our product candidates.• Failure to obtain or maintain adequate insurance coverage and reimbursement for our products,if approved, **or the imposition of price controls or other forms of pricing regulation** could limit our ability to market those products and decrease our ability to generate product revenue ~~.• Government price controls could restrict the amount that we are able to charge for any of our products,if approved~~ .Risks Related to Our Business Operations • We may not be successful in our efforts to identify or discover additional product candidates.• ~~We may not successfully execute or achieve the expected benefits of our strategic pipeline prioritization and restructuring plan or other cost-saving measures that we may take in the future.•~~ Our future success depends on our ability to retain key employees,consultants and advisors and to attract qualified personnel.• We may face liability for our conduct and that of our employees,principal investigators,consultants or commercial partners.• We may face product liability lawsuits.• We could become subject to fines or penalties related to the failure to comply with environmental,health and safety laws.• We and our collaborators or other contractors or consultants may suffer cybersecurity breaches.• Our customers are concentrated and therefore the loss of a significant customer may harm our business.Risks Related **to Our Intellectual Property • Our intellectual property rights may be limited by the terms and conditions of licenses granted to us by others.• We must obtain and maintain patent protection for our products and technology to protect our intellectual property rights.** • Our reliance on third parties requires us to share our trade secrets, which increases the possibility that a competitor will discover them or that our trade secrets will be misappropriated or disclosed Risks Related to Manufacturing • Products..... technology to protect our intellectual property rights . • Our intellectual property licenses with third parties may be subject to disagreements. • We are required to comply with the agreements under which we license intellectual property rights from third parties. • We may not be successful in obtaining necessary rights to our product candidates through acquisitions and in- licenses. • We may not be able to protect our intellectual property rights in the United States and throughout the world. • Issued patents covering our NAV Technology Platform or our product candidates could be found invalid or unenforceable. • Third parties may initiate legal proceedings alleging that we are infringing their intellectual property rights. • We may be subject to intellectual property claims.

• Changes in U. S. patent law could diminish the value of patents in general, thereby impairing our ability to protect our products. • We may be unable to obtain patent term extension and data exclusivity for our product candidates. Risks Related to Ownership of Our Common Stock • Our operating results are difficult to predict and could cause the price of our common stock to fluctuate substantially. • Raising additional capital may cause dilution to our existing stockholders, restrict our operations or require us to relinquish proprietary rights. • ~~Future~~ **Strategic partnerships and any other arrangements or** acquisitions ~~or strategic partnerships~~ **that we effect in the future** may increase our capital requirements, dilute our stockholders, cause us to incur debt or assume contingent liabilities and subject us to other risks. • Provisions in our certificate of incorporation and bylaws might discourage, delay or prevent a change in control. • Our certificate of incorporation includes exclusive forum clauses for certain litigation. • Our business could be negatively affected as a result of the actions of activist stockholders. Risks Related to our NAV Technology Platform and the Development of Our Product Candidates Our gene therapy product candidates are based on a novel technology that makes it difficult to predict the time and cost of development and of subsequently obtaining regulatory approval. Only a few gene therapy products have been approved in the United States, the European Union or elsewhere. We have concentrated our research and development efforts on our proprietary AAV gene delivery platform (our NAV Technology Platform), and we have granted licenses to certain intellectual property related to our NAV Technology Platform to our NAV Technology Licensees. Our future success depends on our and our NAV Technology Licensees' successful development and commercialization of viable gene therapy product candidates. There can be no assurance that we or our NAV Technology Licensees will not experience problems or delays in developing current or future product candidates or that such problems or delays will not cause unanticipated costs, or that any such development problems can be solved. We also may experience unanticipated problems or delays in expanding our manufacturing capacity, and this may prevent us from completing our clinical trials, meeting the obligations of our collaborations or commercializing our products on a timely or profitable basis, if at all. For example, we, a partner or another group may uncover one or more previously unknown risks associated with AAV or our NAV Technology Platform, and this may prolong the period of observation required for obtaining regulatory approval, necessitate additional clinical testing or invalidate our NAV Technology. In addition, the clinical trial requirements of the FDA, the EMA and other regulatory authorities and the criteria these regulators use to determine the quality, safety and efficacy of a product candidate vary substantially according to the type, complexity, novelty and intended use and market of such product candidates. The regulatory approval process for novel product candidates such as ours can be significantly more expensive and take longer than for other, better known or more extensively studied product candidates. Only a few gene therapy products have been approved in the United States, the European Union or elsewhere. It is difficult to determine how long it will take or how much it will cost to obtain regulatory approvals for our product candidates in the United States, the European Union or elsewhere, or how long it will take to commercialize our product candidates, if approved. Furthermore, approvals by one regulatory authority may not be indicative of what other regulatory authorities may require for approval, and approvals of ex vivo gene therapy products may not be indicative of what may be required for approval of in vivo gene therapy products. Regulatory requirements governing gene and cell therapy products have changed frequently and may continue to change in the future. Additionally, we may seek regulatory approval in territories outside the United States and the European Union, which may have their own regulatory authorities along with frequently changing requirements or guidelines. The regulatory review committees and advisory groups in the United States, the European Union and elsewhere, and any new guidelines they promulgate, may lengthen the regulatory review process, require us to perform additional studies, increase our development costs, lead to changes in regulatory positions and interpretations, delay or prevent approval and commercialization of 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 and advisory groups, and comply with applicable guidelines. If we fail to do so, we may be required to delay or discontinue development of certain of our product candidates. These additional processes may result in a review and approval process that is longer than we otherwise would have expected. Delay or failure to obtain, or unexpected costs in obtaining, the regulatory approval necessary to bring a potential product to market could decrease our ability to generate product revenue, and our business, financial condition, results of operations and prospects would be materially harmed. Our business depends substantially on the success of our lead product candidates. If we are unable to obtain regulatory approval for, or successfully commercialize, our lead product candidates, our business will be materially harmed. **Our** ~~Some of our lead product candidates are in the early stages of development and all of our~~ product candidates will require substantial clinical development and testing, manufacturing bridging studies and process validation and regulatory approval prior to commercialization. Successful continued development and ultimate regulatory approval of our lead product candidates is critical for our future business success and our ability to generate product revenue. We have invested, and will continue to invest, a significant portion of our financial resources in the development of our lead product candidates. We will need to raise sufficient funds for, and successfully complete, our clinical trials of our lead product candidates in appropriate subjects. The future regulatory and commercial success of these product candidates is subject to a number of risks, including the following: • we may not have sufficient financial and other resources or patient availability to complete the necessary clinical trials for our lead product candidates; • we may not be able to provide evidence of quality, efficacy and safety for our lead product candidates; • we do not know the degree to which our lead product candidates will be accepted by patients, the medical community and third-party payors as a therapy for the respective diseases to which they relate, even if approved; • the results of our clinical trials may not meet the level of statistical or clinical significance required by the FDA, EMA or comparable foreign regulatory bodies for marketing approval, and modifications to the design of our clinical trials could delay their enrollment, commencement or completion; • subjects in our clinical trials may die or suffer other adverse effects for reasons that may or may not be related to our lead product candidates; • subjects in clinical trials undertaken by our licensees or collaborators, or undertaken by others using AAV, may die or suffer other adverse effects for reasons that may or may not be related to our NAV Technology Platform or AAV; • certain patients' immune systems might prohibit the successful delivery of certain gene therapy products to the target

tissue, thereby limiting the treatment outcomes; • we may not successfully establish commercial manufacturing capabilities; • if approved for treatment of the expected conditions, our lead product candidates will likely compete with other treatments then available, including the off- label use of products already approved for marketing and other therapies currently available or which may be developed; • our products and products developed by our licensees and collaborators may not maintain a continued acceptable safety profile following regulatory approval; • we may not maintain compliance with post- approval regulation and other requirements; and • we may not be able to obtain, maintain or enforce our rights under our licensed patents and other intellectual property rights. Of the large number of biologics and drugs in development in the biopharmaceutical industry, only a small percentage result in the submission of a BLA to the FDA or marketing authorization application (MAA) to the EMA and even fewer are approved for commercialization. ~~Due to several of the risk factors identified in this Annual Report on Form 10- K, we may not achieve our goal to have multiple AAV vector- based gene therapies that are approved or in pivotal trials through our internal and partnered programs by the end of 2025.~~ Furthermore, even if we do receive regulatory approval to market our lead product candidates, any such approval may be subject to limitations on the indicated uses for which we may market the product. Accordingly, even if we are able to obtain the requisite financing to continue to fund our development programs, we cannot assure you that our lead product candidates will be successfully developed or commercialized. If we or any of our future development partners are unable to develop, ~~or~~ obtain regulatory approval for, or, if approved, successfully commercialize, our lead product candidates, we may not be able to generate sufficient revenue to continue our business. We have limited clinical results for ~~most~~ **some** of our product candidates and success in preclinical studies or early clinical trials may not be indicative of results obtained in later trials. Gene therapy development has inherent risks. ~~Most~~ **Some** of our lead product candidates have limited clinical and preclinical results and we may experience unexpected results in the future. We or any of our future development partners will be required to demonstrate through adequate and well- controlled clinical trials that our product candidates containing our proprietary vectors are safe and effective, with a favorable benefit- risk profile, for use in their target indications before we can seek regulatory approvals for their commercial sale. Drug development is a long, expensive and uncertain process, and delay or failure can occur at any stage of development, including after commencement of any of our clinical trials. The results of preclinical studies and early clinical trials are not always predictive of future results. Any product candidate we or any of our future development partners advance into clinical trials, including our lead product candidates, may not have favorable results in later clinical trials, if any, or receive regulatory approval. There is a high failure rate for drugs and biologic products proceeding through clinical trials. Data obtained from preclinical and clinical activities are subject to varying interpretations that may delay, limit or prevent regulatory approval. In addition, we may experience regulatory delays or rejections as a result of many factors, including due to changes in regulatory policy during the period of our product candidate development. Any such delays could materially harm our business, financial condition, results of operations and prospects. Because we are developing **certain** product candidates for the treatment of ~~certain~~ diseases in which there is little clinical experience and we are using new endpoints and methodologies, there is increased risk that the FDA, the EMA or other regulatory authorities may not consider the endpoints of our clinical trials to provide clinically meaningful results and that these results may be difficult to analyze. During the FDA review process, we will need to identify success criteria and endpoints such that the FDA will be able to ~~determine~~ **subsequently evaluate** the clinical efficacy and safety profile of our product candidates. As we are developing novel treatments for diseases in which there is little clinical experience with new endpoints and methodologies, there is heightened risk that the FDA, the EMA or other regulatory bodies may not consider the clinical trial endpoints **that we select** to provide clinically meaningful results (reflecting a tangible benefit to patients). In addition, the resulting clinical data and results may be difficult to analyze. Even if the FDA does find our success criteria to be sufficiently validated and clinically meaningful, we may not achieve the pre- specified endpoints to a degree of statistical significance. Further, even if we do achieve the pre- specified criteria, we may produce results that are unpredictable or inconsistent with the results of the non- primary endpoints or other relevant data. The FDA also weighs the benefits of a product against its risks, and the FDA may view the efficacy results in the context of safety as not being supportive of regulatory approval. The EMA and other regulatory authorities in the European Union and other countries may make similar comments with respect to these endpoints and data **, which may jeopardize or preclude our ability to obtain regulatory approvals in the European Union and other jurisdictions** . The results from our preclinical studies or clinical trials for our product candidates may not support as broad a marketing approval as we seek, and the FDA, the EMA or other regulatory authorities may require us to conduct additional clinical trials or evaluate subjects for an additional follow- up period. While we believe our product candidates should be applicable for the treatment of patients with certain conditions, the results from our preclinical and planned clinical trials may not support as broad of a marketing approval as we seek. Even if we obtain regulatory approval for our product candidates, we may be required by the FDA, the EMA or other regulatory bodies to conduct additional clinical trials to support approval of our product candidates for patients diagnosed with different mutations of the respective diseases to which our product candidates relate. This could result in significant cost increases and substantial delays in obtaining, or never obtaining, marketing approval for our product candidates to treat patients. The inability to market our product candidates to treat patients for the intended indications would materially harm our business, financial condition, results of operations and prospects. Before obtaining marketing approval from regulatory authorities for the sale of our product candidates, we must conduct extensive clinical trials to demonstrate ~~the~~ **their** safety and efficacy ~~of the product candidates~~ . Clinical testing is expensive, time- consuming and uncertain as to outcome. A failure of one or more clinical trials can occur at any stage of testing. Events that may prevent successful or timely commencement and completion of preclinical and clinical development include: • delays in reaching a consensus with regulatory authorities on trial design; • delays in reaching agreement on acceptable terms with prospective CROs and clinical trial sites; • delays in opening clinical trial sites or obtaining required institutional review board or independent Ethics Committee approval at each clinical trial site; • delays in recruiting and enrolling suitable subjects to participate in our clinical trials, due to factors such as the size of the trial or subject population, process for identifying subjects, design or

expansion of protocols, eligibility and exclusive criteria, perceived risks and benefits of the relevant product candidate or gene therapy generally, availability of competing therapies and trials, severity of the disease under investigation, need and length of time required to discontinue other potential therapies, availability of genetic testing, availability and proximity of trial sites for prospective subjects, ability to obtain subject consent and referral practices of physicians; • imposition of a clinical hold by regulatory authorities ~~, including as a result of a serious adverse event or after an inspection of our clinical trial operations or trial sites~~ ; • failure by us, any CROs we engage or any other third parties to adhere to clinical trial requirements; • failure to perform in accordance with GCP, or applicable regulatory guidelines in the European Union and other countries; • delays in the testing, validation, manufacturing and delivery of our product candidates to the clinical sites, including delays by third parties with whom we have contracted to perform; • delays in having subjects complete participation in a trial or return for post-treatment follow- up; • clinical trial sites or subjects dropping out of a trial; • selection of clinical endpoints that require prolonged periods of clinical observation or analysis of the resulting data; • occurrence of serious adverse events associated with the product candidate that are viewed to outweigh its potential benefits; • occurrence of serious adverse events in trials of the same class of agents conducted by other sponsors; or • changes in regulatory requirements and guidance that require amending or submitting new clinical protocols. Any inability to successfully complete research studies and preclinical and clinical development could result in additional costs to us or impair our ability to generate revenues from product sales, regulatory and commercialization milestones and royalties. In addition, if we make manufacturing or formulation changes to our product candidates, we may need to conduct additional studies to bridge our modified product candidates to earlier versions. Clinical trial delays also could shorten any periods during which we may have the exclusive right to commercialize our product candidates, if approved, or allow our competitors to bring products to market before we do, which could impair our ability to successfully commercialize our product candidates, if approved, and may harm our business, financial condition, results of operations and prospects. If the results of our planned clinical trials are inconclusive or if there are safety concerns or serious adverse events associated with our product candidates, we may: • **receive a clinical hold for a particular product candidate, which, if not lifted, could require that we discontinue development of a product candidate; • be required to conduct additional studies or clinical trials with respect to a product candidate or for a potential indication, which may result in additional significant expense and delays in seeking regulatory approval; •** be delayed in obtaining ~~marketing~~ **regulatory** approval for our product candidates, if at all; • obtain approval for indications or patient populations that are not as broad as intended or desired; • obtain approval with labeling that includes significant use or distribution restrictions or safety warnings; • be subject to changes in the way the product is administered; • be required to perform additional clinical trials to support approval or be subject to additional post- marketing testing or other requirements; • have regulatory authorities withdraw, vary or suspend their approval of the product or impose restrictions on its distribution in the form of a modified risk evaluation and mitigation; • be subject to the addition of labeling statements, such as warnings or contraindications; • be sued; or • experience damage to our reputation. Our NAV Technology Platform, our product candidates or our licensees' or collaborators' product candidates, and the process for administering such product candidates, may cause undesirable side effects or have other properties that could delay or prevent regulatory approval of product candidates, limit the commercial potential or result in significant negative consequences following any potential marketing approval. There have been several significant adverse side effects in gene therapy treatments in the past, including reported cases of leukemia in trials using lentivirus vectors and death seen in trials sponsored by other companies using adenovirus vectors and AAV vectors, including NAV vectors. Gene therapy is still a relatively new approach to disease treatment and additional adverse side effects could ~~develop~~ **be identified** . There also is the potential risk of delayed adverse events following exposure to gene therapy products due to persistent biologic activity of the genetic material or other components of products used to carry the genetic material. Possible adverse side effects that could occur with treatment with gene therapy products include an immunologic reaction early after administration which could substantially limit the effectiveness of the treatment. In previous clinical trials involving AAV vectors for gene therapy, some subjects experienced the development of a T- cell response, whereby after the vector is within the target cell, the cellular immune response system triggers the removal of transduced cells by activated T- cells. Furthermore, in clinical trials sponsored by other companies involving AAV vectors administered intravitreally for the treatment of retinal conditions, serious adverse reactions, such as panuveitis and loss of vision, have occurred. In addition to side effects caused by product candidates, the administration process or related procedures also can cause adverse side effects. If any such adverse events occur in our or third-party trials, our clinical trials could be suspended or terminated. As a result of these concerns, we may decide, or the FDA, the European Commission, the EMA or other regulatory authorities could order us, to halt, delay or amend preclinical development or clinical development of our product candidates or we may be unable to receive regulatory approval of our product candidates for any or all targeted indications. Even if we are able to demonstrate that all future serious adverse events are not product-related, such occurrences could affect patient recruitment or the ability of enrolled patients to complete the trial. Moreover, if we elect, or are required, to delay, suspend or terminate any clinical trial of any of our product candidates, the commercial prospects of such product candidates may be harmed and our ability to generate product revenues from any of these product candidates may be delayed or eliminated. Any of these occurrences may harm our ability to develop other product candidates and may harm our business, financial condition and prospects significantly. Additionally, if any of our product candidates receives marketing approval, the FDA could require us to adopt a **Risk Evaluation and Mitigation Strategy (** REMS **)** and other regulatory authorities could impose other specific obligations as a condition of approval to ensure that the benefits of our product candidates outweigh their risks, which could delay approval of our product candidates. A REMS may include, among other things, a medication guide outlining the risks of the product for distribution to patients; a communication plan to health care practitioners or patients; and elements to assure safe use, which can severely restrict the distribution of a product by, for example, requiring that health care providers receive particular training and obtain special certification prior to prescribing and dispensing the product, limiting the healthcare settings in which the product may be dispensed and subjecting patients to

monitoring and enrollment in a registry. If the FDA requires us to adopt a REMS for our products and we are unable to comply with its requirements, the FDA may deem our products to be misbranded and we may be subject to civil money penalties. The European Commission, the EMA and other regulatory authorities may, following grant of marketing authorization in their territory, impose similar obligations. Any of these events could prevent us from achieving or maintaining market acceptance of our NAV Technology Platform and our product candidates and could materially harm our business, prospects, financial condition and results of operations. Even if we complete the necessary preclinical studies and clinical trials, we cannot predict when, or if, we will obtain regulatory approval to commercialize a product candidate in the United States or elsewhere, and the approval may be for a narrower indication than we seek. We cannot commercialize a product candidate until the appropriate regulatory authorities have reviewed and approved the product candidate. Even if our product candidates meet their safety and efficacy endpoints in clinical trials, the regulatory authorities may not complete their review processes in a timely manner or we may not be able to obtain regulatory approval **. The ability of the FDA to review and approve new products can be affected by a variety of factors, including government budget and funding levels, ability to hire and retain key personnel and accept the payment of user fees, and statutory, regulatory and policy changes. Average review times at the FDA have fluctuated in recent years as a result. Disruptions at the FDA and other agencies may also slow the time necessary for new product candidates to be reviewed and approved by necessary government agencies, which could adversely affect our business. In addition, government funding of other government agencies on which our operations may rely, including those that fund research and development activities, is subject to the political process, which is inherently fluid and unpredictable** . Additional delays may result if an FDA Advisory Committee or other regulatory authority recommends non- approval or restrictions on approval. In addition, we may experience delays or rejections based on additional government regulation from future legislation or administrative action or based on changes in regulatory authority policy during the period of product development, clinical trials and the review process. Regulatory authorities also may approve a product candidate for more limited indications than requested or they may impose significant limitations in the form of narrow indications, warnings or a REMS. These regulatory authorities may require precautions or contra- indications with respect to conditions of use or they may grant approval subject to the performance of costly post- marketing clinical trials. In addition, regulatory authorities may not approve the labeling claims that are necessary or desirable for the successful commercialization of our product candidates. Any of the foregoing scenarios could materially harm the commercial prospects for our product candidates and materially harm our business, financial condition, results of operations and prospects. Further, the regulatory authorities may require concurrent approval or the CE mark (a mandatory conformity assessment marking for certain products sold within the European Economic Area (the EEA)) of a companion diagnostic device, since it may be necessary to use FDA- cleared or FDA- approved, or CE-marked, diagnostic tests or diagnostic tests approved by other comparable foreign regulatory authorities to diagnose patients or to assure the safe and effective use of our product candidates in trial subjects. FDA refers to such tests as in vitro companion diagnostic devices. The FDA has articulated a policy position that, when safe and effective use of a therapeutic product depends on a diagnostic device, the FDA generally will require approval or clearance of the companion diagnostic device at the same time that the FDA approves the therapeutic product. The FDA' s guidance allows for two exceptions to the general rule of concurrent drug / device approval, namely, when the therapeutic product is intended to treat serious and life- threatening conditions for which no alternative exists, and when a serious safety issue arises for an approved therapeutic agent, and no FDA-cleared or FDA- approved companion diagnostic test is yet available. It is unclear how the FDA will apply this policy to our current or future gene therapy product candidates. Should the FDA deem genetic tests used for diagnosing patients for our therapies to be in vitro companion diagnostics requiring FDA clearance or approval, we may face significant delays or obstacles in obtaining approval of a BLA for our product candidates. In the European Union, companion diagnostics are subject to the European Union Directive on in vitro diagnostic medical devices and its implementation in the European Union Member States. Recently revised European Union laws on in vitro diagnostics applied beginning in 2022, which provide for stricter requirements for in vitro diagnostic medical devices and impose additional obligations on manufacturers of in vitro diagnostic medical devices that may impact the development and authorization of our product candidates in the European Union. For example, the new regulation extends the requirement for performance assessment procedures and requires greater involvement of notified bodies in the development of in vitro diagnostic medical devices. This may result in additional regulatory and premarket requirements to market new in vitro diagnostic medical devices. Companies producing in vitro diagnostic medical devices will be required to have a responsible person to oversee regulatory compliance. In addition, the new regulation introduces risk classification of in vitro diagnostic medical devices and significantly increases the number of products that will be subject to stricter regulation. It also introduces the requirement to involve a notified body in the conformity assessment procedure. Approval of a product candidate in the United States by the FDA does not ensure approval of such product candidate by regulatory authorities in other countries or jurisdictions, and approval by one foreign regulatory authority does not ensure approval by regulatory authorities in other foreign countries or by the FDA. Sales of our product candidates outside of the United States will be subject to foreign regulatory requirements governing clinical trials and marketing approval. Even if the FDA grants marketing approval for a product candidate, comparable regulatory authorities of foreign countries also must approve the manufacturing and marketing of the product candidates in those countries. Approval procedures vary among jurisdictions and can involve requirements and administrative review periods different from, and more onerous than, those in the United States, including additional preclinical studies or clinical trials. In many countries outside the United States, a product candidate must be approved for reimbursement before it can be approved for sale in that country. In some cases, the price that we intend to charge for our products, if approved, is also subject to approval. We intend to submit a marketing authorization application to EMA for approval of our product candidates by the European Commission in the European Union. However, obtaining such approval from the European Commission following the opinion of EMA is a lengthy and expensive process. Additionally, the UK has its own separate approval procedures for our product candidates following the UK' s exit from the

European Union. Even if a product candidate is approved, the FDA or the European Commission, as the case may be, may limit the indications for which the product may be marketed, require extensive warnings on the product labeling or require expensive and time- consuming additional clinical trials or reporting as conditions of approval. Regulatory authorities in countries outside of the United States and the European Union also have requirements for approval of product candidates with which we must comply prior to marketing in those countries. Obtaining foreign regulatory approvals and compliance with foreign regulatory requirements could result in significant delays, difficulties and costs for us and could delay or prevent the introduction of our product candidates in certain countries. Further, clinical trials conducted in one country may not be accepted by regulatory authorities in other countries. Also, regulatory approval for any of our product candidates may be withdrawn. If we fail to comply with the regulatory requirements, our target market will be reduced and our ability to realize the full market potential of our product candidates will be harmed and our business, financial condition, results of operations and prospects will be harmed. We face significant competition in an environment of rapid technological change and there is a possibility that our competitors may achieve regulatory approval before us or develop products that are safer, less expensive or more convenient or effective than ours, which may harm our financial condition and our ability to successfully market or commercialize our product candidates. The biotechnology and pharmaceutical industries, including the gene therapy field, are characterized by rapidly changing technologies, significant competition and a strong emphasis on intellectual property. We face substantial competition from many different sources, including large and specialty pharmaceutical and biotechnology companies, academic research institutions, government agencies and public and private research institutions. We are aware of a number of companies focused on developing gene therapies in various indications, as well as a number of companies addressing other methods for modifying genes and regulating gene expression. Any advances in gene therapy technology made by a competitor may be used to develop therapies that could compete against any of our product candidates. Many of our potential competitors, alone or with their strategic partners, have substantially greater financial, technical and other resources, such as larger research and development, clinical, marketing and manufacturing organizations. Mergers and acquisitions in the biotechnology and pharmaceutical industries may result in even more resources being concentrated among a smaller number of competitors. Our commercial opportunity could be reduced or eliminated if competitors develop and commercialize products that are safer, more effective, have fewer or less severe side effects, are more convenient or are less expensive than any products that we may develop. Competitors also may obtain FDA or other regulatory approval for their products more rapidly or earlier than we may obtain approval for ours, **or have already obtained,** which could result in our competitors establishing a strong market position before we are able to enter the market. Additionally, technologies developed by our competitors may render our potential product candidates uneconomical or obsolete, and we may not be successful in marketing our product candidates against those of competitors. The availability of our competitors' products could limit the demand, and the price we are able to charge, for any products that we may develop and commercialize. Even though we have obtained orphan drug exclusivity for certain product candidates, that exclusivity may not effectively protect the product candidate from competition because the FDA may subsequently approve another drug for the same condition if the FDA concludes that the latter drug is not the same drug or is clinically superior in that it is shown to be safer, more effective or makes a major contribution to patient care. In the European Union, marketing authorization may be granted to a similar medicinal product for the same orphan indication if: • the second applicant can establish in its application that its medicinal product, although similar to the orphan medicinal product already authorized, is safer, more effective or otherwise clinically superior ; • the holder of the marketing authorization for the original orphan medicinal product consents to a second orphan medicinal product application ; or • the holder of the marketing authorization for the original orphan medicinal product cannot supply sufficient quantities of orphan medicinal product. We have incurred cumulative net losses and have had few profitable quarters since inception. We expect to regularly incur losses until we have successfully commercialized one or more product candidates and may never achieve or maintain profitability in the future. Since inception, we have incurred cumulative net losses. We have historically financed our operations primarily through private and public offerings of our equity securities, collaborations and licensing rights to our NAV Technology Platform, including milestone payments and royalties from our NAV Technology Licensees. We have devoted substantially all of our efforts to research and development, including preclinical and clinical development of our product candidates, and licensing our NAV Technology Platform, as well as to building out our team. We expect that it could be ~~several~~ years **before we commercialize most of our product candidates** . ~~if~~ **and we can provide no assurance that we will** ever **be able to do so** ~~, before we commercialize a product candidate~~ . We license certain intellectual property related to our NAV Technology Platform to our NAV Technology Licensees and collaborators. Our NAV Technology Licensees and collaborators have multiple preclinical studies and clinical trials in progress. However, only one gene therapy product based on our licensing program, Novartis AG' s Zolgensma, has been approved or commercialized. Other than revenue in connection with sales of Zolgensma, we may generate only limited recurring revenue in the near term from our current NAV Technology Licensees and collaborators. We expect to continue to incur significant expenses and regularly incur operating losses for the foreseeable future. The net losses we incur may fluctuate significantly from quarter to quarter. We anticipate that our expenses will increase substantially if, and as, we: • continue our research studies and preclinical and clinical development of our product candidates, including our lead product candidates; • initiate additional preclinical studies and clinical trials for our lead product candidates and future product candidates, if any; • initiate additional activities relating to manufacturing, including building out additional laboratory and manufacturing capacity; • seek to identify additional product candidates; • prepare our BLA and MAA for our lead product candidates and seek marketing approvals for any of our other product candidates that successfully complete clinical trials, if any; • further develop our NAV Technology Platform; • establish a sales, marketing and distribution infrastructure to commercialize any product candidates for which we may obtain marketing approval, if any; • maintain, expand and protect our intellectual property portfolio and enforce our intellectual property rights; and • acquire or in- license other product candidates and technologies. For us to become consistently profitable, we and our licensees and collaborators must develop and

commercialize product candidates with significant market potential. This will require us and our licensees and collaborators to be successful in a range of business challenges, including expansion of the licensing of our NAV Technology Platform, completing preclinical studies of product candidates, commencing and completing clinical trials of product candidates, obtaining marketing approval for these product candidates, manufacturing, marketing and selling those products for which we may obtain marketing approval and satisfying any post- marketing requirements. We may never succeed in any or all of these activities and, even if we do, we may never consistently generate revenues that are sufficient to achieve profitability, and we may not be able to sustain or increase profitability on a quarterly or annual basis. Our failure to become consistently profitable and remain profitable would decrease the value of our company and could impair our ability to raise capital, maintain our research and development efforts, expand our business or continue our operations. A decline in the value of our company also could cause you to lose all or part of your investment. We ~~may~~ **will** need to raise additional funding, which may not be available on acceptable terms, or at all. Failure to obtain this necessary capital when needed may force us to delay, limit or terminate certain of our licensing activities, product development efforts or other operations. We expect to require substantial future capital in order to complete research studies, preclinical and clinical development for our current product candidates and any future product candidates, and potentially commercialize these product candidates, if approved. We expect our spending levels to increase in connection with our preclinical and clinical trials of our product candidates. In addition, if we obtain marketing approval for any of our product candidates, we expect to incur significant expenses related to product sales, medical affairs, marketing, manufacturing and distribution. Accordingly, we will need to obtain substantial additional funding in connection with our continuing operations. If we are unable to raise capital when needed or on favorable terms, we could be forced to delay, reduce or eliminate certain of our licensing activities, our research and development programs or other operations. Our operations have consumed significant amounts of cash since inception. Our future capital requirements will depend on many factors, including: • the timing of enrollment, commencement and completion of our clinical trials; • the results of our clinical trials; • the results of our preclinical studies for our product candidates and any subsequent clinical trials; • the scope, progress, results and costs of drug discovery, laboratory testing, preclinical development and clinical trials for our product candidates; • the costs associated with building out additional laboratory and manufacturing capacity; • the costs, timing and outcome of regulatory review of our **product candidates; • the impact of any government- imposed tariffs on cost of goods and services, particularly related to partnered** product candidates; • the costs of future product sales, medical affairs, marketing, manufacturing and distribution activities for any of our product candidates for which we receive marketing approval; • revenue, if any, received from commercial sales of our products, should any of our product candidates receive marketing approval; • revenue received from commercial sales of Zolgensma and the timing and amount of Zolgensma royalties paid to HCR under our royalty purchase agreement; • revenue received from other commercial sales of our licensees' and collaborators' products, should any of their product candidates receive marketing approval, and other revenue received under our licensing agreements and collaborations; • the costs of preparing, filing and prosecuting patent applications, maintaining and enforcing our intellectual property rights and defending any intellectual property- related claims; • our current licensing agreements or collaborations remaining in effect, including the AbbVie Collaboration and License Agreement **relating to ABBV- RGX- 314 and the Nippon Shinyaku Collaboration and License Agreement relating to RGX- 121 and RGX- 111** , and our ability to timely achieve any milestones set forth in such agreements or collaborations; • our ability to establish and maintain additional licensing agreements or collaborations on favorable terms, if at all; and • the extent to which we acquire or in- license other product candidates and technologies. ~~Many of these factors are outside of our control. Identifying potential product candidates and conducting preclinical testing and clinical trials is a time- consuming, expensive and uncertain process that takes years to complete, and we may never generate the necessary data or results required to obtain regulatory and marketing approval and achieve product sales. In addition, our product candidates, if approved, may not achieve commercial success. Our product revenues, if any, and any commercial milestones or royalty payments under our licensing agreements, will be derived from or based on sales of products that may not be commercially available for many years, if at all. In addition, revenue from our NAV Technology Platform licensing is dependent in part on the clinical and commercial success of our licensing partners, including the commercialization of Zolgensma, and in part on maintaining our license agreements with our licensor partners, including GSK and Penn. Accordingly, we will need to continue to rely on additional financing to achieve our business objectives.~~ The issuance of additional securities, whether equity or debt, by us, including through our at- the- market program, or the possibility of such issuance, may cause the market price of our common stock to decline. Adequate additional financing may not be available to us on acceptable terms, or at all. We also could be required to seek funds through arrangements with partners or otherwise that may require us to relinquish rights to our intellectual property, our product candidates or otherwise agree to terms unfavorable to us. Although we have generated significant revenues from licensing our NAV Technology Platform and our other intellectual property, such as our licensing pursuant to the AbbVie **Collaboration and License Agreement and may do so under the Nippon Shinyaku** Collaboration and License Agreement, we have never generated revenue from sales of our product candidates and may never do so in the future. We have generated significant revenues from licensing our NAV Technology Platform, including sublicense fees, milestone payments and royalties on net sales of a licensed product, Zolgensma, and licensing our intellectual property to AbbVie pursuant to the AbbVie **Collaboration and License Agreement and may do so in the future pursuant to the Nippon Shinyaku** Collaboration and License Agreement. However, our ability to generate revenue from sales of our internal product candidates will depend on our ability, alone or with partners, to successfully complete the development of, and obtain the regulatory approvals necessary to commercialize, our product candidates. Our ability to generate future revenues from sales of our product candidates and in connection with sales of our licensees' and collaborators' products depends heavily on our, and our licensees' and collaborators', success in: • completing research studies and preclinical and clinical development of product candidates and identifying new gene therapy product candidates; • obtaining regulatory and marketing approvals for product candidates for which clinical trials are completed; • commercializing product candidates for

which regulatory and marketing approval is obtained by establishing a sales force, marketing and distribution infrastructure or, alternatively, collaborating with a commercialization partner; • negotiating favorable terms in any collaboration, licensing or other arrangements into which we or our licensees and collaborators may enter and performing our obligations in such collaborations; • qualifying for adequate coverage and reimbursement by government and third- party payors for product candidates; • maintaining and enhancing a sustainable, scalable, reproducible and transferable manufacturing process for our vectors and product candidates; • establishing and maintaining supply and manufacturing relationships with third parties that can provide adequate, in both amount and quality, products and services to support clinical development and the market demand for product candidates, if approved; • obtaining market acceptance of product candidates as a viable treatment option; • competing effectively when other companies may develop products that are priced lower, reimbursed more favorably by government or other third- party payors, safer, more effective or more convenient to use than our products, if any, or our licensees' and collaborators' products; • implementing additional internal systems and infrastructure, as needed; • negotiating favorable terms in any collaboration, licensing or other arrangements into which we may enter and performing our obligations in such collaborations; • maintaining, protecting and expanding our portfolio of intellectual property rights, including patents, trade secrets and know- how; • avoiding and defending against third- party interference, infringement and other intellectual property related claims; and • attracting, hiring and retaining qualified personnel. Many of these factors as they relate to our licensees' and collaborators' products, including Zolgensma, will be outside our control, and future revenues in connection with sales of such products may be precluded or limited by any of these factors. ~~Under our AbbVie Collaboration and License Agreement, we will have limited influence and control over the ABBV- RGX- 314 development and commercialization activities of AbbVie in markets outside the United States, and future revenues in connection with sales of licensed products under such agreement may be precluded or limited by any of these factors.~~ Even if one or more of the product candidates that we develop is approved for commercial sale, we ~~anticipate incurring~~ **may incur** significant costs associated with commercializing any approved product candidate. Our expenses could increase beyond expectations if we are required by the FDA, the EMA or other regulatory authorities to perform clinical and other studies in addition to those that we currently anticipate. Even if we are able to generate revenues from sales of any of our product candidates or in connection with sales of any of our licensees' or collaborators' products, we may not become profitable and may need to obtain additional funding to continue operations. We rely on third parties to conduct **aspects of our clinical trials and** certain preclinical research ~~and~~ development activities ~~and aspects of our clinical trials~~ . If these third parties do not meet our deadlines or otherwise conduct the preclinical research and development activities and trials as required, our **clinical and** preclinical ~~and clinical~~ development programs could be delayed or unsuccessful. We do not have the ability to conduct all aspects of our preclinical research and development activities or clinical trials ourselves. We are dependent on third parties to conduct certain aspects of our clinical trials and, therefore, the timing of the initiation and completion of these trials may be controlled by such third parties and may occur on substantially different timing from our estimates. **We also** ~~Specifically, we~~ rely on third parties to conduct a portion of our preclinical research and development activities and we may also rely on CROs, medical institutions, clinical investigators, consultants or other third parties to conduct our clinical trials in accordance with our clinical protocols and regulatory requirements. A loss or deterioration of our relationships with such third parties or the principal investigators for our preclinical and clinical programs could materially harm our business. There is no guarantee that any third party on which we rely for our preclinical research and development activities and the administration and conduct of our clinical trials will devote adequate time and resources to such activities or trials or perform as contractually required. If any such third party fails to meet expected deadlines, fails to adhere to our preclinical or clinical protocols or otherwise performs in a substandard manner, our preclinical programs and clinical trials may be extended, delayed or terminated, which could materially harm our business. Additionally, if any of our clinical trial sites terminates for any reason, we may experience the loss of follow- up information on subjects enrolled in our ongoing clinical trials unless we are able to transfer those subjects to another qualified clinical trial site. Furthermore, principal investigators for our clinical trials may serve as scientific advisors or consultants to us from time to time and may receive cash or equity compensation in connection with such services. If these relationships and any related compensation result in perceived or actual conflicts of interest, the integrity of the data generated at the applicable clinical trial site may be jeopardized, which could result in substantial delays in our clinical trials and materially harm our business. We have in the past, and in the future may, enter into licensing agreements or collaborations with third parties licensing parts of our NAV Technology Platform for the development of product candidates. If these licensing arrangements or collaborations are not successful, our business could be harmed. We have entered into agreements involving the licensing of parts of our NAV Technology Platform and relating to the development and commercialization of certain product candidates and plan to enter into additional licensing agreements or collaborations in the future. We have limited control over the amount and timing of resources that our current and future licensees and collaborators dedicate to the development or commercialization of product candidates or of products utilizing licensed components of our NAV Technology Platform. Our ability to generate revenues from these arrangements will depend on our and our licensees' and collaborators' abilities to successfully perform the functions assigned to each of us in these arrangements. In addition, our licensees and collaborators have the ability to abandon research or development projects and terminate applicable agreements. Moreover, an unsuccessful outcome in any clinical trial for which our licensee or collaborator is responsible could be harmful to the public perception and prospects of our NAV Technology Platform or product candidates. Any current or future licensing agreements or future collaborations we enter into may pose additional risks, including the following: • subjects in clinical trials undertaken by our licensees and collaborators may suffer adverse effects, including death; • our licensees and collaborators may not pursue development and commercialization of any product candidates that achieve regulatory approval or may elect not to continue or renew development or commercialization programs based on clinical trial results, changes in the licensees' or collaborators' strategic focus or available funding or external factors, such as an acquisition, that divert resources or create competing priorities; • we may not have access to, or may be restricted from disclosing, certain information regarding

product candidates being developed or commercialized under a collaboration and, consequently, may have limited ability to inform our stockholders about the status of such product candidates; • our licensees or collaborators could independently develop, or develop with third parties, products that compete directly or indirectly with our product candidates if the licensees or collaborators believe that competitive products are more likely to be successfully developed or can be commercialized under terms that are more economically attractive than ours; • product candidates developed in collaboration with us may be viewed by our licensees or collaborators as competitive with their own product candidates or products, which may cause licensees or collaborators to cease to devote resources to the commercialization of our product candidates; • a licensee or collaborator with marketing and distribution rights to one or more of our product candidates that achieve regulatory approval may not commit sufficient resources to the marketing and distribution of any such product candidate; • our licensees or collaborators may breach their reporting, payment, intellectual property or other obligations to us, which could prevent us from complying with our contractual obligations to ~~GSK and Penn~~ **our upstream licensors** ; • disagreements with licensees or collaborators, including disagreements over intellectual property and other proprietary rights, payment obligations, contract interpretation or the preferred course of development of any product candidates, may cause delays or termination of the research, development or commercialization of such product candidates, may lead to additional responsibilities for us with respect to such product candidates or may result in litigation or arbitration, any of which would be time- consuming and expensive and could potentially lessen the value of such agreements and collaborations; • our licensees or collaborators may not properly maintain or defend our intellectual property rights or may use our proprietary information in such a way as to invite litigation that could jeopardize or invalidate our intellectual property or proprietary information or expose us to potential litigation; • disputes may arise with respect to the ownership of our other rights to intellectual property developed pursuant to our licensing agreements or collaborations; • **depending on the terms of the licensing agreement, the licensee or collaborator may have sole discretion regarding material aspects of the development, marketing or sale of a product candidate; •** our licensees or collaborators may infringe or otherwise violate the intellectual property rights of third parties, which may expose us to litigation and potential liability; and • licensing agreements or collaborations may be terminated for the convenience of the licensee or collaborator and, if terminated, we could be required to raise additional capital to pursue further development or commercialization of the applicable product candidates ~~. For example, under our AbbVie Collaboration and License Agreement, we will have limited influence and control over the ABBV- RGX- 314 development and commercialization activities of AbbVie in markets outside the United States. Failure by AbbVie to meet its obligations under our AbbVie Collaboration and License Agreement, apply sufficient efforts at developing and commercializing licensed products, or comply with applicable legal or regulatory requirements, may materially adversely affect our business~~ . If our licensing agreements or collaborations do not result in the successful development and commercialization of products, or if one of our licensees or collaborators terminates its agreement with us, we may not receive any future milestone or royalty payments ~~, as applicable, under the license agreement or collaboration~~ . If we do not receive the payments we expect under these agreements, our development of product candidates could be delayed and we may need additional resources to develop our product candidates. In addition, if one of our licensees or collaborators terminates its agreement with us, we may find it more difficult to attract new licensees or collaborators and the perception of us in the business and financial communities could be harmed. Each of our licensees and collaborators is subject to similar risks with respect to product development, regulatory approval and commercialization, and any such risk could result in its business being harmed, which could adversely affect our collaboration. We may in the future decide to partner or collaborate with pharmaceutical and biotechnology companies for the development and potential commercialization of our product candidates. These relationships, or those like them, may require us to incur non- recurring and other charges, increase our near- and long- term expenditures, issue securities that dilute our existing stockholders or disrupt our management and business. In addition, we could face significant competition in seeking appropriate collaborators and the negotiation process is time- consuming and complex. Our ability to reach a definitive licensing agreement or collaboration agreement will depend, among other things, upon our assessment of the collaborator' s resources and expertise, the terms and conditions of the proposed collaboration and the proposed collaborator' s evaluation of a variety of factors. We may not be successful in our efforts to establish such a strategic partnership or other alternative arrangements for our product candidates because our research and development pipeline may be insufficient, our product candidates may be deemed to be at too early of a stage of development for collaborative effort or third parties may not view our product candidates as having the requisite potential to demonstrate safety and efficacy or market opportunity. In addition, we may be restricted under existing collaboration agreements from entering into future agreements with potential collaborators. If we license rights to product candidates, we may not be able to realize the benefit of such transactions if we are unable to successfully integrate the licensed product candidates with our existing operations. If we are unable to reach agreements with suitable licensees or collaborators on a timely basis, on acceptable terms or at all, we may have to curtail the development of a product candidate, reduce or delay its development program, delay its potential commercialization, reduce the scope of any sales or marketing activities or increase our expenditures and undertake development or commercialization activities at our own expense. If we elect to fund development or commercialization activities on our own, we may need to obtain additional expertise and additional capital, which may not be available to us on acceptable terms or at all . Because we rely on third parties..... our competitive position and harm our business . Products intended for use in gene therapies are novel, complex and difficult to manufacture. We could experience production problems that result in delays in our development or commercialization programs, limit the supply of our products or otherwise harm our business. We currently have development, manufacturing and testing agreements with third parties to manufacture supplies of our product candidates, in addition to our internal manufacturing laboratory. Several factors could cause production interruptions, including equipment malfunctions, facility contamination, raw material shortages or contamination, a decline in stability of a product that reduces its shelf life, natural disasters, public health crises, disruption in utility services, human error or disruptions in the operations of suppliers. Our product candidates require processing steps that are more complex

than those required for most chemical pharmaceuticals. Moreover, unlike chemical pharmaceuticals, the physical and chemical properties of biologics such as ours generally cannot be fully characterized. As a result, assays of the finished product may not be sufficient to ensure that the product will perform in the intended manner. Accordingly, we employ multiple steps to control our manufacturing process to assure that the process works and the product candidate is made strictly and consistently in compliance with the process. Problems with the manufacturing process, even minor deviations from the normal process, could result in product defects or manufacturing failures that may not be detected in standard release testing, which could result in lot failures, product recalls, declines in stability, product liability claims or insufficient inventory. We may encounter problems achieving adequate quantities and quality of clinical- grade materials that meet FDA, EMA or other applicable foreign standards or specifications with consistent and acceptable production yields and costs. In addition, the FDA, the EMA and other foreign regulatory authorities may require us to submit samples of any lot of any approved product together with the protocols showing the results of applicable tests at any time. Under some circumstances, the FDA, the EMA or other foreign regulatory authorities may require that we not distribute a lot or batch until the competent authority authorizes its release. Slight deviations in the manufacturing process, including those affecting quality attributes and stability, may result in unacceptable changes in the product that could result in lot / batch failures or product recalls. Lot / batch failures, which we have experienced in the past, or product recalls could cause us to delay clinical trials or product launches which could be costly to us and otherwise harm our business, financial condition, results of operations and prospects. We also may encounter problems hiring and retaining the experienced scientific, quality control and manufacturing personnel needed to operate our manufacturing process which could result in delays in our production or difficulties in maintaining compliance with applicable regulatory requirements. Any problems in our manufacturing process or the facilities with which we contract could make us a less attractive collaborator for potential partners, including larger pharmaceutical companies and academic research institutions, which could limit our access to additional attractive development programs. Problems in third- party manufacturing processes or facilities also could restrict our ability to meet market demand for our products. Additionally, should our manufacturing agreements with third parties be terminated for any reason, there may be a limited number of manufacturers who would be suitable replacements and it could take a significant amount of time to transition the manufacturing to a replacement. Delays in obtaining regulatory approval of our manufacturing process or disruptions in our manufacturing process, including the development of our cGMP production facility, may delay or disrupt our commercialization efforts. Before we can begin to commercially manufacture our product candidates in third- party or our own facilities, we must obtain regulatory approval from the FDA, which includes a review of the manufacturing process and facility. A manufacturing authorization must also be obtained from the appropriate European Union Member State regulatory authorities and may be required by other foreign regulatory authorities. The timeframe required to obtain such approval or authorization is uncertain. In order to obtain approval, we will need to ensure that all of our processes, methods and equipment are compliant with cGMP, and perform extensive audits of vendors, contract laboratories and suppliers. If we or any of our vendors, contract laboratories or suppliers are found to be out of compliance with cGMP, we may experience delays or disruptions in manufacturing while we work to remedy the violation or while we work to identify suitable replacement vendors, contract laboratories or suppliers. The cGMP requirements govern quality control of the manufacturing process and documentation policies and procedures. In complying with cGMP, we will be obligated to expend time, money and effort in production, record keeping and quality control to assure that the product meets applicable specifications and other requirements. If we fail to comply with these requirements, we would be subject to possible regulatory action, which could result in fines or reputational harm, and we may not be permitted to sell any products that we may develop. We currently rely and expect to continue to rely on third parties to conduct **parts of** our product manufacturing, and these third parties may not perform satisfactorily. We currently plan to have some of the material manufactured for our planned preclinical and clinical programs by third parties. We currently rely, and expect to continue to rely, on third parties for the production of a portion of our preclinical study and planned clinical trial materials and, therefore, we can control only certain aspects of their activities. We rely on additional third parties to manufacture ingredients of our product candidates and to perform quality testing, and reliance on these third parties entails risks to which we would not be subject if we manufactured the product candidates ourselves, including: • reduced control for certain aspects of manufacturing activities; • termination or nonrenewal of manufacturing and service agreements with third parties in a manner or at a time that is costly or damaging to us; • disruptions to the operations of our third- party manufacturers and service providers caused by conditions unrelated to our business or operations, including the bankruptcy of, or legal or regulatory actions against, the manufacturer or service provider; • reduced capacity of our third- party manufacturers and service providers caused by increased demand by their other customers; • discovery of data integrity issues with our third- party manufacturers and service providers which directly or indirectly impact our ability to use our product candidates; and • legal or regulatory actions against our third- party manufacturers and service providers which adversely affect our ability to use our product candidates. FDA, EMA or other regulatory authority action could include injunction, recall, seizure or total or partial suspension of product manufacture or manufacturing authorization. Any of these events could lead to clinical trial delays or failure to obtain regulatory approval, or impact our ability to successfully commercialize future product candidates, and therefore may cause our business, financial condition, results of operations and prospects to be materially harmed. Failure to comply with ongoing manufacturing regulatory requirements could cause us to suspend production or put in place costly or time- consuming remedial measures, and shortages of resources or raw materials could result in delays in our research studies, preclinical and clinical development or marketing schedules. Regulatory authorities may, at any time following approval of a product for sale, audit the manufacturing facilities for such product. If any such inspection or audit identifies a failure to comply with applicable regulations, or if a violation of product specifications or applicable regulations occurs independent of such an inspection or audit, the relevant regulatory authority may require remedial measures that may be costly or time- consuming to implement and that may include the temporary or permanent suspension of a clinical trial or commercial sales or the temporary or permanent closure of a manufacturing facility. Any such remedial measures imposed upon us or any of

our third- party manufacturers could materially harm our business, financial condition, results of operations and prospects. If we or any of our third- party manufacturers fail to comply with applicable cGMP regulations, regulatory authorities can impose ~~regulatory~~ sanctions including, among other things, refusal to approve a pending application for a new product candidate or suspension or revocation of a pre- existing approval. Such an occurrence may cause our business, financial condition, results of operations and prospects to be materially harmed. Additionally, if supply from a manufacturing facility is interrupted, there could be a significant disruption in commercial supply of our products. An alternative manufacturer would need to be qualified, through a supplement to its regulatory filing, which could result in further delay. Regulatory authorities also may require additional trials if a new manufacturer is relied upon for commercial production. Switching manufacturers may involve substantial costs and could result in a delay in our desired clinical and commercial timelines. Given the nature of biologics manufacturing, there is a risk of contamination during manufacturing. Any contamination could materially harm our ability to produce product candidates on schedule and could harm our results of operations and cause reputational damage. Some of the resources, raw materials and components required in our manufacturing or research and development processes are derived from biologic sources, and we normally rely on suppliers to provide such resources, raw materials and components. These may be difficult to procure and subject to contamination or recall. Certain resources, raw materials and components, especially those that are specifically catered to the gene therapy industry, may become unavailable to us in sufficient quantities from time to time due to increased demand. A material shortage, contamination, recall or restriction on the use of biologically derived substances in the manufacture of our product candidates may be beyond our control and could adversely impact or disrupt the commercial manufacturing or the production of clinical material, which could materially harm our development timelines and our business, financial condition, results of operations and prospects. **If we are unable to adequately establish sales and marketing capabilities or enter into agreements with third parties to market and sell our product candidates, if approved, we may be unable to generate any product revenue.** We currently have no products to sell ~~and therefore no~~ **. We have a limited** product sales and marketing organization. To successfully commercialize any products that may result from our development programs, we will need to **further** develop these capabilities, either on our own or with others. The **expansion** ~~establishment and development~~ of our own commercial team or the establishment of a contract sales force to market any products we may develop will be expensive and time- consuming and could delay any product launch. Moreover, we cannot be certain that we will be able to successfully develop this capability. We may enter into **additional** ~~collaborations~~ **collaboration arrangements** regarding one or more of our product candidates with other entities to utilize their marketing and distribution capabilities, such as our collaboration with AbbVie **and Nippon Shinyaku** , but we may be unable to enter into such agreements on favorable terms, if at all. If any current licensees or collaborators, or future licensees or collaborators, do not commit sufficient resources to commercialize our products, if approved, or we are unable to develop the necessary capabilities on our own, we will be unable to generate sufficient product revenue to sustain our business. We compete with many companies that currently have extensive, experienced and well- funded marketing and sales operations to recruit, hire, train and retain marketing and sales personnel. We also face competition in our search for third parties to assist us with the sales and marketing efforts of our product candidates. Without an **adequate** internal team or the support of a third party to perform marketing and sales functions, we may be unable to compete successfully against these more established companies. Our efforts to educate the medical community and third- party payors on the benefits of our product candidates may require significant resources and may never be successful. Such efforts may require more resources than are typically required due to the complexity and uniqueness of our potential products. If any of our product candidates is approved but fails to achieve market acceptance among physicians, patients or third- party payors, we will not be able to generate significant revenues from such product, which could materially harm our business, financial condition, results of operations and prospects. If we do not achieve our projected development goals in the timeframes we announce and expect, the commercialization of our products may be delayed and, as a result, our stock price may decline. From time to time, we estimate the timing of the accomplishment of various scientific, clinical, regulatory and other product development goals, which we sometimes refer to as milestones. These milestones may include, but are not limited to, the commencement or completion of scientific studies and clinical trials, the submission of regulatory filings, the announcement of results from scientific studies or clinical trials and the announcement of additional product candidates. From time to time, we may publicly announce the expected timing of some of these milestones. All of these milestones are and will be based on numerous assumptions. The actual timing of these milestones can vary dramatically compared to our estimates, in some cases for reasons beyond our control. If we do not meet these milestones as publicly announced, or at all, the commercialization of our products may be delayed or never achieved and, as a result, our stock price may decline. Even if we receive regulatory approval, we still may not be able to successfully commercialize our lead product candidates or any future product candidate, and the revenue that we generate from any approved product' s sales, if any, could be limited ~~. Ethical, social and legal concerns about gene therapy could result in additional regulations restricting or prohibiting our products~~ . From time to time, public sentiment may be more adverse to commercialization of gene therapy as a therapeutic technique. Even with the requisite approvals from the FDA, the EMA and other regulatory authorities, the commercial success of our product candidates will depend, in part, on the acceptance of physicians, patients and health care payors of gene therapy products in general, and our product candidates in particular, as medically necessary, cost- effective and safe. Any product that we commercialize may not gain acceptance by physicians, patients, health care payors and others in the medical community. If these products do not achieve an adequate level of acceptance, we may not generate significant product revenue and may not become profitable. The degree of market acceptance of our product candidates will depend on a number of factors, including: • demonstration of clinical efficacy and safety compared to other more- established products; • limitation of our targeted patient population and other limitations or warnings contained in any FDA or European Commission labeling, or other comparable foreign regulatory authority- approved labeling; • acceptance of a new formulation by health care providers and their patients; • the prevalence and severity of any adverse effects; • new procedures or methods of treatment that may be more effective in treating or may reduce the conditions

which our products are intended to treat; • pricing and cost- effectiveness; • the effectiveness of our or any future collaborators' sales and marketing strategies; • our ability to obtain and maintain sufficient third- party coverage and reimbursement from government health care programs, including Medicare and Medicaid, private health insurers and other third- party payors; • unfavorable publicity and negative public opinion relating to product candidates or gene therapy generally, including due to serious adverse events in gene therapy trials; and • the willingness of patients to pay out- of- pocket in the absence of third-party coverage or reimbursement. If any product candidate is approved but does not achieve an adequate level of acceptance by physicians, hospitals, healthcare payors or patients, we may not generate sufficient revenue from that product candidate and may not become or remain profitable. Our efforts to educate the medical community and third- party payors on the benefits of our lead product candidates or any future product candidates may require significant resources and may never be successful. In addition, our ability to successfully commercialize our product candidates will depend on our ability to differentiate our products from competing products and defend and enforce our intellectual property rights relating to our products. Additionally, if the market opportunities for our lead product candidates or any future product candidates are smaller than we believe they are, our product revenues may be harmed and our business may suffer. We focus our research and product development on treatments for severe genetic and orphan diseases. Our understanding of both the number of people who have these diseases, as well as the subset of people with these diseases who have the potential to benefit from treatment with our product candidates, are based on estimates. These estimates may prove to be incorrect and new studies may reduce the estimated incidence or prevalence of these diseases. The number of patients in the United States, the European Union and elsewhere may turn out to be lower than expected, may not be otherwise amenable to treatment with our products or patients may become increasingly difficult to identify and access, all of which would harm our business, financial condition, results of operations and prospects. Further, there are several factors that could contribute to making the actual number of patients who receive any products we develop less than the potentially addressable market. These include the lack of widespread availability of, and limited reimbursement for, new therapies in many underdeveloped markets. Further, the severity of the progression of a disease up to the time of treatment, especially in certain degenerative conditions such as the conditions our lead product candidates are intended to treat, will likely diminish the therapeutic benefit conferred by a gene therapy due to irreversible cell death. Lastly, certain patients' immune systems might prohibit the successful delivery of certain gene therapy products to the target tissue, thereby limiting the treatment outcomes. The insurance coverage and reimbursement status of newly approved products is uncertain. Failure to obtain or maintain adequate coverage and reimbursement for our products, if approved, **or the imposition of price controls or other forms of pricing regulation** could limit our ability to market those products and decrease our ability to generate product revenue. We expect the cost of a single administration of gene therapy products, such as those we are developing, to be substantial, when and if they achieve regulatory approval. We expect that coverage and reimbursement by government and private payors will be essential for most patients to be able to afford these treatments. Accordingly, sales of our product candidates will depend substantially, both domestically and abroad, on the extent to which the prices of our product candidates will be paid by health maintenance, managed care, pharmacy benefit and similar healthcare management organizations, or will be reimbursed by government authorities, private health coverage insurers and other third- party payors. Coverage and reimbursement by a third- party payor may depend upon several factors, including the third- party payor' s determination that use of a product is: • a covered benefit under its health plan; • safe, effective and medically necessary; • appropriate for the specific patient; • cost- effective; and • neither experimental nor investigational. Obtaining coverage and reimbursement for a product from third- party payors is a time- consuming and costly process that could require us to provide to the payor supporting scientific, clinical and cost- effectiveness data. We may not be able to provide data sufficient to gain coverage and reimbursement. If coverage and reimbursement are not available, or are available only at limited levels, we may not be able to successfully commercialize our product candidates. Even if coverage is provided, the approved reimbursement amount may not be adequate to realize a sufficient return on our investment. There is significant uncertainty related to third- party coverage and reimbursement of newly approved products, including potential one- time gene therapies. In the United States, third- party payors, including government payors such as the Medicare and Medicaid programs, play an important role in determining the extent to which new drugs and biologics will be covered and reimbursed. The Medicare and Medicaid programs increasingly are used as models for how private payors and other government payors develop their coverage and reimbursement policies. It is difficult to predict what the CMS, the agency responsible for administering the Medicare program, will decide with respect to coverage and reimbursement for fundamentally novel products such as ours, as there is little body of established practices and precedents for these types of products. We cannot be assured that Medicare or Medicaid will cover any of our products, if approved, or provide reimbursement at adequate levels to realize a sufficient return on our investment. In addition, government regulators and legislative bodies in the United States have enacted laws and are considering numerous proposals that may result in limitations on the prices at which we could charge customers for our products if we have products that are approved for sale. For example, the IRA enacted in 2022, permits HHS to engage in price- capped negotiation to set the price of certain drugs and biologics reimbursed under Medicare Part B and Part D. The IRA contains statutory exclusions to the negotiation program, including for certain orphan designated drugs for which the only approved indication is for the orphan disease or condition. Should our product candidates be approved and covered by Medicare Part B or Part D, and fail to fall within a statutory exclusion, such as that for an orphan drug, those products could, after a period of time (e. g., ~~13~~ **11** years after FDA approval of biologics, including gene therapies), be selected for negotiation and become subject to prices representing a significant discount from average prices to wholesalers and direct purchasers. The IRA also establishes a rebate obligation for drug manufacturers that increase prices of Medicare Part B and Part D covered drugs at a rate greater than the rate of inflation. The inflation rebates may require us to pay rebates if we **were to** ~~increased~~ **increase** the cost of a covered Medicare Part B or Part D covered product faster than the rate of inflation. At this time, we are unable to predict how these recent legislative changes or any future legislation might affect our business. Moreover, reimbursement agencies in the European Union may be more conservative than

CMS. It is difficult to predict what third- party payors will decide with respect to the coverage and reimbursement for our product candidates. Outside the United States, international operations generally are subject to extensive government price controls and other market regulations, and increasing emphasis on cost- containment initiatives in the European Union and other countries may put pricing pressure on us. In many countries, the prices of medical products are subject to varying price control mechanisms as part of national health systems. It also can take a significant amount of time after approval of a product to secure pricing and reimbursement for such product in many countries outside the United States. In general, the prices of medicines under such systems are substantially lower than in the United States. Other countries allow companies to fix their own prices for medical products, but monitor and control company profits. Additional foreign price controls or other changes in pricing regulation could restrict the amount that we are able to charge for our product candidates. Accordingly, in markets outside the United States, the reimbursement for our products may be reduced compared with the reimbursement in the United States and may be insufficient to generate commercially reasonable product revenues. Moreover, increasing efforts by government and third- party payors in the United States and abroad to cap or reduce healthcare costs **has led to increased pressure on the healthcare industry to reduce costs and** may **, in the future,** cause such organizations to limit both coverage and the level of reimbursement for new products approved and, as a result, they may not cover or provide adequate payment for our product candidates. Payors increasingly are considering new metrics as the basis for reimbursement rates, and the existing data for reimbursement based on some of these metrics is limited. Therefore, it may be difficult to project the impact of these evolving reimbursement metrics on the willingness of payors to cover candidate products that we or our partners are able to commercialize. We expect to experience pricing pressures in connection with the sale of any of our product candidates due to the trend toward managed healthcare, the increasing influence of health maintenance organizations and additional legislative changes. Additionally, our lead product candidates are designed to provide therapeutic benefit after a single administration and, therefore, the pricing and reimbursement of a single administration of our lead product candidates, if approved, must be adequate to support our commercial infrastructure. The downward pressure on healthcare costs in general, particularly prescription drugs and surgical procedures and other treatments, has become intense. As a result, increasingly high barriers are being erected to the entry of new products. If we are unable to obtain adequate levels of reimbursement, our ability to successfully market and sell our product candidates will be harmed. The manner and level at which reimbursement is provided for services related to our product candidates (e. g., for administration of our product to patients) is also important. Inadequate reimbursement for such services may lead to physician resistance and limit our ability to market or sell our products. ~~Government price controls or other changes in pricing regulation could restrict the amount that we are able to charge for any of our product candidates, if approved, which would adversely affect our revenue and results of operations. We expect that coverage and reimbursement of drugs and biologics may be increasingly restricted in the United States and internationally. The escalating cost of health care has led to increased pressure on the health care industry to reduce costs. In particular, pricing by biopharmaceutical companies recently has come under increased scrutiny and continues to be subject to intense political and public debate in the United States and abroad. Government and private third- party payors have proposed health care reforms and cost reductions of drugs and biologics. A number of federal and state proposals to control the cost of health care have been made in the United States. Specifically, there have been several recent U. S. Congressional inquiries and proposed federal and state bills designed to, among other things, bring more transparency to pricing, review the relationship between pricing and manufacturer patient programs and reform government program reimbursement methodologies. In some international markets, the government controls drug and biologic pricing, which can affect profitability.~~ We cannot predict the extent to which our business may be affected by these or other potential future legislative or regulatory developments. However, future price controls or other changes in pricing regulation or negative publicity related to the pricing of drugs and biologics generally could restrict the amount that we are able to charge for our future products, if any, which could adversely affect our revenue and results of operations. We may not be successful in our efforts to identify or discover additional product candidates and may fail to capitalize on programs or product candidates that may be a greater commercial opportunity or for which there is a greater likelihood of success. **Our main goals continue to include the** ~~The success of our business depends upon our~~ ability to identify, develop and commercialize product candidates based on our NAV Technology Platform. Research programs to identify new product candidates require substantial technical, financial and human resources. Although certain of our product candidates are currently in research studies or preclinical development, we may fail to identify potential product candidates for clinical development for several reasons. For example, our research may be unsuccessful in identifying potential product candidates or our potential product candidates may be shown to have harmful side effects, may be commercially impracticable to manufacture or may have other characteristics that may make the products unmarketable or unlikely to receive marketing approval ~~. Additionally, because we have limited resources, we may forego or delay pursuit of opportunities with certain programs or product candidates or for indications that later prove to have greater commercial potential~~ . Our spending on current and future research and development programs may not yield any commercially viable products. If we do not accurately evaluate the commercial potential for a particular product candidate, we may relinquish valuable rights to that product candidate through strategic collaboration, licensing or other arrangements in cases in which it would have been more advantageous for us to retain sole development and commercialization rights to such product candidate. Alternatively, we may allocate internal resources to a product candidate in a therapeutic area in which it would have been more advantageous to enter into a partnering arrangement. If any of these events occur, we may be forced to abandon our development efforts with respect to a particular product candidate or fail to develop a potentially successful product candidate, which could materially harm our business, financial condition, results of operations and prospects. ~~We may not successfully execute or achieve the expected benefits of our strategic pipeline prioritization and restructuring plan or other cost- saving measures that we may take in the future, and our efforts may result in further actions or additional asset impairment charges, any of which may have a material adverse effect our business, financial condition and results of operations. In November 2023, we announced a strategic pipeline prioritization and restructuring plan to~~

~~increase our focus on our large commercial opportunities in retinal and neuromuscular disease from our strong pipeline of AAV therapeutics. We continue to take actions intended to address the short- term health of our business as well as our long- term objectives based on our current estimates, assumptions and forecasts. These measures are subject to known and unknown risks and uncertainties, including whether we have targeted the appropriate areas for our prioritization and cost- saving efforts and at the appropriate scale, and whether, if required in the future, we will be able to appropriately target any additional areas for our cost- saving efforts. As such, the actions we are taking under the pipeline prioritization and restructuring plan and that we may decide to take in the future may not be successful in yielding our intended results and may not appropriately address either or both of the short- term and long- term strategy for our business. Implementation of the strategic pipeline prioritization and restructuring plan and any other cost- saving initiatives may be costly and disruptive to our business, the expected costs and charges may be greater than we have forecasted, and the estimated cost savings may be lower than we have forecasted. Certain aspects of the restructuring plan, such as severance costs in connection with reducing our headcount, could negatively impact our cash flows. In addition, our initiatives could result in personnel attrition beyond our planned reduction in headcount or reduced employee morale, which could in turn adversely impact productivity, including through a loss of continuity, loss of accumulated knowledge or inefficiency during transitional periods, or our ability to attract and retain highly skilled employees. In addition, the pipeline prioritization and restructuring plan has required, and may continue to require, a significant amount of management' s and other employees' time and focus, which may divert attention from effectively operating and growing our business.~~ Our future success depends on our ability to retain key employees, consultants and advisors and to attract, retain and motivate qualified personnel. **Our** ~~We are highly dependent on members of our~~ executive team **underwent significant transition in 2024** , **including the appointment of a new Chief Executive Officer and Chief Financial Officer, as well as the appointment of our former Chief Executive Officer as Chairman of the Board of Directors. If we are unable to successfully transition to our new leadership team, or if we were to lose the services of any of the members of our executive team, we may be unable to achieve our strategic priorities. In addition, our success depends in large part on the performance of our team. If we were to lose the services of a significant number of employees, consultants or advisors, or** ~~the~~ **those who sit in key positions, including scientific and technical roles, such** loss **could impede our ability to** ~~of any of whose services may adversely impact the achievement~~ ---- **achieve** ~~of~~ our **research, development, licensing and commercialization** objectives ~~. While we have entered into employment agreements with each of our executive officers, any of them could leave our employment at any time, as all of our employees are " at- will " employees~~ . We currently do not have " key person " insurance on any of our employees. ~~The loss of the services of one or more of our current employees, consultants and advisors might impede the achievement of our research, development, licensing and commercialization objectives.~~ Recruiting and retaining other qualified employees, consultants and advisors for our business, including scientific and technical personnel is, and will continue to be, critical to our success. There currently is a shortage of skilled individuals with substantial gene therapy experience ~~, which we believe is likely to continue~~ . As a result, competition for skilled personnel, including in gene therapy research and vector manufacturing, is intense and the turnover rate can be high. We may not be able to attract and retain personnel on acceptable terms given the competition among numerous pharmaceutical and biotechnology companies and academic institutions for individuals with similar skill sets. In addition, failure to succeed in preclinical studies or clinical trials or applications for marketing approval may make it more challenging to recruit and retain qualified personnel. The inability to recruit, or loss of services of any of our key executives, employees, consultants or advisors may impede the progress of our research, development, licensing and commercialization objectives and materially harm our business, financial condition, results of operations and prospects. If we are successful in executing our business strategy, we will need to expand our managerial, operational, financial **, compliance** and other systems and resources to manage our operations, continue our research and development and licensing activities, and, in the longer term, build a sales and marketing infrastructure to support commercialization of any of our product candidates that are approved for sale. Future growth would impose significant added responsibilities on members of management. It is likely that our management, finance, development personnel, systems and facilities currently in place may not be adequate to support this future growth. Our need to effectively manage our operations, growth and product candidates requires that we continue to develop more robust business processes and improve our systems and procedures in each of these areas and to attract and retain sufficient numbers of talented employees. We may be unable to successfully implement these tasks on a larger scale and, accordingly, may not achieve our research, development and growth goals. If our employees, principal investigators, consultants or commercial partners engage in misconduct, or if we are unable to comply with federal and state healthcare fraud and abuse laws, false claims laws, health information privacy and security laws or other applicable laws or regulations, then we could face substantial penalties. We are exposed to the risk of fraud or other misconduct by our employees, principal investigators, consultants and commercial partners. Misconduct by these parties could include intentional failures to **:** comply with FDA regulations or the regulations applicable in the European Union and other jurisdictions ~~,~~ **;** provide accurate information to the FDA, the European Commission and other regulatory authorities ~~,~~ **;** comply with healthcare fraud and abuse laws and regulations in the United States and abroad ~~,~~ **;** report financial information or data accurately **;** or disclose unauthorized activities to us. In particular, sales, marketing and business arrangements in the healthcare industry are subject to extensive laws and regulations intended to prevent fraud, misconduct, kickbacks, self- dealing and other abusive practices. These laws and regulations restrict or prohibit a wide range of pricing, discounting, marketing and promotion, sales commission, customer incentive programs and other business arrangements. Such misconduct also could involve the improper use of information obtained in the course of clinical trials or interactions with the FDA or other regulatory authorities, which could result in regulatory sanctions and cause serious harm to our reputation. We have adopted a code of business conduct applicable to all of our employees, but it is not always possible to identify and deter employee misconduct, and the precautions we take to detect and prevent this activity may not be effective in controlling unknown or unmanaged risks or losses or in protecting us from government investigations or other actions or lawsuits stemming from a failure to comply with these

laws or regulations. If any such actions are instituted against us ~~,~~ and we are not successful in defending ourselves or asserting our rights, those actions could have a significant **adverse** impact on our business, financial condition, results of operations and prospects, including the imposition of significant fines or other sanctions. If we obtain the approval of the FDA, the European Commission or other regulatory authorities for any of our product candidates and begin commercializing those products in the United States or outside the United States, our operations will be directly, or indirectly through our prescribers, customers and purchasers, subject to various federal, state and foreign fraud and abuse laws and regulations, including, without limitation, the federal Health Care Program Anti- Kickback Statute, the federal civil and criminal False Claims Act and Physician Payments Sunshine Act and regulations, and similar laws in foreign jurisdictions. These laws will impact, among other things, our proposed sales, marketing and educational programs. In addition, we may be subject to patient privacy laws by both the federal government and the states in which we conduct our business. The laws that will affect our operations include, but are not limited to: • the federal Health Care Program Anti- Kickback Statute, which prohibits, among other things, persons or entities from knowingly and willfully soliciting, receiving, offering or paying any remuneration (including any kickback, bribe or rebate), directly or indirectly, overtly or covertly, in cash or in kind, in return for the purchase, recommendation, leasing or furnishing of an item or service reimbursable under a federal healthcare program, such as the Medicare and Medicaid programs. This statute has been interpreted to apply to arrangements between pharmaceutical manufacturers on the one hand, and prescribers, purchasers and formulary managers on the other. Liability may be established under the federal Anti- Kickback Statute without proving actual knowledge of the statute or specific intent to violate it; • federal civil and criminal false claims laws and civil monetary penalty laws which prohibit, among other things, individuals or entities from knowingly presenting, or causing to be presented, claims for payment or approval from Medicare, Medicaid or other government payors that are false or fraudulent. PPACA provides and recent government cases against pharmaceutical and medical device manufacturers support the view that federal Anti- Kickback Statute violations and certain marketing practices, including off- label promotion, may implicate the False Claims Act; • HIPAA, which created new federal criminal statutes that prohibit a person from knowingly and willfully executing a scheme or from making false or fraudulent statements to defraud any healthcare benefit program, regardless of the payor (e. g., public or private); • HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act (HITECH), and its implementing regulations, and as amended again by the final HIPAA omnibus rule, Modifications to the HIPAA Privacy, Security, Enforcement, and Breach Notification Rules Under HITECH and the Genetic Information Nondiscrimination Act; • Other Modifications to HIPAA, which imposes certain requirements relating to the privacy, security and transmission of individually identifiable health information without appropriate authorization by entities subject to the rule, such as health plans, health care clearinghouses and health care providers; • federal transparency laws, including the federal Physician Payment Sunshine Act, that require disclosure of payments and other transfers of value provided to physicians and teaching hospitals, and ownership and investment interests held by physicians and other healthcare providers and their immediate family members and applicable group purchasing organizations; • national laws, industry codes and professional codes of conduct applicable to certain European Union Member States which require payments made to physicians to be publicly disclosed and agreements with physicians to often be the subject of prior notification and approval by the physicians' employer, his or her competent professional organization and / or the regulatory authorities of the individual Member States; • federal, state and foreign laws relating to the processing, storage and transfer of personal data, including, but not limited to, the California Consumer Privacy Act and the European Union' s General Data Protection Regulation, which may require us to incur substantial costs or change our business practices with respect to the treatment of personal data; and • state and foreign law equivalents of each of the above federal laws, state laws that require drug manufacturers to report information related to payments and other transfers of value to physicians and other healthcare providers or marketing expenditures and state laws governing the privacy and security of health information in certain circumstances, many of which differ from each other in significant ways and may not have the same effect, thus complicating compliance efforts in certain circumstances, such as specific disease states. Because of the breadth of these laws and the narrowness of the statutory exceptions and safe harbors available, it is possible that some of our business activities could be subject to challenge under one or more of such laws. If our operations are found to be in violation of any of the laws described above or any other government regulations that apply to us, we may be subject to penalties, including civil and criminal penalties, damages, fines, exclusion from participation in government health care programs, such as Medicare and Medicaid, ~~imprisonment,~~ reputational harm, public reprimands, third-party actions, such as cease and desist letters or injunctions, and the curtailment or restructuring of our operations, any of which could harm our ability to operate our business and our results of operations. The provision of benefits or advantages to physicians to induce or encourage the prescription, recommendation, endorsement, purchase, supply, order or use of medicinal products is prohibited in the European Union. The provision of benefits or advantages to physicians is also governed by the national anti- bribery laws of European Union Member States **and** ~~, such as the~~ **other** ~~UK Bribery Act 2010~~ **jurisdictions in which we may operate** . Infringement of these laws could result in substantial fines and imprisonment. Product liability lawsuits against us could cause us to incur substantial liabilities and could limit licensing of our NAV Technology Platform or commercialization of any product candidates that we may develop. We face an inherent risk of product liability exposure related to our licensed NAV Technology Platform and the testing of our product candidates in clinical trials and may face an even greater risk if products utilizing our NAV Technology Platform are commercialized. If we cannot successfully defend ourselves against claims that our technology or product candidates caused injuries, we could incur substantial liabilities. Regardless of merit or eventual outcome, liability claims may result in: • decreased demand for our technology, including any product candidates that we may develop; • loss of revenue; • substantial monetary awards to trial participants or patients; • significant time and costs to defend the related litigation; • withdrawal of clinical trial participants; • the inability to license our NAV Technology Platform or commercialize any product candidates that we may develop; and • injury to our reputation and significant negative media attention. Although we maintain product liability insurance coverage, this insurance may not be

adequate to cover all liabilities that we may incur. We anticipate that we will evaluate the need to increase our insurance coverage each time we commence a clinical trial and may from time to time purchase additional coverage for clinical trials. We may need to increase our product liability insurance coverage if we successfully commercialize any product candidates. Insurance coverage is increasingly expensive and we may not be able to maintain insurance coverage at a reasonable cost or in an amount adequate to satisfy any liability that may arise. If we, our development partners, including our licensees and collaborators, or our third- party manufacturers or suppliers fail to comply with environmental, health and safety laws and regulations, we could become subject to fines or penalties or incur costs that could materially harm the success of our business. We, our development partners, including our licensees and collaborators, and our third- party manufacturers and suppliers are subject to numerous environmental, health and safety laws and regulations, including those governing laboratory procedures and the generation, handling, use, storage, treatment, manufacture, transportation and disposal of, and exposure to, hazardous materials and wastes, as well as laws and regulations relating to occupational health and safety. Our operations involve the use of hazardous and flammable materials, including chemicals and biologic and radioactive materials. Our operations and the operations of our development partners and third- party manufacturers and suppliers also produce hazardous waste products. We cannot eliminate the risk of contamination or injury from these materials. In the event of contamination or injury resulting from the use of hazardous materials by us, our development partners or our third- party manufacturers or suppliers, we could be held liable for any resulting damages, and any liability could exceed our resources. We also could incur significant costs associated with civil or criminal fines and penalties. Although we maintain workers' compensation insurance for certain costs and expenses we may incur due to work- related injuries to our employees, this insurance may not provide adequate coverage against potential liabilities. Although we maintain insurance for claims that may be asserted against us in connection with our storage or disposal of biologic, hazardous or radioactive materials, this insurance may not be adequate to cover all liabilities that we may incur in connection with such claims. In addition, we may incur substantial costs in order to comply with current or future environmental, health and safety laws and regulations, which have tended to become more stringent over time. These current or future laws and regulations may impair us or our development partners', including our licensees' and collaborators', research, development or production efforts. Failure to comply with these laws and regulations also may result in substantial fines, penalties or other sanctions or liabilities, which could materially harm our business, financial condition, results of operations and prospects. Our ~~internal computer~~ **information technology** systems, or those of our **vendors,** collaborators or other contractors or consultants, may fail or suffer security breaches, which could result in a material disruption of our business or financial operations, including our licensing and product development programs. Our ~~internal computer~~ **information technology** systems and those of our current and any future **vendors,** collaborators and other contractors or consultants are vulnerable to damage from computer viruses, unauthorized access, natural disasters, terrorism, war and telecommunication and electrical failures. Although we have experienced cybersecurity incidents from time to time in the past, we believe we have not experienced any incident that has had a material effect on our business. If such an incident were to occur in the future and cause a material interruption in our operations, it could result in a material disruption of our business or financial operations, including our licensing and development programs **, and potentially subject us to liability** . Unauthorized disclosure of sensitive or confidential patient or employee data, including personally identifiable information, whether through breach of ~~computer~~ **information technology** systems, systems failure, employee negligence, fraud or misappropriation, or otherwise, or unauthorized access to or through our information systems and networks, whether by our employees or third parties, could result in negative publicity, legal liability and damage to our reputation. Unauthorized disclosure of personally identifiable information could also expose us to sanctions for violations of data privacy laws and regulations around the world, especially since the regulatory environment surrounding data privacy laws are increasingly demanding, with frequent imposition of new and changing requirements. To the extent that any disruption or security breach results in a loss of, or damage to, our trade secrets, data or applications, or inappropriate disclosure of confidential or proprietary information, we could incur liability, our competitive position could be harmed and the further licensing of our NAV Technology Platform and development and commercialization of our product candidates could be delayed. For example, the loss of, or damage to, clinical trial data for any of our product candidates could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data. Security breaches pose a risk that sensitive data, including intellectual property, trade secrets or personal information, may be exposed to unauthorized persons or to the public. Cyber- attacks are increasing in their frequency, sophistication and intensity, and have become increasingly difficult to detect. Cybersecurity attacks include, but are not limited to, malicious software (malware, ransomware and viruses), phishing and social engineering, attempts to gain unauthorized access to networks, computer systems and data, malicious or negligent actions of employees (including misuse of information they are entitled to access), cyber extortion, electronic or wire fraud, and other forms of electronic security breaches. These incidents may be caused by failures during routine operations, such as system upgrades, or by user errors, as well as network or hardware failures, malicious or disruptive software, unintentional or malicious actions of employees or contractors, cyberattacks by hackers, criminal groups or nation-state organizations (which may include social engineering, business email compromise, cyber extortion, denial of service, or attempts to exploit vulnerabilities), geopolitical events, natural disasters, failures or impairments of telecommunications networks, or other catastrophic events. Our business partners face similar risks, and a security breach of their systems could adversely affect our security posture. While we have procedures in place for selecting and managing our relationships with third- party service providers and other business partners, we do not have control over their business operations or governance and compliance systems, practices and procedures, and our management of multiple third- party service providers and business partners increases our operational complexity. If we fail to adequately monitor our third- party service providers' and business partners' performance, including for compliance with our agreements and regulatory and legal requirements, we may have to incur additional costs to correct errors, our reputation could be harmed or we could be subject to litigation, claims, legal or regulatory proceedings, inquiries or investigations. Third- party service providers and business partners may experience

cybersecurity incidents that may involve data we share with them or rely on them to provide to us, and the need to coordinate with such third- parties and business partners, including with respect to timely notification and access to personnel and information concerning an incident, may complicate our efforts to resolve any issues that arise. As a result, we are subject to the risk that the activities associated with our third- party service providers and business partners will adversely affect our business, even if the cyber incident does not directly impact our systems or information. While we continue to invest in data protection and information technology, including providing an information security training and compliance program to our employees, there can be no assurance that our efforts will prevent service interruptions or identify breaches in our systems that could adversely affect our business and operations and / or result in the loss of critical or sensitive information, which could result in financial, legal, business or reputational harm. Although we have general liability and cybersecurity insurance coverage, our insurance may not cover all claims, continue to be available on reasonable terms or be sufficient in amount to cover one or more large claims; additionally, the insurer may disclaim coverage as to any claim. The successful assertion of one or more large claims against us that exceed or are not covered by our insurance coverage or changes in our insurance policies, including premium increases or the imposition of large deductible or co- insurance requirements, could materially harm our business, financial condition, results of operations and prospects. Our current revenues are derived from a concentrated customer base. Our revenues for the years ended December 31, **2024 and** 2023 ~~and 2022~~ consisted solely of license and royalty revenue **. One customer accounted for approximately 98 % of our total revenues for the year ended December 31, 2024** . One customer accounted for approximately 95 % of our total revenues for the year ended December 31, 2023 ~~. One customer accounted for approximately 90 % of our total revenues for the year ended December 31, 2022~~ . We expect future license and royalty revenue to be derived from a limited number of licensees and collaborators. Future license and royalty revenue is uncertain due to the contingent nature of our licenses granted to third parties. Our rights to license our NAV Technology Platform and to develop and commercialize our product candidates are subject, in part, to the terms and conditions of licenses granted to us by others. We are heavily reliant upon licenses to certain patent rights and proprietary technology from third parties that are important or necessary to the development of our technology and products, including technology related to our manufacturing process and our gene therapy product candidates. These and other licenses may not provide exclusive rights to use such intellectual property and technology in all relevant fields of use and in all territories in which we may wish to license our platform or develop or commercialize our technology and products in the future. As a result, we may not be able to prevent competitors from developing and commercializing competitive products in territories not included in all of our licenses. For example, under our license agreement with GSK, GSK retained certain exclusive and non- exclusive rights under the patent rights that it licensed from Penn. Licenses to additional third- party technology that may be required for our licensing or development programs may not be available in the future or may not be available on commercially reasonable terms, or at all, which could materially harm our business and financial condition. In some circumstances, we may not have the right to control the preparation, filing and prosecution of patent applications, or to maintain or enforce the patents, covering technology that we license from third parties. For example, under our license agreement with Penn, Penn is entitled to control the preparation, prosecution and maintenance of the patent rights licensed to us. However, if we determine that we desire a greater degree of control over such patent rights, the Penn license agreement provides that Penn will work in good faith with us to enter into an arrangement for such additional control with reimbursement by us of certain expenses. If our licensors fail to maintain such patents, or lose rights to those patents or patent applications, the rights we have licensed may be reduced or eliminated and our right to develop and commercialize any of our products that are the subject of such licensed rights could be impacted. In addition to the foregoing, the risks associated with patent rights that we license from third parties will also apply to patent rights we may own in the future. Furthermore, the research resulting in certain of our licensed patent rights and technology was funded by the U. S. government. As a result, the government may have certain rights, or march- in rights, to such patent rights and technology. When new technologies are developed with government funding, the government generally obtains certain rights in any resulting patents, including a non- exclusive license authorizing the government to use the invention for non- commercial purposes. These rights may permit the government to disclose our confidential information to third parties and to exercise march- in rights to use or allow third parties to use our licensed technology. The government can exercise its march- in rights if it determines that action is necessary because we fail to achieve practical application of the government- funded technology, because action is necessary to alleviate health or safety needs, to meet requirements of federal regulations or to give preference to U. S. industry. In addition, our rights in such inventions may be subject to certain requirements to manufacture products embodying such inventions in the United States. Any exercise by the government of such rights could harm our competitive position, business, financial condition, results of operations and prospects. If we are unable to obtain and maintain patent protection for our products and technology, or if the scope of the patent protection obtained is not sufficiently broad, our competitors could develop and commercialize products and technology similar or identical to ours, and our ability to successfully license our NAV Technology Platform and commercialize our products and technology may be harmed. Our success depends, in ~~large~~ part, on our ability to obtain and maintain patent protection in the United States and other countries with respect to our proprietary NAV Technology Platform, our product candidates and our manufacturing technology. Our licensors have sought and we intend to seek to protect our proprietary position by filing patent applications in the United States and abroad related to many of our novel technologies and product candidates that are important to our business. The patent prosecution process is expensive, time- consuming and complex, and we may not be able to file, prosecute, maintain, enforce or license all necessary or desirable patent applications at a reasonable cost or in a timely manner. In addition, certain patents in the field of gene therapy that may have otherwise potentially provided patent protection for certain of our product candidates have expired or will soon expire. In some cases, the work of certain academic researchers in the gene therapy field has entered the public domain, which we believe precludes our ability to obtain patent protection for certain inventions relating to such work. 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. We are a party to intellectual property

license agreements with GSK and Penn, each of which is important to our business, and other entities and we expect to enter into additional license agreements in the future. Our existing license agreements impose, and we expect that future license agreements will impose, various diligence, development and commercialization timelines, milestone payments, royalties and other obligations on us. If we or our licensees or collaborators fail to comply with our obligations under these agreements, or we are subject to a bankruptcy, the licensor may have the right to terminate the license, in which event we would not be able to market products covered by the license. The patent position of biotechnology and pharmaceutical companies generally is highly uncertain, involves complex legal and factual questions and has, in recent years, been the subject of much litigation. As a result, the issuance, scope, validity, enforceability and commercial value of our patent rights are highly uncertain. Our pending and future patent applications may not result in patents being issued which protect our technology or product candidates or which effectively prevent others from commercializing competitive technologies and product candidates. 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. We may not be aware of all third- party intellectual property rights potentially relating to our technology and product candidates. Publications of discoveries in the scientific literature often lag 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 any owned or any licensed patents or pending patent applications, or that we were the first to file for patent protection of such inventions. Even if the patent applications we license or may own in the future do issue as patents, they may not issue in a form that will provide us with any meaningful protection, prevent competitors or other third parties from competing with us or otherwise provide us with any competitive advantage. Our competitors or other third parties may avail themselves of safe harbor under the Drug Price Competition and Patent Term Restoration Act of 1984 (the Hatch- Waxman Amendments) to conduct research and clinical trials and may be able to circumvent our 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 inventorship, scope, validity or enforceability, and our patents may be challenged in the courts or patent offices in the United States and abroad. Such challenges may result in loss of exclusivity or in patent claims being narrowed, invalidated or held unenforceable, which could limit our ability to stop others from using or commercializing similar or identical technology and products, or limit the duration of the patent protection of our technology and product candidates. Given the amount of time required for the development, testing and regulatory review of new product candidates, patents protecting such candidates might expire before or shortly after such candidates are commercialized. As a result, our intellectual property may not provide us with sufficient rights to exclude others from commercializing products similar or identical to ours. **.** Because we rely on third parties,including contractors,to research,develop and manufacture our product candidates,we must,at times,share trade secrets with them.We seek to protect our proprietary technology in part by entering into confidentiality agreements and,if applicable,material transfer agreements,consulting agreements or other similar agreements with our advisors,employees,third- party contractors and consultants prior to beginning research or disclosing proprietary information.These agreements typically limit the rights of the third parties to use or disclose our confidential information,including our trade secrets.Despite the contractual provisions employed when working with third parties,these provisions may be breached,and the need to share trade secrets and other confidential information increases the risk that such trade secrets become known by our competitors,are inadvertently incorporated into the technology of others,or are disclosed or used in violation of these agreements.Any disclosure,either intentional or unintentional,by our employees,the employees of third parties with whom we share our facilities or third- party consultants and vendors that we engage to perform research,clinical trials or manufacturing activities,or misappropriation by third parties (such as through a cybersecurity breach) of our trade secrets or proprietary information could enable competitors to duplicate or surpass our technological achievements,thus eroding our competitive position in our market.Further,adequate remedies may not exist in the event of unauthorized use or disclosure.Given that our proprietary position is based,in part,on our know- how and trade secrets,a competitor' s independent discovery of our trade secrets or other unauthorized use or disclosure would impair our competitive position and may materially harm our business.In addition,these agreements typically restrict the ability of our advisors,employees,third- party contractors and consultants to publish data potentially relating to our trade secrets,although our agreements may contain certain limited publication rights.For example,any academic institution that we collaborate with,or may collaborate with in the future,will sometimes be granted rights to publish data arising out of such collaboration,provided that we are notified in advance and given the opportunity to delay publication for a limited time period in order for us to secure patent protection of intellectual property rights arising from the collaboration,in addition to the opportunity to remove confidential or trade secret information from any such publication.We may also conduct joint research and development programs that may require us to share trade secrets under the terms of our research and development or similar agreements.Despite our efforts to protect our trade secrets,our competitors may discover our trade secrets,either through breach of our agreements with third parties,independent development or publication of information by any of our third- party collaborators.A competitor' s discovery of our trade secrets would impair our competitive position and harm our business .~~–~~Our intellectual property licenses with third parties may be subject to disagreements over contract interpretation, which could narrow the scope of our rights to the relevant intellectual property or technology, increase our financial or other obligations to our licensors or other parties, or decrease financial or other obligations of our licensees and collaborators. The agreements under which we currently license intellectual property or technology from or to third parties, including the AbbVie Collaboration and License Agreement **, the Nippon Shinyaku Collaboration and License Agreement and our license agreements with GSK and Penn** , are complex, and certain provisions in such agreements may be susceptible to multiple interpretations. The resolution of any contract interpretation disagreement that may arise could narrow what we believe to be the scope of our rights to the relevant intellectual property or technology, increase what we believe to be our financial or other obligations under the relevant agreement, or decrease what we believe to be the financial or other obligations of our licensee under the relevant agreement, any of which could materially harm our business, financial

condition, results of operations and prospects. Moreover, if disputes over intellectual property that we have licensed prevent or impair our ability to maintain our current licensing arrangements or obtain additional licensing arrangements on commercially acceptable terms, we may be unable to successfully develop and commercialize the affected products or product candidates, which could have a material adverse effect on our business, financial conditions, results of operations, and prospects. If we fail to comply with our obligations in the agreements under which we license intellectual property rights from third parties or otherwise experience disruptions to our business relationships with our licensors, we could lose license rights that are important to our business. We have entered into license agreements with third parties and may need to obtain additional licenses from others to advance our research, to expand our licensing program or to allow commercialization of our product candidates. It is possible that we may be unable to obtain additional licenses at a reasonable cost or on reasonable terms, if at all. In that event, we may be required to expend significant time and resources to redesign our technology or product candidates or the methods for manufacturing them or to develop or license replacement technology, all of which may not be feasible on a technical or commercial basis. If we are unable to do so, we may be unable to redesign our platform technology or to develop or commercialize the affected product candidates, which could materially harm our business. We cannot provide any assurances that third- party patents do not exist or will not be issued, which might be enforced against our current platform technology, manufacturing methods, product candidates or future methods or products, resulting in either an injunction prohibiting our licensing, manufacture or sales, or, with respect to our sales, an obligation on our part to pay royalties and / or other forms of compensation to third parties. In many of our existing license agreements, patent prosecution of our licensed technology is controlled primarily by the licensor, and we are required to reimburse the licensor for certain costs of patent prosecution and maintenance. If our licensors fail to obtain and maintain patent or other protection for the proprietary intellectual property we license from them, 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. Further, in our license agreements, we could be responsible for bringing actions against any third party for infringing on the patents we have licensed. Certain of our license agreements in which we are the licensee also require us to meet development milestones to maintain the license, including establishing a set timeline for developing and commercializing products and minimum diligence obligations in developing and commercializing the product. Disputes may arise regarding intellectual property subject to a licensing agreement, including: • the scope of rights granted under the license agreement and other interpretation- related issues; • the extent to which our technology and processes infringe on or otherwise violate intellectual property of the licensor that is not subject to the licensing agreement; • the sublicensing and corresponding payment obligations of patent and other intellectual property rights under our collaborative development relationships; • our diligence obligations under the license agreement and what activities satisfy those diligence obligations; • the inventorship or ownership of inventions and know- how resulting from the joint creation or use of intellectual property by our licensors and us and our partners; and • the priority of invention of patented technology. If disputes over intellectual property that we have licensed prevent or impair our ability to maintain our current licensing arrangements on acceptable terms, we may be unable to successfully develop and commercialize the affected product candidates. We currently have rights to intellectual property, through licenses from third parties, to develop our product candidates. Because our programs may require the use of intellectual property or other proprietary rights held by third parties, the growth of our business may depend, in part, on our ability to acquire, in- license or use such intellectual property and proprietary rights. We may be unable to acquire or in- license any compositions, methods of use, processes (and patents for such technology) or other intellectual property rights from third parties that we identify as necessary for our technology platform and product candidates. The licensing or acquisition of third- party intellectual property rights is a competitive area, and several more established companies may pursue strategies to license or acquire third- party intellectual property rights that we may consider attractive. These established companies may have a competitive advantage over us due to their size, capital resources and greater clinical development and commercialization capabilities. In addition, companies that perceive us to be a competitor may be unwilling to license rights to us. We also may be unable to license or acquire third party intellectual property rights on terms that would allow us to make an appropriate return on our investment. We sometimes collaborate with non- profit and academic institutions to accelerate our preclinical research or development under written agreements with these institutions. Some of these institutions may provide us with an option to negotiate a license to any of the institution' s rights in technology resulting from the collaboration. Regardless of such option, we may be unable to negotiate a license within the specified timeframe or under terms that are acceptable to us. If we are unable to do so, the institution may offer the intellectual property rights to other parties, potentially blocking our ability to pursue our program. If we are unable to successfully obtain rights to required third- party intellectual property rights or maintain the existing intellectual property rights we have, we may have to abandon development of the relevant program or product candidate and our business, financial condition, results of operations and prospects could suffer. Filing, prosecuting and defending patents on our platform technology or product candidates in all countries throughout the world would be prohibitively expensive, and our intellectual property rights in some countries outside the United States could be less extensive than those in the United States. Although our license agreements with GSK and Penn grant us worldwide rights, certain of our in- licensed U. S. patent rights lack corresponding foreign patents or patent applications. In addition, the laws of some foreign countries do not protect intellectual property rights to the same extent as federal and state laws in the United States. Consequently, we may not be able to prevent third parties from practicing our inventions in all countries outside the United States, or from selling or importing products made using our inventions in and into the United States or other jurisdictions. Competitors may use our technologies in jurisdictions where we have not obtained patent protection to develop their own products and, further, may export otherwise infringing products to territories where we have patent protection, but enforcement is not as strong as that in the United States. These products may compete with our products and our patents or other intellectual property rights may not be effective or sufficient to prevent them from competing. Many companies have encountered significant problems in protecting and defending intellectual property rights in foreign jurisdictions. The legal

systems of certain countries, particularly certain developing countries, do not favor the enforcement of patents, trade secrets and other intellectual property protection, particularly those relating to biotechnology products, 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 jurisdictions could result in substantial costs and divert our efforts and attention from other aspects of our business, could put our patents at risk of being invalidated or interpreted narrowly and our patent applications at risk of not issuing and could provoke third parties to assert claims against us. We may not prevail in any lawsuits that we initiate and the damages or other remedies awarded, if any, may not be commercially meaningful. Accordingly, our efforts to enforce our intellectual property rights around the world may be inadequate to obtain a significant commercial advantage from the intellectual property that we develop or license. In this regard, we are engaged in patent litigation with Sarepta Therapeutics, Inc. (Sarepta) arising from its use of cultured host cell technology, which we believe is claimed in a patent we licensed from Penn, to make gene therapy products to treat Duchenne muscular dystrophy and Limb- girdle muscular dystrophy, among other products. In January 2024 **,** the U. S. District Court for the District of Delaware granted Sarepta' s motion for summary judgment dismissing the case. Although we have appealed this decision our litigation against Sarepta will have an uncertain outcome and may not result in the patent enforcement we desire. Competitors may infringe our patents or the patents of our licensing partners, or we may be required to defend against claims of infringement or that our intellectual property is invalid or unenforceable. To counter infringement or unauthorized use claims or to defend against claims of infringement or other intellectual property related claims can be expensive and time consuming. Even if resolved in our favor, litigation or other legal proceedings relating to intellectual property claims may cause us to incur significant expenses, and could distract our technical and management personnel from their normal responsibilities. In addition, there could be public announcements of the results of hearings, motions or other interim proceedings or developments and if securities analysts or investors perceive these results to be negative, it could materially harm the price of our common stock. Such litigation or proceedings could substantially increase our operating losses and reduce the resources available for development activities or any future sales, marketing or distribution activities. We may not have sufficient financial or other resources to adequately conduct such litigation or proceedings. Some of our competitors may be able to sustain the costs of such litigation or proceedings more effectively than we can because of their greater financial resources and more mature and developed intellectual property portfolios. An adverse result in any litigation proceeding could put one or more of our patents or licensed patents at risk of being invalidated, held unenforceable or interpreted narrowly, and could put any of our patent applications at risk of not yielding an issued patent. 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 or trade secrets could be compromised by disclosure during this type of litigation. Uncertainties resulting from the initiation and continuation of patent and other intellectual property litigation or proceedings could materially harm our ability to compete in the marketplace. Periodic maintenance fees, renewal fees, annuity fees and various other government fees on patents and / or applications will be due to be paid to the U. S. Patent and Trademark Office (the USPTO) and various patent agencies outside of the United States over the lifetime of our licensed patents and / or applications and any patent rights we may own or license in the future. We may rely on our licensing partners to pay these fees due to non- U. S. patent agencies with respect to our licensed patent rights. The USPTO and various non- U. S. patent agencies require compliance with several procedural, documentary, fee payment and other similar provisions during the patent application process. We employ reputable law firms and other professionals to help us comply and we are also dependent on our licensors to take the necessary action to comply with these requirements with respect to our licensed intellectual property. In many cases, an inadvertent lapse can be cured by payment of a late fee or by other means in accordance with the applicable rules. There are situations, however, in which non- compliance can result in abandonment or lapse of the patent or patent application, resulting in partial or complete loss of patent rights in the relevant jurisdiction. In such an event, potential competitors might be able to enter the market and this circumstance could materially harm our business. If we or our licensors fail to maintain the patents and patent applications covering our product candidates, it could have a material adverse effect on our business. We have registered trademarks with the USPTO, including for the marks " AAVIATE, " **"** AFFINITY **," "** **AFFINITY BEYOND** ," " AFFINITY DUCHENNE, " " ALTITUDE, " " ATMOSPHERE, " " CAMPSIITE, " " NAV, " **"** ~~NAVXCELL~~ **" NAVXcell ", " NAVXpress** " and " REGENXBIO, " as well as for the REGENXBIO logos. Our trademarks or trade names may be challenged, infringed, circumvented or declared generic or determined to be infringing on other marks. We may not be able to protect our rights to these trademarks and trade names, which we need to build name recognition among potential partners or customers in our markets of interest. At times, competitors may adopt trade names or trademarks similar to ours, thereby impeding our ability to build brand identity and possibly leading to market confusion. In addition, there could be potential trade name or trademark infringement claims brought by owners of other registered trademarks or trademarks that incorporate variations of our registered or unregistered trademarks or trade names. Over the long- term, if we are unable to establish name recognition based on our trademarks and trade names, then we may not be able to compete effectively and our business may be harmed. Our efforts to enforce or protect our proprietary rights related to trademarks, trade secrets, domain names, copyrights or other intellectual property may be ineffective and could result in substantial costs and diversion of resources and could harm our financial condition or results of operations. Many countries have compulsory licensing laws under which a patent owner may be compelled to grant licenses to third parties. In addition, many countries limit the enforceability of patents against government agencies or government contractors. In these countries, the patent owner may have limited remedies, which could materially diminish the value of such patent. If we or any of our licensors is forced to grant a license to third parties with respect to any patents relevant to our business, our competitive position may be impaired, and our business, financial condition, results of operations, and prospects may be adversely affected. Issued patents covering our NAV Technology Platform or our product candidates could be found invalid or unenforceable if challenged in court. We may not be able to protect our trade secrets in court. If one of our licensing partners or we initiate legal proceedings against a third party to enforce a patent

covering our NAV Technology Platform or one of our product candidates, the defendant could counterclaim that the patent covering our product candidate is invalid or unenforceable. In patent litigation in the United States, defendant counterclaims alleging invalidity or unenforceability are commonplace. Grounds for a validity challenge could be an alleged failure to meet any of several statutory requirements, including subject- matter eligibility, novelty, non- obviousness, written description or enablement. Grounds for an unenforceability assertion could be an allegation that someone connected with prosecution of the patent withheld information material to patentability from the USPTO, or made a misleading statement, during prosecution. Third parties also may raise similar claims before administrative bodies in the United States or abroad, even outside the context of litigation. Such mechanisms include re- examination, post grant review, inter partes review and equivalent proceedings in foreign jurisdictions. Such proceedings could result in the revocation or cancellation of or amendment to our patents in such a way that they no longer cover our NAV Technology Platform or our product candidates. The outcome following legal assertions of invalidity and unenforceability is unpredictable. With respect to the validity question, for example, we cannot be certain that there is no invalidating prior art, of which the patent examiner and we or our licensing partners were unaware during prosecution. If a defendant were to prevail on a legal assertion of invalidity or unenforceability, we could lose at least part, and perhaps all, of the patent protection on one or more of our product candidates. Such a loss of patent protection could materially harm our business. In addition to the protection afforded by patents, we rely on trade secret protection and confidentiality agreements to protect proprietary know- how that is not patentable or that we elect not to patent, processes for which patents are difficult to enforce and any other elements of our technology, product candidate discovery and development processes that involve proprietary know- how, information or technology that is not covered by patents. However, trade secrets can be difficult to protect and some courts inside and outside the United States are less willing or unwilling to protect trade secrets. We seek to protect our proprietary technology and processes, in part, by entering into confidentiality agreements with our employees, consultants, scientific advisors and contractors. We cannot guarantee that we have entered into such agreements with each party that may have or have had access to our trade secrets or proprietary technology and processes. We also seek to preserve the integrity and confidentiality of our data and trade secrets by maintaining physical security of our premises and physical and electronic security of our information technology systems. While we have confidence in these individuals, organizations and systems, agreements or security measures may be breached, and we may not have adequate remedies for any breach. In addition, our trade secrets may otherwise become known or be independently discovered by competitors. Third parties may initiate legal proceedings alleging that we are infringing their intellectual property rights, the outcome of which would be uncertain and could materially harm our business. Our commercial success depends, in part, upon our ability to license our NAV Technology Platform, and upon our ability and our licensees' and collaborators' ability to develop, manufacture, market and sell products and use our proprietary technologies without infringing or otherwise violating the proprietary rights and intellectual property of third parties. The biotechnology and pharmaceutical industries are characterized by extensive and complex litigation regarding patents and other intellectual property rights. We **or our collaborators** may in the future become party to, or be threatened with, adversarial proceedings or litigation regarding intellectual property rights with respect to our product candidates and technology, including interference proceedings, post grant review and inter partes review before the USPTO. Third parties may assert infringement claims against us **or our collaborators** based on existing patents or patents that may be granted in the future, regardless of their merit. There is a risk that third parties may choose to engage in litigation with us **or our collaborators** to enforce or to otherwise assert their patent rights against us. Even if we believe such claims are without merit, a court of competent jurisdiction could hold that these third- party patents are valid, enforceable and infringed, which could materially harm our ability to license our technology platform or commercialize our lead product candidates or any future product candidates or technologies covered by the asserted third- party patents. In order to successfully challenge the validity of any such U. S. patent in federal court, we would need to overcome a presumption of validity. As this burden is a high one requiring us to present clear and convincing evidence as to the invalidity of any such U. S. patent claim, there is no assurance that a court of competent jurisdiction would invalidate the claims of any such U. S. patent. If we are found to infringe a third party' s valid and enforceable intellectual property rights, we could be required to obtain a license from such third party to continue licensing, developing, manufacturing and marketing our product candidates and technology. However, we may not be able to obtain any required license on commercially reasonable terms or at all. Even if we were able to obtain a license, it could be non- exclusive, thereby giving our competitors and other third parties access to the same technologies licensed to us, and it could require us to make substantial licensing and royalty payments. We **or our collaborators** could be forced, including by court order, to cease licensing, developing, manufacturing and commercializing the infringing technology or product candidates. In addition, we could be found liable for monetary damages, including treble damages and attorneys' fees, if we are found to have willfully infringed a patent or other intellectual property right **or if our collaborators are found to have willfully infringed a patent or other intellectual property right, for which we may be required to indemnify our collaborators** . A finding of infringement could prevent us from licensing our technology platform or manufacturing and commercializing our product candidates or force us to cease some of our business operations, which could materially harm our business. Claims that we have misappropriated the confidential information or trade secrets of third parties could similarly harm our business, financial condition, results of operations and prospects. We may be subject to claims asserting that our employees, consultants or advisors have wrongfully used or disclosed alleged trade secrets of their current or former employers or claims asserting ownership of what we regard as our own intellectual property. Many of our employees, consultants or advisors are currently, or were previously, employed at universities or other biotechnology or pharmaceutical companies, including our competitors or potential competitors. Although we try to ensure that our employees, consultants and advisors do not use the proprietary information or know- how of others in their work for us, we may be subject to claims that these individuals or we have used or disclosed intellectual property, including trade secrets or other proprietary information, of any such individual' s current or former employer. Litigation may be necessary to defend against these claims. If we fail in defending any such claims, in addition to paying monetary damages, we

may lose valuable intellectual property rights or personnel. Even if we are successful in defending against such claims, litigation could result in substantial costs and be a distraction to management. In addition, while it is our policy to require our employees and contractors who may be involved in the conception or development of intellectual property to execute agreements assigning such intellectual property to us, we may be unsuccessful in executing such an agreement with each party who, in fact, conceives or develops intellectual property that we regard as our own. The assignment of intellectual property rights may not be self-executing or the assignment agreements may be breached, and we may be forced to bring claims against third parties, or defend claims that they may bring against us, to determine the ownership of what we regard as our intellectual property. The patent positions of companies engaged in the development and commercialization of biologics and pharmaceuticals are particularly uncertain. On March 20, 2012, the Supreme Court issued a decision in Mayo Collaborative Services v. Prometheus Laboratories, Inc. (Prometheus), a case involving patent claims directed to a process of measuring a metabolic product in a patient to optimize a drug dosage for the patient. According to the Supreme Court, the addition of well- understood, routine or conventional activity such as " administering " or " determining " steps was not enough to transform an otherwise patent-ineligible natural phenomenon into patent- eligible subject matter. On June 13, 2013, the Supreme Court issued its decision in Association for Molecular Pathology v. Myriad Genetics, Inc. (Myriad), a case involving patent claims held by Myriad relating to the breast cancer susceptibility genes BRCA1 and BRCA2. Myriad held that an isolated segment of naturally occurring DNA, such as the DNA constituting the BRCA1 and BRCA2 genes, is not patent eligible subject matter, but that complementary DNA, which is an artificial construct that may be created from RNA transcripts of genes, may be patent eligible. The USPTO has issued a number of guidance memoranda and updates to instruct USPTO examiners on the ramifications of the Prometheus, Myriad and other court rulings and the application of the rulings to natural products and principles including all naturally occurring nucleic acids. USPTO guidance may be further updated in view of developments in the case law and in response to public feedback. Patents for certain of our product candidates contain claims related to specific DNA sequences that are naturally occurring and, therefore, could be the subject of future challenges made by third parties. In addition, USPTO guidance or changes in guidance or procedures issued by the USPTO could make it impossible for us to pursue similar patent claims in patent applications we may prosecute in the future. We cannot assure you that our efforts to seek patent protection for our technology and products will not be negatively impacted by the decisions described above, rulings in other cases or changes in guidance or procedures issued by the USPTO. We cannot fully predict what ongoing impact the Supreme Court' s decisions in Prometheus and Myriad may have on the ability of life science companies to obtain or enforce patents relating to their products and technologies in the future. These decisions, the guidance issued by the USPTO and rulings in other cases or changes in USPTO guidance or procedures could materially harm our existing patent portfolio and our ability to protect and enforce our intellectual property in the future. Moreover, although the Supreme Court has held in Myriad that isolated segments of naturally occurring DNA are not patent- eligible subject matter, certain third parties could allege that activities that we may undertake infringe other gene- related patent claims, and we may deem it necessary to defend ourselves against these claims by asserting non- infringement and / or invalidity positions, or paying to obtain a license to these claims. In any of the foregoing or in other situations involving third- party intellectual property rights, if we are unsuccessful in defending against claims of patent infringement, we could be forced to pay damages or be subjected to an injunction that would prevent us from utilizing the patented subject matter. Such outcomes could harm our business, financial condition, results of operations or prospects. If we do not obtain patent term extension and data exclusivity for our product candidates, our business may be materially harmed. Depending upon the timing, duration and specifics 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 Hatch- Waxman Amendments. The Hatch-Waxman Amendments permit a patent extension term of up to five years as compensation for patent term lost during the FDA regulatory review process. A patent term extension cannot extend the remaining term of a patent beyond a total of 14 years from the date of product approval, only one patent may be extended and only those claims covering the approved drug, a method for using it or a method for manufacturing it may be extended. However, we may not be granted an extension because of, for example, failing to exercise due diligence during the testing phase or regulatory review process, failing to apply within applicable deadlines, failing to apply prior to expiration of relevant patents or otherwise failing to satisfy applicable requirements. Moreover, the applicable time period or the scope of patent protection afforded could be less than we request. If we are unable to obtain patent term extension or the term of any such extension is less than we request, our competitors may obtain approval of competing products following our patent expiration, and our revenue could be reduced, possibly materially. Further, for our licensed patents, we may not have the right to control prosecution, including filing with the U. S. Patent and Trademark Office, a petition for patent term extension under the Hatch- Waxman Act. Thus, if one of our licensed patents is eligible for patent term extension under the Hatch- Waxman Act, we may not be able to control whether a petition to obtain a patent term extension is filed, or obtained, from the U. S. Patent and Trademark Office. Our operating results may fluctuate substantially, which makes our future operating results difficult to predict and could cause the price of our common stock to fluctuate substantially. We expect our operating results to be subject to fluctuations. Our net income or loss and other operating results may be affected by numerous factors, including: • any variations in the level of expenses related to our NAV Technology Platform, lead product candidates or future product candidates and technologies; • the addition or termination of any clinical trials and the timing and outcomes of clinical trials; • any regulatory or clinical developments affecting our lead product candidates, any future product candidates or our licensees' product candidates; • our execution of any collaborative, licensing or similar arrangements and the timing of any payments we may make or receive under these arrangements; • changes in the competitive landscape of our industry, including consolidation among our competitors or partners; • the nature and terms of any stock- based compensation grants; • any intellectual property infringement lawsuits in which we may become involved; • our ability to adequately support future growth; • potential unforeseen business disruptions that increase our costs or expenses; • future accounting pronouncements or changes in our accounting policies; and • the changing and volatile global economic

environment. The cumulative effect of these factors could result in large fluctuations and unpredictability in our quarterly and annual operating results. As a result, we believe that comparing our operating results on a period- to- period basis is not necessarily meaningful. Investors should not rely on our past results as an indication of our future performance. This variability and unpredictability could also result in our ~~failing~~ **failure** to meet the expectations of securities or industry analysts or investors for any period. If our operating results fall below the expectations of investors or analysts, the price of our common stock could decline substantially. Furthermore, any quarterly or annual fluctuations in our operating results may, in turn, cause the price of our stock to fluctuate substantially. Such a stock price decline could occur even when we have met any previously publicly stated revenue or earnings guidance we have provided. We have raised significant capital through public offerings of our common stock in order to fund our operations, which has caused dilution to our stockholders. We may seek to raise additional capital through public or private equity offerings, debt financings, strategic partnerships, licensing arrangements or other means. We have an effective shelf registration statement on file with the SEC, which allows us to access capital in a timely manner. To the extent that we raise additional capital by issuing equity securities, including through our at- the- market program, the share ownership of existing stockholders will be diluted. Any future debt financing may involve covenants that restrict our operations, including limitations on our ability to incur liens or additional debt, pay dividends, redeem our stock, make certain investments or engage in certain merger, consolidation, or asset sale transactions. In addition, if we seek funds through arrangements with collaborative partners, these arrangements may require us to relinquish rights to some of our technologies or products or otherwise agree to terms unfavorable to us. If we engage in future acquisitions or strategic partnerships, this may increase our capital requirements, dilute our stockholders, cause us to incur debt or assume contingent liabilities and subject us to other risks. We may evaluate various acquisitions and strategic partnerships, including licensing or acquiring complementary products, intellectual property rights, technologies, or businesses. Any potential acquisition or strategic partnership may entail numerous risks, including: • increased operating expenses and cash requirements; • the assumption of additional indebtedness or contingent liabilities; • the issuance of our equity securities; • assimilation of operations, intellectual property and products of an acquired company, including difficulties associated with integrating new personnel; • the diversion of our management ~~'~~**'** s attention from our existing product programs and initiatives in pursuing such a strategic merger or acquisition; • retention of key employees, the loss of key personnel, and uncertainties in our ability to maintain key business relationships; • risks and uncertainties associated with the other party to such a transaction, including the prospects of that party and their existing products or product candidates and regulatory approvals; and • our inability to generate revenue from acquired technology and / or products sufficient to meet our objectives in undertaking the acquisition or even to offset the associated acquisition and maintenance costs. In addition, if we undertake acquisitions, we may issue dilutive securities, assume or incur debt obligations, incur large one- time expenses and acquire intangible assets that could result in significant future amortization expense. Moreover, we may not be able to locate suitable acquisition opportunities and this inability could impair our ability to grow or obtain access to technology or products that may be important to the development of our business. Provisions in our ~~restated~~ certificate of incorporation and ~~amended and restated~~ bylaws and under Delaware law might discourage, delay or prevent a change in control of our company or changes in our board of directors and, therefore, depress our stock price. Our restated certificate of incorporation and amended and restated bylaws contain provisions that could depress the market price of our common stock by acting to discourage, delay or prevent a change in control of our company or changes in our board of directors that the stockholders of our company may deem advantageous. Among other things, these provisions: • establish a classified board of directors so that not all members of our board are elected at one time; • permit the board of directors to establish the number of directors; • provide that directors may only be removed " for cause " **and only upon the vote of the holders of at least two- thirds of our outstanding shares** ; • require super- majority voting to amend some provisions in our restated certificate of incorporation and amended and restated bylaws; • authorize the issuance of " blank check " preferred stock that our board of directors could use to implement a stockholder rights plan; • eliminate the ability of our stockholders to call special meetings of stockholders; • prohibit stockholder action by written consent, which requires all stockholder actions to be taken at a meeting of our stockholders; • provide that the board of directors is expressly authorized to adopt, amend or repeal our bylaws; and • establish advance notice requirements for nominations for election to our board of directors or for proposing matters that can be acted upon by stockholders at annual stockholder meetings. In addition, Section 203 of the Delaware General Corporation Law may discourage, delay or prevent a change in control of our company. Section 203 imposes certain restrictions on merger, business combinations and other transactions between us and holders of 15 % or more of our common stock. Our restated certificate of incorporation includes exclusive forum clauses for certain litigation that may be initiated by our stockholders, which could limit our stockholders' ability to obtain a favorable judicial forum for disputes with us or our directors, officers or employees. Pursuant to our restated certificate of incorporation, unless we consent in writing to the selection of an alternative forum, the Court of Chancery of the State of Delaware (or, if the Court of Chancery does not have jurisdiction, the federal district court for the District of Delaware), will be the sole and exclusive forum for (i) any derivative action or proceeding brought on our behalf, (ii) any action asserting a claim of breach of a fiduciary duty owed by any of our directors, officers or other employees to us or our stockholders, (iii) any action asserting a claim arising pursuant to any provision of the Delaware General Corporation Law, our restated certificate of incorporation or our amended and restated bylaws or (iv) any action asserting a claim governed by the internal affairs doctrine. Additionally, if the subject matter of any action within the scope of the preceding sentence is filed in a court other than a court located with the State of Delaware (a Foreign Action) in the name of any stockholder, such stockholder shall be deemed to have consented to (a) the personal jurisdiction of the state and federal courts located within the State of Delaware in connection with any action brought in any such court to enforce the preceding sentence and (b) having service of process made upon such stockholder in any such action by service upon such stockholder' s counsel in the Foreign Action as agent for such stockholder. Additionally, our restated certificate of incorporation provides that, unless we consent in writing to the selection of an alternative forum, the federal district

courts of the United States of America shall, to the fullest extent permitted by law, be the exclusive forum for the resolution of any complaint asserting a cause of action arising under the Securities Act. Any person or entity purchasing or otherwise acquiring any interest in any of our securities shall be deemed to have notice of and to have consented to this provision. The forum selection clauses in our restated certificate of incorporation may limit our stockholders' ability to obtain a favorable judicial forum for disputes with us, our directors, officers or other employees. Alternatively, if a court were to find the choice of forum provisions contained in our restated certificate of incorporation to be inapplicable or unenforceable in an action, we may incur additional costs associated with resolving such action in other jurisdictions, which could adversely affect our business and financial condition. Proxy contests have been waged against many companies in the biopharmaceutical industry over the last several years, and proxy advisory firms or investors may recommend changes to our business operations, provisions in our restated certificate of incorporation or amended and restated bylaws, or the composition of our board of directors or its committees. If faced with a proxy contest or other type of stockholder activism, or a proxy advisory firm recommendation that is adverse to a management proposal, we may not be able to respond successfully to the contest or dispute, which could be disruptive to our business. Even if we are successful, our business could be adversely affected by such a contest or dispute involving us or our partners because: • responding to proxy contests or other actions by activist stockholders, or adverse proxy advisory firm recommendations, can be costly and time- consuming, disrupting operations and diverting the attention of management and employees; • perceived uncertainties as to future direction may result in the loss of potential acquisitions, collaborations or licensing opportunities, and may make it more difficult to attract and retain qualified personnel and business partners; and • if individuals are elected to our board of directors with a specific agenda, it may adversely affect our ability to effectively and timely implement our strategic plan and create additional value for our stockholders. These actions could cause our stock price to decrease and experience periods of increased volatility.