Risk Factors Comparison 2025-03-24 to 2024-03-18 Form: 10-K

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Investing in shares of our common stock involves a high degree of risk. You should carefully consider the following risks and uncertainties, together with all of the other information contained in this Annual Report on Form 10- K before making an investment decision. The occurrence of any of the following risks could materially and adversely affect our business, financial condition, reputation, or results of operations. In such case, the trading price of shares of our common stock could decline, and you may lose all or part of your investment. It is not possible to predict or identify all such risks; our operations could also be affected by factors, events or uncertainties that are not presently known to us or that we currently do not consider to present significant risks to our operations. Therefore, you should not consider the following risks to be a complete statement of all the potential risks or uncertainties that we face. **Moreover, some of the factors, events and contingencies discussed below may have occurred in the past, but the disclosures below are not representations as to whether or not the factors, events or contingencies have occurred in the past, and instead reflect our beliefs and opinions as to the factors, events or contingencies that could materially and adversely affect us in the future.** Summary of Risk Factors • We have a limited operating history, have not completed any clinical trials, and have no products approved for commercial sale, and our results may vary from quarter to quarter. • We will require substantial additional capital to finance our operations in the future. If we are unable to raise such capital when needed, or on acceptable terms, we may be forced to delay, reduce or eliminate clinical trials, product development programs or future commercialization efforts. • We have incurred significant losses since inception, ~~and~~ expect to incur significant losses for the foreseeable future and may not be able to achieve or sustain profitability in the future. We have no products for sale, have not generated any product revenue and may never generate product revenue or become profitable. • NGN- 401 ~~, NGN- 101~~ and our other programs are in early stages of development and may fail in development or suffer delays that materially and adversely affect their commercial viability **. If we or our current or future collaborators are unable to complete development of, or commercialize, our product candidates, or experience significant delays in doing so, our business will be materially harmed** . • We are substantially dependent on the success of our most advanced product ~~candidates~~ **candidate** , NGN- 401 ~~and NGN- 101~~ , and our ongoing and anticipated clinical trials of ~~such candidates~~ **NGN- 401** may not be successful. • Delays in developing our manufacturing capabilities or failure to achieve operating efficiencies from such capabilities may require us to devote additional resources and management time to manufacturing operations and may delay our product development timelines. • We have a number of academic collaborations, and currently rely on our collaboration with the University **Court of the University** of Edinburgh for certain aspects of our preclinical research and development programs, including working in collaboration to discover and preclinically develop **potential** ~~our lead~~ product ~~candidate~~ **candidates** for ~~Rett syndrome and~~ our near- term future pipeline. Failure or delay of the University of Edinburgh or any other collaborator to fulfil all or part of its obligations under our ~~agreement~~ **agreements** , a breakdown in collaboration between the parties or a complete or partial loss of the relationship would materially harm our business. • In order to successfully implement our plans and strategies, we will need to grow the size of our organization and we may experience difficulties in managing this growth. • The regulatory approval processes of the U. S. Food and Drug Administration (“ FDA ”) and other comparable foreign regulatory authorities are lengthy, time- consuming and inherently unpredictable. If we are not able to obtain, or if there are delays in obtaining, required regulatory approvals for our product candidates, we will not be able to commercialize, or will be delayed in commercializing, such product candidates, and our ability to generate revenue will be materially impaired. • The market price of our common stock may continue to be volatile. • ~~If our~~ **We may be required to allocate resources to fulfilling the requirements of the Contingent Value Rights Agreement entered into in connection with the Reverse Merger related to certain** legacy lease obligations **which** ~~are not subleased, assigned, terminated or otherwise addressed or the legacy assets subject to the CVR Agreement are not sold, respectively, in a timely manner, we~~ may ~~have to incur time and resources to~~ take ~~such actions~~ **away from our core programs and create a distraction for our management and employees** . • Future sales of shares by existing stockholders could cause our stock price to decline. • Our executive officers, directors and principal stockholders have the ability to control or significantly influence all matters submitted to our stockholders for approval. Risks Related to **Our** ~~Neurogene’ s~~ Limited Operating History, Financial Position and Capital Requirements We are a clinical- stage biotechnology company with limited operating history. Since our inception in 2018, we have incurred significant operating losses and have used substantially all of our resources to conduct research and development activities, preclinical studies and Phase 1 / 2 clinical trials of our most advanced product candidates, establish in- house manufacturing capabilities, including analytical and process development operations to support ongoing manufacturing operations, manufacture product candidates, conduct business planning, develop and maintain our intellectual property portfolio, hire personnel, raise capital, and provide general and administrative support for these activities. We have ~~little~~ **limited** experience as a company in initiating, conducting or completing clinical trials. In part because of this lack of experience, we cannot be certain that our current and planned clinical trials will begin **on time, meet our anticipated timelines for enrollment and data analysis,** or be completed on time, if at all. In addition, while we are conducting a Phase 1 / 2 clinical trial of NGN- 401 in patients with Rett syndrome and **have completed enrollment in** a Phase 1 / 2 clinical trial of NGN- 101 in patients with CLN5 Batten disease, we have not yet demonstrated our ability to successfully complete clinical trials (including Phase 3 or other pivotal clinical trials), obtain regulatory or marketing approvals, manufacture a commercial- scale product or arrange for a third party to do so on our behalf, or conduct sales, marketing and distribution activities necessary for successful product commercialization. Additionally, we expect our financial condition and operating results to continue to fluctuate

significantly from period to period due to a variety of factors, many of which are beyond our control. Consequently, any predictions made about our future success or viability may not be as accurate as they could be if we had a longer operating history. In addition, as our business grows, we may encounter unforeseen expenses, restrictions, difficulties, complications, delays and other known and unknown factors. We will need to transition at some point from a company with an early research and development focus to a company capable of supporting larger pivotal clinical trials and eventually commercial activities, including the manufacture of commercial scale product. We may not be successful in such a transition. Developing biotechnology products is a long, time- consuming, expensive and uncertain process that takes years to complete. Since our inception, we have funded our operations primarily through private financings and have incurred significant recurring losses, including **a cumulative** net ~~losses~~ -- **loss from inception through December 31, 2024** of $ ~~36~~ **262** . 3 million ~~and $ 55. 2 million for the years ended December 31, 2023 and 2022, respectively~~ . We expect our expenses to increase in connection with our ongoing activities, particularly as we continue to conduct a Phase 1 / 2 clinical trial of NGN- 401 in patients with Rett syndrome ~~and a Phase 1 / 2 clinical trial of NGN- 101 in patients with CLN5 Batten disease~~ , with the expectation that we will also initiate additional clinical trials in the future, and continue to research, develop and conduct preclinical studies of our other potential product candidates. **We also anticipate that we may have near term expenses related to NGN- 101 as we continue to evaluate options for the program following the denial by the FDA of an RMAT designation, which precludes our ability to use a streamlined registrational pathway necessary for further investment in the program.** In addition, if we obtain regulatory approval for any product candidate for commercial sale, including NGN- 401 ~~and NGN- 101~~ , we anticipate incurring significant commercialization expenses related to product manufacturing, marketing, sales and distribution activities to launch any such product. Our expenses could increase beyond expectations if we are required by the FDA or other regulatory agencies to perform preclinical studies or clinical trials in addition to those that we currently anticipate. Because the design and outcome of our current, planned and anticipated clinical trials are highly uncertain, we cannot reasonably estimate the actual amount of funding that will be necessary to successfully complete the development and commercialization of any product candidate we develop. Our future capital requirements depend on many factors, including factors that are not within our control. We **have incurred and expect to continue to** incur additional costs associated with operating as a public company, and we do not anticipate achieving any significant revenue in the near term given the development stage of our product candidates. Accordingly, we will require substantial additional funding to continue our operations. Based on our current operating plan, we believe that our existing cash, cash equivalents and short- term investments should be sufficient to fund ~~its~~ **our** operations into the second half of ~~2026~~ **2027** . This estimate is based on assumptions that may prove to be materially wrong, and we could deplete our available capital resources sooner than we currently expect. Our future capital requirements will depend on many factors, including: • the timing and progress of preclinical and clinical development activities **, including any impact to our NGN- 401 clinical trial activities relating to our participation in the FDA' s Support for clinical Trials Advancing Rare disease Therapeutics (" START ") program and the Regenerative Medicine Advanced Therapy (" RMAT ") program** ; • the number and scope of preclinical and clinical programs we pursue to develop our gene therapy candidate pipeline and EXACT **(Expression Attenuation via Construct Timing)** platform; • our ability to secure appropriate animal models for the conduct of investigational new drug (" IND ")- enabling studies in a timely and financially feasible manner, especially large animal models, such as non- human primates (" NHPs ") needed for toxicology studies; • our ability to establish an acceptable safety profile with IND- enabling toxicology studies to enable clinical trials; • successful patient enrollment in, and the initiation and completion of, larger and later- stage clinical trials; • the number of subjects that participate in clinical trials and per subject trial costs; • the number and extent of trials required for regulatory approval; • the countries in which the trials are conducted; • the length of time required to enroll eligible subjects in clinical trials; • the drop- out and discontinuation rate of subjects; • potential additional safety monitoring requested by regulatory agencies; • the duration of subject participation in the trials and follow- up; • the extent to which we encounter any serious adverse events in our clinical trials; • the timing of receipt of regulatory approvals from applicable regulatory authorities, including those required to initiate clinical trials; • the timing, receipt and terms of any marketing approvals and post- marketing approval commitments from applicable regulatory authorities; • the extent to which we establish collaborations, strategic partnerships, or other strategic arrangements with third parties, if any, and the performance of any such third party; • the scale up of our clinical and regulatory capabilities, including establishing our current good manufacturing practices (" cGMP ") manufacturing capabilities to support expansion of our pipeline and future registration- enabling clinical trials, and obtaining cGMP material for clinical trials or potential commercial sales; • hiring and retaining research, clinical, regulatory, manufacturing (including quality control and quality assurance) and administrative personnel; • our arrangements with third- party contract development and manufacturing organizations (" CDMOs ") and contract research organizations (" CROs "); • the **outfitting** ~~build- out~~ and validation of our cGMP manufacturing facility ~~, including expansion to commercial scale~~ ; • the impact of any business interruptions to our operations or to those of the third parties with whom we work; and • obtaining, maintaining, defending and enforcing patent claims and other intellectual property rights. We do not have any committed external sources of funds **,** and adequate additional financing may not be available to us on acceptable terms, or at all. We may be required to seek additional funds sooner than planned through public or private equity offerings, debt financings, collaborations and licensing arrangements or other sources. Such financing may dilute our stockholders or the failure to obtain such financing may restrict our operating activities. Any additional fundraising efforts may divert our management from their day- to- day activities, which may adversely affect our business. To the extent that ~~Neurogene~~ **we** ~~raises~~ **raise** additional capital through the sale of equity or convertible debt securities, your ownership interest will be diluted, and the terms may include liquidation or other preferences and anti- dilution protections that adversely affect your rights as a stockholder. Debt financing may result in imposition of debt covenants, increased fixed payment obligations or other restrictions that may affect our business. If we raise additional funds through upfront payments or milestone payments pursuant to future collaborations with third parties, we may have to relinquish valuable rights to product development programs, or grant

licenses on terms that are not favorable to us. Our ability to raise additional capital may be adversely impacted by global macroeconomic conditions **, geopolitical instability, changes in government regulations** and volatility in the credit and financial markets in the United States and worldwide, over which we may have no or little control. Our failure to raise capital as and when needed or on acceptable terms would have a negative impact on our financial condition and our ability to pursue our business strategy, and we may have to delay, reduce the scope of, suspend or eliminate clinical trials, product development programs or future commercialization efforts ~~. We have incurred significant losses since inception, expect to incur significant losses for the foreseeable future and may not be able to achieve or sustain profitability in the future. We have no products for sale, have not generated any product revenue and may never generate product revenue or become profitable~~ . Investment in biotechnology product development is a highly speculative undertaking and entails substantial upfront expenditures and significant risks that any program will fail to demonstrate adequate efficacy or potency or an acceptable safety profile, gain regulatory approval and become commercially viable. We have no products approved for commercial sale, ~~has~~ **have** not generated any revenue from product sales to date, and ~~continues~~ **continue** to incur significant research and development and other expenses related to our ongoing operations. We do not expect to generate product revenue unless or until we successfully complete clinical development and obtain regulatory approval of, and then successfully commercialize, at least one product candidate. We may never succeed in these activities and, even if we do, we may never generate product revenue or revenues that are significant or large enough to achieve profitability. If we are unable to generate sufficient revenue through the sale of any approved products, we may be unable to continue operations without additional funding. We have incurred significant net losses in each period since we commenced operations in 2018. Our net loss was $ ~~36~~ **75** . ~~3~~ **1** million for the year ended December 31, ~~2023~~ **2024 and our cumulative net loss from inception as of December 31, 2024 was $ 262. 3 million** . We expect to continue to incur significant losses for the foreseeable future. Our operating expenses and net losses may fluctuate significantly from quarter to quarter and year to year. We anticipate that our expenses will increase substantially if and as we: • advance our existing and future programs through preclinical and clinical development, including expansion into additional indications; • seek to identify additional programs and additional product candidates; • continue to develop our gene therapy product candidate pipeline and our EXACT platform; • maintain, expand, enforce, defend and protect our intellectual property portfolio; • seek regulatory and marketing approvals for product candidates; • seek to identify, establish and maintain additional collaborations and license agreements, including those which may enhance the biodistribution and delivery of our product candidates; • ultimately establish a sales, marketing and distribution infrastructure to commercialize any biological products for which we may obtain marketing approval, either by ourselves or in collaboration with others; • generate revenue from commercial sales of products for which we receive marketing approval; • hire additional personnel, including research and development, clinical and commercial; • add operational, financial and management information systems and personnel to support further expansion and operation as a public company; • acquire or in- license products, intellectual property and technologies which may enhance our current technology; and • establish commercial- scale cGMP capabilities through our own or third- party manufacturing facilities. In addition, our expenses will increase if, among other things, we are required by the FDA or other regulatory authorities to perform trials or studies in addition to, or different than, those that we currently anticipate, there are any delays in completing our clinical trials or the development of any product candidates, or there are any third- party challenges to our intellectual property or we need to defend against any intellectual property- related claim. Even if we obtain marketing approval for, and are successful in commercializing, one or more product candidates, we expect to incur substantial additional research and development and other expenditures to develop and market additional programs and / or to expand the approved indications of any marketed product. We may encounter unforeseen expenses, difficulties, complications, delays and other unknown factors that may adversely affect our business. The size of our future net losses will depend, in part, on the rate of future growth of our expenses and our ability to generate revenue. Our failure to become profitable would decrease our value and could impair our ability to raise capital, maintain our research and development efforts, expand our business and / or continue our operations. A decline in our value could also cause you to lose all or part of your investment. Risks Related to Discovery, Development and Commercialization We face competition from entities that have developed or may develop programs for the diseases we plan to address with NGN- 401 and ~~NGN- 101 or~~ other product candidates **in development** . The development and commercialization of biological products is highly competitive. If approved, NGN- 401 ~~and NGN- 101~~ or **any** other product candidates **we may develop** will face significant competition and our failure to effectively compete may prevent us from achieving significant market penetration. We compete with a variety of multinational biopharmaceutical companies, specialized biotechnology companies and emerging biotechnology companies, as well as academic institutions, governmental agencies, and public and private research institutions, among others. Many of the companies with which we are currently competing or will compete against in the future have significantly greater financial resources and expertise in research and development, manufacturing, preclinical testing, conducting clinical trials, obtaining regulatory approvals, and marketing approved products than we do. Mergers and acquisitions in the pharmaceutical and biotechnology industry may result in even more resources being concentrated among a smaller number of our competitors. Smaller or early- stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies. These competitors also compete with us in recruiting and retaining qualified scientific and management personnel, establishing clinical trial sites and patient registration for clinical trials, as well as in acquiring technologies complementary to, or necessary for, NGN- 401 and **any** ~~NGN- 101 or~~ other product candidates **we may develop** . As described in ~~the section above entitled~~ " Business — Competition " **in our Annual Report on Form 10- K** , our competitors have developed, are developing or may develop programs or clinical stage products competitive with NGN- 401 or ~~NGN- 101 or~~ **our** other earlier stage product candidates. Competitive therapeutic treatments include those that have already been approved and accepted by the medical community **for Rett syndrome** and any new treatments for Rett syndrome ~~or for CLN5 Batten disease~~ . Our success will depend partially on our ability to develop and commercialize products that have a competitive safety, efficacy or potency, dosing

and / or presentation profile. Our commercial opportunity and success will be reduced or eliminated if competing products are safer, more effective or potent, have a more attractive or less invasive dosing profile or presentation or are less expensive than any products we may develop, or if competitors develop competing products **that** ~~or if biosimilars~~ enter the market more quickly than we are able to, if we are able to at all, and are able to gain market acceptance. NGN- 401 ~~, NGN- 101~~ and our ~~other~~ **preclinical** programs are in early stages of development and may fail in development or suffer delays that materially and adversely affect their commercial viability. If we or our current or future collaborators are unable to complete development of, or commercialize, our product candidates, or experience significant delays in doing so, our business will be materially harmed. We have no products on the market and NGN- 401 **is in the early stages of clinical development. In addition, we announced that unless we are able to find** ~~and~~ **an alternative pathway for advancement, we will need to discontinue our** NGN- 101 ~~are in~~ **program following** the **denial** ~~early stages~~ of ~~clinical development~~ **RMAT designation for NGN- 101 by the FDA** , ~~while our~~ **which would preclude a streamlined path to regulatory approval. Our** other programs are in early stages of preclinical development. As a result, we expect it will be many years before we commercialize ~~these~~ **our** product candidates and ultimately may not be successful in commercializing any of our product candidates. Our ability to achieve and sustain profitability depends on obtaining regulatory approvals for, and successfully commercializing, our lead product candidate NGN- 401 or other product candidates, ~~including NGN- 101,~~ either alone or with third parties, and we cannot guarantee that we will ever obtain regulatory approval for any product candidates **we may develop** . We have limited experience as a company in conducting and managing the clinical trials necessary to obtain regulatory approvals, including approval by the FDA or comparable foreign regulatory authorities. We have not yet demonstrated our ability to obtain regulatory approvals, manufacture a commercial scale product or arrange for a third party to do so on our behalf, or conduct sales and marketing activities necessary for successful product commercialization. Before obtaining regulatory approval for the commercial distribution of product candidates, we or an existing or future collaborator must conduct extensive preclinical tests and clinical trials to demonstrate the safety, purity and efficacy or potency in humans of such product candidates. **In November 2024, we announced that we do not expect to move forward with the NGN- 101 gene therapy program for CLN5 Batten disease at this time. Given the rarity of the disease, continued investment in the program was predicated on alignment on a streamlined registrational pathway with the FDA. To support a streamlined pathway, we submitted an RMAT application to the FDA. Despite our belief that we met the standard of preliminary clinical evidence required to obtain an RMAT designation, the RMAT application was denied. Similar challenges may prevent our success with or increase the cost of other current, planned or future clinical trials.** We or our collaborators may experience delays in initiating or completing clinical trials, and also may experience unforeseen events during, or as a result of, any current or future clinical trials that could delay or prevent our ability to receive marketing approval or commercialize NGN- 401 ~~or NGN- 101~~ or any other product candidates, including: • regulators or institutional review boards (“ IRBs ”), the FDA or ethics committees may not authorize us or our investigators to commence a clinical trial or conduct a clinical trial at a prospective trial site; • we may experience delays in reaching, or may fail to reach, agreement on acceptable terms with prospective trial sites and prospective CROs, the terms of which can be subject to extensive negotiation and may vary significantly among different CROs and trial sites; • the observation of an actual or suspected unexpected serious adverse reaction, serious adverse events, or adverse events of special interest could result in a partial or complete clinical hold for an unpredictable length of time, delay or halt future enrollment, require increased staggering between patient dosing, require dose reductions that could adversely affect the anticipated efficacy or potency product profile, or require a program discontinuation; • clinical trial sites may fail to meet enrollment targets, may deviate from trial protocol, or may experience patients dropping out of a trial; • clinical trials of any product candidates may fail to show safety or efficacy or potency, or produce negative or inconclusive results, and we may decide, or regulators may require us, to conduct additional preclinical studies or clinical trials or we may decide to abandon product development programs; • the number of subjects required for clinical trials of any of our product candidates may be larger than we anticipate, especially if the effect size observed in future clinical data from a Phase 1 / 2 clinical trial is small or is difficult to ascertain relative to natural history as a comparator, or if regulatory authorities require completion of a sham- controlled clinical trial; • enrollment in clinical trials may be slower than we anticipate or subjects may drop out of clinical trials or fail to return for post- treatment follow- up at a higher rate than we anticipate; • our third- party contractors may fail to comply with regulatory requirements or meet their contractual obligations to us in a timely manner, or at all, or may deviate from the clinical trial protocol or drop out of the trial, which may require that we add new clinical trial sites or investigators; • we may elect to, or regulators, independent data **and** safety monitoring boards (“ DSMBs ”), IRBs or ethics committees may require that we or our investigators suspend or terminate clinical research or trials, or delay further **recruitment, enrollment or** dosing of subjects in clinical trials **or specific trial sites** , for various reasons, including noncompliance with regulatory requirements **, internal processes** or **protocols of the relevant review body,** a finding that the participants in our trials are being exposed to unacceptable health risks **, or any other development that may impact the benefit- risk assessment of our product candidates** ; • the cost of clinical trials of any of our product candidates may be greater than we anticipate; • the quality of our product candidates or other materials necessary to conduct clinical trials of our product candidates may be inadequate to initiate or complete a given clinical trial; • ~~our inability~~ **we may be unable** to manufacture sufficient quantities **at adequate scales** of our product candidates for use in clinical trials; • reports from clinical testing of other therapies may raise safety ~~or~~ **,** efficacy or potency concerns about our product candidates; • ~~our failure~~ **we may fail** to establish an appropriate safety profile for a product candidate based on clinical or preclinical data for such product candidate **and** ~~as well as~~ data emerging from other therapies in the same class as our product candidates; and • the FDA or other regulatory authorities may require us to submit additional data, such as long- term toxicology studies, or impose other requirements before permitting us to initiate a clinical trial. **If safety concerns develop with respect to our product candidates or clinical trial designs, we may be delayed in our development plans as we may need to pause our enrollment in a clinical trial, revise our trial designs, investigate potential safety developments, or take other measures that may**

increase the amount of time and resources required to bring our product candidates forward. For example, on November 11, 2024, we were advised of a severe adverse event (" SAE ") experienced by a participant in the 3E15 vg dose (high dose cohort) of our Phase 1 / 2 clinical trial of NGN- 401 for the treatment of Rett syndrome. The participant subsequently died following complications from a rare and life- threatening hyperinflammatory syndrome associated with systemic exposure to high doses of AAV. The FDA completed a review of the safety data for NGN- 401 and allowed us to continue with the Phase 1 / 2 trial using the 1E15 vg dose (low- dose cohort). We paused further use of the 3E15 vg dose (high- dose cohorts) upon initial notification of the SAE and made the determination to remove that dose level from the trial protocol as we do not plan to enroll any further participants at the 3E15 vg dose level. Commencing clinical trials in the United States is subject to acceptance by the FDA of an IND or, if commenced in other jurisdictions, acceptance by the comparable foreign regulatory agency of a similar application, as well as finalizing the trial design. In the event that the FDA or applicable foreign regulatory agency requires us to complete additional preclinical studies, or we are required to satisfy other regulatory requests prior to commencing clinical trials, the start of our clinical trials may be delayed. Even after we receive and incorporate guidance from these regulatory authorities, the FDA or other regulatory authorities could disagree that we have satisfied their requirements to commence any clinical trial or change their position on the acceptability of our trial design or the clinical endpoints selected, which may require us to complete additional preclinical studies or clinical trials, delay the enrollment of our clinical trials or impose stricter approval conditions than we currently expect. There are equivalent processes and risks applicable to clinical trial applications in other jurisdictions, including the United Kingdom (" UK "), Australia and the European Union **(" EU ")** . We may not have the financial resources to continue development of, or to modify existing collaborations or enter into new collaborations for, a product candidate if we experience any issues that delay or prevent regulatory approval of, or our ability to commercialize, NGN- 401 or ~~NGN- 101 or~~ any other product candidates **we are developing or may develop in the future** . We or our current or future collaborators' inability to complete development of, or commercialize, NGN- 401 ~~or NGN- 101~~ or any other product candidates or significant delays in doing so, could have a material and adverse effect on our business, financial condition, results of operations and prospects. We currently utilize adeno- associated virus serotype 9 (" AAV9 ") capsid for delivery of therapeutic transgenes to deliver our product candidates, which may limit the safety, purity, and efficacy or potency of such product candidates. Our current approach is to identify, develop and commercialize gene therapy product candidates using an AAV9 capsid for delivery of therapeutic transgenes to certain kinds of cells. Although AAV9 has been tested in numerous clinical trials and is an approved serotype for **at least** one gene therapy product, we cannot be certain that our AAV9 product candidates will successfully advance through preclinical studies and clinical trials, or that they will not cause significant adverse events or toxicities **. For more information, please refer to the risk factor below titled " Participants in our clinical trials may experience undesirable side effects, which could cause delays or prevent regulatory approval of our product candidates, limit the commercial potential or create significant negative consequences to our development plans, even if such side effects are ultimately determined not to be attributable or possibly attributable to our product candidates ". In November 2024, a participant who had been recently dosed in the 3E15 vg (high dose) cohort of our Phase 1 / 2 clinical trial of NGN- 401 for the treatment of Rett syndrome experienced an SAE consistent with known risks of AAV gene therapy and ultimately died from this complication. While this reaction was very rare, we cannot ensure that other SAEs related to the use of AAV9 will not occur, or that we will not experience delays or other negative impacts to our clinical trial related to this or other AAV- related SAEs** . We also cannot be certain that we will be able to avoid triggering toxicities in our future preclinical studies or clinical trials or that our chosen routes of administration to deliver such therapies will not cause unforeseen side effects or other challenges. Although AAV9 has been shown to facilitate biodistribution and cell transduction to the central nervous system (" CNS "), the potentially limited levels of AAV9 transduction of cells in the CNS and certain retinal cells may **also** limit the potential efficacy or potency of any of our product candidates, including NGN- 401 ~~and~~ **. Participants in our clinical trials may experience undesirable side effects, which could cause delays or prevent regulatory approval of our product candidates, limit the commercial potential or create significant negative consequences to our development plans, even if such side effects are ultimately determined not to be attributable or possibly attributable to our product candidates. Our primary product candidates, including** NGN- ~~101~~ **401 for the treatment of Rett syndrome, are AAV- based gene therapies. AAV- based gene therapies in development or approved for use carry a risk of certain adverse side effects, known and unknown, including the potential for inflammatory events such as heightened innate or adaptive immune reactions in response to the presence of the AAV vector, including the development of a T- cell and / or B- cell immune response, complement system activation, thrombotic microangiopathy, thrombocytopenia, toxicity due to damage of the dorsal root ganglia, loss of nerve conductivity with or without the diminishment or loss of reflexes and sensory symptoms, increased liver enzymes and liver toxicity, organ damage to kidneys or the heart, or in rare cases, death. In addition, some participants in our AAV- based gene therapy clinical trials may have pre- existing conditions, such as diminished lean muscle mass, impaired function of biological systems or vital organs, or recent viral infections, or complications relating to their genetic makeup, and, as such, those participants may present a different risk profile and may have an increased potential for serious adverse events such as a heightened immune response, the re- activation of a viral infection due to immunosuppression measures that are taken in conjunction with administration of AAV- based gene therapy, or a diminished capacity to withstand treatment- related side effects that might be mild if they were to present in another participant. Because of the novel nature of gene therapy in general and specifically AAV- based gene therapy, not all side effects may have been discovered, and we may not be able to identify all of the increased risk factors for our participants, and additional unexpected serious adverse events may occur as a result. In addition, due to components of our product candidates used to carry the genetic materials, it is possible that some participants could develop delayed side effects from treatment. There can also be significant variability in how patients respond to gene therapy, especially in a mosaic**

disease presentation like Rett syndrome in females where some of the cells carry a correct copy of the DNA sequence for the impacted gene while other cells have a mutated variant. As a result, some patients may not respond as well to gene therapy as others. Serious adverse events related to our trial or to other clinical trials using AAV- based gene therapy, even if those other trials are not related to our product candidates or targeted disease states, and even if such adverse events are not ultimately attributable to the relevant product candidates or products, may result in unfavorable public sentiment about our clinical trial and our product candidates, increased government regulation, potential regulatory delays for approval of our product candidates, stricter labeling requirements, the imposition of additional monitoring of our products if they are approved, challenges in enrolling patients in our clinical trials and a decrease in demand for our product candidates. If our product candidates are believed to be or shown to be associated with side effects that significantly alter the benefit- risk determination of any of our product candidates, we may not be able to continue development of that product candidate. Some product candidates that have shown positive safety results in early clinical testing have later been found to cause side effects that required the abandonment of further development of that product candidate. We may also be required to delay or slow the development of a particular product candidate if there are side effects whose cause is unclear or uncertain in order to further understand the nature of such side effects, which could materially impact our plans for development and financial position . We intend to identify and develop novel gene therapy product candidates, which makes it difficult to predict the time, cost and potential success of product candidate development. A key part of our business strategy is to identify and develop additional product candidates. As such, our future success depends on the successful development of novel therapeutic approaches, including by utilizing our EXACT technology or other ~~gene~~ **transgene** regulation technology. Our preclinical research and clinical trials may initially show promise in identifying potential product candidates, yet fail to yield product candidates for a number of reasons. For example, although EXACT is designed to deliver therapeutic levels of transgene while avoiding **overexpression toxicity and** off- target effects, there can be no assurance that any EXACT ~~gene~~ **transgene** regulation will result in product candidates that are shown in clinical trials to be safe, pure, and effective or potent. To date, very few products that utilize gene transfer have been approved in the United States, Europe or other markets, and no products have been approved using our EXACT ~~(Expression Attenuation via Construct Timing)~~ technology **or technology similar to it** . There have been a limited number of clinical trials of gene transfer technologies, with only very few product candidates ever approved by the FDA or comparable foreign regulatory authorities. As a result, it is difficult for us to predict the time and cost of product candidate development, and we cannot predict whether the application of our approach to gene therapy will result in the identification, development, and regulatory approval of any product candidates, or that other gene therapy programs will not be considered better or more attractive. There can be no assurance that any development problems we experience in the future related to our current gene therapy approaches or product candidates or any of our research programs will not cause significant delays or unanticipated costs, or that such development problems can be solved. Research programs to identify new product candidates require substantial technical, financial, and human resources. If we are unable to identify suitable gene therapy product candidates for preclinical and clinical development, we may not be able to successfully implement our business strategy, and may have to delay, reduce the scope of, suspend or eliminate one or more of our product candidates, clinical trials or future commercialization efforts, which would negatively impact our financial condition. The disorders we seek to treat have low prevalence and it may be difficult to identify and enroll patients with these disorders. If we experience delays or difficulties in the enrollment and / or maintenance of patients in clinical trials, our receipt of necessary regulatory approvals could be delayed or prevented. Successful and timely completion of clinical trials will require that we enroll and maintain a sufficient number of patients. Patient enrollment is affected by many factors, including the size and nature of the patient population and competition for patients with other trials. Genetic diseases generally, and especially the rare diseases for which some of our current product candidates are targeted, have low incidence and prevalence. For example, we estimate global incidence of **Rett syndrome to be** ~~all 13 subtypes of Batten disease is~~ approximately one in ~~100~~ **10** , 000 live **female** births ~~, and the CLN5 Batten disease incidence, which is included in this estimate, is estimated to be even lower~~ . Accordingly, it may be difficult for us to identify and timely recruit a sufficient number of eligible patients to conduct our clinical trials. Further, any natural history studies that we or our collaborators may conduct may fail to provide us with patients for our clinical trials because patients enrolled in the natural history studies may not be good candidates for our clinical trials, or may choose to not enroll in our clinical trials. Trials may be subject to delays as a result of patient enrollment taking longer than anticipated or patient withdrawal. We may not be able to initiate or continue clinical trials for our product candidates if we are unable to locate and enroll a sufficient number of eligible patients to participate in these trials as required by the FDA, the European Medicines Agency (“ EMA ”) or other foreign regulatory authorities. We cannot predict how successful we will be at enrolling subjects in future clinical trials. Subject enrollment is affected by other factors including: • the eligibility criteria for the trial in question; • the timely diagnosis of disease to meet such eligibility criteria; • the size of the patient population and process for identifying patients; • the perceived risks and benefits of the product candidate in the trial, especially by clinician experts and patient advocacy organizations, including relating to AAV9- based gene therapy **, which may evolve over time as more AAV- based gene therapy trials are conducted,** and intracerebral spinal fluid delivery system; • the availability of competing commercially available therapies and other competing therapeutic candidates’ clinical trials; • the willingness of caregivers to enroll their children in our clinical trials; • the efforts to facilitate timely enrollment in clinical trials; • potential disruptions caused by pandemics or other public health crises, including difficulties in initiating clinical sites, enrolling and retaining participants, diversion of healthcare resources away from clinical trials, travel or quarantine policies that may be implemented, and other factors; • the patient referral practices of physicians; • the ability to monitor patients adequately during and after treatment; and • the proximity and availability of clinical trial sites for prospective patients. Even if we are able to enroll a sufficient number of patients in our clinical trials, we may have difficulty maintaining enrollment of such patients. Our inability to enroll or maintain a sufficient number of patients would result in significant delays in completing clinical trials or

receipt of marketing approvals and increased development costs, or may require us to abandon one or more clinical trials altogether. We are substantially dependent on the success of our most advanced product ~~candidates~~ **candidate**, NGN- 401 ~~and NGN- 101~~, and our ongoing and anticipated clinical trials of ~~such candidates~~ **NGN- 401** may not be successful. Our future success is substantially dependent on our ability to timely obtain marketing approval for, and then successfully commercialize, our most advanced product ~~candidates~~ **candidate**, NGN- 401 ~~and NGN- 101~~. We are investing a majority of our efforts and financial resources into the research and development of ~~these~~ **this product** ~~candidates~~ **candidate**. ~~We~~ **, as we** are **currently** conducting a Phase 1 / 2 clinical trial of NGN- 401 in patients with Rett syndrome ~~and a~~ **. Based on the initial interim positive clinical trial data from our 1E15 vg dose of NGN- 401 in our** Phase 1 / 2 clinical trial **for the treatment** of **Rett syndrome** ~~NGN- 101 in patients with CLN5 Batten disease. If topline results from our Phase 1 / 2 clinical trial of NGN- 401 are successful~~, we ~~anticipate initiating~~ **expect to advance that product candidate to** a ~~pivotal~~ **registrational** clinical trial, pending future regulatory feedback on various aspects of development such as the pivotal trial design ~~and manufacturing related requirements. If topline results from our Phase 1 / 2 clinical trial of NGN- 101 are successful, we anticipate initiating a pivotal clinical trial or expanding the current Phase 1 / 2 clinical trial, pending future regulatory feedback on various aspects of development, such as the Phase 3 clinical trial design and manufacturing related requirements~~. NGN- 401 ~~and NGN- 101~~ will require additional clinical development, evaluation of clinical, preclinical and manufacturing activities, marketing approval in multiple jurisdictions, substantial investment and significant marketing efforts before we generate revenues from product sales, if any. We are not permitted to market or promote ~~these~~ **this** product ~~candidates~~ **candidate**, or any other product candidates **we may develop**, before we receive marketing approval from the FDA and / or comparable foreign regulatory authorities, and we may never receive such marketing approvals. The success of NGN- 401 ~~and NGN- 101~~ will depend on a variety of factors. We do not have complete control over many of these factors, including certain aspects of clinical development and the regulatory submission process, potential threats to our intellectual property rights and the manufacturing, marketing, distribution and sales efforts of any future collaborator. Accordingly, we cannot guarantee that we will ever be able to generate revenue through the sale of ~~these~~ **this product** ~~candidates~~ **candidate**, even if approved. If we are not successful in commercializing NGN- 401 **, or are significantly delayed in doing so,** ~~or~~ **our business will be materially harmed. We may not be successful in identifying and advancing a strategy to continue the development of** NGN- 101 ~~, or~~ **for CLN5 Batten disease, and in the meantime, may incur additional costs as we continue the post- dosing phases of our clinical trial for NGN- 101. In November 2024, we announced that we do not expect to move forward with the NGN- 101 CLN5 Batten disease gene therapy program at the present time because of an inability to align on a streamlined registrational pathway with the FDA for that product candidate, but that we would continue to evaluate options for that program. We may consider a range of potential alternatives for the program, which could include continuing to discuss possibilities for a streamlined pathway to registration with the FDA, looking for a partner or out- licensing the product candidate entirely, but there can be no assurance that we will find any alternative to move the program forward. In addition, while we have completed dosing in the Phase 1 / 2 clinical trial of NGN- 101 for CLN5 Batten disease, we do intend to continue to follow the patients who received treatment in the clinical trial and therefore may continue to incur certain incremental costs related to the continued observations in that clinical trial, even if we** are ~~significantly delayed in doing so, our business will be materially harmed~~ **not able to find a future path to commercialization for this product candidate**. Our programs are focused on the development of therapeutics for patients with neurological diseases, which is a rapidly evolving area of science, and the approach we are taking to discover and develop product candidates is novel and may never lead to approved or marketable products. The discovery and development of therapeutics for patients with neurological diseases is an emerging field, and the scientific discoveries that form the basis for our efforts to discover and develop product candidates are relatively new. The scientific evidence to support the feasibility of developing product candidates based on these discoveries is both preliminary and limited. Although we believe, based on our preclinical work, that our programs have the potential to be disease- modifying therapies, clinical results may not confirm this hypothesis or may only confirm it for certain alterations or certain indications. The patient populations for our product candidates are limited to those with specific neurological diseases. We cannot be certain that the patient populations for each specific disease will be large enough to allow us to successfully obtain approval and commercialize our product candidates and achieve profitability. Further, ~~both~~ our Phase 1 / 2 clinical trial of NGN- 401 ~~and Phase 1 / 2 clinical trial of NGN- 101~~ will involve a small patient population. Because of the small sample sizes, the **expansion of our clinical trial to an adolescent / adult cohort and the heterogeneity of the disease state, the** results of ~~these~~ **this** ~~trials~~ **trial** may not be indicative of results of future clinical trials. If we do not achieve our projected development goals in the ~~time frames~~ **timeframes** we announce and expect, the commercialization of NGN- 401 ~~or NGN- 101~~ or any other product candidates may be delayed and, as a result, our stock price may decline. From time to time, we estimate the timing of the anticipated accomplishment of various scientific, clinical, regulatory and other product development goals, which we sometimes refer to as milestones. These milestones may include the commencement or completion of scientific studies and clinical trials and the submission of regulatory filings. 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 NGN- 401 or ~~NGN- 101 or~~ any other product candidates may be delayed or never achieved and, as a result, our stock price may decline. Preclinical and clinical development involves a lengthy and expensive process that is subject to delays and uncertain outcomes, and results of earlier studies and trials may not be predictive of future clinical trial results. If our preclinical studies and clinical trials are not sufficient to support regulatory approval of any of our product candidates, we may incur additional costs or experience delays in completing, or ultimately be unable to complete, the development of such product candidate. Before obtaining marketing approval from regulatory authorities for the sale of any product candidate, we must complete preclinical studies, which are a lengthy, time consuming and expensive process

with **a high** risk of ~~high~~ failure. The length of time of such testing may vary substantially according to the type, complexity and novelty of the program, and often can be several years or more per program. Delays associated with programs for which we are conducting preclinical testing and studies may cause us to incur additional operating expenses. **For example, we depend on the availability of NHPs to conduct certain preclinical studies that we are required to complete prior to submitting an IND and initiating clinical development. A sustained global shortage of NHPs available for biological product development could cause the cost of obtaining NHPs for our future preclinical studies to increase significantly and result in delays to our development timelines.** However, after conducting preclinical studies, we must then conduct extensive clinical trials to demonstrate the safety, purity, and efficacy or potency of our product candidate in humans. Our clinical trials may not be conducted as planned or completed on schedule, if at all ~~. For example, we depend on the availability of NHPs to conduct certain preclinical studies that we are required to complete prior to submitting an IND and initiating clinical development. There is currently a global shortage of NHPs available for biological product development. This could cause the cost of obtaining NHPs for our future preclinical studies to increase significantly and, if the shortage continues, could also result in delays to our development timelines~~ . Furthermore, failure can occur at any time during the preclinical study or clinical trial process, and the outcome of preclinical studies and early- stage clinical trials may not be predictive of the success of later clinical trials, especially as our initial clinical trials do not contain a control arm. In addition, we have designed our initial clinical trials with relatively small cohorts before expanding in size and dosing in subsequent cohorts. If safety issues arise in an early cohort, we may be delayed or prevented from dose escalating or subsequently expanding into larger trial cohorts **. For example, on November 11, 2024, we were advised of an SAE experienced by a recently dosed participant in the 3E15 vg (high dose cohort) of our Phase 1 / 2 clinical trial of NGN- 401 for the treatment of Rett syndrome. The participant subsequently died following complications from a rare and life threatening hyperinflammatory syndrome associated with systemic exposure to high doses of AAV. The FDA completed a review of the safety data for NGN- 401 and allowed us to proceed with the Phase 1 / 2 trial using the 1E15 vg dose (low- dose cohort), although we decided to revise our trial protocol before resuming dosing, and have removed the 3E15 vg dose level from the trial protocol as we do not plan to enroll any further participants at the that dose level** . Moreover, preclinical and clinical data are often susceptible to varying interpretations and analyses, and many companies that have believed their product candidates performed satisfactorily in preclinical studies and clinical trials have nonetheless failed to obtain marketing approval of their product candidates. Earlier gene therapy clinical trials conducted by others also utilized AAV vectors. However, these studies should not be relied upon as evidence that our planned clinical trials will succeed. In addition, we expect to rely on patients **, caregivers and clinicians** to provide feedback on measures, which are subjective and inherently difficult to evaluate. These measures can be influenced by factors outside of our control, and can vary widely from day to day for a particular patient, and from patient to patient **or caregiver to caregiver** and from site to site within a clinical trial. We cannot be sure that the FDA or comparable foreign regulatory authorities will agree with our clinical development plan. We are conducting a Phase 1 / 2 clinical trial of NGN- 401 in patients with Rett syndrome ~~and a Phase 1 / 2 clinical~~ **. If the FDA or any comparable regulatory authorities require us to conduct additional** ~~trial~~ **trials** ~~of~~ **or enroll additional patients, our development timelines may be delayed, or we may not be able to pursue further development due to such delays. For example, in November 2024, we announced that the Company does not expect to move forward with the** NGN- 101 **for** ~~in patients with~~ CLN5 Batten disease **gene therapy program at this time** . ~~If~~ **Given the rarity of the disease, continued investment in the program was predicated on alignment on a streamlined registrational pathway with** the FDA **. To support a streamlined pathway, we submitted an RMAT application to the FDA. Despite** ~~or our comparable regulatory authorities~~ **belief that we met the standard of preliminary clinical evidence** ~~requires~~ **required** ~~us~~ to **obtain an RMAT designation** ~~conduct additional trials or enroll additional patients~~ , **the RMAT application was denied** ~~our development timelines may be delayed~~ . We cannot be sure that submission of an IND application, clinical trial application (" CTA ") or similar application will result in the FDA or comparable foreign regulatory authorities, as applicable, allowing clinical trials to begin in a timely manner, if at all. Moreover, even if these trials begin, issues may arise that could cause regulatory authorities to require us to suspend or terminate such clinical trials. Events that may prevent successful or timely initiation or completion of clinical trials include: inability to generate sufficient preclinical, toxicology or other in vivo or in vitro data to support the initiation or continuation of clinical trials; delays in reaching a consensus with regulatory authorities on study design or implementation of the clinical trials; delays or failure in obtaining regulatory authorization to commence a trial; delays in reaching agreement on acceptable terms with prospective CROs and clinical trial sites, the terms of which can be subject to extensive negotiation and may vary significantly among different CROs and clinical trial sites; delays in identifying, recruiting and training suitable clinical investigators; delays in obtaining required IRB approval at each clinical trial site; difficulties in patient enrollment in our clinical trials for a variety of reasons **; delays related to safety concerns** ; delays in manufacturing, testing, releasing, validating or importing / exporting sufficient stable quantities of our product candidates for use in clinical trials or the inability to do any of the foregoing; failure by our CROs, other third parties or us to adhere to clinical trial protocols; failure to perform in accordance with the FDA' s or any other regulatory authority' s good clinical practices (" GCPs ") or applicable regulatory guidelines in other countries; changes to the clinical trial protocols; clinical sites deviating from trial protocol or dropping out of a trial; changes in regulatory requirements and guidance that require amending or submitting new clinical protocols; selection of clinical endpoints that require prolonged periods of observation or analyses of resulting data; transfer of manufacturing processes to larger- scale facilities operated by a CDMO and delays or failure by our CDMOs or us to make any necessary changes to such manufacturing process **and demonstrate comparability to materials used in earlier clinical phases** ; and third parties being unwilling or unable to satisfy their contractual obligations to us. We could also encounter delays if a clinical trial is placed on clinical hold, suspended or terminated by us, the IRBs of the institutions in which such trials are being conducted, or the FDA, the competent authorities and / or ethics committees of the UK, Australia, EU Member States or other regulatory authorities, if a clinical trial is

recommended for suspension or termination by the DSMB or equivalent body for such trial, or on account of changes to federal, state, or local laws. If we are required to conduct additional clinical trials or other testing of NGN- 401 or ~~NGN- 101 or~~ any other product candidates beyond those that we contemplate, if we are unable to successfully complete clinical trials of NGN- 401 or ~~NGN- 101 or~~ any other product candidates, if the results of ~~these~~ **such** trials are not positive or are only moderately positive or if there are safety concerns, our business and results of operations may be adversely affected and we may incur significant additional costs. In addition, even if we are able to successfully complete **the** clinical ~~trials~~ **trial** for NGN- 401 ~~or NGN- 101~~, we cannot guarantee that the FDA or foreign regulatory authorities will interpret the results as we do, and more trials could be required before we submit our product candidates for approval. This is particularly true for clinical trials in very rare diseases, such as with our Phase 1 / 2 clinical trial of NGN- ~~101 for the treatment of CLN5 Batten disease and Phase 1 / 2 clinical trial of NGN-~~ 401 for the treatment of Rett syndrome, where the very small patient population makes it difficult to conduct two traditional, adequate and well- controlled studies. In such cases, the FDA or comparable foreign regulatory authorities are often required or permitted to exercise flexibility in approving therapies for such diseases, but obtaining flexibility is uncertain and may never occur. Moreover, results acceptable to support approval in one jurisdiction may be deemed inadequate by another regulatory authority to support regulatory approval in the other jurisdiction. To the extent that the results of the trials are not satisfactory to the FDA or applicable regulatory authorities for support of a marketing application, we may be required to expend significant resources, which may not be available to us, to conduct additional trials in support of potential approval of our product candidates. Preliminary, " topline " or interim data from our **preclinical studies and** clinical trials that we announce or publish from time to time may change as more patient data becomes available and are subject to audit and verification procedures. From time to time, we may publicly disclose preliminary, interim or topline data from our preclinical studies and clinical trials, which are based on a preliminary analysis of then- available data, and the results and related findings and conclusions are subject to change following a more comprehensive review of the data. We also make assumptions, estimations, calculations and conclusions as part of our analyses of these data without the opportunity to fully and carefully evaluate complete data. Preliminary, interim or topline results also remain subject to audit and verification procedures that may result in the final data being materially different from the preliminary data previously disclosed. These preliminary, interim or topline data are subject to the risk that one or more of the clinical outcomes may materially change as patient enrollment continues and more patient data become available or as patients from our clinical trials continue other treatments. ~~As~~ **For example, in June 2024, we announced initial safety data related to the dosing of our first four participants in the 1E15 vg dose cohort of our Phase 1 / 2 clinical trial of NGN- 401 for the treatment of Rett syndrome which suggested** a ~~result~~ **favorable safety profile for the 1E15 vg dose. In November 2024, an SAE was reported in a participant who received the 3E15 vg dose (high- dose), which caused us to revise our assumptions regarding the safety profile of the 3E15 vg dose (high- dose). Because of this potential for change**, preliminary, interim and topline data should be viewed with caution until final data are available. Further, others, including regulatory agencies, may not accept or agree with our assumptions, estimates, calculations, conclusions or analyses or may interpret or weigh the importance of data differently, which could impact the value of the particular product candidate, the approvability or commercialization of a particular product candidate and our company in general. In addition, the information we choose to publicly disclose regarding a particular preclinical study or clinical trial is based on what is typically extensive information, and you or others may not agree with what we determine is material or otherwise appropriate information to include in our disclosure. If the preliminary, interim or topline data that we report differ from actual results, or if others, including regulatory authorities, disagree with the conclusions reached, our ability to obtain approval for, and commercialize, NGN- 401 or ~~NGN- 101 or~~ any other product candidate may be harmed, which could harm our business, operating results, prospects or financial condition. In addition, differences between preliminary, interim or topline data and final data could significantly harm our business prospects and may cause the trading price of our common stock to fluctuate significantly. Our current or future clinical trials may reveal significant adverse events or undesirable side effects not seen in our preclinical studies and may result in a safety profile that could halt clinical development, inhibit regulatory approval or limit commercial potential or market acceptance of ~~any of~~ NGN- 401 ~~or NGN- 101~~ or any other product candidates or result in potential product liability claims. Results of our clinical trials could reveal a high and unacceptable severity and prevalence of side effects, adverse events or unexpected characteristics. ~~While our~~ **We believe NGN- 401** ~~Phase~~ **has been generally well-tolerated at the 1E15 vg dose; however** ~~1 / 2 clinical trials have not shown any such characteristics to date~~, we have not yet completed ~~those~~ **this** clinical ~~trials~~ **trial and the benefit- risk assessments of our product candidates remains ongoing. In November 2024, a participant who had recently received the 3E15 vg dose of NGN- 401 experienced an SAE consistent with the known risks of AAV gene therapy and subsequently died following complications from a rare and life-threatening hyperinflammatory syndrome associated with systemic exposure to high doses of AAV. Participants at the 1E15 vg dose level have also experienced adverse events, and may experience adverse events in the future**. If ~~significant~~ **additional SAEs or other** adverse events or other side effects are observed in any of our current or future clinical trials, we may have difficulty recruiting patients to such trials, patients may drop out of our trials, patients may be harmed, or we may be required to **delay enrollment or abandon one or more cohorts of a trial or delay or** abandon the trials or our development efforts of one or more product candidates altogether, including NGN- 401 ~~or NGN- 101~~. We, the FDA, ~~EMA~~ **MHRA**, or other applicable regulatory authorities, or an IRB, may require suspension of any clinical trials of NGN- 401 ~~or NGN- 101~~ or any other product candidates at any time for various reasons, including a finding that subjects or patients in such trials are being exposed to unacceptable health risks or adverse side effects. Some potential products developed in the biotechnology industry that initially showed therapeutic promise in early- stage trials have later been found to cause side effects that prevented their further development. Even if the side effects do not preclude a product candidate from obtaining or maintaining marketing approval, undesirable side effects may inhibit market acceptance of an approved product due to its tolerability versus other therapies. In addition, as gene replacement has a potentially life- long activity, with no ability to withdraw the product as with

other treatment modalities, this profile could prolong the duration of undesirable side effects, which could also inhibit market acceptance. Treatment- emergent adverse events could also affect patient recruitment or the ability of enrolled subjects to complete our clinical trials or could result in potential product liability claims. Potential side effects associated with NGN- 401 or ~~NGN- 101 or~~ any other product candidates may not be appropriately recognized or managed by the treating medical staff, as toxicities resulting from NGN- 401 or ~~NGN- 101 or~~ any other product candidates may not be normally encountered in the general patient population and by medical personnel. Any of these occurrences could harm our business, financial condition, results of operations and prospects significantly. In addition, even if we successfully advance NGN- 401 or ~~NGN- 101 or~~ any other product candidates through clinical trials, such trials will only include a limited number of patients and limited duration of follow up to such product candidates. As a result, we cannot be assured that adverse effects of NGN- 401 ~~or NGN- 101~~ or any other product candidates will not be uncovered when a significantly larger number of patients are exposed to such product candidate after approval, or a significantly longer follow up post- dosing is obtained as part of regulators' recommendations for long- term follow up of clinical study subjects treated with gene therapy. Further, any clinical trials may not be sufficient to determine the effect and safety consequences of using our product candidates over a multi- year period. We have expended substantial efforts and costs testing our EXACT technology in preclinical studies of NGN- 401, including completing toxicology studies prior to the FDA providing clearance of the IND for NGN- 401. However, we cannot guarantee that significant adverse effects will not be seen in clinical trials for NGN- 401, which could result in clinical holds, delays, suspension or withdrawal of our IND. If any of the foregoing events occur or if NGN- 401 ~~or~~ **,** NGN- 101 or any other product candidates prove to be unsafe, our entire pipeline could be affected, which would have a material adverse effect on our business, financial condition, results of operations and prospects. We may expend our limited resources to pursue a particular product candidate, such as NGN- 401 ~~or NGN- 101~~ , and fail to capitalize on candidates that may be more profitable or for which there is a greater likelihood of success. Because we have limited financial and managerial resources, we intend to focus our research and development efforts on certain selected product candidates. For example, **to date** we **have allocated** ~~are initially allocating~~ significant resources to our most advanced product candidates, NGN- 401 and NGN- 101. As a result, we may forgo or delay pursuit of opportunities with other potential candidates that may later prove to have greater commercial potential **. For example, in November 2024, we announced that the Company does not expect to move forward with the NGN- 101 for CLN5 Batten disease gene therapy program at this time. Given the rarity of the disease, continued investment in the program was predicated on a streamlined registrational pathway with the FDA. To support a streamlined pathway, we submitted an RMAT application to the FDA. Despite our belief that we met the standard of preliminary clinical evidence required to obtain an RMAT designation, the RMAT designation was denied** . Our resource allocation decisions may cause us to fail to capitalize on viable commercial products or profitable market opportunities. Our spending on current and future research and development programs for specific indications may not yield any commercially viable product candidates. If we do not accurately evaluate the commercial potential or target market for a particular product candidate, we may relinquish valuable rights to that candidate through collaboration, licensing or other royalty arrangements in cases in which it would have been more advantageous for us to retain sole development and commercialization rights to such candidate. Even if regulatory approval is obtained, any approved products resulting from NGN- 401 ~~or NGN- 101~~ or any other product candidate may not achieve adequate market acceptance among clinicians, patients, healthcare third- party payors and others in the medical community necessary for commercial success and we may not generate any future revenue from the sale or licensing of such products. Even if regulatory approval is obtained for NGN- 401 or ~~NGN- 101 or~~ any other product candidates, our product candidates may not gain market acceptance among physicians, patients, healthcare payors or the medical community. We may not generate or sustain revenue from sales of the product due to factors such as whether the product can be sold at a competitive cost and whether it will otherwise be accepted in the market. There is currently one FDA- approved product and multiple other product candidates in various stages of development for the treatment of Rett syndrome. Market participants with significant influence over acceptance of new treatments, such as clinicians and third- party payors, may not adopt a gene therapy replacement with a target product profile such as that of NGN- 401 or ~~NGN- 101 or~~ for ~~their~~ **its** targeted indications, and we may not be able to convince the medical community and third- party payors to accept and use, or to provide favorable reimbursement for, any product candidates developed by us or our existing or future collaborators. Market acceptance of NGN- 401 ~~or NGN- 101~~ or any other product candidates will depend on many factors, including factors that are not within our control. Sales of biological products also depend on the willingness of clinicians to prescribe the treatment. We cannot predict whether clinicians, clinicians' organizations, hospitals, other healthcare providers, government agencies or private insurers will determine that any of our approved products are safe, therapeutically effective or potent, cost effective or less burdensome as compared with competing treatments. If NGN- 401 ~~or NGN- 101~~ or any other product candidate is approved but does not achieve an adequate level of acceptance by such parties, we may not generate or derive sufficient revenue from that product and may not become or remain profitable. We have never commercialized a product candidate and may lack the necessary expertise, personnel and resources to successfully commercialize a product candidate on our own or together with suitable collaborators. We have never commercialized a product candidate and currently have no sales force, marketing or distribution capabilities. To achieve commercial success for a product candidate, we may opt to license such product candidate to others, in which case we may rely on the assistance and guidance of our collaborators on that license arrangement. For a product candidate for which we retain commercialization rights and marketing approval, we will have to develop our own sales, marketing and supply organization or outsource these activities to a third party. Factors that may affect our ability to commercialize a product candidate, if approved, on our own include recruiting and retaining adequate numbers of effective sales and marketing personnel, developing adequate educational and marketing programs to increase public acceptance of our approved product candidate, ensuring regulatory compliance of our company, employees and third parties under applicable healthcare laws and other unforeseen costs associated with creating an independent sales and marketing organization. Developing a sales and marketing organization will be expensive

and time- consuming and could delay the launch of a product candidate upon approval. Moreover, we may not be able to build an effective sales and marketing organization. If we are unable to build our own distribution and marketing capabilities or to find suitable partners for the commercialization of an approved product candidate, we may not generate revenues from them or be able to reach or sustain profitability. We have never completed any late- stage clinical trials and may not be able to file an IND application or other applications for regulatory approval to commence additional clinical trials on the timelines we expect. Even if we are able to complete such trials, the FDA or comparable foreign regulatory authorities may not permit us to proceed or could suspend or terminate any such trial after it has been initiated. We are early in our development efforts and will need to successfully complete later- stage and pivotal clinical trials in order to obtain FDA or comparable foreign regulatory approval to market our product candidates. Carrying out clinical trials and the submission of a successful IND or CTA is a complicated process. **Even though our product candidate NGN- 401 for Rett syndrome has been accepted into the FDA' s START program and RMAT program, the combination of which is expected to allow access to frequent advice from FDA staff, intensive guidance on efficient drug development and eligibility for an Accelerated Approval pathway and Priority Review, our lack of experience with FDA submissions may still slow our progress towards FDA approval.** We have not yet completed a Phase 1 / 2 clinical trial and have limited experience as a company in preparing, submitting and prosecuting regulatory filings. ~~If topline results~~ **Based on the initial positive interim data** from **the 1E15 vg dose of NGN- 401 in** our Phase 1 / 2 clinical trial of NGN- 401 ~~are successful~~, we intend to engage with the FDA and other comparable foreign regulators to determine the requirements to support initiation of a pivotal clinical trial. ~~If topline results from our Phase 1 / 2 clinical trial of NGN- 101 are successful, we intend to engage with the FDA and other comparable foreign regulators to determine if there is a streamlined pathway to approval for NGN- 101 for the treatment of CLN5 Batten disease.~~ However, regulatory authorities may recommend changes to the study ~~designs~~ **design** for NGN- 401 ~~or NGN- 101~~, including the number and size of registrational clinical trials required to be conducted in ~~such~~ **that** ~~programs~~ **program**. In addition, regulatory authorities could require manufacturing changes or have us implement additional analytical processes prior to initiation of a future clinical trial. Consequently, we may be unable to successfully and efficiently execute and complete necessary clinical trials in a way that leads to regulatory submission and approval of our product candidates **or we may determine that the regulatory requirements for submission are too burdensome to support continued development of one or more of our product candidates, as we did with our NGN- 101 product candidate for CLN5 Batten disease, which we plan to terminate due to a lack of alignment with the FDA on a streamlined pathway to registration**. Additionally, even if regulatory authorities agree with the design and implementation of the clinical trials set forth in a regulatory meeting, such regulatory authorities may change their requirements in the future. The FDA or comparable foreign regulatory authorities may require the analysis of data from trials assessing different doses of the product candidate alone or in combination with other therapies to justify the selected dose prior to the initiation of large trials in a specific indication. Any delays or failure to initiate clinical trials or obtain regulatory approvals for our trials may prevent us from completing our clinical trials or commercializing our products on a timely basis, if at all. We are subject to similar risks related to the review and authorization of our protocols and amendments by comparable foreign regulatory authorities. For our preclinical pipeline, if the IND- enabling studies support a decision to advance into clinical development, we would plan to submit an IND or CTA with a foreign regulatory authority. We may not be able to file the IND or CTA in accordance with our desired timelines for future product candidates. For example, we may experience manufacturing delays or other delays with IND- enabling studies, including with suppliers, study sites, or third- party contractors and vendors on which we depend. Moreover, we cannot be sure that submission of an IND application will result in the FDA or comparable foreign regulatory authorities allowing further clinical trials to begin, or that, once begun, issues will not arise that lead us to suspend or terminate such clinical trials. Risks Related to Manufacturing Gene therapies are novel, complex and difficult to manufacture. We could experience manufacturing problems that result in delays in the development or commercialization of our product candidates or otherwise harm our business. The manufacture of gene therapy products is technically complex and necessitates substantial expertise and capital investment. Production difficulties caused by unforeseen events may delay the availability of material for our clinical studies. While we **have** ~~are currently establishing~~ **established** our own manufacturing facility to provide clinical and commercial supply of our product candidates, we expect to rely on contract manufacturers for certain portions of our manufacturing needs for the foreseeable future, such as those related to research grade material for our early preclinical studies ~~. We have also relied on a third- party contract manufacturer to manufacture clinical supply for our Phase 1 / 2 clinical trial of NGN- 101~~. The manufacturers of biological and pharmaceutical products must comply with strictly enforced cGMP requirements, state and federal regulations, as well as foreign requirements when applicable. Any failure of us or our CDMOs to adhere to or document compliance with such regulatory requirements could lead to a delay or interruption in the availability of our program materials for clinical trials or enforcement action from the FDA, EMA or other foreign regulatory authorities. If we or our manufacturers were to fail to comply with the FDA, EMA or other regulatory authority, it could result in sanctions being imposed on us, including clinical holds, fines, injunctions, civil penalties, delays, suspension or withdrawal of approvals, license revocation, seizures or recalls of product candidates or products, operating restrictions and criminal prosecutions, any of which could significantly and adversely affect supplies of our product candidates. Our potential future dependence upon others for the manufacture of our product candidates may also adversely affect our future profit margins and our ability to commercialize any product candidates that receive regulatory approval on a timely and competitive basis. Biological products are inherently difficult to manufacture. Although we believe that the manufacture of our product candidates may be simplified due to their shared raw materials and other similarities, we cannot be certain that this will be the case and we may be required to develop manufacturing methods that ultimately differ significantly between product candidates, which would require that we invest substantial time and capital to develop suitable manufacturing methods. Our program materials are manufactured using technically complex processes requiring specialized equipment and facilities, highly specific raw materials, cells, and reagents, and other production constraints. Our production

process requires a number of highly specific raw materials, cells and reagents with limited suppliers. Even though we aim to have backup supplies of raw materials, cells and reagents whenever possible, we cannot be certain those supplies will be sufficient if our primary sources are unavailable. **One or more of our suppliers is the sole source of certain materials used by us in our manufacturing process, and a disruption of the supply of those materials could also negatively impact our ability to manufacture clinical supply as we would have to suspend or revise our operations to accommodate for any disruption in the supply of those materials.** A shortage of a critical raw material, cell line, or reagent, or a technical issue during manufacturing, may lead to delays in clinical development or commercialization plans. We are particularly susceptible to any shortages, delays or inability to obtain suitable raw materials, given that all of our current and planned product candidates require this starting material. Any changes in the manufacturing of components of the raw materials we use could result in unanticipated or unfavorable effects in our manufacturing processes, resulting in delays. Once the biological products are manufactured, the product must be analyzed utilizing assays and meet pre- determined specifications in order to be used in certain preclinical studies, in any clinical trial, and, if approval is obtained, for commercial distribution. This testing is performed in- house and at third- party contract manufacturers. Delays or other unexpected obstacles **in developing analytical methods or** in performing the tests and obtaining the results in- house or at a third- party contractor could result in unanticipated impact to our ability to supply material as needed for pre- clinical, clinical, or commercial needs. ~~Neurogene~~ **We** and our contract manufacturers for AAV9 are subject to significant regulation with respect to manufacturing of our products. The third- party manufacturing facilities on which we rely, our in- house manufacturing facility, and any manufacturing facility that we may have in the future, may have limited capacity or fail to meet the applicable stringent regulatory requirements. We currently have relationships with a limited number of suppliers for the raw materials, including plasmids and virus banks, required by the manufacturing processes of our product candidates. Virus intended for use in our early preclinical studies has been and can be externally supplied; however, if we experience slowdowns or problems with our in- house manufacturing facility and are unable to establish or scale our internal manufacturing capabilities, we will need to continue to contract with manufacturers to produce the preclinical, clinical and commercial supply and such supply will be more uncertain and subject to delays. In addition, each supplier may require licenses to manufacture certain components of the supply if such processes are not owned by the supplier or in the public domain and we may be unable to license such intellectual property rights on reasonable commercial terms or to transfer or sublicense the intellectual property rights we may have with respect to such activities. All entities involved in the preparation of therapeutics for clinical trials or commercial sale, including our existing contract manufacturers for components of our product candidates, are subject to extensive regulation. Components of a finished therapeutic product approved for commercial sale or used in late- stage clinical trials must be manufactured in accordance with cGMP. These regulations govern manufacturing processes and procedures (including recordkeeping) and the implementation and operation of quality systems to control and assure the quality of investigational products and products approved for sale. Poor control of production processes can lead to the introduction of adventitious agents or other contaminants, or to inadvertent changes in the properties or stability of our product candidates that may not be detectable in final product testing. We or our contract manufacturers must supply all necessary documentation in support of a biologics license application (" BLA ") or marketing authorization application (" MAA ") on a timely basis. Our facilities and quality systems and the facilities and quality systems of some or all of our third- party contractors must pass a pre- approval inspection for compliance with the applicable regulations as a condition of regulatory approval of our current or future product candidates. In addition, regulatory authorities may, at any time, audit or inspect a manufacturing facility involved with the preparation of our current or future product candidates or the associated quality systems for compliance with the regulations applicable to the activities being conducted, and they could put a hold on one or more of our clinical trials if the facilities of our CDMOs do not pass such audit or inspections. If these facilities do not pass a pre- approval plant inspection, the FDA or other foreign regulatory agency approval of the products will not be granted. Regulatory authorities also may, at any time following approval of a product for sale, inspect or audit our manufacturing facilities or those of our third- party contractors. If any such inspection or audit identifies a failure to comply with applicable regulations or if a violation of our product specifications or applicable regulations occurs independent of such an inspection or audit, we or the relevant regulatory authority may require remedial measures that may be costly and / or time- consuming for us or a third party to implement, and that may include the temporary or permanent suspension of a clinical trial or commercial sales or the temporary or permanent closure of a facility. Any such remedial measures imposed upon us or third parties with whom we contract could harm our business. If we or any of our third- party manufacturers fail to maintain regulatory compliance, the FDA or other foreign regulatory agencies can impose regulatory sanctions including, among other things, refusal to approve a pending application for a new drug product or biologic product, or revocation of a pre- existing approval. As a result, our business, financial condition and results of operations may be harmed. Additionally, if supply from one approved manufacturer is interrupted, there could be a significant disruption in commercial supply. An alternative manufacturer would need to be qualified through a BLA and / or MAA supplement, which could result in further delay. The regulatory agencies may also require additional studies if a new manufacturer is relied upon for commercial production. Switching manufacturers may involve substantial costs and is likely to result in a delay in our desired clinical and commercial timelines. These factors could cause the delay of clinical trials, regulatory submissions, required approvals or commercialization of our product candidates, cause us to incur higher costs and prevent us from commercializing our products successfully, if approved. Further, if our suppliers fail to meet contractual requirements, and we are unable to secure one or more replacement suppliers capable of production at a substantially equivalent cost, our clinical trials may be delayed or we could lose future potential revenue, if any. We depend on third- party suppliers for materials used in the manufacture of our product candidates, and the loss of these third- party suppliers or their inability to supply us with adequate materials could harm our business. We rely on third- party suppliers for certain materials and components required for the production of our product candidates. Our dependence on these third- party suppliers and the challenges we may face in obtaining adequate supplies of materials involve

several risks, including limited control over pricing, availability and quality of supplies and delivery schedules. There is substantial demand and limited supply for certain of the raw materials used to manufacture gene therapy products. As a small company, our negotiation leverage is limited and we are likely to get lower priority than our larger competitors. We cannot be certain that our suppliers will continue to provide us with the quantities of raw materials that we require or satisfy our anticipated specifications and quality requirements. **One or more of our suppliers is the sole source of certain materials used by us in our manufacturing process, and a disruption of the supply of those materials could also negatively impact our ability to manufacture clinical supply as we would have to suspend or revise our operations to accommodate for any disruption in the supply of those materials.** Any supply interruption in limited or sole sourced raw materials could materially harm our ability to manufacture our product candidates until a new source of supply, if any, could be identified and qualified. We may be unable to find a sufficient alternative supply channel in a reasonable time or on commercially reasonable terms. Any performance failure on the part of our suppliers could delay the development and potential commercialization of our product candidates, including limiting supplies necessary for clinical trials and regulatory approvals, which would have a material adverse effect on our business. Delays in developing our manufacturing capabilities or failure to achieve operating efficiencies from such capabilities may require us to devote additional resources and management time to manufacturing operations and may delay our product development timelines. We have a GMP manufacturing facility located in Houston, Texas that includes process, analytical and bioanalytical development labs with experienced teams. NGN- 401 was manufactured at our Houston facility and clinical- grade product is available for dosing in the Phase 1 / 2 clinical trial of NGN- 401 that is currently enrolling patients. However, we will need to conduct additional NGN- 401 manufacturing campaigns to generate additional clinical supply, as well as supply for our preclinical studies for our discovery programs, and we may not be able to satisfy such supply through production at our own facility **and may need to outsource some or all of our production work** . Other risks relating to the manufacture of biologics and drug products include: production interruptions, delays in quality / release testing, equipment malfunctions, facility contamination, labor problems, natural disasters, disruption in utility services, terrorist activities, war, cases of force majeure **, weather- related events** , acts of god (such as public health crises) or other events beyond our control and, in each case, could result in delays in our production or difficulties in maintaining compliance with applicable regulatory requirements. Any contamination or interruption in our manufacturing process, shortages of raw materials or failure of our suppliers to deliver necessary components could result in delays in our clinical development or marketing schedules. Given the nature of gene therapy manufacturing, there is a risk of contamination. Any contamination could adversely affect our ability to produce product candidates on schedule and could, therefore, harm our results of operations and cause reputational damage. Some of the raw materials required in our manufacturing process are derived from biologic sources. Such raw materials are difficult to procure and may be subject to contamination or recall. A material shortage, contamination, recall or restriction on the use of biologically derived substances in the manufacture of our product candidates could adversely impact or disrupt the commercial manufacturing or the production of clinical material, which could adversely affect our development timelines and our business, financial condition, results of operations and prospects. We may not be able to successfully manufacture our product candidates in sufficient quality and quantity, which would delay or prevent us from developing our product candidates and commercializing resulting approved products, if any. To date, we have manufactured NGN- 401 in quantities and quality adequate for preclinical, toxicology and clinical studies. In order to conduct clinical trials for a product candidate and for commercialization of the resulting product if that product candidate is approved for sale, we will need to manufacture product candidates in additional cGMP campaigns or in larger batch sizes. We may not be able to successfully repeat or increase the manufacturing capacity for any of our product candidates in a timely or cost- effective manner or at all. Significant changes or scale- up of manufacturing may require additional validation studies **and / or analytical comparability studies** , which are costly and which regulatory authorities must review and approve. In addition, quality issues may arise during those changes or scale- up activities. If we are unable to successfully manufacture any of our product candidates in sufficient quality and quantity, the development of that product candidate and regulatory approval or commercial launch for any resulting products may be delayed or there may be a shortage in supply, which could significantly harm our business. Changes in methods of product candidate manufacturing or formulation may result in additional costs or delay. As product candidates proceed through preclinical studies to late- stage clinical trials towards potential approval and commercialization, it is common that various aspects of the development program, such as manufacturing methods and formulation, are altered along the way in an effort to optimize processes and product characteristics. Such changes carry the risk that they will not achieve our intended objectives. Any such changes could cause our product candidates to perform differently and affect the results of planned clinical trials or other future clinical trials conducted with the materials manufactured using altered processes. Such changes may also require additional testing, FDA notification or approval from the FDA or foreign regulatory agencies. This could delay completion of clinical trials, require the conduct of bridging clinical trials or the repetition of one or more clinical trials, increase clinical trial costs, delay approval of our product candidates and jeopardize our ability to commence sales and generate revenue. In addition, we may be required to make significant changes to our upstream and downstream processes across our pipeline, which could delay the development of our future product candidates. Risks Related to Our Reliance on Third Parties **We have a number of academic collaborations, and currently rely on our collaboration with the University of Edinburgh for certain aspects of our preclinical research and development programs, including working in collaboration to discover and preclinically develop our potential product candidates for our near- term future pipeline. Failure or delay of the University of Edinburgh or any other collaborator to fulfil all or part of its obligations under our agreements, a breakdown in collaboration between the parties or a complete or partial loss of the relationship would materially harm our business.** Our discovery engine is supplemented by academic collaborations to expand our platform, which we rely upon to advance discovery and development of product candidates. For example, our collaboration with the University of Edinburgh is critical to our business. In December 2020, we entered into a Master Collaboration Agreement (the " MCA ") with the

University of Edinburgh, which we rely on to conduct certain aspects of the preclinical development of our pipeline candidates, including NGN- 401 and all of our early- stage pipeline product candidates. Further, in March 2022, we entered into an exclusive license agreement with the University of Edinburgh for, with respect to certain University of Edinburgh- owned technology, a worldwide, exclusive, sublicensable license to develop, have developed, use, manufacture, have manufactured, supply, have supplied, sell, have sold, offer for sale, commercialize, import, export, register, reproduce, dispose of or otherwise exploit any products, processes, components, services and / or technologies incorporating the technology for the prevention or treatment of disease or medical or genetic conditions in humans. We also currently rely on the University of Edinburgh for portions of preclinical research capabilities under the direction of Dr. Stuart Cobb, Professor in Translational Neuroscience at the University of Edinburgh and our Chief Scientific Officer. Pursuant to the MCA, we and the University of Edinburgh agreed to collaborate on certain research and development projects (the " Projects "), and we agreed to provide funding for such Projects. In exchange for such funding, the University of Edinburgh grants us an option to exclusively license any intellectual property arising from such Projects. Either party has the right in certain circumstances to terminate the collaboration pursuant to the terms of the MCA. If the MCA is not renewed or is terminated, our pipeline of product candidates would be significantly adversely affected, and our business would be materially harmed. Following an amendment to the MCA in November 2023, the term of the research funding portion of the MCA, under which we have the ability to acquire exclusive rights to additional technology and gene therapy products, now expires in December 2026. If we need to extend the term of this provision beyond that date, we will need to negotiate an additional extension with the University of Edinburgh, and we may not be able to agree on such an extension on terms that are acceptable to us, or at all. We may have disagreements with the University of Edinburgh with respect to the interpretation of the MCA, use of resources or otherwise that could cause our relationship to deteriorate. As a result, the University of Edinburgh may reduce focus on, and resources allocated to, our programs, potentially delaying or terminating our ability to advance product candidates through preclinical studies. Additionally, if Dr. Cobb were to leave the University of Edinburgh or to otherwise no longer be meaningfully involved with us, our preclinical research and development capabilities may be substantially reduced. Further, under the MCA, the University of Edinburgh is primarily responsible for prosecuting and maintaining our licensed intellectual property, and it may fail to properly prosecute, maintain or defend such intellectual property. In such event, if we are unable to otherwise maintain or defend such intellectual property, we could face the potential invalidation of the intellectual property or be subjected to litigation or arbitration, any of which would be time-consuming and expensive. To enforce the licensed intellectual property rights under the MCA, we will need to coordinate with the University of Edinburgh, which could slow down or hamper our ability to enforce our licensed intellectual property rights. If this happens, we could face increased competition that could materially and adversely affect our business. For a further description of the MCA, see " ~~Business~~ **Management' s Discussion and Analysis of Financial Condition and Results of Operations** — License **and Collaboration** Agreements. " ~~Additionally, in May 2019, we entered into an exclusive license agreement with the University of North Carolina (" UNC ") for, with respect to the UNC invention known as " Optimized CLN5 Genes and Expression Cassettes and Their Use, " a worldwide, exclusive, sublicensable license to make, use, sell, have made, have sold, offer for sale and import any method or process, composition, product, or component part thereof for the prevention or treatment of disease or medical or genetic conditions, including CLN5 Batten disease or other diseases stemming from dysfunction of the CLN5 gene.~~ We also currently have or may in the future engage in other academic collaborations to supplement our internal discovery and product development program. While these academic institutions have contractual obligations to us, they are independent entities and are not under our control or the control of our officers or directors. Our research and licensing agreements with academic collaborators generally provide academic collaborators with license maintenance fees, development and regulatory milestone payments, royalties on net sales of products and a portion of sublicense income that we receive. Upon the scheduled expiration of any academic collaboration, we may not be able to renew the related agreement, or any renewal could be on terms less favorable to us than those contained in the existing agreement. Furthermore, either we or the academic institution generally may terminate the sponsored research agreement for convenience following a specified notice period. If any of these academic institutions decides to not renew or to terminate the related agreement or decides to devote fewer resources to such activities, our discovery efforts would be diminished, while our royalty obligations, if any, would continue unmodified. We currently rely, and intend in the future to rely, on third parties to conduct a significant portion of our preclinical studies and existing clinical trials and potential future clinical trials for product candidates, and those third parties may not perform satisfactorily, including failing to meet deadlines for the completion of such trials. We have engaged CROs or other third parties to conduct preclinical and IND enabling studies and our clinical trials, including our Phase 1 / 2 clinical trial of NGN- 401 ~~and Phase 1 / 2 clinical trial of NGN- 101~~. We expect to continue to rely on third parties, including CROs, medical institutions and clinical investigators, to conduct those clinical trials. Any of these third parties may terminate their engagements with us, some in the event of an uncured material breach and some at any time for convenience. If any of our relationships with these third parties terminate, we may not be able to timely enter into arrangements with alternative third parties or do so on commercially reasonable terms, if at all. Switching or adding CROs involves substantial cost and requires management time and focus. In addition, there is a natural transition period when a new CRO commences work. As a result, delays occur, which can materially impact our ability to meet our desired clinical development timelines. Though we intend to carefully manage our relationships with CROs, there can be no assurance that we will not encounter challenges or delays in the future or that these delays or challenges will not have a material adverse impact on our business and financial condition. In addition, any third parties conducting our clinical trials will not be our employees, and except for remedies available to us under our agreements with such third parties, we cannot control whether or not such third parties devote sufficient time and resources to our clinical programs. If these third parties do not successfully carry out their contractual duties or obligations or meet expected deadlines, if they need to be replaced or if the quality or accuracy of the clinical data they obtain is compromised due to the failure to adhere to our clinical protocols, regulatory requirements or for other reasons, our clinical

trials may be extended, delayed or terminated **, we may incur additional and unexpected costs related to such failures,** and we may not be able to obtain regulatory approval for or successfully commercialize our product candidates. Consequently, our results of operations and the commercial prospects for our product candidates would be harmed, our costs could increase substantially and our ability to generate revenue could be delayed significantly. Further, while our reliance on these third parties for research and development activities will reduce our control over these activities, we will not be relieved of our responsibilities for ensuring that each of our studies and trials is conducted in accordance with the applicable protocol, legal, regulatory and scientific standards. For example, we will remain responsible for ensuring that each of our clinical trials is conducted in accordance with the general investigational plan and protocols for the trial. Moreover, the FDA requires us to comply with GCPs for conducting, recording and reporting the results of clinical trials to assure that data and reported results are credible and accurate and that the rights, integrity and confidentiality of trial participants are protected. We also are required to register ongoing clinical trials and post the results of completed clinical trials on a government- sponsored database, ClinicalTrials. gov, within specified timeframes. Failure to do so can result in fines, adverse publicity and civil and criminal sanctions. If we or any of our CROs or other third parties, including trial sites, fail to comply with applicable GCPs, the clinical data generated in our clinical trials may be deemed unreliable and the FDA, EMA or comparable foreign regulatory authorities may require us to perform additional clinical trials before approving our marketing applications. We cannot assure you that upon inspection by a given regulatory authority, such regulatory authority will determine that any of our clinical trials complies with GCP regulations. In addition, our clinical trials must be conducted with product produced under cGMP conditions. Our failure to comply with these regulations may require us to repeat clinical trials, which would delay the regulatory approval process. In addition, principal investigators for our clinical trials may serve as scientific advisors or consultants to us from time to time and receive compensation in connection with such services. Under certain circumstances, we may be required to report some of these relationships to the FDA. The FDA may conclude that a financial relationship between us and a principal investigator has created a conflict of interest or otherwise affected interpretation of the trial. The FDA may therefore question the integrity of the data generated at the applicable clinical trial site and the utility of the clinical trial itself may be jeopardized. This could result in a delay in approval, or rejection, of our marketing applications by the FDA and may ultimately lead to the denial of marketing approval of NGN- 401 ~~and NGN- 101~~ or any other product candidates. We currently store drug product for clinical trial sites in the United States, and currently rely on and ~~expects~~ **expect** in the future to rely on third parties to distribute product supplies for our clinical trials, as well as to store and distribute supply for clinical trial sites outside of the United States. Any performance failure on the part of ~~Neurogene~~ **us** or our distributors could **result in an unexpected increase in costs to us,** delay clinical development or marketing approval of our product candidates or commercialization of our products, if approved, producing additional losses and depriving us of potential revenue. Risks Related to Our Business and Operations Over time, we expect to experience significant growth in the number of our employees and the scope of our operations, particularly in the areas of preclinical and clinical biological product development, technical operations, clinical operations, regulatory affairs, manufacturing and, potentially, sales, marketing and distribution. To manage our anticipated future growth, we must continue to implement and improve our managerial, operational and financial personnel and systems, expand our facilities and recruit and train additional qualified personnel. Due to our limited financial resources and the limited experience of our management team working together in managing a company with such anticipated growth, we may not be able to effectively manage the expansion of our operations or recruit and train additional qualified personnel. The expansion of our operations may lead to significant costs and may divert our management and business development resources. Any inability to manage growth could delay the execution of our business plans or disrupt our operations. We are highly dependent on our key personnel and anticipate hiring new key personnel. If we are not successful in attracting and retaining highly qualified personnel, we may not be able to successfully implement our business strategy. Our ability to compete in the highly competitive biotechnology and pharmaceutical industries depends upon our ability to attract and retain highly qualified managerial, scientific and medical personnel. We are highly dependent on our managerial, scientific and medical personnel, including our Founder and Chief Executive Officer, President and Chief Financial Officer, and Chief Scientific Officer, as well as other key members of our leadership team. Our executive officers may terminate their employment with us at any time. We do not maintain " key person " insurance for any of our executives or other employees. The loss of the services of our executive officers or other key employees could impede the achievement of our research, development and commercialization objectives and seriously harm our ability to successfully implement our business strategy. Furthermore, replacing executive officers and key personnel may be difficult and may take an extended period of time. Failure to attracting and retaining qualified personnel could materially and adversely affect our business, financial condition and results of operations. We could in the future have difficulty attracting and retaining experienced personnel and may be required to expend significant financial resources on our employee recruitment and retention efforts. Our future growth may depend, in part, on our ability to operate in foreign markets, where we would be subject to additional regulatory burdens and other risks and uncertainties. Our future growth may depend, in part, on our ability to develop and commercialize NGN- 401 or ~~NGN- 101 or~~ other product candidates in foreign markets for which we may rely on collaborations with third parties. We are not permitted to market or promote any product candidates before we receive regulatory approval from the applicable foreign regulatory authority, and may never receive such regulatory approval for any product candidates. To obtain separate regulatory approval in many other countries, we must comply with numerous and varying regulatory requirements of such countries regarding safety and efficacy and governing, among other things, clinical trials and commercial sales, pricing and distribution of NGN- 401 or ~~NGN- 101 or~~ other product candidates, and we cannot predict success in these jurisdictions. If we fail to comply with the regulatory requirements in international markets or to receive applicable marketing approvals, our target market will be reduced and our ability to realize the full market potential of NGN- 401 or ~~NGN- 101 or~~ other product candidates will be harmed and our business will be adversely affected. Moreover, even if we obtain approval of NGN- 401 ~~or NGN- 101~~ or other product candidates and ultimately commercialize such product candidates in

foreign markets, we would be subject to the risks and uncertainties of operating in such foreign markets, including the burden of complying with complex and changing foreign regulatory, tax, accounting and legal requirements and reduced protection of intellectual property rights in some foreign countries. Our employees, independent contractors, consultants, commercial collaborators, principal investigators, CROs, CDMOs, suppliers and vendors may engage in misconduct or other improper activities, including noncompliance with regulatory standards and requirements. We are exposed to the risk that our employees, independent contractors, consultants, commercial collaborators, principal investigators, CROs, CDMOs, suppliers and vendors acting for or on our behalf may engage in misconduct or other improper activities. It is not always possible to identify and deter misconduct by these parties and the precautions we take to detect and prevent this activity may not be effective in controlling unknown or unmanaged risks or losses or in protecting us from governmental investigations or other actions or lawsuits stemming from a failure to comply with these laws or regulations. Our ~~internal computer systems have suffered a security breach and in the future our~~ systems, or those of any of our CROs, manufacturers, other contractors, third party service providers or consultants or potential future collaborators, may fail or suffer ~~additional~~ security or data privacy breaches or other unauthorized or improper access to, use of, or destruction of our proprietary or confidential data, employee data or personal data, which could result in additional costs, loss of revenue, significant liabilities, harm to our brand and material disruption of our operations. Despite the implementation of security measures in an effort to protect systems that store our information, given the size and complexity of such systems and the increasing amounts of information maintained on our internal information technology systems and those of our third- party CROs, other contractors (including sites performing our clinical trials), third-party service providers and supply chain companies, consultants and other partners, these systems are potentially vulnerable to breakdown or other damage or interruption from service interruptions, system malfunction, natural disasters, terrorism, war **,** and telecommunication and electrical failures, as well as security breaches from inadvertent or intentional actions by our employees, contractors, consultants, business partners and / or other third parties, or from cyber- attacks by malicious third parties, which may compromise our system infrastructure or lead to the loss, destruction, alteration or dissemination of, or damage to, our data. From time to time, we are subject to business email compromise attack attempts. In August 2023, we discovered a business email compromise attack that resulted in the misappropriation of approximately $ 0. 9 million. While we have implemented remedial measures in response to this incident **and recovered $ 0. 8 million of those losses through insurance claims** , we cannot guarantee that such measures will prevent additional related, as well as unrelated incidents, or that we will be able to defend against or successfully remediate any such attacks that may occur in the future. If a material system failure, accident or security breach were to occur and cause interruptions in our operations or the operations of third- party collaborators, service providers, contractors and consultants, it could result in a material disruption of our development programs and significant reputational, financial, legal, regulatory, business or operational harm. Further, since we sponsor clinical trials, any breach that compromises patient data and identities causing a breach of privacy could have significant adverse consequences on our business. For example, the loss of clinical trial data from completed or future clinical trials could affect trust in us, negatively impacting our ability to recruit for future clinical trials, result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data. To the extent that any disruption or security breach were to result in a loss, destruction, unavailability, alteration or dissemination of, or damage to, our data or applications, or inappropriate disclosure of confidential proprietary information, or for it to be believed or reported that any of these occurred, we could incur liability and reputational damage and the development and commercialization of ~~NGN- 101 or~~ NGN- 401 or other product candidates could be delayed. As our employees work remotely and use network connections, computers, and devices outside of our premises or network, including working at home, while in transit and in public locations, there are risks to our information technology systems and data. Additionally, business transactions (such as acquisitions or integrations) could expose us to additional cybersecurity risks and vulnerabilities, as our systems could be negatively affected by vulnerabilities present in acquired or integrated entities' systems and technologies. While we have implemented security measures designed to protect against security incidents, there can be no assurance that these measures will be effective. We may be unable in the future to detect vulnerabilities in our information technology systems because such threats and techniques change frequently, are often sophisticated in nature, and may not be detected until after a security incident has occurred. Further, we may experience delays in developing and deploying remedial measures designed to address any such identified vulnerabilities. Applicable data privacy and security obligations may require us to notify relevant stakeholders, patients or other individuals, regulators or, in certain circumstances, the media of security incidents. Such disclosures are costly, and the disclosure or the failure to comply with such requirements could lead to adverse consequences, including damage to our reputation. We rely on third- party service providers and technologies to operate critical business systems, including to process sensitive information in a variety of contexts. Our ability to monitor these third parties' information security practices is limited, and these third parties may not have adequate information security measures in place. If our third- party service providers experience a security incident or other interruption, we could experience adverse consequences as a result. While we may be entitled to damages if our third- party service providers fail to satisfy their privacy or security- related obligations to us, any award may be insufficient to cover our monetary, reputational and other damages, or we may be unable to recover such award. In addition, supply- chain attacks have increased in frequency and severity, and we cannot guarantee that third parties' infrastructure in our supply chain or our third- party partners' supply chains have not been and will not be compromised. If we (or a third party upon whom we rely) experiences a security incident or is perceived to have experienced a security incident, we may experience adverse consequences, such as government enforcement actions (for example, investigations, fines, penalties, audits, and inspections); additional reporting requirements and / or oversight; restrictions on processing personal information (including sensitive data); litigation (including class claims); indemnification obligations; negative publicity; reputational harm; monetary fund diversions; interruptions in our operations (including availability of data); increased investigation and compliance costs; financial loss; and other similar harms. Security incidents and attendant consequences may cause our stakeholders (including investors and potential customers) to stop

supporting our business, deter new customers from our products, deter patients from participating in clinical trials and negatively impact our ability to grow and operate our business. Our contracts may not contain limitations of liability, and even where they do, there can be no assurance that limitations of liability in our contracts are sufficient to protect us from liabilities, damages, or claims related to our data privacy and security obligations. We cannot be sure that our insurance coverage will be adequate or sufficient to protect us from or to mitigate liabilities arising out of our privacy and security practices or from disruptions in, or failure or security breach of, our systems or third- party systems where information important to our business operations or commercial development is stored, or that such coverage will continue to be available on commercially reasonable terms or at all, or that such coverage will pay future claims. We are subject to stringent and changing laws, regulations and standards, and contractual obligations relating to privacy, data protection, and data security. The actual or perceived failure to comply with such obligations could lead to government enforcement actions (which could include civil or criminal penalties), fines and sanctions, private litigation, injunctive restrictions on data processing and / or adverse publicity and could negatively affect our operating results and business. We, and third parties with whom we work, are or may become subject to numerous domestic and foreign laws, regulations, and standards relating to privacy, data protection, and data security, the scope of which are changing, subject to differing applications and interpretations, and may be inconsistent among countries, or conflict with other rules. **In addition, there is proposed legislation in the U. S. Congress that could restrict working with certain biotech providers who are deemed " companies of concern " which may impact the ability of certain third parties with whom we work to meet their performance obligations.** We are or may become subject to the terms of contractual obligations related to privacy, data protection, and data security. Our obligations may also change or expand as our business grows. The actual or perceived failure by us or third parties related to us to comply with such laws, regulations and obligations could increase our compliance and operational costs, expose us to regulatory scrutiny, actions, fines and penalties, result in reputational harm, lead to a loss of customers, result in litigation and liability, subject us to injunctive restrictions on data processing **, adversely impact our ability to appropriately manage third parties with whom we work** and otherwise cause a material adverse effect on our business, financial condition, and results of operations. See ~~the sections entitled~~ " Business — Government Regulation — Data Privacy and Security " and " — Other Regulatory Matters " **in our Annual Report on Form 10- K** for a more detailed description of the laws that may affect our ability to operate. If we fail to comply with environmental, health and safety laws and regulations, we could become subject to fines or penalties or incur costs that could have a material adverse effect on the success of our business. We are subject to numerous environmental, health and safety laws and regulations, including those governing laboratory procedures and the handling, use, storage, treatment and disposal of hazardous materials and wastes. Our operations may involve the use of hazardous and flammable materials, including chemicals and biological and radioactive materials. In addition, we may incur substantial costs in order to comply with current or future environmental, health and safety laws and regulations. These current or future laws and regulations may impair our research, development or commercialization efforts. Failure to comply with these laws and regulations also may result in substantial fines, penalties or other sanctions. Our ability to utilize our net operating loss carryforwards and certain other tax attributes may be limited. As of December 31, ~~2023~~ **2024** , ~~Neurogene~~ **we** had net operating loss **(" NOL ")** carryforwards for federal and state income tax purposes of $ ~~277~~ **319** . ~~9~~ **8** million and $ ~~35~~ **39** . ~~1~~ **6** million, respectively. The federal **NOLs** ~~net operating losses~~ will not be subject to expiration and can be carried forward indefinitely; however, they are limited to a deduction to 80 % of annual taxable income. The state **NOLs** ~~net operating losses~~ begin to expire in 2038. To the extent that our taxable income exceeds any current year operating losses, we plan to use our carryforwards to offset income that would otherwise be taxable. Also, for state income tax purposes, the extent to which states will conform to the federal laws is uncertain and there may be periods during which the use of **NOL** ~~net operating loss~~ carryforwards are suspended or otherwise limited, which could accelerate or permanently increase state taxes owed. In addition, under Section 382 of the Code, changes in our ownership may limit the amount of our **NOL** ~~net operating loss~~ carryforwards and tax credit carryforwards that could be utilized annually to offset our future taxable income, if any. This limitation would generally apply in the event of a cumulative change in ownership of more than 50 % (as measured by value) among a stockholder or one or more groups of stockholders who own at least 5 % of our stock within a three- year period. We have not performed an analysis to determine whether there has been an ownership change pursuant to Section 382. Any such limitation may significantly reduce our ability to utilize our **NOL** ~~net operating loss~~ carryforwards and tax credit carryforwards before they expire. Any such limitation, whether as the result of a public offering, private placements, sales of our common stock by our existing stockholders or additional sales of our common stock by us, could have a material adverse effect on our results of operations in future years. We may be subject to adverse legislative or regulatory tax changes that could negatively impact our financial condition. The rules dealing with U. S. federal, state and local income taxation are constantly under review by persons involved in the legislative process and by the Internal Revenue Service and the U. S. Treasury Department. Changes to tax laws (which changes may have retroactive application) could adversely affect us or our stockholders. We assess the impact of various tax reform proposals and modifications to existing tax treaties in all jurisdictions where we have operations **and employees** to determine the potential effect on our business and any assumptions we ~~have made~~ **make** about our future taxable income. We cannot predict whether any specific proposals will be enacted, the terms of any such proposals or what effect, if any, such proposals would have on our business if they were to be enacted. For example, the United States recently enacted the Inflation Reduction Act of 2022, which implements, among other changes, a 1 % excise tax on certain stock buybacks. In addition, beginning in 2022, the Tax Cuts and Jobs Act ~~eliminates~~ **eliminated** the ~~currently available~~ option to deduct research and development expenditures and requires taxpayers to amortize them generally over five years. The U. S. Congress is considering legislation that would restore the current deductibility of research and development expenditures, however, there is no assurance that the provision will be repealed or otherwise modified. Such changes, among others, may adversely affect our effective tax rate, results of operation and general business condition. We may acquire businesses or products, or form strategic alliances, in the future, and may not realize the benefits of such acquisitions. We may acquire

additional businesses or products, form strategic alliances, or create joint ventures with third parties that we believe will complement or augment our existing business. If we acquire businesses with promising markets or technologies, we may not be able to realize the benefit of acquiring such businesses if we are unable to successfully integrate them with our existing operations and company culture. We may encounter numerous difficulties in developing, manufacturing and marketing any new product candidates or products resulting from a strategic alliance or acquisition that delay or prevent us from realizing their expected benefits or enhancing our business. There is no assurance that, following any such acquisition, we will achieve the synergies expected in order to justify the transaction, which could result in a material adverse effect on our business and prospects. We maintain our cash at financial institutions, at times in balances that exceed federally- insured limits. The failure of financial institutions could adversely affect our ability to pay our operational expenses or make other payments. Our cash held in non- interest- bearing and interest- bearing accounts at financial institutions can at times exceed the Federal Deposit Insurance Corporation (" FDIC ") insurance limits. If such banking institutions were to fail, we could lose all or a portion of those amounts held in excess of such insurance limitations. For example, the FDIC took control of Silicon Valley Bank on March 10, 2023. The Federal Reserve subsequently announced that account holders would be made whole. However, the FDIC may not make all account holders whole in the event of future bank failures. In addition, even if account holders are ultimately made whole with respect to a future bank failure, account holders' access to their accounts and assets held in their accounts may be substantially delayed. Any material loss that we may experience in the future or inability for a material time period to access our cash and cash equivalents could have an adverse effect on our ability to pay our operational expenses or make other payments, which could adversely affect our business. At the end of August 2023, we identified a material weakness in our internal control over financial reporting and may identify additional material weaknesses in the future that may cause us to fail to meet our reporting obligations or result in material misstatements of our financial statements. If our internal control over financial reporting or our disclosure controls and procedures are not effective, we may not be able to accurately report our financial results, prevent fraud or file our periodic reports in a timely manner, which may cause investors to lose confidence in our reported financial information and may lead to a decline in our share price. Our internal controls related to the cash disbursements process were not adequately designed to identify unauthorized payment requests, resulting in the identification of a material weakness. Specifically, at the end of August 2023, we discovered that we were subject to a business email compromise attack by a third party. This deficiency in our controls resulted in the diversion of payments to fraudulent bank accounts. We determined that certain internal controls required for safeguarding our cash assets were not properly designed due to insufficient specificity regarding our policies and procedures surrounding supplier banking information changes, not identifying segregation of duties, and insufficient training on exercising professional skepticism. We therefore implemented steps to remediate this control deficiency, including increasing communication of and training around our controls relating to changes made to information, emphasizing security awareness and the importance of professional skepticism and designing a process to review supplier information changes prior to release of payments. While our management determined based on the assessment of internal control over financial reporting that as of December 31, 2023, this material weakness had been remediated, there can be no assurance that the remediation plans we implemented relating to this business email compromise attack will be successful in preventing a repeat of that attack or that we will be able to avoid potential future material weaknesses. If we are unable to successfully remediate existing or any future material weakness in our internal control over financial reporting, or if we identify any additional material weaknesses, the accuracy and timing of our financial reporting may be adversely affected, we may be unable to maintain compliance with securities law and applicable stock exchange listing requirements regarding timely filing of periodic reports, investors may lose confidence in our financial reporting, and our stock price may decline as a result. We also could become subject to investigations by Nasdaq, the SEC or other regulatory authorities. Risks Related to Intellectual Property Our ability to protect our patents and other proprietary rights is uncertain, exposing us to the possible loss of competitive advantage. We rely and expect to continue to rely upon a combination of patents, trademarks, trade secret protection and confidentiality agreements to protect the intellectual property related to our product candidates and technologies and to prevent third parties from unfairly competing with us. Our success depends in large part on our ability to obtain and maintain patent protection for platform technologies, including our EXACT ~~gene~~ **transgene** regulation platform, product candidates and their uses, as well as the ability to operate without infringing on or violating the proprietary rights of others. As of December 31, ~~2023~~ **2024** , we license ~~17~~ **29** patent applications, including U. S. patent applications, international patent applications under the Patent Cooperation Treaty or otherwise, and expect to continue to file patent applications in the United States and abroad related to discoveries and technologies that are important to our business. However, we may not be able to protect our intellectual property rights throughout the world and the legal systems in certain countries may not favor enforcement or protection of patents, trade secrets and other intellectual property. Filing, prosecuting and defending patents on product candidates worldwide would be prohibitively expensive and our intellectual property rights in some foreign jurisdictions may be less extensive than those in the United States. As such, we do not have patents in all countries or all major markets and may not be able to obtain patents in all jurisdictions even if we apply for them. Competitors may operate in countries where we do not have patent protection and could then freely use our technologies and discoveries in such countries to the extent such technologies and discoveries are publicly known or disclosed in countries where patent protection has not been requested **. In addition, competitors may be able to design around our patents to create technologies that directly compete with ours without infringing our intellectual property** . Our intellectual property portfolio is at an early stage. As of December 31, ~~2023~~ **2024** , we do not own or in- license any issued patents. Our pending and future patent applications may not result in patents being issued. Any issued patents may not afford sufficient protection of our product candidates or their intended uses against competitors, nor can there be any assurance that the patents issued will not be infringed, designed around, invalidated by third parties, or effectively prevent others from commercializing competitive technologies, products or product candidates. Even if these patents are granted, they may be difficult to enforce. Further, any issued patents that may be licensed or owned covering

our product candidates could be narrowed or found invalid or unenforceable if challenged in court or before administrative bodies in the United States or abroad, including the United States Patent and Trademark Office (" USPTO "). Further, if we encounter delays in any clinical trials or delays in obtaining regulatory approval, the period of time during which we could market product candidates under patent protection would be reduced. Thus, the patents that we may own or license may not afford any meaningful competitive advantage. In addition to seeking patents for some of our technology and product candidates, we may also rely on trade secrets, including unpatented know- how, technology and other proprietary information, to maintain our competitive position. Any disclosure, either intentional or unintentional, by our employees, the employees of third parties with whom we share facilities or third- party consultants and vendors that we engage to perform researches, 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 the market. In order to protect our proprietary technology and processes, we rely in part on confidentiality agreements with collaborators, employees, consultants, outside scientific collaborators and sponsored researchers and other advisors. These agreements may not effectively prevent disclosure of confidential information and may not provide an adequate remedy in the event of unauthorized disclosure of confidential information. We may need to share our proprietary information, including trade secrets, with future business partners, collaborators, contractors and others located in countries at heightened risk of theft of trade secrets, including through direct intrusion by private parties or foreign actors and those affiliated with or controlled by state actors. In addition, while we undertake efforts to protect our trade secrets and other confidential information from disclosure, others may independently discover trade secrets and proprietary information, and in such cases, we may not be able to assert any trade secret rights against such party. Costly and time- consuming litigation could be necessary to enforce and determine the scope of our proprietary rights and failure to obtain or maintain trade secret protection could adversely affect our competitive business position. Lastly, if our trademarks and trade names are not registered or adequately protected, then we may not be able to build name recognition in markets of interest and our business may be adversely affected. We may not be successful in obtaining or maintaining necessary rights to product candidates through acquisitions and in- licenses. Because our development programs require and may in the future require the use of proprietary rights held by third parties, the growth of our business may depend in part on our ability to acquire, in- license, or use these third- party proprietary rights. We may be unable to acquire or in- license any compositions, methods of use, processes or other third- party intellectual property rights from third parties that we identify as necessary for product candidates. The licensing and acquisition of third- party intellectual property rights is a competitive area, and a number of more established companies may pursue strategies to license or acquire third- party intellectual property rights that we may consider attractive or necessary. 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 assign or 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 investment or at all. 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 product candidate, which could have a material adverse effect on our business, financial condition, results of operations, and prospects. While we will normally seek to obtain the right to control prosecution, maintenance and enforcement of the patents relating to a product candidate, there may be times when the filing and prosecution activities for patents and patent applications relating to a product candidate are controlled by future licensors or collaboration partners. For example, we currently license several patent families from the University of Edinburgh covering the EXACT ~~gene~~ **transgene** regulation platform, as well as the NGN- 401 product candidate and its uses ~~. We also license a patent family covering the NGN- 101 product candidate and its uses from UNC~~ . If any of such licensors or collaboration partners fail to prosecute, maintain and enforce such patents and patent applications in a manner consistent with the best interests of our business, including by payment of all applicable fees for patents covering a product candidate, we could lose rights to the intellectual property or exclusivity with respect to those rights, our ability to develop and commercialize such candidates may be adversely affected and we may not be able to prevent competitors from making, using and selling competing products. In addition, even where we have the right to control patent prosecution of patents and patent applications which may be licensed to and from third parties, we may still be adversely affected or prejudiced by actions or inactions of licensees, future licensors and their counsel that took place prior to the date upon which we assumed control over patent prosecution. Our future licensors may rely on third- party consultants or collaborators or on funds from third parties such that future licensors are not the sole and exclusive owners of the patents we in- license. If other third parties have ownership rights to future in- licensed patents, they may be able to license such patents to our competitors, and the competitors could market competing products and technology. This could have a material adverse effect on our competitive position, business, financial conditions, results of operations, and prospects. It is possible that we may be unable to obtain licenses at a reasonable cost or on reasonable terms, if at all. Even if we are able to obtain a license, it may be non- exclusive, thereby giving competitors access to the same technologies licensed to us. In that event, we may be required to expend significant time and resources to redesign our technology, product candidates, or the methods for manufacturing the same, 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 develop or commercialize the affected product candidates, which could harm our business, financial condition, results of operations, and prospects significantly. We cannot provide any assurances that third- party patents do not exist which might be enforced against our current technology or manufacturing methods, our product candidates, or future methods or product candidates, resulting in either an injunction prohibiting manufacture or future sales, or, with respect to future sales, an obligation on our part to pay royalties and / or other forms of compensation to third parties, which could be significant. Disputes may arise between us and our future licensors regarding intellectual property subject to a license agreement, including: the scope of rights granted under the license agreement and other interpretation- related issues; whether

and to what extent to which our technology and processes infringe on intellectual property of the licensor that is not subject to the licensing agreement; our right to sublicense patents and other rights to third parties; our right to transfer or assign the license; the inventorship and ownership of inventions and know- how resulting from the joint creations or use of intellectual property by future licensors and us and / or our partners; and the priority date of an invention of patented technology. Certain of our current product candidates and research programs are licensed from or based upon licenses from a third party and are field limited to certain indications. If these license agreements are terminated or interpreted to narrow our rights, our ability to advance our current product candidates or develop new product candidates based on these technologies will be materially adversely affected. We depend on, and will continue to depend on, our current licenses with ~~UNC, the~~ University of Edinburgh, Virovek, Inc. (" Virovek ") ~~and~~ **,** Sigma- Aldrich Co. LLC (" Sigma **"), and Leland Stanford Junior University (" Stanford** "), and on licenses and sublicenses from other third parties, as well as potentially on other strategic relationships with third parties, for the research, development, manufacturing and commercialization of our current product candidates. If any of our licenses or relationships or any in- licenses on which our licenses are based are terminated or breached, we may: • lose our rights to develop and market our current product candidates; • lose patent or trade secret protection for our current product candidates; • experience significant delays in the development or commercialization of our current product candidates; • not be able to obtain any other licenses on acceptable terms, if at all; or • incur liability for damages. Additionally, even if not terminated or breached, our intellectual property licenses or sublicenses may be subject to disagreements over contract interpretation, which could narrow the scope of our rights to the relevant intellectual property or technology or increase our financial or other obligations. If we experience any of the foregoing, it could have a materially adverse effect on our business and could force us to cease operations. If we fail to comply with our obligations in any agreements under which we may 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 are party to license agreements with ~~UNC, the~~ University of Edinburgh, Virovek ~~and~~ **,** Sigma **and Stanford** and may from time to time in the future be party to other license and collaboration agreements with third parties to advance our research or allow commercialization of current or future product candidates. Such agreements may impose numerous obligations, such as development, diligence, payment, commercialization, funding, milestone, royalty, sublicensing, insurance, patent prosecution, enforcement and other obligations on us and may require us to meet development timelines, or to exercise commercially reasonable efforts to develop and commercialize licensed products, in order to maintain the licenses ~~. See " Business — License Agreements " for more information regarding our license agreements with UNC, the University of Edinburgh, Virovek and Sigma~~ . Despite our best efforts, our licensors might conclude that we have materially breached our license agreements and might therefore terminate the license agreements, thereby removing or limiting our ability to develop and commercialize products and technologies covered by these license agreements. If these licenses are terminated for any reason, or if the underlying patents fail to provide the intended exclusivity, we could lose significant rights and our ability to commercialize our current or future product candidates may be harmed, and competitors or other third parties would have the freedom to seek regulatory approval of, and to market, products identical to ours and we may be required to cease our development and commercialization of certain of our current or future product candidates. Any of the foregoing could have a material adverse effect on our competitive position, business, financial conditions, results of operations, and prospects. Disputes may also arise between us and our licensors regarding intellectual property subject to a license agreement, including: • the scope of rights granted under the license agreement and other interpretation- related issues; • whether and the extent to which our technology and processes infringe, misappropriate or otherwise violate intellectual property rights of the licensor that is not subject to the licensing agreement; • our right to sublicense patent and other rights to third parties under collaborative development relationships; • our diligence obligations with respect to the use of the licensed technology in relation to the development and commercialization of our current or future product candidates, and what activities satisfy those diligence obligations; • the priority of invention of any patented technology; and • the ownership of inventions and know- how resulting from the joint creation or use of intellectual property by our current or future licensors and by us and our other partners. In addition, the agreements under which we may license intellectual property or technology from third parties are likely to be 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, or increase what we believe to be our financial or other obligations under the relevant agreement, either of which could have a material adverse effect on our business, financial condition, results of operations and prospects. Moreover, if disputes over intellectual property that we may license prevent or impair our ability to maintain future licensing arrangements on acceptable terms, we may be unable to successfully develop and commercialize the affected current or future product candidates, which could have a material adverse effect on our business, financial conditions, results of operations and prospects. We may be subject to patent infringement claims or may need to file claims to protect our intellectual property, which could result in substantial costs, liability and diversion of resources, and prevent or delay us from commercializing potential products. Because the intellectual property landscape in the biotechnology industry is rapidly evolving and interdisciplinary, it is difficult to conclusively assess our freedom to operate and guarantee that we can operate without infringing on or violating third party rights. If certain of our product candidates are ultimately granted regulatory approval, patent rights held by third parties, if found to be valid and enforceable, could be alleged to render one or more of such product candidates infringing. We cannot be certain that patents owned or licensed by us will not be challenged by others in the course of litigation. If a third party successfully brings a claim against us, we may be required to pay substantial damages, be forced to abandon any affected product candidate and / or seek a license from the patent holder. In addition, any uncertainties resulting from the initiation and continuation of any litigation could have a material adverse effect on our business. Competitors may infringe or otherwise violate our patents, trademarks, copyrights or other intellectual property. To counter infringement or other violations, we may be required to file claims, which can be expensive and time- consuming. Any such claims could

provoke these parties to assert counterclaims against us, including claims alleging that our intellectual property, methods or products infringes their patents or other intellectual property rights. In addition, in a patent infringement proceeding, a court or administrative body may decide that one or more of the patents we assert is invalid or unenforceable, in whole or in part, construe the patent' s claims narrowly or refuse to prevent the other party from using the technology at issue on the grounds that our patents do not cover the technology. Similarly, if we assert trademark infringement claims, a court or administrative body may determine that the marks asserted are invalid or unenforceable or that the party against whom we have asserted trademark infringement has superior rights to the marks in question. In such a case, we could ultimately be forced to cease use of such marks. In any intellectual property litigation, even if we are successful, any award of monetary damages or other remedy received may not be commercially valuable. Further, we may be required to protect our patents through procedures created to attack the validity of a patent at the USPTO. An adverse determination in any such submission or proceeding could reduce the scope or enforceability of, or invalidate, our patent rights, which could adversely affect our competitive position. Because of a lower evidentiary standard in USPTO proceedings compared to the evidentiary standard in U. S. federal courts necessary to invalidate a patent claim, a third party could potentially provide evidence in a USPTO proceeding sufficient for the USPTO to hold a claim invalid even though the same evidence would be insufficient to invalidate the claim if first presented in a district court action. If we are required to defend intellectual property actions brought by third parties, or if we sue to protect our own intellectual property rights or otherwise to protect our proprietary information and to prevent its disclosure, or if we are involved in other litigation, whether as a plaintiff or defendant, and whether or not successful, we may incur substantial legal expenses and the attention of our management and key personnel may be diverted from business operations. Further, some of our competitors may be able to sustain the costs of complex intellectual property litigation more effectively than we can because they have substantially greater resources. In addition, if our product candidates are found to infringe the intellectual property rights of third parties, these third parties may assert infringement claims against our future licensees and other parties with whom we have business relationships and we may be required to indemnify those parties for any damages they suffer as a result of these claims, which may require us to initiate or defend protracted and costly litigation on behalf of licensees and other parties regardless of the merits of such claims. If any of these claims succeed, we may be forced to pay damages on behalf of those parties or may be required to obtain licenses for the products they use, and may not be able to obtain such licenses on terms acceptable to us, if at all. Furthermore, because of the substantial amount of discovery required in connection with intellectual property litigation or other legal proceedings relating to our intellectual property rights, there is a risk that some of our confidential information could be compromised by disclosure during this type of litigation or other proceedings. We may be subject to claims that we have wrongfully hired an employee from a competitor or that employees, consultants or independent contractors have wrongfully used or disclosed confidential information of third parties. As is common in the biotechnology industry, in addition to employees, we engage consultants to assist in the development of our product candidates. Many of these consultants, and many of our employees, were or may have been previously employed at, or may have previously provided or may be currently providing consulting services to, other biotechnology or pharmaceutical companies including our competitors or potential competitors. We could in the future be subject to claims that we or our employees or consultants working on our behalf have inadvertently or otherwise used or disclosed alleged trade secrets or other confidential information of former employers or competitors. Although we try to ensure that our employees and consultants do not use the intellectual property, proprietary information, know- how or trade secrets of others in their work for us, we may become subject to claims that we caused an employee to breach the terms of his or her non- competition or non- solicitation agreement, or that we or these individuals have, inadvertently or otherwise, used or disclosed the alleged trade secrets or other proprietary information of a former employer or competitor. We may litigate to defend ourselves against these claims, and even if we are successful, litigation could result in substantial costs and could be a distraction to management. If our defenses to these claims fail, in addition to requiring us to pay monetary damages, a court could prohibit us from using technologies or features that are essential to our product candidates, if such technologies or features are found to incorporate or be derived from the trade secrets or other proprietary information of the former employers. Moreover, any such litigation or the threat thereof may adversely affect our reputation, our ability to form strategic alliances or sublicense our rights to collaborators, engage with scientific advisors or hire employees or consultants, each of which would have an adverse effect on our business, operations and financial condition. Changes to patent laws in the United States and other jurisdictions could diminish the value of patents in general, thereby impairing our ability to protect our products. Changes in either the patent laws or interpretation of patent laws in the United States, including patent reform legislation such as the Leahy- Smith America Invents Act (the " Leahy- Smith Act "), could increase the uncertainties and costs surrounding the prosecution of our owned and any future in- licensed patent applications and the maintenance, enforcement or defense of our owned and any future in- licensed issued patents. The Leahy- Smith Act includes a number of significant changes to U. S. patent law. These changes include provisions that affect the way patent applications are prosecuted, redefine prior art, provide more efficient and cost- effective avenues for competitors to challenge the validity of patents, and enable third- party submission of prior art to the USPTO during patent prosecution along with additional procedures to attack the validity of a patent at USPTO- administered post- grant proceedings, including post- grant review, inter partes review, and derivation proceedings. Assuming that other requirements for patentability are met, prior to March 16, 2013, in the United States, the first to invent the claimed invention was entitled to the patent, while outside the United States, the first to file a patent application was entitled to the patent. After March 16, 2013, under the Leahy- Smith Act, the United States transitioned to a first- to- file system in which, assuming that the other statutory requirements for patentability are met, the first inventor to file a patent application will be entitled to the patent on an invention regardless of whether a third party was the first to invent the claimed invention. As such, the Leahy- Smith Act and its implementation could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents, all of which could have a material adverse effect on our business, financial condition, our operations and prospects. In

addition, the patent positions of companies in the development and commercialization of biologics and pharmaceuticals are particularly uncertain. U. S. Supreme Court and U. S. Court of Appeals for the Federal Circuit rulings have narrowed the scope of patent protection available in certain circumstances and weakened the rights of patent owners in certain situations, including in the antibody arts. This combination of events has created uncertainty with respect to the validity and enforceability of patents once obtained. Depending on future actions by the U. S. Congress, the federal courts and the USPTO, the laws and regulations governing patents could change in unpredictable ways that could have a material adverse effect on our patent rights and our ability to protect, defend and enforce our patent rights in the future. Geopolitical actions in the United States and in foreign countries could increase the uncertainties and costs surrounding the prosecution or maintenance of patent applications and the maintenance, enforcement or defense of issued patents. Accordingly, our competitive position may be impaired, and our business, financial condition, operations and prospects may be adversely affected. In addition, a European Unified Patent Court (" UPC ") came into force in June 2023. The UPC is a common patent court to hear patent infringement and revocation proceedings effective for member states of the **EU** ~~European Union~~ . This could enable third parties to seek revocation of a European patent in a single proceeding at the UPC rather than through multiple proceedings in each of the jurisdictions in which the European patent is validated. We currently have three pending European applications, and if we obtain such patents and applications in the future, any such revocation and loss of patent protection could have a material adverse impact on our business and our ability to commercialize or license our technology and products. Moreover, the controlling laws and regulations of the UPC will develop over time, and may adversely affect our ability to enforce or defend the validity of any European patents obtained. We may decide to opt out from the UPC for any future European patent applications that we may file and any patents we may obtain. If certain formalities and requirements are not met, however, such European patents and patent applications could be challenged for non- compliance and brought under the jurisdiction of the UPC. We cannot be certain that future European patents and patent applications will avoid falling under the jurisdiction of the UPC, if we decide to opt out of the UPC. Obtaining and maintaining patent protection depends on compliance with various procedural, document submissions, fee payment and other requirements imposed by governmental patent agencies, and our patent protection could be reduced or eliminated for non- compliance with these requirements. Periodic maintenance fees, renewal fees, annuities fees and various other governmental fees on patents and / or patent applications are due to be paid to the USPTO and foreign patent agencies in several stages over the lifetime of the patent and / or patent application. The USPTO and various foreign governmental patent agencies also require compliance with a number of procedural, documentary, fee payment and other similar provisions during the patent application process. While an inadvertent lapse can in many cases be cured by payment of a late fee or by other means in accordance with the applicable rules, there are situations in which noncompliance can result in abandonment or lapse of the patent or patent application, resulting in partial or complete loss of patent rights in the relevant jurisdiction. Non-compliance events that could result in abandonment or lapse of a patent or patent application include, but are not limited to, failure to respond to official actions within prescribed time limits, non- payment of fees and failure to properly legalize and submit formal documents. If we fail to maintain the patents and patent applications covering our product candidates, our competitive position would be adversely affected. We may not identify relevant third- party patents or may incorrectly interpret the relevance, scope or expiration of a third- party patent, which might adversely affect our ability to develop and market our products. We cannot guarantee that any of our patent searches or analyses, including the identification of relevant patents, the scope of patent claims or the expiration of relevant patents, are complete or thorough, nor can we be certain that we have identified each and every third- party patent and pending application in the United States and abroad that is relevant to or necessary for the commercialization of our product candidates in any jurisdiction. The scope of a patent claim is determined by an interpretation of the law, the written disclosure in a patent and the patent' s prosecution history. Our interpretation of the relevance or the scope of a patent or a pending application may be incorrect. For example, we may incorrectly determine that our products are not covered by a third- party patent or may incorrectly predict whether a third party' s pending application will issue with claims of relevant scope. Our determination of the expiration date of any patent in the United States or abroad that we consider relevant may be incorrect. Our failure to identify and correctly interpret relevant patents may negatively impact our ability to develop and market our products. In addition, because some patent applications in the United States may be maintained in secrecy until the patents are issued, patent applications in the United States and many foreign jurisdictions are typically not published until 18 months after filing, and publications in the scientific literature often lag behind actual discoveries, we cannot be certain that others have not filed patent applications for technology covered by our pending applications or any future issued patents, or that we were the first to invent the technology. Our competitors may have filed, and may in the future file, patent applications covering our products or technology similar to ours. Any such patent application may have priority over our patent applications or patents, which could require us to obtain rights to issued patents covering such technologies. We may become subject to claims challenging the inventorship or ownership of our patents and other intellectual property. We may be subject to claims that former employees, collaborators or other third parties have an interest in our patents or other intellectual property as an inventor or co- inventor. The failure to name the proper inventors on a patent application can result in the patents issuing thereon being unenforceable. Inventorship disputes may arise from conflicting views regarding the contributions of different individuals named as inventors, the effects of foreign laws where foreign nationals are involved in the development of the subject matter of the patent, conflicting obligations of third parties involved in developing our product candidates or as a result of questions regarding co- ownership of potential joint inventions. Litigation may be necessary to resolve these and other claims challenging inventorship and / or ownership. Alternatively, or additionally, we may enter into agreements to clarify the scope of our rights in such intellectual property. If we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights, such as exclusive ownership of, or right to use, valuable intellectual property. Such an outcome could have a material adverse effect on our business. Even if we are successful in defending against such claims, litigation could result in substantial costs and be a distraction to management and other

employees. Our current or future licensors may have relied on third- party consultants or collaborators or on funds from third parties, such as the U. S. government or academic institutions, such that our licensors are not the sole and exclusive owners of the patents we in- licensed. If other third parties have ownership rights or other rights to our in- licensed patents, they may be able to license such patents to our competitors, and our competitors could market competing products and technology. This could have a material adverse effect on our competitive position, business, financial conditions, operations, and prospects. Patent terms may be inadequate to protect our competitive position on our product candidates for an adequate amount of time. Patents have a limited lifespan. In the United States, if all maintenance fees are timely paid, the natural expiration of a patent is generally 20 years from its earliest U. S. non- provisional filing date. Various extensions may be available, but the life of a patent, and the protection it affords, is limited. Even if patents covering our product candidates are obtained, once the patent life has expired, we may be open to competition from competitive products, including generics or biosimilars. Given the amount of time required for the development, testing and regulatory review of new product candidates, patents protecting such product candidates might expire before or shortly after such product candidates are commercialized. As a result, our owned and future licensed patent portfolio may not provide us with sufficient rights to exclude others from commercializing products similar or identical to ours. Some intellectual property that we have in- licensed may have been discovered through government funded programs and thus may be subject to federal regulations such as " march- in " rights, certain reporting requirements and a preference for U. S.- based companies. Compliance with such regulations may limit our exclusive rights, and limit our ability to contract with non- U. S. manufacturers. Certain of the intellectual property rights we have licensed are generated through the use of U. S. government funding and are therefore subject to certain federal regulations. As a result, the U. S. government may have certain rights to intellectual property embodied in our current or future product candidates pursuant to the Bayh- Dole Act of 1980 (the " Bayh- Dole Act ") and implementing regulations. These U. S. government rights in certain inventions developed under a government- funded program include a non- exclusive, non- transferable, irrevocable worldwide license to use inventions for any governmental purpose. In addition, the U. S. government has the right to require our or our licensors' to grant exclusive, partially exclusive, or non- exclusive licenses to any of these inventions to a third party if it determines that: (i) adequate steps have not been taken to commercialize the invention; (ii) government action is necessary to meet public health or safety needs; or (iii) government action is necessary to meet requirements for public use under federal regulations (also referred to as " march- in rights "). The U. S. government also has the right to take title to these inventions if we fail, or the applicable licensor, fails to disclose the invention to the government and fails to file an application to register the intellectual property within specified time limits. These time limits have recently been changed by regulation, and may change in the future. Intellectual property generated under a government funded program is also subject to certain reporting requirements, compliance with which may require us or the applicable licensor to expend substantial resources. In addition, the U. S. government requires that any products embodying the subject invention or produced through the use of the subject invention be manufactured substantially in the United States. The manufacturing preference requirement can be waived if the owner of the intellectual property can show that reasonable but unsuccessful efforts have been made to grant licenses on similar terms to potential licensees that would be likely to manufacture substantially in the United States or that under the circumstances domestic manufacture is not commercially feasible. This preference for U. S. manufacturers may limit our ability to contract with non- U. S. product manufacturers for products covered by such intellectual property. To the extent any of our current or future intellectual property is generated through the use of U. S. government funding, the provisions of the Bayh- Dole Act may similarly apply. Risks Related to Government Regulation The regulatory approval processes of the FDA and other comparable foreign regulatory authorities are lengthy, time- consuming and inherently unpredictable. If we are not able to obtain, or if there are delays in obtaining, required regulatory approvals for our product candidates, **or if we determine that we are not willing or able to complete the regulatory approval process given the resources required to do so,** we will not be able to commercialize, or will be delayed in commercializing, such product candidates, and our ability to generate revenue will be materially impaired. The process of obtaining regulatory approvals, both in the United States and abroad, is unpredictable, expensive and typically takes many years following commencement of clinical trials, if approval is obtained at all, and can vary substantially based upon a variety of factors, including the type, complexity and novelty of the product candidates involved **. While our product candidate NGN- 401 for Rett syndrome has been accepted into the FDA' s START program and the RMAT program, which we expect together will allow us to have access to more frequent advice from FDA staff, intensive guidance on efficient drug development and eligibility for an Accelerated Approval pathway and Priority Review, participation in these programs is not a guarantee that our approval process with the FDA will be faster or that we will ultimately achieve approval of a pivotal trial design or approval of NGN- 401 as an accepted therapy for Rett syndrome** . We cannot commercialize product candidates in the United States without first obtaining regulatory approval from the FDA **. In addition, we may determine that the resources required to complete the regulatory approval process are in excess of what we are able or willing to expend on a particular program. For example, in November 2024, we announced that we do not expect to move forward with the NGN- 101 gene therapy program for for CLN5 Batten disease. Given the rarity of the disease, continued investment in the program was predicated on an alignment on a streamlined registrational pathway with the FDA. To support a streamlined pathway, we submitted and RMAT application to the FDA. Despite our belief that we met the standard of preliminary clinical evidence required to obtain an RMAT designation, the RMAT application was denied. We are currently evaluation options for the program** . Similarly, we cannot commercialize product candidates outside of the United States without obtaining regulatory approval from comparable foreign regulatory authorities. Before obtaining regulatory approvals for the commercial sale of our product candidates, including our most advanced product ~~candidates~~ **candidate** , NGN- 401 ~~and NGN- 101~~ , we must demonstrate through lengthy, complex and expensive preclinical and clinical trials that such product candidates are safe, pure and effective or potent for each targeted indication. Securing regulatory approval also requires the submission of information about the biological

product manufacturing process to, and inspection of manufacturing facilities by, the relevant regulatory authority. Further, a product candidate may not be effective or potent, may be only moderately effective or potent or may prove to have undesirable or unintended side effects, toxicities or other characteristics that may preclude its obtaining marketing approval. The FDA and comparable foreign regulatory authorities have substantial discretion in the approval process and may refuse to accept any application or may decide that our data are insufficient for approval and require additional preclinical, clinical or other data. A product candidate could be delayed in receiving, or fail to receive, regulatory approval for many reasons, including: the FDA or comparable foreign regulatory authorities may disagree with the design or implementation of our clinical trials; we may be unable to demonstrate to the satisfaction of the FDA or comparable foreign regulatory authorities that a product candidate is safe, pure, and effective or potent for its proposed indication; the results of clinical trials may not meet the level of statistical significance required by the FDA or comparable foreign regulatory authorities for approval; serious and unexpected product-related side effects may be experienced by participants in our clinical trials or by individuals using drugs or biological products similar to a product candidate; we may be unable to demonstrate that a candidate' s clinical and other benefits outweigh its safety risks; the FDA or comparable foreign regulatory authorities may disagree with our interpretation of data from preclinical studies or clinical trials; the data collected from clinical trials of a product candidate may not be acceptable or sufficient to support the submission of a BLA or other submission or to obtain regulatory approval in the United States or elsewhere, and we may be required to conduct additional clinical trials; the FDA or the applicable foreign regulatory authority may disagree regarding the formulation, labeling and / or the specifications of a product candidate; the FDA or comparable foreign regulatory authorities may fail to approve the manufacturing processes or facilities of third- party manufacturers with which we contract for clinical and commercial supplies; and the approval policies or regulations of the FDA or comparable foreign regulatory authorities may significantly change in a manner rendering our clinical data insufficient for approval. Of the large number of products in development, only a small percentage successfully complete the FDA or foreign regulatory approval processes and are commercialized. The lengthy approval process as well as the unpredictability of future clinical trial results may result in us failing to obtain regulatory approval to market NGN- 401 or ~~NGN- 101 or~~ other product candidates, which would significantly harm our business, results of operations and prospects. If we were to obtain approval, regulatory authorities may approve any such product candidate for fewer or more limited indications than we request, including failing to approve the most commercially promising indications, may grant approval contingent on the performance of costly post- marketing clinical trials, or may approve a product candidate with a label that does not include the labeling claims necessary or desirable for the successful commercialization of that product candidate. If we are not able to obtain, or if there are delays in obtaining, required regulatory approvals for a product candidate, we will not be able to commercialize, or will be delayed in commercializing, such product candidate and our ability to generate revenue may be materially impaired **. In addition, the FDA and foreign regulatory authorities may change their policies, issue additional regulations or revise existing regulations, or take other actions, such as those implemented by the recently established Department of Government Efficiency (" DOGE "), which may prevent or delay approval of our product candidates under development on a timely basis. Such policy or regulatory changes could impose additional requirements upon us that could delay our ability to obtain approvals and increase the costs of compliance. Additionally, changes in the leadership of the FDA and other federal agencies under President Trump' s administration (the " Trump Administration ") may also lead to new policies and changes in the regulations and operations of the FDA, which may impact our clinical development plans** . Because gene therapy is novel and the regulatory landscape that governs any product candidates we may develop is rigorous, complex, uncertain and subject to change, we cannot predict the time and cost of obtaining regulatory approval, if received at all, for any product candidates we may develop. The regulatory requirements that will govern any novel gene therapy product candidates we develop are not entirely clear and are subject to change. Within the broader genetic medicine field, very few therapeutic products have received marketing authorization from the FDA or the EMA. Even with respect to more established products that fit into the categories of gene therapies or cell therapies, the regulatory landscape is still developing. Regulatory requirements governing gene therapy products and cell therapy products have changed frequently and will likely continue to change in the future. Moreover, there is substantial overlap in those responsible for review and regulation of existing gene therapy products and cell therapy products. For example, in the United States, the FDA has established the Office of Therapeutic Products within its Center for Biologics Evaluation and Research (" CBER "), as part of its reorganization of the Office of Tissues and Advanced Therapies, to consolidate the review of gene therapy and related products. In addition, the Cellular, Tissue and Gene Therapies Advisory Committee advises CBER on its review. Our product candidates will need to meet safety, purity and efficacy or potency standards applicable to any new biologic under the regulatory framework administered by the FDA. In addition to FDA oversight and oversight by IRBs under guidelines promulgated by the National Institutes of Health (" NIH ") gene therapy clinical trials are also subject to review and oversight by an institutional biosafety committee (" IBC "), a local institutional committee that reviews and oversees research utilizing recombinant or synthetic nucleic acid molecules at that institution. The IBC assesses the safety of the research and identifies any potential risk to public health or the environment. While the NIH guidelines are not mandatory unless the research in question is being conducted at or sponsored by institutions receiving NIH funding of recombinant or synthetic nucleic acid molecule research, many companies and other institutions not otherwise subject to the NIH guidelines voluntarily follow them. Although the FDA decides whether individual gene therapy protocols may proceed, the review process and determinations of other reviewing bodies can impede or delay the initiation of a clinical trial, even if the FDA has reviewed the trial and approved its initiation. The same applies in the **EU** ~~European Union~~ . The EMA' s Committee for Advanced Therapies (" CAT ") is responsible for assessing the quality, safety, and efficacy of advanced-therapy medicinal products. Advanced- therapy medicinal products include gene therapy medicines, somatic- cell therapy medicines and tissue- engineered medicines. The role of the CAT is to prepare a draft opinion on an application for marketing authorization for a gene therapy medicinal candidate that is submitted to the EMA. In the **EU** ~~European Union~~ , the

development and evaluation of a gene therapy product must be considered in the context of the relevant EU guidelines. The EMA may issue new guidelines concerning the development and marketing authorization for gene therapy products and require that we comply with these new guidelines. As a result, the procedures and standards applied to gene therapy products and cell therapy products may be applied to any gene therapy product candidate we may develop, but that remains uncertain at this point. Adverse developments in preclinical studies or clinical trials conducted by others in the field of gene therapy and gene regulation products may cause the FDA, the EMA, and other regulatory authorities to revise the requirements for approval of any product candidates we may develop or limit the use of products utilizing gene regulation technologies, either of which could harm our business. In addition, the clinical trial requirements of the FDA, the EMA, and other regulatory authorities and the criteria these regulators use to determine the safety, purity and efficacy or potency of a product candidate vary substantially according to the type, complexity, novelty, and intended use and market of the potential products. ~~The~~ **Because of this complexity, even though our product candidate NGN- 401 for Rett syndrome has been accepted into the FDA' s START program and the RMAT program, which we expect together will allow us to have access to more frequent advice from FDA staff, intensive guidance on efficient drug development and eligibility for an Accelerated Approval pathway and Priority Review, the** regulatory approval process for product candidates such as those being developed by us can be more expensive and take longer than for other, better known, or more extensively studied pharmaceutical or other product candidates. Further, as we are developing novel potential treatments for diseases in which, in some cases, there is little clinical experience with potential new endpoints and methodologies, heightened risk that the FDA, the EMA or other regulatory authorities may not consider the clinical trial endpoints to provide clinically meaningful results, and the resulting clinical data and results may be more difficult to analyze. In addition, we may not be able to identify or develop appropriate animal disease models to enable or support planned clinical development. Any natural history studies that we may conduct or rely upon in our clinical development may not be accepted by the FDA, EMA or other regulatory authorities. Regulatory agencies administering existing or future regulations or legislation may not allow production and marketing of products utilizing gene regulation technology in a timely manner or under technically or commercially feasible conditions. In addition, regulatory action or private litigation could result in expenses, delays, or other impediments to our research programs or the commercialization of resulting products. Further, approvals by one regulatory agency may not be indicative of what other regulatory agencies may require for approval. The regulatory review committees and advisory groups described above and the new guidelines they promulgate may lengthen the regulatory review process, require us to perform additional preclinical studies or clinical trials, increase our development costs, lead to changes in regulatory positions and interpretations, delay or prevent approval and commercialization of these treatment candidates, or lead to significant post- approval limitations or restrictions. As we advance our research programs and develop future product candidates, we will be required to consult with these regulatory and advisory groups and to comply with applicable guidelines. If we fail to do so, we may be required to delay or discontinue development of any product candidates we identify and develop. These additional processes may result in a review and approval process that is longer than we otherwise would have expected. Delays as a result of an increased or lengthier regulatory approval process or further restrictions on the development of our product candidates can be costly and could negatively impact our ability to complete clinical trials and commercialize our current and future product candidates in a timely manner, if at all. Disruptions at the FDA and other regulatory authorities could negatively affect the review of our regulatory submissions, which could negatively impact our business. The ability of the FDA and other regulatory authorities to review and approve regulatory submissions can be affected by a variety of factors, including ~~understaffing~~ **statutory, regulatory and policy changes, inadequate government budget funding levels or a reduction in the FDA' s workforce and its ability to hire and retain key personnel** , disruptions caused by government shutdowns and public health crises. **There have been mass layoffs of federal employees since the start of the Trump Administration in January 2025, the impact of which is unclear at this time.** Such disruptions could significantly impact the ability of the FDA or other regulatory authorities to timely review and process our regulatory submissions **, which could have a material adverse effect on our business. In addition, the Trump Administration has led and is expected to continue to lead to changes in the leadership of various U. S. federal regulatory agencies and changes to U. S. federal government policy that have led to, in some cases, legal challenges and uncertainty around the funding, functioning and policy priorities of the U. S. federal regulatory agencies. We are unable to predict the extent to which the current U. S. federal administration may impose or seek to impose leadership or policy changes at the U. S. federal regulatory agencies responsible for regulating our business or changes to rules and policies impacting our operations. It is unclear how these executive actions or other potential actions by the Trump Administration or other parts of the federal government will impact the FDA or other regulatory authorities that oversee our business. Government proposals to reduce or eliminate budgetary deficits may include reduced allocations to the FDA and other related government agencies. These budgetary pressures may reduce the FDA' s ability to perform its responsibilities. If a significant reduction in the FDA' s workforce occurs, the FDA' s budget is significantly reduced or a prolonged government shutdown occurs, it could significantly impact the ability of the FDA to timely review and process our regulatory submissions or take other actions critical to the development or marketing of our most advanced product candidate, NGN- 401, or other product candidates, if approved** , which could have a material adverse effect on our business. We may not be able to meet requirements for the chemistry, manufacturing and control of our product candidates. In order to receive approval of our products by the FDA and comparable foreign regulatory authorities, we must show that we and our contract manufacturing partners are able to characterize, control and manufacture our biological products safely and in accordance with regulatory requirements. This includes manufacturing the drug substance, developing an acceptable formulation, performing tests to adequately characterize the formulated product, documenting a repeatable manufacturing process, and demonstrating that our biological products meet stability requirements. Meeting these chemistry, manufacturing and control (" CMC ") requirements is a complex task that requires specialized expertise. If we are not able to meet the CMC requirements, we may not be successful in getting our

products approved. We intend to deliver our product candidates via a drug delivery device that will have its own regulatory, development, supply and other risks. We intend to deliver our product candidates via a drug delivery device, such as a catheter or other delivery system. There may be unforeseen technical complications related to the development activities required to bring such a product to market, including primary container compatibility and / or dose volume requirements **. We expect to use drug delivery devices authorized for marketing under clearances or approvals held by third parties** . Our product candidates may not be approved or may be substantially delayed in receiving approval if the devices do not gain and / or maintain their own regulatory approvals or clearances. Where approval of the drug product and device is sought under a single application, the increased complexity of the review process may delay approval. In addition, some drug delivery devices are provided by single- source unaffiliated third- party companies. We may be dependent on the sustained cooperation and effort of those third- party companies both to supply the devices and, in some cases, to conduct the studies required for approval or other regulatory clearance of the devices. Even if approval is obtained, we may also be dependent on those third- party companies continuing to maintain such approvals or clearances once they have been received. Failure of third- party companies to supply the devices, to successfully complete studies on the devices in a timely manner, or to obtain or maintain required approvals or clearances of the devices could result in increased development costs, delays in or failure to obtain regulatory approval and delays in product candidates reaching the market or in gaining approval or clearance for expanded labels for new indications. We currently and may in the future conduct clinical trials for our product candidates at sites outside the United States, and the FDA may not accept data from trials conducted in such locations. We plan to conduct clinical trials outside the United States, including in Australia, the UK, Europe or other foreign jurisdictions. For example, we **are** currently ~~intend to conduct~~ **conducting** our Phase 1 / 2 clinical ~~trial~~ **trials** for NGN- 401 in the United States **,** ~~and outside the United States. Our Phase 1 / 2 clinical trial for NGN- 101 is currently being conducted in the United States and in~~ the UK **and Australia** . In cases where data from clinical trials conducted outside the United States are intended to serve as the sole basis for marketing approval in the United States, the FDA will generally not approve the application on the basis of foreign data alone unless (i) the data are applicable to the United States population and United States medical practice; (ii) the trials were performed by clinical investigators of recognized competence and (iii) the data may be considered valid without the need for an on- site inspection by the FDA or, if the FDA considers such an inspection to be necessary, the FDA is able to validate the data through an on- site inspection or other appropriate means. Additionally, the FDA' s clinical trial requirements, including sufficient size of patient populations and statistical powering, must be met. Many foreign regulatory authorities have similar approval requirements. In addition, such foreign trials would be subject to the applicable local laws of the foreign jurisdictions where the trials are conducted. There can be no assurance that the FDA or any similar foreign regulatory authority will accept data from trials conducted outside of the United States or the applicable jurisdiction. If the FDA or any similar foreign regulatory authority does not accept such data, it would result in the need for additional trials, which would be costly and time- consuming and delay aspects of our business plan, and which may result in our product candidates not receiving approval or clearance for commercialization in the applicable jurisdiction. Even if the FDA accepts such data, it could require us to modify our planned clinical trials to receive clearance to initiate such trials in the United States or to continue such trials once initiated. Other risks inherent in conducting international clinical trials include: foreign regulatory requirements, differences in healthcare services, and differences in cultural customs that could restrict or limit our ability to conduct our clinical trials; administrative burdens of conducting clinical trials under multiple sets of foreign regulations; foreign exchange fluctuations; diminished protection of intellectual property in some countries; and political and economic risks relevant to foreign countries. Our product candidates for which we intend to seek approval as biologics may face competition sooner than anticipated. The Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act, includes a subtitle called the Biologics Price Competition and Innovation Act of 2009 (" BPCIA "), which created an abbreviated approval pathway for biological products that are biosimilar to or interchangeable with an FDA- licensed reference biological product. Under the BPCIA, an application for a highly similar or " biosimilar " product may not be submitted to the FDA until four years following the date that the reference product was first approved by the FDA. In addition, the approval of a biosimilar product may not be made effective by the FDA until 12 years from the date on which the reference product was first approved. During this 12- year period of exclusivity, another company may still market a competing version of the reference product if the FDA approves a full BLA for the competing product containing the sponsor' s own preclinical data and data from adequate and well- controlled clinical trials to demonstrate the safety, purity and potency of its product. Our investigational biological products, if approved, could be considered reference products entitled to 12- year exclusivity. However, there is a risk that this exclusivity could be shortened due to congressional action or otherwise, or that the FDA will not consider a product candidate to be reference products for competing products, potentially creating the opportunity for competition sooner than anticipated. Other aspects of the BPCIA, some of which may impact the BPCIA exclusivity provisions, have also been the subject of recent litigation. Moreover, the extent to which a biosimilar, once approved, will be substituted for any reference products in a way that is similar to traditional generic substitution for non- biological products is not yet clear, and will depend on a number of marketplace and regulatory factors that are still developing. Even if we receive regulatory approval of NGN- 401 or ~~NGN- 101 or~~ other product candidates, we will be subject to extensive ongoing regulatory obligations and continued regulatory review, which may result in significant additional expense and we may be subject to penalties if we fail to comply with regulatory requirements or experience unanticipated problems with our product candidates. Any regulatory approvals that we may receive for NGN- 401 ~~or NGN- 101~~ or other product candidates will require the submission of reports to regulatory authorities and surveillance to monitor the safety, purity and efficacy or potency of such product candidates, may contain significant limitations related to use restrictions for specified age groups, warnings, precautions or contraindications, and may include burdensome post- approval study or risk management requirements. For example, the FDA may require a risk evaluation and mitigation strategy in order to approve a product candidate, which could entail requirements for a medication guide, physician training and communication

plans or additional elements to ensure safe use, such as restricted distribution methods, patient registries and other risk minimization tools. In addition, if the FDA or comparable foreign regulatory authorities approve a product candidate, the products and the activities associated with their development and commercialization, including their design, testing, manufacture, safety, purity, efficacy or potency, recordkeeping, labeling, storage, approval, advertising, promotion, sale, distribution, import and export will be subject to comprehensive regulation by the FDA and other regulatory agencies in the United States and by comparable foreign regulatory authorities. These requirements include submissions of safety and other post- marketing information and reports, registration, as well as on- going compliance with current cGMPs and GCPs for any clinical trials that we conduct following approval. In addition, manufacturers of drug products and their facilities are subject to continual review and periodic, unannounced inspections by the FDA and other regulatory authorities for compliance with cGMPs. If we or a regulatory authority discovers previously unknown problems with a product, such as adverse events of unanticipated severity or frequency, or problems with the facilities where the product is manufactured, a regulatory authority may impose restrictions on that product, the manufacturing facility or us, including requiring recall or withdrawal of the product from the market or suspension of manufacturing, requiring the addition of labeling statements, such as a " black box " warning or a contraindication, requiring creation of a medication guide outlining the risk of such side effects for distribution to patients, withdrawal or suspension of existing approvals or licenses, refusal to approve pending applications or supplements, restrictions on our ability to conduct clinical trials, including full or partial clinical holds on ongoing or planned trials, restrictions on the manufacturing process, warning or untitled letters, civil and criminal penalties, injunctions, product seizures, detentions or import bans, voluntary or mandatory publicity requirements and imposition of restrictions on operations, including costly new manufacturing requirements. The occurrence of any event or penalty described above may inhibit our ability to commercialize NGN- 401 or ~~NGN- 101 or~~ other product candidates and generate revenue and could require us to expend significant time and resources in response and could generate negative publicity. We may face difficulties from healthcare legislative reform measures. Existing regulatory policies may change, and additional government regulations may be enacted that could prevent, limit or delay regulatory approval of NGN- 401 or ~~NGN- 101 or~~ other product candidates. We cannot predict the likelihood, nature or extent of government regulation that may arise from future legislation or administrative action, either in the United States or abroad. If we are slow or unable to adapt to changes in existing requirements or the adoption of new requirements or policies, or if we are not able to maintain regulatory compliance, we may lose any marketing approval that we may have obtained and we may not achieve or sustain profitability. See ~~the section entitled~~ " Business — Government Regulation — Healthcare Reform " **in our Annual Report on Form 10- K** for a more detailed description of healthcare reforms measures that may prevent us from being able to generate revenue, attain profitability, or commercialize product candidates. Our business operations and current and future arrangements with investigators, healthcare professionals, consultants, third- party payors, patient organizations and customers will be subject to applicable healthcare regulatory laws, which could expose us to penalties. Our business operations and current and future arrangements with investigators, healthcare professionals, consultants, third- party payors, patient organizations and customers may expose us to broadly- applicable fraud and abuse and other healthcare laws and regulations. These laws may constrain the business or financial arrangements and relationships through which we conduct our operations, including how we research, market, sell and distribute our product candidates, if approved. See ~~the section entitled~~ " Business — Government Regulation — Other Healthcare Laws and Compliance Requirements " **in our Annual Report on Form 10- K** for a more detailed description of the laws that may affect our ability to operate. Ensuring that our internal operations and future business arrangements with third parties comply with applicable healthcare laws and regulations will involve substantial costs. If our operations are found to be in violation of any of these laws or any other governmental laws and regulations that may apply to us, we may be subject to significant penalties, including civil, criminal and administrative penalties, damages, fines, exclusion from government- funded healthcare programs, integrity oversight and reporting obligations to resolve allegations of non- compliance, disgorgement, individual imprisonment, contractual damages, reputational harm, diminished profits and the curtailment or restructuring of our operations. Further, defending against any such actions can be costly and time- consuming and may require significant personnel resources. Therefore, even if we are successful in defending against any such actions that may be brought against us, our business may be impaired. Even if we are able to commercialize NGN- 401 or ~~NGN- 101 or~~ other product candidates, due to unfavorable pricing regulations and / or third- party coverage and reimbursement policies, we may not be able to offer such products at competitive prices which would seriously harm our business. We intend to seek approval to market NGN- 401 and ~~NGN- 101 and~~ other product candidates in both the United States and in selected foreign jurisdictions. If we obtain approval in one or more foreign jurisdictions for such product candidates, we will be subject to rules and regulations in those jurisdictions. Our ability to successfully commercialize any product candidates that we may develop will depend in part on the extent to which reimbursement for these products and related treatments will be available from government health administration authorities, private health insurers and other organizations. Government authorities and other third- party payors, such as private health insurers and health maintenance organizations, decide which medications they will pay for and establish reimbursement levels. Government authorities and other third- party payors have attempted to control costs by limiting coverage and the amount of reimbursement for particular medications. These entities may create preferential access policies for a competitor' s product, including a branded or generic / biosimilar product, over our products in an attempt to reduce their costs, which may reduce our commercial opportunity. Additionally, if any of our product candidates are approved and we are found to have improperly promoted off- label uses of those programs, we may become subject to significant liability, which would materially adversely affect our business and financial condition. See ~~the sections entitled~~ " Business — Government Regulation — Coverage and Reimbursement " and " — Regulation in the European Union " **in our Annual Report on Form 10- K** for a more detailed description of the government regulations and third- party payor practices that may affect our ability to commercialize product candidates. We are subject to U. S. and certain foreign export and import controls, sanctions, embargoes, anti- corruption laws, and anti- money laundering laws and regulations. We

can face criminal liability and other serious consequences for violations, which can harm our business. We are subject to export control and import laws and regulations, including the U. S. Export Administration Regulations, U. S. Customs regulations, various economic and trade sanctions regulations administered by the U. S. Treasury Department' s Office of Foreign Assets Controls, the U. S. Foreign Corrupt Practices Act of 1977, as amended, the U. S. domestic bribery statute contained in 18 U. S. C. § 201, the U. S. Travel Act, the USA PATRIOT Act, and other state and national anti- bribery and anti- money laundering laws in the countries in which we conduct activities **. In addition, the U. S. Congress is currently contemplating legislation that could have the impact of limiting the ability of us and certain of our vendors to work with certain designated biotech companies from China and other nations** . Anti- corruption laws are interpreted broadly and prohibit companies and their employees, agents, contractors, and other collaborators from authorizing, promising, offering, or providing, directly or indirectly, improper payments or anything else of value to or from recipients in the public or private sector. We may engage third parties to sell products outside the United States, to conduct clinical trials, and / or to obtain necessary permits, licenses, patent registrations, and other regulatory approvals. We have direct or indirect interactions with officials and employees of government agencies or government- affiliated hospitals, universities, and other organizations. We can be held liable for the corrupt or other illegal activities of our employees, agents, contractors, and other collaborators, even if we do not explicitly authorize or have actual knowledge of such activities. Any violations of the laws and regulations described above may result in substantial civil and criminal fines and penalties, imprisonment, the loss of export or import privileges, debarment, tax reassessments, breach of contract and fraud litigation, reputational harm, and other consequences. Governments outside the United States may impose strict price controls, which may adversely affect our revenue, if any. In some countries, particularly member states of the **EU** ~~European Union~~ , the pricing of prescription drugs is subject to governmental control. In these countries, pricing negotiations with governmental authorities can take considerable time after receipt of marketing approval for a therapeutic. In addition, there can be considerable pressure by governments and other stakeholders on prices and reimbursement levels, including as part of cost containment measures. Political, economic and regulatory developments may further complicate pricing negotiations, and pricing negotiations may continue after reimbursement has been obtained. Reference pricing used by various EU member states and parallel distribution, or arbitrage between low- priced and high- priced member states, can further reduce prices. To obtain coverage and reimbursement or pricing approvals in some countries, we or current or future collaborators of ours may be required to conduct a clinical trial or other studies that compare the cost- effectiveness of a product to other available therapies in order to obtain or maintain reimbursement or pricing approval. Publication of discounts by third- party payors or authorities may lead to further pressure on the prices or reimbursement levels within the country of publication and other countries. If reimbursement of any product approved for marketing is unavailable or limited in scope or amount, or if pricing is set at unsatisfactory levels, our business, financial condition, results of operations or prospects could be materially and adversely affected. Brexit could lead to legal uncertainty and potentially divergent national laws and regulations, including those related to the pricing of prescription pharmaceuticals, as the UK determines which EU laws to replicate or replace. If the UK were to significantly alter its regulations affecting the pricing of prescription pharmaceuticals, we could face significant new costs. While we have received Fast Track designation for NGN- 401 for the treatment of Rett syndrome and ~~for NGN- 101 for the treatment of CLN5 Batten disease and we may seek certain designations for our other product candidates, including Breakthrough Therapy and Priority Review designations in the United States, we may not receive such designations, and even if we do, such designations may not lead to a faster development or regulatory review or approval process. We have received Fast Track designation in the United States for~~ NGN- 401 **has been accepted into the FDA' s START Pilot Program and RMAT program and has received PRIME designation from the EMA, such designations may not lead to a faster development or regulatory review or approval process. In 2024, the FDA began accepting applications from sponsors** for the **START pilot program with the purpose of further accelerating the pace of development of novel drug and biological products that are intended to address an unmet medical need as a** treatment ~~of Rett syndrome and~~ for ~~NGN~~ **rare disease. The pilot is designed to be milestone** ~~- 101~~ **driven (i. e. to facilitate the progression of a development program to pivotal clinical study stage** ~~for - or the treatment of CLN5 Batten disease, and we may seek additional designations for one or more of our other --~~ **the pre- BLA meeting stage) where** product ~~candidates that~~ **development programs selected** ~~could~~ **would** ~~expedite review and approval by~~ **benefit from enhanced communication with** the FDA. **The START pilot program** ~~A Breakthrough Therapy product is defined as a product that~~ is intended ~~, alone~~ **to provide a mechanism** ~~or~~ **for addressing** ~~in combination with one or more other products, to treat a serious or life threatening disease or condition, and preliminary clinical evidence indicates that the product may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in~~ clinical development **issues** ~~. For products~~ that ~~have been designated~~ **otherwise would delay or prevent a promising novel drug or biological product from progressing to the pivotal clinical trial stage or pre- BLA meeting stage. Participants in the START Pilot Program will receive enhanced communications with the FDA review staff. These enhanced communications will include at a minimum an initial meeting to review features of the pilot, discuss a pathway intended to support a marketing application, and to discuss specific issues for which a sponsor requests enhanced communications with the FDA. Additional communications will include ongoing interactions via email or teleconference that take place on a scheduled and / or** as **needed basis as agreed upon by** ~~Breakthrough Therapies, interaction and communication between the FDA and~~ the sponsor **information on how best** ~~of the trial can help~~ to **facilitate more** ~~identify the most~~ efficient ~~path for clinical~~ development ~~while minimizing the number~~ of **potentially** ~~patients placed in ineffective control regimens. The FDA may also designate a product for Fast Track review if it is intended, whether alone or in combination with one or more other products, for the treatment of a serious or~~ life- **saving therapies** ~~threatening disease or condition, and it demonstrates the potential to address unmet medical needs~~ for **rare** ~~such a disease~~ **diseases and help** ~~or condition. For Fast Track products,~~ sponsors **generate high- quality** ~~may have greater interactions with the FDA and the FDA may initiate review of sections of a Fast Track product' s application before the application is~~

~~complete. This rolling review may be available if the FDA determines~~ , **actionable** ~~after preliminary evaluation of clinical~~ data ~~submitted by the sponsor~~ **to support future new drug or biologics license applications. In June 2024** , **we announced** that ~~a Fast Track product may be effective. We may also seek a priority review designation for one or~~ **our** ~~more of our product candidates. If the FDA determines that a~~ product candidate offers a treatment for a serious condition..... the Fast Track designation we received for NGN- 401 **had** ~~. In addition, even~~ **been** ~~if one or~~ **accepted into the FDA' s START Pilot Program, which we expect will allow us to have access to** more **frequent advice from** ~~of our product candidates qualifies for these designations, the~~ FDA ~~may later decide that~~ **staff to address product- specific development issues, possibly including clinical study design, choice of control group, patient population choices and** ~~the~~ **other early development issues. The** ~~product candidates no longer meet the conditions for qualification or decide that the time period for~~ FDA **' s** ~~review or approval will not be shortened. The RMAT designation by the FDA for any of our product candidates may not lead to a faster development or regulatory review or approval process and it does not increase the likelihood that our product candidates will receive marketing approval. We may seek an RMAT designation for our product candidates if the clinical data support such a designation for one or more product candidates. The~~ RMAT designation program is intended to fulfill the requirement of the 21st Century Cures Act that the FDA facilitate an efficient development program for, and expedite review of, any product that meets the following criteria: (1) it qualifies as ~~a~~ **an** RMAT, which is defined as a cell therapy, therapeutic tissue engineering product, human cell and tissue product, or any combination product using such therapies or products, with limited exceptions; (2) it is intended to treat, modify, reverse, or cure a serious or life- threatening disease or condition; and (3) preliminary clinical evidence indicates that the product has the potential to address unmet medical needs for such a disease or condition. ~~Like breakthrough therapy~~ **In 2024, the FDA granted an RMAT** designation ~~,~~ **for NGN- 401 for the treatment of Rett syndrome.** RMAT designation provides potential benefits that include more frequent meetings with FDA to discuss the development plan for the product candidate, and eligibility for rolling review and priority review. Products granted RMAT designation may also be eligible for accelerated approval on the basis of a surrogate or intermediate endpoint reasonably likely to predict long- term clinical benefit, or may be able to rely upon data obtained from a meaningful number of sites, including through expansion to additional sites. RMAT designation does not change the standards for product approval, and there is no assurance that such designation will result in expedited review or approval or that the approved indication will not be narrower than the indication covered by the RMAT designation. Additionally, RMAT designation can be revoked if the criteria for eligibility cease to be met as clinical data emerges. ~~If the FDA determines that a product candidate~~ offers a treatment for a serious condition,and if approved,would provide a significant improvement in safety or effectiveness where no adequate therapy exists,the FDA may designate the product candidate for priority review.A priority review designation means that the goal for the FDA to review an application is six months,rather than the standard review period of ten months. ~~As with the Fast Track designations and selection for participation in the START Pilot Program,these~~ **These** designations are within the discretion of the FDA. **Accordingly,** ~~Even~~ **even** if we believe that one ~~or more~~ of our product candidates meets the criteria for these designations,the FDA may ~~not agree~~ **disagree** and instead determine **not** to ~~not~~ make such **designation.Further,even if we receive** a designation ~~.Even if one or more of our product candidates qualifies for either or both of these designations~~ ,the **receipt of** ~~FDA may later decide that~~ such **designation for a** product candidate **may** ~~no~~ **not** ~~longer meets~~ **result in a faster development or regulatory review or approval process compared to products considered for approval under conventional FDA procedures and does not assure ultimate approval by** the ~~conditions~~ **FDA,including the Fast Track designation we received** for We have received orphan drug designation for NGN- 401 for the treatment of Rett syndrome and ~~for NGN- 101 for the treatment of CLN5 Batten disease, and~~ we may seek orphan drug designation for certain future product candidates, but we may be unable to obtain such designations or to maintain the benefits associated with orphan drug designation, including market exclusivity, which may cause our revenue, if any, to be reduced. We have received orphan drug designation from the FDA **and orphan drug designation and advanced therapy medicinal product designation from the European Medicines Agency (EMA)** for NGN- 401 for the treatment of Rett syndrome ~~and have also received orphan drug designation from the FDA and European Medicines Agency for NGN- 101 for the treatment of CLN5 Batten disease~~ . Although we may seek orphan product designation for some or all of our other product candidates, we may never receive such designations. Under the Orphan Drug Act, the FDA may designate a drug or biological product as an orphan drug if it is intended to treat a rare disease or condition, defined as a patient population of fewer than 200, 000 in the United States, or a patient population greater than 200, 000 in the United States where there is no reasonable expectation that the cost of developing the drug will be recovered from sales in the United States. Orphan drug designation must be requested before submitting a BLA. In the **EU** ~~European Union~~ , the EMA' s Committee for Orphan Medicinal Products grants orphan drug designation to promote the development of products that are intended for the diagnosis, prevention, or treatment of a life- threatening or chronically debilitating condition affecting not more than five in 10, 000 persons in the **EU** ~~European Union~~ . Additionally, designation is granted for products intended for the diagnosis, prevention, or treatment of a life- threatening, seriously debilitating or serious and chronic condition when, without incentives, it is unlikely that sales of the drug in the **EU** ~~European Union~~ would be sufficient to justify the necessary investment in developing the drug or biological product or where there is no satisfactory method of diagnosis, prevention, or treatment, or, if such a method exists, the medicine must be of significant benefit to those affected by the condition. In the United States, orphan drug designation entitles a party to financial incentives such as opportunities for grant funding towards clinical trial costs, tax advantages, and application fee waivers. After the FDA grants orphan drug designation, the generic identity of the drug and its potential orphan use are disclosed publicly by the FDA. In addition, if a product receives the first FDA approval for the indication for which it has orphan designation, the product is entitled to orphan drug exclusivity, which means the FDA may not approve any other application to market the same drug for the same indication for a period of seven years, except in limited circumstances, such as a showing of clinical superiority over the product with orphan exclusivity or where the manufacturer is unable to assure sufficient product quantity for the orphan patient population. Exclusive marketing rights in the United States

may also be unavailable if we or our collaborators seek approval for an indication broader than the orphan designated indication and may be lost if the FDA later determines that the request for designation was materially defective. In the **EU** ~~European Union~~, orphan drug designation entitles a party to financial incentives such as reduction of fees or fee waivers and ten years of market exclusivity following drug or biological product approval. This period may be reduced to six years if the orphan drug designation criteria are no longer met, including where it is shown that the product is sufficiently profitable not to justify maintenance of market exclusivity. Even with an orphan drug designation for our current and potential future product candidates, we may not be the first to obtain marketing approval for any particular orphan indication due to the uncertainties associated with developing pharmaceutical products. Further, even if we obtain orphan drug exclusivity for an existing or future product candidate, that exclusivity may not effectively protect the product from competition because different drugs with different active moieties still can be approved for the same condition even with an orphan drug designation. Even after an orphan drug is approved, the FDA can subsequently approve the same drug with the same active moiety for the same condition if the FDA concludes that the later drug is clinically superior in that it is safer, more effective, or makes a major contribution to patient care. Orphan drug designation neither shortens the development time or regulatory review time of a drug or biologic nor gives the drug or biologic any advantage in the regulatory review or approval process. We have received Rare Pediatric Disease designation by the FDA for NGN- 401 for the treatment of Rett syndrome ~~and for NGN- 101 for the treatment of CLN5 Batten disease~~. However, Rare Pediatric Disease designation for any of our product candidates does not guarantee that the BLA for the product will qualify for a priority review voucher upon approval, and it does not lead to a faster development or regulatory review process, or increase the likelihood that our product candidates will receive marketing approval. Under the Rare Pediatric Disease Priority Review Voucher program, upon the approval of a qualifying BLA for the treatment of a rare pediatric disease, the sponsor of such an application would be eligible for a rare pediatric disease priority review voucher that can be used to obtain priority review for a subsequent BLA or NDA. If a product candidate is designated before September 30, 2024, it is eligible to receive a voucher if it is approved before September 30, 2026. While we have obtained Rare Pediatric Disease ~~designations~~ **designation** for NGN- 401 for the treatment of Rett syndrome ~~and for NGN- 101 for the treatment of CLN5 Batten disease~~, it is unlikely that ~~these~~ **this** product ~~candidates~~ **candidate** will be approved by September 30, 2026. If approval is not obtained by then, we would not be in a position to obtain a priority review voucher, unless Congress further reauthorizes the program beyond the current sunset ~~date~~ **dates** ~~in~~**, which require that a product designated as being for a rare pediatric disease be approved by** September **30,** ~~2024~~ **2026**. Additionally, designation of a biological product for a rare pediatric disease does not guarantee that a BLA will meet the eligibility criteria for a rare pediatric disease priority review voucher at the time the application is approved. Finally, a Rare Pediatric Disease designation does not lead to faster development or regulatory review of the product or increase the likelihood that it will receive marketing approval. General Risk Factors Our estimates of market opportunity and forecasts of market growth may prove to be inaccurate, and even if the markets in which we compete achieve the forecasted growth, our business may not grow at similar rates, or at all. Our market opportunity estimates and growth forecasts are subject to significant uncertainty and are based on assumptions and estimates which may not prove to be accurate. Our estimates and forecasts relating to size and expected growth of our target market may prove to be inaccurate. Even if the markets in which we compete meet our size estimates and growth forecasts, our business may not grow at similar rates, or at all. Our growth is subject to many factors, including our success in implementing our business strategy, which is subject to many risks and uncertainties. Our revenue will be dependent, in part, upon the size of the markets in the territories for which we gain regulatory approval, the accepted price for the product, the ability to obtain coverage and reimbursement and whether we own the commercial rights for that territory. If the number of our addressable patients is not as significant as we estimate, the indication approved by regulatory authorities is narrower than we expect or the treatment population is narrowed by competition, physician choice or treatment guidelines, we may not generate significant revenue from sales of such products, even if approved. We may become exposed to costly and damaging liability claims, either when testing a product candidate in the clinical or at the commercial stage, and our product liability insurance may not cover all damages from such claims. We are exposed to potential product liability and professional indemnity risks that are inherent in the research, development, manufacturing, marketing, and use of pharmaceutical products. While we currently have no products that have been approved for commercial sale, the current and future use of a product candidate in clinical trials, and the sale of any approved products in the future, may expose us to liability claims. These claims may be made by patients that use the product, healthcare providers, pharmaceutical companies, or others selling such product. Any claims against us, regardless of their merit, could be difficult and costly to defend and could materially and adversely affect the market for our products or any prospects for commercialization of our products. Although we believe we currently maintain adequate product liability insurance for NGN- 401 ~~and~~**,** NGN- 101 and other product candidates, it is possible that our liabilities could exceed our insurance coverage or that in the future we may not be able to maintain insurance coverage at a reasonable cost or obtain insurance coverage that will be adequate to satisfy any liability that may arise. If a successful product liability claim or series of claims is brought against us for uninsured liabilities or in excess of insured liabilities, our assets may not be sufficient to cover such claims and our business operations could be impaired **. Our manufacturing facility is located in Houston, Texas, making us vulnerable to risks (including weather- related risks) associated with maintaining those operations in a single geographic area. Our manufacturing facility is located in Houston, Texas, which is subject to extreme weather events such as hurricanes and other significant storms, which can cause interruption to our utilities and potentially result in damage to our facility, limit the ability of suppliers to reach us during such disruptions and adversely impact our manufacturing processes. For example, in July 2024, our facility in Houston sustained five days of power loss from the impact of Hurricane Beryl, which was a Category 1 hurricane. While we were able to maintain power to critical systems through the use of our generators, the outage caused a minor delay in our development activities and caused disruptions in our manufacturing processes, including in our clean rooms. The impact of Hurricane Beryl was not material to our operations, however,**

future weather events could cause more disruption, including the potential for a sustained loss of power that could result in costly delays to our manufacturing process or the loss of certain materials stored in our facility, which could in turn have a material adverse effect on our product development timeline and results of operations . Litigation costs and the outcome of litigation could have a material adverse effect on our business. From time to time we may be subject to litigation claims through the ordinary course of our business operations regarding, but not limited to, employment matters, security of patient and employee personal information, contractual relations with collaborators and intellectual property rights. Litigation to defend ourselves against claims by third parties, or to enforce any rights that we may have against third parties, may continue to be necessary, which could result in substantial costs and diversion of our resources, causing a material adverse effect on our business, financial condition, results of operations or cash flows. Our business could be adversely affected by economic downturns, inflation, increases in interest rates, natural disasters, public health crises such as the COVID- 19 pandemic, political crises, geopolitical events, such as conflicts between Russia and Ukraine and between Israel and the surrounding regions, or other macroeconomic conditions, which could have a material and adverse effect on our results of operations and financial condition. The global economy, including credit and financial markets, has experienced extreme volatility and disruptions, including, among other things, diminished liquidity and credit availability, declines in consumer confidence, declines in economic growth, supply chain shortages, increases in inflation rates, higher interest rates, and uncertainty about economic stability. For example, the COVID- 19 pandemic resulted in widespread unemployment, economic slowdown and extreme volatility in the capital markets. The Federal Reserve has raised interest rates multiple times in response to concerns about inflation and it may not reduce interest rates in the near term or may raise them again. Higher interest rates, coupled with reduced government spending and volatility in financial markets, may increase economic uncertainty and affect consumer spending. Similarly, the ongoing military conflict between Russia and Ukraine, as well as the conflict between Israel and the surrounding regions, and rising tensions with China have created extreme volatility in the global capital markets and may have further global economic consequences, including disruptions of the global supply chain. Any such volatility and disruptions may adversely affect our business or the third parties on whom we rely. If the equity and credit markets deteriorate, including as a result of economic or political uncertainty, political unrest or war, it may make any necessary debt or equity financing more costly, more dilutive, or more difficult to obtain in a timely manner or on favorable terms, if at all. Increased inflation rates can adversely affect us by increasing our costs, including labor and employee benefit costs. We may in the future experience disruptions as a result of such macroeconomic conditions, including delays or difficulties in initiating or expanding clinical trials and manufacturing sufficient quantities of materials. Any one or a combination of these events could have a material and adverse effect on our results of operations and financial condition. Risks Related to Owning Our Stock The market price of our common stock following the merger has been and may continue to be subject to significant fluctuations. Some of the factors that may cause the market price of our common stock to fluctuate include: • timing and results of clinical trials and preclinical studies of our product candidates, or those of our competitors or our existing or future collaborators; • failure to meet or exceed financial and development projections that we may provide to the public; • failure to meet or exceed the financial and development projections of the investment community; • failure to achieve the perceived benefits of the merger as rapidly or to the extent anticipated by financial or industry analysts; • announcements of significant acquisitions, strategic collaborations, joint ventures or capital commitments by us or our competitors; • actions taken by regulatory agencies with respect to our product candidates, clinical studies, manufacturing process or sales and marketing terms; • disputes or other developments relating to proprietary rights, including patents, litigation matters, and our ability to obtain patent protection for our technologies; • additions or departures of key personnel; • significant lawsuits, including patent or stockholder litigation; • if securities or industry analysts do not publish research or reports about our business, or if they issue adverse or misleading opinions regarding our business and stock; • changes in the market valuations of similar companies; • general market ~~or ,~~ macroeconomic **or geopolitical** conditions or market conditions in the pharmaceutical and biotechnology sectors; • sales of securities by us or our securityholders in the future; • if we fail to raise an adequate amount of capital to fund our operations or continued development of our product candidates; • trading volume of our common stock; • announcements by competitors of new commercial products, clinical progress or lack thereof, significant contracts, commercial relationships or capital commitments; • adverse publicity relating to precision medicine product candidates, including with respect to other products in such markets; • the introduction of technological innovations or new therapies that compete with our products; and • period- to- period fluctuations in our financial results. Moreover, the stock markets in general have experienced substantial volatility that has often been unrelated to the operating performance of individual companies. These broad market fluctuations may also adversely affect the trading price of our common stock. In addition, a recession, depression or other sustained adverse market event could materially and adversely affect our business and the value of our common stock. In the past, following periods of volatility in the market price of a company' s securities, stockholders have often instituted class action securities litigation against such companies. Furthermore, market volatility may lead to increased shareholder activism if we experience a market valuation that activists believe is not reflective of our intrinsic value. Activist campaigns that contest or conflict with our strategic direction or seek changes in the composition of our board of directors could have an adverse effect on our operating results, financial condition and cash flows. **We may be required to allocate resources to fulfill the requirements of the CVR Agreement entered into in connection with the Reverse Merger (as defined below) related to certain legacy lease obligations which may take away from our core programs and create a distraction for our management and employees.** On December 18, 2023, we completed our business combination with **our wholly owned subsidiary incorporated in the state of Nevada and also named Neurogene Inc. (“** Neurogene OpCo **”)** in accordance with the terms of the Agreement and Plan of Merger, dated as of July 17, 2023 **(the “ Merger Agreement ”)** , by and among the Company, Project North Merger Sub, Inc., a Delaware corporation and a wholly owned subsidiary of the Company **(“ Merger Sub ”)** , and Neurogene OpCo, pursuant to which, among other matters, Merger Sub merged with and into Neurogene OpCo, with Neurogene OpCo surviving as a wholly owned subsidiary of the Company

(the " Reverse Merger "). In connection with the Reverse Merger, we declared a dividend, to each person who, as of immediately prior to the effective time of the Reverse Merger, was a stockholder of the Company or had the right to receive our common stock pursuant to an existing pre- funded warrant, of the right to receive one non- transferable contingent value right (each, a " CVR ") for each then outstanding share of our common stock (before giving effect to a 1- for- 4 reverse stock split (the " Reverse Stock Split ") that was implemented immediately prior to the effective time), each representing the non- transferable contractual right to receive certain contingent payments from the Company upon the occurrence of certain events within agreed time periods. Holders of options to purchase our common stock outstanding immediately prior to the effective time of the merger will also ~~received~~ **receive** four CVRs for each share of our common stock that may be issued upon exercise of such option, such that they will receive the same number of CVRs as they would have received if the option had been exercised before the Reverse Stock Split, subject to certain conditions set forth in the CVR Agreement. ~~Further, pursuant~~ **Pursuant** to the terms of the CVR Agreement, the holders of our common stock prior to the effective time of the Reverse Merger, including holders of existing pre- funded warrants and holders of options to purchase our common stock outstanding immediately prior to the effective time of the merger and exercised after the effective time of the merger, rather than all of our current holders of our common stock, are the primary recipients of any net proceeds of the disposition of the legacy assets related the business of Neoleukin Therapeutics, Inc. **as it existed** prior to the effective time of the Reverse Merger, the mitigation of legacy lease obligations related the business of Neoleukin Therapeutics, Inc. **as it existed** prior to the effective time of the Reverse Merger or receipt of any sales tax refund from the State of Washington based on tax returns filed by the Company prior to the effective time of the Reverse Merger. **While we have entered into agreements for the disposition of certain legacy assets of Neoleukin, we are still pursuing a resolution of the legacy lease obligations of Neoleukin and expect that we will need to allocate resources, including payment of certain up- front costs, and time from employees and management to complete the resolution of such obligations and to administer the provisions of the CVR Agreement and distribution of any payments to holders of the CVRs.** Accordingly, we may be required to allocate a portion of our funds, time and resources to such activities and not our core programs and the foregoing could be a distraction to our management and employees. As a result, our operations and financial condition may be adversely affected. We have incurred, and will continue to incur additional costs and increased demands upon management as a result of complying with the laws and regulations affecting public companies. We have incurred and will continue to incur significant legal, accounting and other expenses as a public company that may not be reflected in our historical financial statements, which reflect ~~the~~ **our** operation ~~of Neurogene~~ as a private company. Some of these additional expenses include costs associated with public company reporting obligations under the Securities Exchange Act of 1934, as amended (the " Exchange Act "). Our management team consists of ~~the~~ **our** executive officers ~~of Neurogene~~ prior to the merger. These executive officers and other personnel will need to devote substantial time to complying with public company reporting requirements and compliance with applicable laws and regulations to ensure that we comply with all of these requirements. Any changes we make to comply with these obligations may not be sufficient to allow us to satisfy our obligations as a public company on a timely basis, or at all. These reporting requirements, rules and regulations, coupled with the increase in potential litigation exposure associated with being a public company, could also make it more difficult for us to attract and retain qualified persons to serve on the board of directors or on board committees or to serve as executive officers, or to obtain certain types of insurance, including directors' and officers' insurance, on acceptable terms. Once we are no longer a smaller reporting company or otherwise no longer qualify for applicable exemptions, we will be subject to additional laws and regulations affecting public companies that will increase our costs and the demands on management and could harm our operating results and cash flows. We are subject to the reporting requirements of the Exchange Act, which requires, among other things, that we file with the SEC annual, quarterly and current reports with respect to our business and financial condition as well as other disclosure and corporate governance requirements. We expect to still qualify as a " smaller reporting company, " as such term is defined in Rule 12b- 2 under the Exchange Act, in at least the near term, which allows us to take advantage of many exemptions from disclosure requirements, including not being required to comply with the auditor attestation requirements of Section 404 of the Sarbanes- Oxley Act and reduced disclosure obligations regarding executive compensation in our periodic reports and proxy statements. Once we are no longer a smaller reporting company or otherwise no longer qualify for this exemption, we will be required to comply with these additional legal and regulatory requirements applicable to public companies and will incur significant additional legal, accounting and other expenses to do so. If we are not able to comply with the requirements in a timely manner or at all, our financial condition or the market price of our common stock may be harmed. For example, if we or our independent auditor identifies deficiencies in our internal control over financial reporting that are deemed to be material weaknesses, we could face additional costs to remedy those deficiencies, the market price of our stock could decline or we could be subject to sanctions or investigations by the SEC or other regulatory authorities, any of which would require additional financial and management resources. If we fail to maintain proper and effective internal controls, our ability to produce accurate financial statements on a timely basis could be impaired. We are subject to the reporting requirements of the Exchange Act, the Sarbanes- Oxley Act and the rules and regulations of Nasdaq. The Sarbanes- Oxley Act requires, among other things, that we maintain effective disclosure controls and procedures and internal control over financial reporting. We must perform system and process evaluation and testing of our internal control over financial reporting to allow management to report on the effectiveness of our internal control over financial reporting in each Annual Report on Form 10- K, as required by Section 404 of the Sarbanes- Oxley Act. Prior to the merger in December 2023, ~~the~~ **our** operating and finance teams ~~of Neurogene~~ were part of a private company, and therefore were not previously required to test internal controls within a specified period. As a result, we have incurred and may continue to incur substantial professional fees and internal costs to expand our accounting and finance functions as well as to expend significant management efforts. We may discover weaknesses in our system of internal financial and accounting controls and procedures that could result in a material misstatement of our financial statements. Our internal control over financial reporting will not prevent or detect all errors and all fraud. A control

system, no matter how well designed and operated, can provide only reasonable, not absolute, assurance that the control system' s objectives will be met. Because of the inherent limitations in all control systems, no evaluation of controls can provide absolute assurance that misstatements due to error or fraud will not occur or that all control issues and instances of fraud will be detected. For example, our internal controls related to the cash disbursements process were not adequately designed to identify unauthorized payment requests, resulting in the identification of a material weakness. Specifically, at the end of August 2023, we discovered that we were subject to a business email compromise attack by a third party. This deficiency in our controls resulted in the diversion of payments to a fraudulent bank account. While management has determined in its assessment of our internal control over financial reporting as of December 31, 2023, that we have remediated this material weakness, there can be no assurance that the remediation will prevent similar attacks in the future or that we will not identify other material weaknesses in the future. If we are unable to successfully remediate a material weakness in our internal control over financial reporting, or if we identify any other material weaknesses, the accuracy and timing of our financial reporting may be adversely affected, we may be unable to maintain compliance with securities law requirements regarding timely filing of periodic reports in addition to applicable stock exchange listing requirements, investors may lose confidence in our financial reporting, and our stock price may decline as a result. We also could become subject to investigations by Nasdaq, the SEC or other regulatory authorities. If we are not able to comply with the requirements of Section 404 of the Sarbanes- Oxley Act, or if we are unable to maintain proper and effective internal controls, we may not be able to produce timely and accurate financial statements. If that were to happen, the market price of our common stock could decline and we could be subject to sanctions or investigations by Nasdaq, the SEC or other regulatory authorities. Our certificate of incorporation and bylaws, as well as provisions under Delaware law, could make an acquisition of the company more difficult and may prevent attempts by our stockholders to replace or remove management. Provisions in our certificate of incorporation and bylaws may discourage, delay or prevent a merger, acquisition or other change in control of the company that stockholders may consider favorable, including transactions in which our common stockholders might otherwise receive a premium price for their shares. These provisions could also limit the price that investors might be willing to pay in the future for shares of our common stock, thereby depressing the market price of our common stock. In addition, because our board of directors will be responsible for appointing the members of our management team, these provisions may frustrate or prevent any attempts by our stockholders to replace or remove current management by making it more difficult for stockholders to replace members of our board of directors. Among other things, these provisions: • establish a classified board of directors such that all members of the board are not elected at one time; • allow the authorized number of our directors to be changed only by resolution of our board of directors; • limit the manner in which stockholders can remove directors from the board; • establish advance notice requirements for nominations for election to the board of directors or for proposing matters that can be acted on at stockholder meetings; • require that stockholder actions must be effected at a duly called stockholder meeting and prohibit actions by our stockholders by written consent; • limit who may call a special meeting of stockholders; • authorize our board of directors to issue preferred stock without stockholder approval, which could be used to institute a " poison pill " that would work to dilute the stock ownership of a potential hostile acquirer, effectively preventing acquisitions that have not been approved by our board of directors; and • require the approval of the holders of at least 66 2 / 3 % of the votes that all stockholders would be entitled to cast to amend or repeal certain provisions of our charter or bylaws. Moreover, because we are incorporated in Delaware, we are governed by the provisions of Section 203 of the DGCL, which prohibits stockholders owning in excess of 15 % of our outstanding voting stock from merging or combining with us. Although we believe these provisions collectively will provide for an opportunity to receive higher bids by requiring potential acquirors to negotiate with our board of directors, they would apply even if the offer may be considered beneficial by some stockholders. Our governing documents provide that, unless we consent in writing to the selection of an alternative forum, certain designated courts will be the sole and exclusive forum for certain legal actions between us and our stockholders, which could limit our stockholders' ability to obtain a favorable judicial forum for disputes with us or our directors, officers, employees or agents. Our governing documents provide that, unless we consent in writing to an alternative forum, the Court of Chancery of the State of Delaware is the sole and exclusive forum for state law claims for (i) any derivative action or proceeding brought on the company' s behalf, (ii) any action asserting a claim of or based on a breach of a fiduciary duty owed by any of our current or former directors, officers, or other employees to the company or our stockholders, (iii) any action asserting a claim arising pursuant to any provision of the DGCL, the certificate of incorporation or the bylaws, (iv) any action to interpret, apply, enforce or determine the validity of the certificate of incorporation or bylaws, or (v) any action asserting a claim that is governed by the internal affairs doctrine, in each case subject to the Court of Chancery having personal jurisdiction over the indispensable parties named as defendants therein, which for purposes of this risk factor refers to herein as the " Delaware Forum Provision. " Our governing documents further provide that, unless we consent in writing to an alternative forum, the federal district courts of the United States will be the exclusive forum for resolving any complaint asserting a cause of action arising under the Securities Act, which for purposes of this risk factor refers to herein as the " Federal Forum Provision. " Neither the Delaware Forum Provision nor the Federal Forum Provision will apply to any causes of action arising under the Exchange Act. In addition, any person or entity purchasing or otherwise acquiring any interest in shares of our capital stock will be deemed to have notice of and consented to the foregoing Delaware Forum Provision and Federal Forum Provision; provided, however, that stockholders cannot and will not be deemed to have waived our compliance with the U. S. federal securities laws and the rules and regulations thereunder. The Delaware Forum Provision and the Federal Forum Provision may impose additional litigation costs on our stockholders in pursuing any such claims, particularly if such stockholders do not reside in or near the State of Delaware. Additionally, these forum selection clauses may limit our stockholders' ability to bring a claim in a judicial forum that they find favorable for disputes with us or our directors, officers or employees, which may discourage such lawsuits against us and our directors, officers and employees even though an action, if successful, might benefit our stockholders. If our existing stockholders sell, or indicate an intention to sell, substantial amounts of our common stock in the public market, the trading price

of our common stock could decline. Based on shares outstanding as of December 31, ~~2023~~ **2024** , there **are** approximately ~~16~~ **20** , ~~887~~ **979** , ~~026~~ **721** shares of our common stock outstanding or issuable on exercise of **pre-** ~~prefunded~~ --- **funded** warrants to purchase common stock. ~~Of these shares, approximately 3, 637, 374 shares outstanding or issuable upon exercise of prefunded warrants or vested options to purchase common stock will be available for sale in the public market beginning June 15, 2024, which is 180 days after the closing of the merger on December 18, 2023 (the " Closing "), as a result of the expiration of lock-up agreements between us and certain of our securityholders.~~ All ~~other~~ outstanding shares of common stock and any shares issuable on exercise of **pre-** ~~prefunded~~ --- **funded** warrants or vested options to purchase our common stock, other than shares held by our affiliates or otherwise subject to restrictions on vesting and exercise, are freely tradable, without restriction, in the public market. If **a significant number of** these shares are sold, the trading price of our common stock could decline. Our executive officers, directors and principal stockholders beneficially own a significant percentage of our outstanding common stock. As a result, if these stockholders were to choose to act together, they would be able to control or significantly influence all matters submitted to our stockholders for approval, as well as our management and affairs. For example, these stockholders, if they choose to act together, would control or significantly influence the election of directors and approval of any merger, consolidation or sale of all or substantially all of our assets. This concentration of voting power could delay or prevent ~~an~~ **our** acquisition ~~of Neurogene~~ on terms that other stockholders may desire. We may be exposed to increased litigation, including stockholder litigation, which could have an adverse effect on our business and operations. We may be exposed to increased litigation from stockholders, suppliers and other third parties, which may have an adverse impact on our business and results of operations or may cause disruptions to our operations. In the past, stockholders have initiated class action lawsuits against biotechnology companies following periods of volatility in the market prices of these companies' stock, and we may also be subject to threats of litigation based on our recent merger activity. Such litigation, if instituted against us, could cause us to incur substantial costs and divert management' s attention and resources, which could have a material adverse effect on our business, financial condition and results of operations. If equity research analysts do not publish research or reports, or publish unfavorable research or reports, about us, our business or our market, our stock price and trading volume could decline. The trading market for our common stock will be influenced by the research and reports that equity research analysts publish about us and our business. Equity research analysts may elect to not provide research coverage of our common stock, and such lack of research coverage may adversely affect the market price of our common stock. If we do have equity research analyst coverage, we will not have any control over the analysts or the content and opinions included in their reports. The price of our common stock could decline if one or more equity research analysts downgrade our stock or issue other unfavorable commentary or research. If one or more equity research analysts ceases coverage of ~~Neurogene~~ **us** or fails to publish reports on us regularly, demand for our common stock could decrease, which in turn could cause our stock price or trading volume to decline. ~~The rules dealing with U. S. federal, state and local income taxation are constantly under review by persons involved in the legislative process and by the Internal Revenue Service and the U. S. Treasury Department. Changes to tax laws (which changes may have retroactive application) could adversely affect us or our stockholders. We continue to assess the impact of various tax reform proposals and modifications to existing tax treaties in all jurisdictions where we have operations or employees to determine the potential effect on our business and any assumptions we make about our future taxable income. We cannot predict whether any specific proposals will be enacted, the terms of any such proposals or what effect, if any, such proposals would have on our business if they were to be enacted. For example, the United States recently enacted the Inflation Reduction Act of 2022, which implements, among other changes, a 1 % excise tax on certain stock buybacks. In addition, beginning in 2022, the Tax Cuts and Jobs Act eliminated the option to deduct research and development expenditures and requires taxpayers to amortize them generally over five years. The U. S. Congress is considering legislation that would restore the current deductibility of research and development expenditures; however, there is no assurance that the current provision will be repealed or otherwise modified. Such changes, among others, may adversely affect our effective tax rate, results of operation and general business condition.~~