Risk Factors Comparison 2025-03-24 to 2024-03-11 Form: 10-K

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Investing in our common stock involves a high degree of risk. You should consider carefully the risks and uncertainties described below, together with all of the other information in this Annual Report, including **the section titled " Management' s Discussion and Analysis of Financial Condition and Results of Operations " and** our financial statements and related notes **included elsewhere in this Annual Report** , before deciding whether to **invest in** ~~purchase shares of~~ our common stock. The risks and uncertainties described below are not the only ones we face. Additional risks and uncertainties that we are unaware of, or that we currently believe are not material, may also become important factors that affect us. If any of the following risks are realized, our business, financial condition, results of operations and prospects could be materially and adversely affected. In that event, the ~~trading~~ price of our common stock could decline, and you could lose part or all of your investment **. We cannot provide assurance that any of the events discussed below will not occur** . Risk Factors Summary Investing in our common stock involves a high degree of risk because our business is subject to numerous risks and uncertainties, as fully described below. The principal factors and uncertainties that make investing in our common stock risky include, among others: • we have incurred significant losses since our inception, and we expect to incur significant net losses for the foreseeable future and may not be able to achieve or sustain revenue or profitability in the future; • we have a limited operating history, have not completed any clinical trials, and have no products approved for commercial sale; • if we are unable to raise capital when needed, we could be forced to curtail our planned operations and the pursuit of our growth strategy; • raising additional capital may cause dilution to our stockholders, restrict our operations or require us to relinquish rights to our technologies or product candidates; • our business is dependent on our ability to advance our current and future product candidates through preclinical studies and clinical trials, obtain marketing approval and ultimately commercialize them. If we are unable to or experience significant delays in doing so, our business will be materially harmed; • we are developing novel gene therapy product candidates, which makes it difficult to predict the time, cost and potential success of product candidate development; • 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 with certainty the geographic areas in which we could obtain regulatory approval or the time and cost of obtaining regulatory approval, if we receive it at all, for any product candidates we may develop; • preclinical studies and clinical trials are expensive, time- consuming, difficult to design and implement and involve an uncertain outcome. Further, we may encounter substantial delays in completing the development of our product candidates; • the regulatory approval processes of the FDA, EMA and comparable foreign authorities are lengthy, time- consuming and inherently unpredictable. If we are not able to obtain required regulatory approval for our product candidates, our business will be substantially harmed; • success in preclinical studies or earlier clinical trials may not be indicative of results in future clinical trials; • interim " top- line " and preliminary results from our clinical trials that we announce, publish or present from time to time may change as more patient data become available and are subject to audit and verification procedures that could result in material changes in the final data; • our preclinical studies and clinical trials may fail to demonstrate the safety and efficacy of our product candidates, or serious adverse or unacceptable side effects may be identified during the development of our product candidates, which could prevent or delay regulatory approval and commercialization, increase our costs or necessitate the abandonment or limitation of the development of some of our product candidates. We may also identify safety and efficacy concerns after the approval of a product candidate which can result in negative consequences to our business and results of operations; • some of the diseases we initially seek to treat have low prevalence and it may be difficult to identify and enroll patients with these diseases. 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; • we may seek Orphan Drug designation or Rare Pediatric Disease designation for some of our product candidates and we may be unsuccessful, or may be unable to maintain the benefits associated with Orphan Drug designation, including the potential for market exclusivity, for product candidates for which we obtain Orphan Drug designation; • Fast Track, Breakthrough Therapy, or Regenerative Medicine Advanced Therapy designation that we may receive from the FDA may not actually lead to a faster development or regulatory review or approval process, and does not assure FDA approval of our product candidates; • we have received Rare Pediatric Disease designation from the FDA for LX2006 for the treatment of FA and we may seek such designation for future product candidates **if Congress extends the rare pediatric disease priority review program** . However, a marketing application for these product candidates, if approved, may not meet the eligibility criteria for a rare pediatric disease priority review voucher; • we and our contract manufacturers are subject to significant regulation with respect to manufacturing our products. The third- party manufacturing facilities on which we rely, and any manufacturing facility that we may have in the future, may have limited capacity or fail to meet the applicable stringent regulatory requirements; • 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; • 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; • even if any of our product candidates receive marketing approval, they may fail to achieve the degree of market acceptance by physicians, patients, third- party payors and others in the medical community necessary for commercial success; • ~~currently,~~ we rely on our collaborations with ~~Cornell University and UCSD~~ **several leading academic institutions** to conduct research and development for many of our pipeline programs, including conducting preclinical and IND- enabling studies for portions of our near- term future pipeline. Failure or delay of ~~Cornell University or~~ **our** ~~UCSD~~ **academic partners** to fulfill all or part of their

respective obligations to us under our agreements, a breakdown in collaboration between the parties or a complete or partial loss of either of these relationships could materially harm our business; • we intend to continue to rely on third parties to conduct a significant portion of our 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; • if we are unable to obtain or protect intellectual property rights related to any of our product candidates, we may not be able to compete effectively in our market **; • changes in the FDA and other government agencies, regulatory actions and other actions under the new Trump administration could hinder their ability to hire and retain key leadership and other personnel, or otherwise prevent new products and services from being developed or commercialized in a timely manner, which could negatively impact our business** ; and • we are currently subject to a lawsuit claiming, among other things, that we misappropriated the confidential information and trade secrets of Rocket, and which seeks unspecified damages and asks the court to enjoin us from competing and working in the market for gene therapy treatments targeting cardiac diseases. In the future, we may be subject to additional claims that we and our employees, consultants ~~,~~ or independent contractors have wrongfully used or disclosed confidential information or trade secrets of third parties. Risks related to our financial position and capital needs We have incurred significant losses since our inception. We expect to incur losses over the next several years and may never achieve or maintain profitability. Since our inception, we have incurred significant net losses, and we expect to continue to incur significant expenses and operating losses for the foreseeable future. For the fiscal years ended December 31, **2024 and** 2023 ~~and 2022~~ , we incurred net losses of $ **98. 3 million and $** 66. 4 ~~million and $ 59. 3~~ million, respectively, and we had an accumulated deficit of $ ~~181~~ **280** . ~~8~~ **2** million as of December 31, ~~2023~~ **2024** . We have primarily financed our operations with ~~approximately of $ 100. 3 million of~~ net proceeds raised in our **initial public offering, or** IPO **,** and the subsequent partial exercise of the underwriters' 30- day option to purchase additional shares of common stock, as well as ~~totals of $ 185. 0 million and $ 3. 9 million of~~ net proceeds from **the sale of our common stock in a private placement, or the Private Placement,** sales of our convertible equity securities and ~~a~~ **the** convertible SAFE Note ~~, respectively~~ . We have no products approved for commercialization and have never generated any revenue from product sales. We are still in the early clinical stages of development of our lead product candidates. We expect to continue to incur significant expenses and operating losses over the next several years. 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 as we: • continue to advance the preclinical and clinical development of our product candidates and discovery programs; • initiate and complete additional clinical trials of our current and future product candidates; • seek regulatory approval for any product candidates that successfully complete clinical trials; • continue to develop our gene therapy product candidate pipeline; • scale up our clinical and regulatory capabilities; • work with our third party manufacturing partners to produce material in accordance with cGMP for clinical trials or potential commercial sales; • establish, either alone or with a third party, a commercialization infrastructure and scale up manufacturing and distribution capabilities to commercialize any product candidates for which we may obtain regulatory approval; • adapt our regulatory compliance efforts to incorporate requirements applicable to marketed products; • maintain, expand and protect our intellectual property portfolio and patent claims; • hire additional clinical, quality control, regulatory, manufacturing, scientific and administrative personnel; • add operational, financial and management information systems and personnel, including personnel to support our product development and planned future commercialization efforts; and • incur additional legal, accounting and other expenses in operating as a public company. To date, we have not generated any revenue from the commercialization of our product candidates. To become and remain profitable, we must succeed in developing and eventually commercializing product candidates that generate significant revenue. This will require us to be successful in a range of challenging activities, including completing preclinical testing and clinical trials of our product candidates, obtaining regulatory approval, and manufacturing, marketing and selling any product candidates for which we may obtain regulatory approval, as well as discovering and developing additional product candidates. We are only in the preliminary stages of most of these activities and all of our product candidates are in early clinical trials or preclinical development. We may never succeed in these activities and, even if we do, may never generate any revenue or revenue that is significant enough to achieve profitability. Even if we achieve profitability, we may not be able to sustain or increase profitability on a quarterly or annual basis. Our failure to become and remain profitable would depress the value of our company and could impair our ability to raise capital, expand our business, maintain our development efforts, obtain product approvals, diversify our offerings or continue our operations. A decline in the value of our company could also cause you to lose all or part of your investment. We have a limited operating history and no history of commercializing products, which may make it difficult for an investor to evaluate the success of our business to date and to assess our future viability. We are a clinical stage genetic medicine company with a limited operating history. We commenced substantive business operations in 2020, and our operations to date have been largely focused on organizing and staffing our company, business planning, raising capital ~~and~~ **,** entering into collaboration and license agreements for conducting preclinical and clinical research and development activities for our product candidates and gene therapy pipeline **, and conducting clinical trials for our product candidates through CROs and other third parties** . To date, we have not yet demonstrated our ability to successfully ~~complete internally sponsored clinical trials,~~ complete pivotal clinical trials, manufacture a product on a commercial scale or arrange for a third party to do so on our behalf, or conduct sales and marketing activities necessary for successful commercialization. Consequently, any predictions you make about our future success or viability may not be as accurate as they could be if we had a longer operating history or a history of successfully developing and commercializing products. We will need substantial additional funding to meet our financial obligations and to pursue our business objectives. If we are unable to raise capital when needed, we could be forced to curtail our planned operations and the pursuit of our growth strategy. We will require substantial future capital in order to complete planned and future clinical development for our lead product candidates, preclinical development for our other product candidates, and potential commercialization of these product candidates, if any are approved. We expect our spending levels to significantly increase in connection with our planned clinical

trials of our lead product candidates. In addition, if we obtain marketing approval for any of our product candidates, we expect to incur significant expenses related to product sales, medical affairs, marketing, manufacturing and distribution. We also expect to incur additional costs associated with operating as a public company. Accordingly, we will need to obtain substantial additional funding in connection with our continuing operations. If we are unable to raise capital when needed or on acceptable terms, we would be forced to delay, reduce or eliminate certain of our clinical trials, our research and development programs or other operations. As of December 31, ~~2023~~ **2024**, we had cash ~~and ,~~ cash equivalents **, and investments** of $ ~~121~~ **128**. 5 million. **We** ~~Following the $ 95. 0 million of gross proceeds expected to be received upon the anticipated closing of our private placement equity financing in March 2024, we~~ believe that our cash ~~and ,~~ cash equivalents **, and investments balances** will be sufficient to fund our operating expenses and capital requirements into 2027. This estimate is based on assumptions that may prove to be wrong, and we could use our available capital resources sooner than we expect. Changes may occur beyond our control that would cause us to consume our available capital before that time, including changes in and progress of our development activities, acquisitions of additional product candidates, and changes in regulation. Our future capital requirements will depend on many factors, including: • the costs of and investment in ongoing and future development of our gene therapy product candidates; • the scope, progress, costs and results of discovery, preclinical development, laboratory testing and clinical trials for our product candidates; • the extent to which we develop, in- license or acquire other product candidates and technologies in our product candidate pipeline; • the costs and timing of process development and manufacturing scale- up activities associated with our product candidates and other programs as we advance them through preclinical and clinical development; • the number of, and development requirements for, product candidates that we may pursue; • the costs, timing and outcome of regulatory review of our product candidates; • our headcount growth and associated costs as we expand our research and development capabilities and establish a commercial infrastructure; • the costs of establishing and maintaining commercial- scale cGMP manufacturing capabilities, either internally or with third parties; • the costs and timing of future commercialization activities, including product manufacturing, marketing, sales and distribution, for any of our product candidates for which we receive marketing approval; • the costs and timing of preparing, filing and prosecuting patent applications, maintaining and enforcing our intellectual property rights and defending any intellectual property- related claims; • the revenue, if any, received from commercial sales of our product candidates for which we receive marketing approval; • our ability to establish and maintain additional collaborations on favorable terms, if at all; • the success of any collaborations that we may establish and our license agreements; • the outcome of **any** legal proceedings involving us; • the achievement of milestones or occurrence of other developments that trigger payments under our collaboration agreement or any additional collaboration agreements we may enter into; and • the costs of operating as a public company. We will require additional capital to achieve our business objectives. While the long- term economic impact of ~~either the COVID- 19 pandemic or~~ the ongoing geopolitical conflicts in Ukraine and ~~Israel~~ **the Middle East** is difficult to assess or predict, each of these events has caused significant disruptions to the global financial markets and contributed to a general global economic slowdown. Furthermore, inflation rates, particularly in the United States, have increased recently to levels not seen in decades. Increased inflation may result in increased operating costs and may affect our operating budgets, specifically with respect to increased labor costs and associated difficulties in recruiting qualified personnel. In addition, the U. S. Federal Reserve has raised, and may further raise, interest rates in response to concerns about inflation. Increases in interest rates, especially if coupled with reduced government spending and volatility in financial markets, may further increase economic uncertainty and heighten these risks. If the disruptions and slowdown deepen or persist, we may not be able to access additional capital on favorable terms, or at all, which could in the future negatively affect our financial condition and we could be forced to curtail our planned operations and the pursuit of our growth strategy. Raising additional capital may cause dilution to our stockholders, restrict our operations or require us to relinquish rights to our technologies or product candidates. Until such time, if ever, as we can generate substantial revenue, we may finance our cash needs through a combination of equity offerings, government or private party grants, debt financings and license and collaboration agreements. We do not currently have any committed external source of funds. To the extent that we raise additional capital through the sale of equity or convertible debt securities, your ownership interest will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect your rights as a common stockholder. Debt financing and preferred equity financing, if available, may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends. If we raise additional funds through collaborations, strategic alliances or marketing, distribution or licensing arrangements with third parties, we may be required to relinquish valuable rights to our technologies, future revenue streams or product candidates, grant licenses on terms that may not be favorable to us or commit to future payment streams. If we are unable to raise additional funds through equity or debt financings when needed, we may be required to delay, limit, reduce or terminate our product development or future commercialization efforts or grant rights to develop and market product candidates that we would otherwise prefer to develop and market ourselves. Risks related to the development of our product candidates Our business is dependent on our ability to advance our current and future product candidates through preclinical studies and clinical trials, obtain marketing approval and ultimately commercialize them. If we are unable to or experience significant delays in doing so, our business will be materially harmed. We have invested a significant portion of our time and financial resources in the development of our product candidates and technology platforms. Our business is dependent on our ability to successfully complete preclinical and clinical development of, obtain regulatory approval for, and, if approved, successfully commercialize LX2006, ~~LX1001,~~ LX2020 and any other product candidates in a timely manner. Each of our product candidates and programs will require additional preclinical and / or clinical development, regulatory approval and significant marketing efforts, and we will be required to obtain manufacturing supply and expertise and to build a commercial organization or successfully outsource commercialization before we generate any revenue from product sales. We do not have any products that are approved for commercial sale, and we may never be able to develop or commercialize marketable

products. Our ability to generate revenue from our product candidates, which **may** ~~we do~~ not ~~expect to~~ occur for several years ~~, if ever~~ , will depend heavily on the successful development, regulatory approval and eventual commercialization of our product candidates. The success of our lead product candidates, or any other product candidates that we develop or otherwise may acquire will depend on ~~several~~ **various** factors, including: • timely and successful completion of preclinical studies, including toxicology studies, biodistribution studies and minimally efficacious dose studies in animals, where applicable, under GLPs; • the availability or development of suitable animal disease models for nonclinical studies to enable us to proceed into clinical development or support the submission of a marketing application; • effective IND applications from the FDA or comparable foreign applications that allow commencement of our planned clinical trials or future clinical trials for our product candidates; • sufficiency of our financial and other resources to complete the necessary preclinical studies and clinical trials; • successful enrollment and completion of clinical trials, including under cGCPs; • establishment of our own manufacturing capabilities and / or arrangements with third- party manufacturers for our commercial manufacturing processes for any of our product candidates that receive regulatory approval; • receipt of timely marketing approvals from applicable regulatory authorities; • launch of commercial sales of products, if approved, whether alone or in collaboration with others; • acceptance of the benefits and use of our products, including method of administration, if and when approved, by patients, the medical community and third- party payors, for their approved indications; • the prevalence and severity of adverse events experienced with any of our product candidates; • the availability, perceived advantages, cost, safety and efficacy of alternative therapies for any diseases for which we are developing our product candidates; • our ability to produce our product candidates on a commercial scale; • attainment and maintenance of patent, trademark and trade secret protection and regulatory exclusivity for our product candidates and otherwise protecting our rights in our intellectual property portfolio; • maintenance of compliance with regulatory requirements such as cGMPs; • attainment and maintenance of third- party coverage and adequate reimbursement for our product candidates and patients' willingness to pay out- of- pocket in the absence of such coverage and adequate reimbursement; and • maintenance of a continued acceptable safety, tolerability and efficacy profile of our products following approval. If we are not successful with respect to one or more of these factors in a timely manner or at all, we could experience significant delays or an inability to successfully commercialize the product candidates we develop, which would materially harm our business. If we do not receive marketing approvals for any product candidate we develop, we may not be able to continue our operations. We are developing novel gene therapy product candidates, which makes it difficult to predict the time, cost and potential success of product candidate development. Our future success depends on the successful development of a novel therapeutic approach. To date, very few products that utilize gene transfer have been approved in the United States or Europe. There have been a limited number of clinical trials using AAVrh10. Although gene therapies have been studied in human clinical trials for over 30 years, only a limited number of AAV- based gene therapy products have been approved by the FDA. We cannot be certain that our AAVrh10- based gene therapy product candidates will successfully complete clinical trials or that any future product candidates utilizing this or other vector constructs will successfully complete preclinical studies or clinical trials. We may not be successful in developing product candidates that avoid triggering toxicities or other side effects in preclinical studies or clinical trials. Our intravenous ~~, intracisternal~~ and intrathecal routes of administration may cause unforeseen side effects or present other challenges. Any such results could impact our ability to develop a product candidate, including our ability to enroll patients in our clinical trials. As a result of these factors, it is more difficult for us to predict the time and cost of product candidate development, and we cannot predict whether the application of our approach to gene therapy, or any similar or competitive programs, will result in the identification, development, and regulatory approval of any product candidate, or that other gene therapy programs will not be considered better or more favorable. There can be no assurance that any development problems we experience in the future related to our current gene therapy product candidates or any of our research programs will not cause significant delays or unanticipated costs, or that such development problems can be solved. We may also experience delays and challenges in achieving sustainable, reproducible, and scalable production. Any of these factors may prevent us from completing our preclinical studies or clinical trials or commercializing any product candidates we may develop on a timely or profitable basis, if at all. 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 with certainty the geographic areas in which we could obtain regulatory approval ~~or~~ **nor** the time and cost of obtaining regulatory approval, if we receive it 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. The novel nature of our capsids makes it difficult to determine how long it will take or how much it will cost to obtain regulatory approvals for our product candidates in the United States, the European Union or other jurisdictions. Within the broader genetic medicine field, very few gene therapy products have received marketing authorization from the FDA or the European Medicines Agency, or EMA. Even with respect to gene therapies, the regulatory landscape is still developing. Regulatory requirements governing gene therapy products have changed frequently and will likely continue to change in the future, including with respect to those responsible for regulation of existing gene therapy products. For example, in 2016, the FDA established the Office of Tissues and Advanced Therapies, or OTAT, within the Center for Biologics Evaluation and Research, or CBER, to consolidate the review of gene therapy and related products, and to advise the CBER on its review. In ~~September~~ **March** ~~2022~~ **2023** , the FDA **retitled the** ~~announced retitling of~~ OTAT to the Office of Therapeutic Products, or OTP, and **elevated** ~~elevation of~~ OTP to a " Super Office " to meet its growing cell and gene therapy workload. Our product candidates will need to meet safety and efficacy standards applicable to any new biologic being pursued for a given disease under the regulatory framework administered by the FDA. Although the FDA decides whether individual gene therapy protocols may proceed, the review process and determinations of other reviewing bodies, including IRBs, can impede or delay the initiation of a clinical trial. The same applies in the ~~EU~~ **European Union** . The EMA' s Committee for Advanced Therapies, or 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. This could mean that any gene therapy product candidate we may develop in the future could be required to comply with additional and / or more stringent gene therapy guidelines in the ~~EU~~ **European Union**. 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 bodies 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 and efficacy of a product candidate vary substantially according to the type, complexity, novelty, and intended use and market of the potential products. The regulatory approval process for product candidates such as ours 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, there is heightened risk that the FDA, the EMA or other regulatory bodies 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 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 any 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 product 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. Preclinical studies and clinical trials are expensive, time- consuming, difficult to design and implement and involve an uncertain outcome. Further, we may encounter substantial delays in completing the development of our product candidates. All of our product candidates are in preclinical or early clinical development, and the risk of failure is high. The preclinical studies, clinical trials and manufacturing of our product candidates are, and the manufacturing and marketing of our products, if approved, will be, subject to extensive and rigorous review and regulation by numerous government authorities in the United States and in other countries where we may test and market our product candidates. Before obtaining regulatory approvals for the commercial sale of any of our product candidates, we must demonstrate through lengthy, complex and expensive preclinical testing and clinical trials that our product candidates are both safe and effective for use in each target disease. In particular, because our product candidates are subject to regulation as biologics, we will need to demonstrate that they are sufficiently safe and of sufficient purity and potency for use in their target diseases. Each product candidate must demonstrate an adequate risk- versus- benefit profile in its intended patient population and for its intended use. Clinical testing is expensive, can take many years to complete and is subject to uncertainty. We cannot guarantee that any clinical trials will be initiated on schedule, conducted as planned or completed on schedule, if at all. To date, we are sponsoring clinical trials of ~~LX1001,~~ LX2006 and LX2020, but we have not successfully completed any clinical trial that we have internally sponsored. Failure can occur at any time during the clinical trial process. Even if our ongoing and future clinical trials are completed as planned, we cannot be certain that their results will support the safety and effectiveness of our product candidates for their targeted diseases or support continued clinical development of such product candidates. Our future clinical trial results may not be successful. In addition, even if such trials are successfully completed, 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 rare diseases, where the small patient populations make it difficult or impossible to conduct two traditional, adequate and well- controlled trials, and therefore the FDA or comparable foreign regulatory authorities are often required to exercise flexibility in approving therapies for such diseases. Moreover, results acceptable to support approval in one jurisdiction may be deemed inadequate by another regulatory authority to support regulatory approval in that other jurisdiction. To the extent that the results of the trials are not satisfactory to the FDA or foreign 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. We may experience delays in initiating and conducting clinical trials of our lead product candidates and we do not know whether our clinical trials will begin on time, need to be redesigned, recruit and enroll patients on time or be completed on schedule, or at all. Events that may prevent successful or timely completion of clinical development include: • inability to generate sufficient preclinical, toxicology, or other in vivo or in vitro data to support the initiation of clinical trials; • delays in sufficiently developing, characterizing or controlling a manufacturing process suitable for advanced clinical trials; •

delays in sourcing or qualifying ancillaries required for administration of our clinical drug product (such as vials, stoppers, or tubing); • delays in developing suitable assays for screening patients for eligibility for trials with respect to certain product candidates; • delays in reaching agreement with the FDA, EMA or other regulatory authorities as to the design or implementation of our clinical trials; • failure to obtain regulatory approval to commence a clinical trial; • failure to reach an agreement on acceptable terms with clinical trial sites or prospective CROs, the terms of which can be subject to extensive negotiation and may vary significantly among different clinical trial sites; • inability to obtain IRB approval for each clinical trial site; • inability to recruit suitable patients to participate in a clinical trial in a timely manner; • failure to have patients complete a clinical trial or return for post- treatment follow- up; • deviations by clinical trial sites, CROs or other third parties from trial protocol; • failure to perform our planned clinical trials in accordance with the FDA' s cGCP requirements, or applicable regulatory guidelines in other countries; • inability to address patient- safety concerns that arise during the course of a trial, including occurrence of adverse events associated with the product candidate that are viewed to outweigh its potential benefits; • failure to initiate a sufficient number of clinical trial sites; or • delays in manufacturing sufficient quantities of a product candidate for use in clinical trials. We may experience numerous unforeseen events during, or as a result of, clinical trials that could delay or prevent our ability to receive marketing approval or commercialize our product candidates or significantly increase the cost of such trials, including: • we may experience changes in regulatory requirements or guidance, or receive feedback from regulatory authorities **,** that ~~requires~~ **require** us to modify the design of our clinical trials; • clinical trials of our product candidates may produce negative or inconclusive results, and we may decide, or regulators may require us, to conduct additional clinical trials or halt development programs; • the number of patients required for clinical trials of our product candidates may be larger than we anticipate, enrollment in these clinical trials may be slower than we anticipate, or participants may drop out of these clinical trials 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; • we, our investigators or regulators may suspend or terminate clinical trials of our product candidates for various reasons, including non-compliance with regulatory requirements, a finding that our product candidates have undesirable side effects or other unexpected characteristics, or a finding that the participants are being exposed to unacceptable health risks; • the cost of clinical trials of our product candidates may be greater than we anticipate, and we may not have funds to cover the costs; • the supply or quality of our product candidates or other materials necessary to conduct clinical trials of our product candidates may be insufficient or inadequate; • regulators may revise the requirements for approving our product candidates, or such requirements may not be as we anticipate; and • any future collaborators that conduct clinical trials may face any of the above issues and may conduct clinical trials in ways they view as advantageous to them but that are suboptimal for us. If we are required to conduct additional clinical trials or other testing of our product candidates beyond those that we currently contemplate, if we are unable to successfully initiate or complete clinical trials of our product candidates or other testing, if the results of these trials or tests are not positive or are only modestly positive or if there are safety concerns, we may: • incur unplanned costs; • be delayed in obtaining marketing approval for our product candidates or not obtain marketing approval at all; • obtain marketing approval in some countries and not in others; • obtain marketing approval for diseases or patient populations that are not as broad as intended or desired; • obtain marketing approval with labeling that includes significant use or distribution restrictions or safety warnings, including boxed warnings or REMS; • be subject to additional post- marketing testing requirements; or • have the product removed from the market after obtaining marketing approval. We could encounter delays if a clinical trial is suspended or terminated by us, by the IRBs of the institutions in which such trials are being conducted, by the Data Safety Monitoring Board for such trial or by the FDA, EMA or other regulatory authorities. Such authorities may impose such a suspension or termination due to a number of factors, including failure to conduct the clinical trial in accordance with regulatory requirements or our clinical protocols, inspection of the clinical trial operations or trial site by the FDA, EMA or other regulatory authorities resulting in the imposition of a clinical hold, unforeseen safety issues or adverse side effects, failure to demonstrate a benefit from using a drug, changes in governmental regulations or administrative actions or lack of adequate funding to continue the clinical trial. All of our product candidates will require extensive clinical testing before we are prepared to submit a BLA or marketing authorization application, or MAA, for regulatory approval. We cannot predict with any certainty if or when we might complete the clinical development for our product candidates and submit a BLA or MAA for regulatory approval of any of our product candidates or whether any such BLA or MAA will be approved. We may also seek feedback from the FDA, EMA or other regulatory authorities on our clinical development program, and the FDA, EMA or such regulatory authorities may not provide such feedback on a timely basis, or such feedback may not be favorable, which could further delay our development programs. We cannot predict with any certainty whether or when we might complete a given clinical trial. If we experience delays in the commencement or completion of our clinical trials, or if we terminate a clinical trial prior to completion, the commercial prospects of our product candidates could be harmed, and our ability to generate revenues from our product candidates may be delayed or lost. In addition, any delays in our clinical trials could increase our costs, slow down the development and approval process and jeopardize our ability to commence product sales and generate revenues. Any of these occurrences may harm our business, financial condition and results of operations. In addition, many of the factors that cause, or lead to, a delay in the commencement or completion of clinical trials may also ultimately lead to the denial of regulatory approval of our product candidates. The regulatory approval processes of the FDA, EMA and comparable foreign authorities are lengthy, time- consuming and inherently unpredictable. If we are not able to obtain required regulatory approval for our product candidates, our business will be substantially harmed. The time required to obtain approval or other marketing authorizations by the FDA, EMA and comparable foreign authorities is unpredictable, and it typically takes many years following the commencement of clinical trials and depends upon numerous factors, including the substantial discretion of the regulatory authorities. In addition, approval policies, regulations, and the type and amount of clinical data necessary to gain approval may change during the course of a product candidate' s clinical development and may vary among jurisdictions. We have not

obtained regulatory approval for any product candidate, and it is possible that we may never obtain regulatory approval for any product candidates we may seek to develop in the future. Neither we nor any collaborator is permitted to market any of our biologic product candidates in the United States until we receive regulatory approval of a BLA from the FDA, and we cannot market any of our product candidates in the European Union until we receive approval for an MAA from the EMA, or other required regulatory approval in other countries. Prior to obtaining approval to commercialize any product candidate in the United States or abroad, we must demonstrate with substantial evidence from well- controlled clinical trials, and to the satisfaction of the FDA or foreign regulatory agencies, that such product candidates are safe, effective and of sufficient purity for their intended uses. Results from preclinical studies and clinical trials can be interpreted in different ways. Even if we believe the preclinical or clinical data for our product candidates meet regulatory standards, such data may not be sufficient to support approval by the FDA and other regulatory authorities. The FDA may also require us to conduct additional preclinical studies or clinical trials for our product candidates either prior to or after approval, or it may object to elements of our clinical development programs, or require changes to our manufacturing approaches. 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 and marketing authorization process as well as the unpredictability of future clinical trial results may result in our failing to obtain regulatory approval and marketing authorization to market our product candidates, which would significantly harm our business, financial condition, results of operations and prospects. We have invested a significant portion of our time and financial resources in the development of our product candidates and technology platforms. Our business is dependent on our ability to successfully complete preclinical and clinical development of, obtain regulatory approval for, and, if approved, successfully commercialize LX2006, LX2020, ~~LX1001~~ and our other product candidates in a timely manner. Even if we eventually complete clinical testing and receive approval of a BLA or foreign marketing application for any of our product candidates, the FDA, EMA or the applicable foreign regulatory agency may grant approval or other marketing authorization contingent on the performance of costly additional clinical trials, including post- marketing clinical trials. The FDA, EMA or the applicable foreign regulatory agency also may approve or authorize for marketing a product candidate for a more limited disease or patient population than we originally request, and the FDA, EMA or applicable foreign regulatory agency may not approve or authorize the labeling that we believe is necessary or desirable for the successful commercialization of a product candidate. Any delay in obtaining, or inability to obtain, applicable regulatory approval or other marketing authorization would delay or prevent commercialization of that product candidate and would materially adversely impact our business and prospects. In addition, the FDA, EMA and other regulatory authorities may change their policies, issue additional regulations or revise existing regulations, or take other actions, which may prevent or delay approval of our future products 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, increase the costs of compliance or restrict our ability to maintain any marketing authorizations we may have obtained. In ~~addition~~ **June 2024** , ~~if~~ the Supreme Court **overruled** ~~reverses or curtails~~ the Chevron doctrine, which **had** ~~gives~~ **given** deference to regulatory agencies **' statutory interpretations of ambiguous regulations** in litigation against **federal government agencies, such as the** FDA **. The overruling of the Chevron doctrine may significantly increase the number of challenges brought by companies** and other **stakeholders against federal** agencies **such as the** ~~, more companies may bring lawsuits against~~ FDA ~~to challenge~~ **and its** longstanding decisions and policies ~~of~~ **, including the** FDA **' s statutory interpretations of market exclusivities and the " substantial evidence " requirements for drug approvals** , which could undermine **the** FDA' s authority, lead to uncertainties in the industry, and disrupt **the** FDA' s normal operations, **any of** which could delay **the** FDA' s review of our ~~marketing applications~~ **regulatory submissions. We cannot predict the full impact of this decision, future judicial challenges brought against the FDA, or the nature or extent of government regulation that may arise from future legislation or administrative action** . Success in preclinical studies or earlier clinical trials may not be indicative of results in future clinical trials. Success in preclinical testing and early clinical trials does not ensure that later clinical trials will generate the same results or otherwise provide adequate data to demonstrate the efficacy and safety of a product candidate. Preclinical tests and Phase 1 and Phase 2 clinical trials are primarily designed to test safety, to study pharmacokinetics and pharmacodynamics and to understand the side effects of product candidates at various doses and schedules. Success in preclinical or animal studies and early clinical trials does not ensure that later larger- scale efficacy and safety trials will be successful, nor does it predict final results. For example, we may be unable to identify suitable animal disease models for our product candidates, which could delay or frustrate our ability to proceed into clinical trials or obtain marketing approval. In addition, the preclinical studies conducted by Stelios (an entity that we acquired in 2021) and **The Regents of** UCSD for our product candidates LX2021 and LX2022 employed an AAV9- based formulation and studies using this vector may not be predictive of future testing we intend to conduct using an AAVrh10- based formulation or other potential capsid serotypes. Our product candidates may fail to show the desired safety and efficacy in clinical development despite positive results in preclinical studies or having successfully advanced through initial clinical trials. Furthermore, our currently ongoing and most future clinical trials involve or will involve a small patient population. Because of the small sample sizes studied in our trials thus far, the results of these trials may not be indicative of results of future clinical trials. Additionally, some of our ongoing and planned clinical trials utilize, or may utilize, an " open- label " trial design. An " open- label " clinical trial is one where both the patient and investigator know whether the patient is receiving the investigational product candidate or either an existing approved drug or placebo. Most typically, open- label clinical trials test only the investigational product candidate and sometimes may do so at different dose levels. Open- label clinical trials are subject to various limitations that may exaggerate any therapeutic effect as patients in open- label clinical trials are aware when they are receiving treatment. Open- label clinical trials may be subject to a " patient bias " where patients perceive their symptoms to have improved merely due to their awareness of receiving an experimental treatment. In addition, open- label clinical trials may be subject to an " investigator bias " where those assessing and reviewing the physiological outcomes of the clinical trials are aware of which patients have

received treatment and may interpret the information of the treated group more favorably given this knowledge. The results from an open- label trial may not be predictive of future clinical trial results when studied in a controlled environment with a placebo or active control. Many companies in the pharmaceutical and biotechnology industries have suffered significant setbacks in late- stage clinical trials even after achieving promising results in preclinical testing and earlier- stage clinical trials. Data obtained from preclinical and clinical activities are subject to varying interpretations, which may delay, limit or prevent regulatory approval. In addition, we may experience regulatory delays or rejections as a result of many factors, including changes in regulatory policy during the period of our product candidate development. Any such delays could negatively impact our business, financial condition, results of operations and prospects. Interim " top- line " and preliminary results from our clinical trials that we announce, publish or present from time to time may change as more patient data become available and are subject to audit and verification procedures that could result in material changes in the final data. From time to time, we **have and** may **continue to** publish or present interim top- line or preliminary results from our clinical trials. Interim results from clinical trials that we may complete are subject to the risk that one or more of the clinical outcomes may materially change as patient enrollment continues and more patient data become available. Preliminary or top- line results also remain subject to audit and verification procedures that may result in the final data being materially different from the preliminary data we previously published. As a result, interim and preliminary data should be viewed with caution until the final data are available. Differences between preliminary or interim data and final data could significantly harm our business prospects and may cause the trading price of our common stock to fluctuate significantly. Our preclinical studies and clinical trials may fail to demonstrate the safety and efficacy of our product candidates, or serious adverse or unacceptable side effects may be identified during the development of our product candidates, which could prevent or delay regulatory approval and commercialization, increase our costs or necessitate the abandonment or limitation of the development of some of our product candidates. We may also identify safety and efficacy concerns after the approval of a product candidate which can result in negative consequences to our business and results of operations. Before obtaining regulatory approvals for the commercial sale of our product candidates, we must demonstrate through lengthy, complex and expensive preclinical testing and clinical trials that our product candidates are safe, effective and of sufficient purity for use in each target disease, and failures can occur at any stage of testing. Preclinical studies and clinical trials often fail to demonstrate safety or efficacy of the product candidate studied for the target disease. While we have developed our AAVrh10- mediated gene therapy product candidates to leverage the low seropositivity of AAVrh10, any gene therapy product based on viral vectors carries the risks of immunogenicity, elevated liver enzymes and insertional oncogenesis, which is the process whereby the insertion of a functional gene near a gene that is important in cell growth or division results in uncontrolled cell division, which could potentially enhance the risk of malignant transformation. In one of our preclinical studies of LX2006, we observed four cases of hepatocellular carcinoma, or HCC, in wild- type mice at 10 months post- treatment. Although data reported by the FDA Cellular, Tissue, and Gene Therapies Advisory Committee in September 2021 suggests that HCC observed in mice after AAV treatment is unlikely to translate to risks for humans, any future instances of HCC in our clinical trials could result in delays or the abandonment of our trials. Health authorities also ask that sponsors closely monitor the risk of elevated liver enzymes and abnormal liver ultrasound on a routine basis in patients participating in gene therapy clinical trials. Possible adverse side effects that could occur with treatment with gene therapy products include an immunologic reaction early after administration, which, while not necessarily adverse to the patient' s health, could substantially limit the effectiveness of the treatment. For example, in previous third- party clinical trials involving AAV capsids for gene therapy, some subjects experienced the development of a T- cell antibody response, whereby after the vector is within the target cell types, the cellular immune response system triggers the removal of transduced cell types by activated T- cells. If any of our product candidates demonstrate a similar effect, we may decide or be required to perform additional preclinical studies or to halt or delay further clinical development of our product candidates. In addition to side effects caused by the product candidate, the administration process or related procedures also can cause adverse side effects. Our APOE- associated Alzheimer' s disease product candidates are designed to be delivered via intracisternal administration. While the intracisternal method of administration has been available for some years, its use for gene therapies is new and no gene therapy is currently approved for this method of administration. Intracisternal administration may have greater risk and / or be perceived as having greater risk than more common methods of administration, such as intravenous injection. Other gene therapy product candidates in clinical development utilizing intracisternal delivery could also generate data that could adversely affect the clinical, regulatory or commercial perception of our product candidates. If adverse events occur, either as a result of the product candidate or administration process, our clinical trials could be suspended or terminated. If we cannot demonstrate that any adverse events were not caused by the drug or administration process or related procedures, the FDA, EMA or foreign regulatory authorities could order us to cease further development of, or deny approval of, our product candidates for any or all targeted diseases. Even if we are able to demonstrate that ~~all future~~ serious adverse events are not product- related, such occurrences could affect patient recruitment or the ability of enrolled patients to complete the trial. Moreover, if we elect, or are required, to not initiate, delay, suspend or terminate any future clinical trial of any of our product candidates, the commercial prospects of such product candidates may be harmed and our ability to generate product revenues from any of these product candidates may be delayed or eliminated. Any of these occurrences may harm our ability to develop other product candidates, and may harm our business, financial condition and prospects significantly. Furthermore, negative results in our development of LX2006 ~~, LX1001,~~ or LX2020 could be interpreted as a failure to achieve proof of concept for our technology and result in the abandonment of other development programs. In addition, gene therapy is still a relatively new approach to disease treatment and additional adverse side effects could develop. There also is the potential risk of delayed adverse events following exposure to gene therapy products due to persistent biologic activity of the genetic material or other components of products used to carry the genetic material. If our product candidates are associated with side effects in clinical trials or have characteristics that are unexpected, we may need to abandon their development or limit development to more narrow uses in which the side effects or other

characteristics are less prevalent, less severe or more acceptable from a risk- benefit perspective. The FDA or an IRB may also require that we suspend, discontinue, or limit our clinical trials based on safety information, or that we conduct additional animal or human studies regarding the safety and efficacy of our product candidates which we have not planned or anticipated. Such findings could further result in regulatory authorities failing to provide marketing authorization for our product candidates or limiting the scope of the approved indication, if approved. Many product candidates that initially showed promise in early-stage testing have later been found to cause side effects that prevented further development of the product candidate. Additionally, if one or more of our product candidates receives marketing approval, and we or others identify undesirable side effects caused by such products or the administration procedure, a number of potentially significant negative consequences could result, including: • regulatory authorities may withdraw approvals of such product; • regulatory authorities may require additional warnings on the labels; • we may be required to create a medication guide outlining the risks of such side effects for distribution to patients or other requirements subject to a REMS; • we could be sued and held liable for harm caused to patients; • we may not be able to obtain or maintain third- party payor coverage and adequate reimbursement; and • our reputation and physician or patient acceptance of our products may suffer. There can be no assurance that we will resolve any issues related to any product- related adverse events to the satisfaction of the FDA or foreign regulatory agency in a timely manner or at all. Moreover, any of these events could prevent us from achieving or maintaining market acceptance of the particular product candidate, if approved, and could significantly harm our business, results of operations and prospects. Some of the diseases we initially seek to treat have low prevalence and it may be difficult to identify and enroll patients with these diseases. 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 a sufficient number of patients. Patient enrollment, a significant factor in the timing of clinical trials, is affected by many factors, including the size and nature of the patient population and competition for patients with other trials. The rare genetic diseases which some of our product candidates are designed to target have low incidence and prevalence and may be difficult to diagnose. In particular, because we are focused on patients with specific genetic mutations, our ability to enroll eligible patients may be limited or enrollment may be slower than we anticipate. For example, we estimate that approximately 6, 600 people in the United States have FA and that approximately 80 % of these patients will develop the cardiac manifestation of FA, or FA cardiomyopathy, and accordingly it may be difficult for us to identify and timely recruit a sufficient number of eligible patients to conduct our clinical trials. While the patient population for LX2020, our program targeting PKP2- ACM, is significantly larger than FA, we may face challenges in identifying and recruiting eligible patients to conduct our clinical trial given competing clinical trials. Even for more prevalent conditions such as Alzheimer' s disease, it may be difficult to recruit patients to clinical trials due to the number of approved products, difficulty identifying patients with the specific genotype we are studying, and the number of clinical trials being conducted in this indication. Our 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, 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 severity of the disease under investigation; • the eligibility criteria for the trial in question; • the size of the patient population and process for identifying patients; • the perceived risks and benefits of the product candidate under study; • clinicians' and patients' perceptions as to the potential advantages of the product candidate being studied in relation to other available therapies, including any new drugs that may be approved for the diseases we are investigating; • the availability of competing commercially available therapies and other competing therapeutic product candidates' clinical trials; • the efforts to facilitate timely enrollment in clinical trials; • the risk that patients enrolled in clinical trials will drop out of the clinical trials before completion of their treatment; • 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. Our inability to enroll a sufficient number of patients for clinical trials would result in significant delays and could require us to abandon one or more clinical trials altogether. Enrollment delays in these clinical trials may result in increased development costs for our product candidates, which would cause the value of our company to decline and limit our ability to obtain additional financing. Furthermore, we rely on CROs and clinical trial sites to help ensure the proper and timely conduct of our clinical trials and we may have limited influence over their performance. For additional information, see the risk factor in this section under the heading " We intend to continue to rely on third parties to conduct a significant portion of our 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. " Furthermore, even if we are able to enroll a sufficient number of patients for our clinical trials, we may have difficulty maintaining enrollment of such patients in our clinical trials. We may seek Orphan Drug designation or Rare Pediatric Disease designation for some of our product candidates and we may be unsuccessful, or may be unable to maintain the benefits associated with Orphan Drug designation, including the potential for market exclusivity, for product candidates for which we obtain Orphan Drug designation. Regulatory authorities in some jurisdictions, including the United States, may designate drugs or biologics intended to treat relatively small patient populations as orphan drug products. Under the Orphan Drug Act, the FDA may designate a drug or biologic as an orphan drug if it is intended to treat a rare disease or condition, which is generally defined as a patient population of fewer than 200, 000 individuals in the United States, or a patient population of 200, 000 or more 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. In the United States, Orphan Drug designation entitles a party to potential financial incentives such as tax advantages and user fee waivers. Opportunities for grant funding toward clinical trial costs may also be available for clinical trials of drugs or biologics for rare diseases, regardless of whether the drugs or biologics are designated for the orphan use. In addition, if a drug or biologic with an Orphan Drug designation subsequently receives the

first marketing approval for the disease for which it has such designation, the product is entitled to a seven- year period of marketing exclusivity, which precludes the FDA from approving another marketing application for the same drug and disease for that time period, except in limited circumstances. If our competitors are able to obtain orphan drug exclusivity prior to us, for products that constitute the " same drug " and treat the same diseases as our product candidates, we may not be able to have competing products approved by the applicable regulatory authority for a significant period of time. Similarly, in the European Union, the European Commission, upon the recommendation of the EMA' s Committee for Orphan Medicinal Products, grants Orphan Drug designation to promote the development of drugs that are intended for the diagnosis, prevention or treatment of life- threatening or chronically debilitating conditions and either the prevalence of the condition is not more than 5 in 10, 000 persons in the European Union, or, without incentives, it is unlikely that sales of the drug in the European Union would be sufficient to justify the necessary investment in developing the drug. In each case, there must be no satisfactory method of diagnosis, prevention or treatment of the condition that has been authorized, or, if such a method exists, the product in question must be of significant benefit to those affected by such condition. In the European Union, Orphan Drug designation entitles a party to financial incentives such as reduction of fees or fee waivers. We have obtained from the FDA Orphan Drug designation for LX2006 for **the** treatment of FA **cardiomyopathy** and for LX2020 for the treatment of PKP2- ACM **. LX2006 and LX2020 have also received Orphan Medicinal Product designation from the European Commission** . We may seek orphan designation for some or all of our product candidates in orphan indications in which there is a medically plausible basis for the use of these product candidates. However, we may be unsuccessful in obtaining Orphan Drug designation and may be unable to maintain the benefits associated with such designations. Even if we obtain orphan drug exclusivity for any of our product candidates, that exclusivity may not effectively protect those product candidates from competition because different drugs can be approved for the same condition, and orphan drug exclusivity does not prevent the FDA from approving the same or a different drug in another indication. Even after an orphan drug is granted orphan exclusivity and approved, the FDA can subsequently approve a later application for the same drug for the same condition before the expiration of the seven- year exclusivity period if the FDA concludes that the later drug is clinically superior in that it is shown to be safer in a substantial portion of the target populations, more effective or makes a major contribution to patient care. On August 3, 2017, Congress passed the FDA Reauthorization Act of 2017, or FDARA. FDARA, among other things, codified the FDA' s preexisting regulatory interpretation, to require that a drug sponsor demonstrate the clinical superiority of an orphan drug that is otherwise the same as a previously approved drug for the same rare disease in order to receive orphan drug exclusivity. The statute supplants prior precedent holding that the Orphan Drug Act unambiguously requires that the FDA recognize the orphan exclusivity period regardless of a showing of clinical superiority. Moreover, in the Consolidated Appropriations Act of 2021, Congress clarified that the interpretation of orphan drug exclusivity codified in FDARA would apply in cases where the FDA issued an orphan designation before the enactment of FDARA but where product approval came after the enactment of FDARA. In addition, a designated Orphan Drug may not receive orphan drug exclusivity if it is approved for a use that is broader than the disease for which it received orphan designation. ~~On January 24~~ **In Catalyst Pharms.** , **Inc. v. Becerra, 14 F. 4th 1299 (11th Cir.** ~~2023~~ **2021)** , **the court disagreed with** the FDA **' s** ~~announced its intention to apply its existing regulations and long- standing~~ **longstanding position that** ~~approach to grant~~ orphan drug exclusivity ~~based on~~ **only applies to** the **approved use or indication within an eligible disease. This decision created uncertainty in the application of orphan drug exclusivity. In January 2023, the FDA published a notice in the Federal Register to clarify that while the agency complies with the court' s order in Catalyst, the FDA intends to continue to apply its longstanding interpretation of the regulations to matters outside of the scope of the Catalyst order – that is, the agency will continue tying the scope of orphan- drug exclusivity to the uses or** indications for which ~~the~~ **a** drug is approved ~~rather than granting~~ **, which permits** ~~the~~ **other** ~~exclusivity~~ **sponsors to obtain approval of a drug for new uses** ~~for~~ **or indications within** the ~~entire rare~~ **same orphan designated** disease or condition that **have not yet been approved** ~~was the subject of the orphan drug designation, in response to the U~~ . ~~S~~ **In view of the overturn of the Chevron doctrine in Loper Bright Enterprises v** . **Raimondo, this landmark Supreme** Court ~~of Appeals for the Eleventh Circuit' s September 30, 2021,~~ decision **may invite various stakeholders to bring lawsuits against the** ~~in Catalyst Pharms., Inc. v. Becerra. The~~ FDA ~~may~~ **to challenge longstanding decisions and policies, including regulatory exclusivities, which could lead to uncertainties in the industry.** ~~further~~ **Further** ~~reevaluate its~~ **, changes in the leadership of the FDA and other federal agencies under the Trump administration may lead to new policies and changes in the** regulations and ~~policies under~~ **operations of** the ~~Orphan Drug Act~~ **FDA, which may impact our clinical development plans** . We do not know if, when, or how the FDA, Congress, or future judicial challenges may change the orphan drug regulations and policies in the future, and it is uncertain how any changes might affect our business. Depending on what changes the FDA may make to its orphan drug regulations and policies, our business could be adversely impacted. Moreover, orphan drug- exclusive marketing rights in the United States may be lost if the FDA later determines that the request for designation was materially defective or if we are unable to manufacture sufficient quantities of the product to meet the needs of patients with the rare disease or condition. Orphan Drug designation neither shortens the development time or regulatory review time of a drug nor gives the drug any advantage in the regulatory review or approval process. Fast Track, Breakthrough Therapy, or Regenerative Medicine Advanced Therapy designation that we may receive from **the** FDA may not actually lead to a faster development or regulatory review or approval process, and does not assure FDA approval of our product candidates. We may seek Fast Track, Breakthrough Therapy or RMAT designation from the FDA for some or all of our product candidates, but we may be unable to obtain such designations or to maintain the benefits associated with such designations. The FDA' s Fast Track, Breakthrough Therapy, and RMAT designation programs are intended to expedite the development of certain qualifying product candidates intended for the treatment of serious diseases and conditions. If a product candidate is intended for the treatment of a serious or life- threatening condition and preclinical or clinical data demonstrate the product' s potential to address an unmet medical need for this condition, the sponsor may apply for FDA Fast Track designation. A product candidate may be

designated as a breakthrough therapy if it is intended, alone or in combination with one or more other drugs or biologics to treat a serious or life- threatening condition and preliminary clinical evidence indicates that the product candidate may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints. For product candidates that have been designated as breakthrough therapies, interaction and communication between the FDA and the sponsor of the trial can help to identify the most efficient path for clinical development while minimizing the number of patients placed in ineffective control regimens. Drugs and biologics designated as breakthrough therapies by the FDA may also be eligible for accelerated approval. A product candidate may receive RMAT designation if it is a regenerative medicine therapy that is intended to treat, modify, reverse or cure a serious or life- threatening disease or condition, and preliminary clinical evidence indicates that the product candidate has the potential to address an unmet medical need for such condition. RMAT designation allows companies developing regenerative medicine therapies to work more closely and frequently with the FDA, and RMAT- designated product candidates may be eligible for priority review and accelerated approval. FDA has confirmed that gene therapies, including genetically modified cells, that lead to a sustained effect on cells or tissues may meet the definition of a regenerative medicine therapy. For product candidates that have received an RMAT designation, interaction and communication between the FDA and the sponsor of the trial can help to identify the most efficient path for clinical development while minimizing the number of patients placed in ineffective control regimens. We have received Fast Track designation for ~~LX1001~~ **LX2006** for the treatment of **FA cardiomyopathy** ~~patients with early Alzheimer' s disease who are APOE4 homozygous~~ , ~~to slow disease progression~~ and ~~have received Fast Track designation~~ for LX2020 for the treatment of PKP2- ACM **. We have also received RMAT designation for LX2006 for the treatment of FA cardiomyopathy** . While we may seek Fast Track, Breakthrough Therapy and / or RMAT designation for some or all of our product candidates, there is no guarantee that we will be successful in obtaining any such designation. Even if we do obtain such designation, we may not experience a faster development process, review or approval compared to conventional FDA procedures. A Fast Track, Breakthrough Therapy, or RMAT designation does not ensure that the product candidate will receive marketing approval or that approval will be granted within any particular time frame. In addition, the FDA may withdraw Fast Track, Breakthrough Therapy, or RMAT designation if it believes that the designation is no longer supported by data from our clinical development program. Fast Track, Breakthrough Therapy and / or RMAT designation alone does not guarantee qualification for the FDA' s priority review procedures. We have received Rare Pediatric Disease designation from the FDA for LX2006 for the treatment of FA and we may seek such designation for future product candidates. However, a marketing application for these product candidates, if approved, may not meet the eligibility criteria for a rare pediatric disease priority review voucher. We have received Rare Pediatric Disease designation from the FDA for LX2006 for the treatment of FA and LX1004 for the treatment of CLN2 disease and we may seek Rare Pediatric Disease designation for future product candidates. The FDA defines " rare pediatric disease " as a (i) serious or life- threatening disease in which the serious or life- threatening manifestations primarily affect individuals aged from birth to 18 years, including age groups often called neonates, infants, children, and adolescents; and (ii) a rare disease or condition within the meaning of the Orphan Drug Act. Designation of a product candidate as a product for a rare pediatric disease does not guarantee that a marketing application for such product candidate will meet the eligibility criteria for a rare pediatric disease priority review voucher at the time the application is approved. Under the FDCA, we will need to request a rare pediatric disease priority review voucher in our original marketing application for our product candidates for which we have received Rare Pediatric Disease designation. The FDA may determine that a marketing application for any such product candidates, if approved, does not meet the eligibility criteria for a priority review voucher, including for the following reasons: • the rare pediatric disease that received such designation no longer meets the definition of a " rare pediatric disease "; • the marketing application contains an active ingredient (including any ester or salt of the active ingredient) that has been previously approved in a marketing application **;** • the marketing application is not deemed eligible for priority review **;** • the marketing application does not rely on clinical data derived from studies examining a pediatric population and dosages of the product intended for that population (that is, if the marketing application does not contain sufficient clinical data to allow for adequate labeling for use by the full range of affected pediatric patients); or • the marketing application is approved for a different adult indication than the rare pediatric disease for which our product candidates are designated. Under the current statutory sunset provisions, after September 30, 2024, the FDA may only award a priority review voucher, or PRV, for an approved rare pediatric disease product application if the sponsor has Rare Pediatric Disease designation for the drug or biologic that is the subject of such application, and that designation was granted by September 30, 2024. After September 30, 2026, the FDA may not award any rare pediatric disease **PRVs** ~~priority review vouchers~~ . However, it is possible the authority for FDA to award rare pediatric disease **PRVs** ~~priority review vouchers~~ will be further extended by Congress. As such, if we do not obtain approval of a marketing application for LX2006 in patients with FA on or before September 30, 2026, and if the **PRV** ~~priority review voucher~~ program is not extended by Congressional action, we may not receive a **PRV. Congress did not reauthorize the rare pediatric disease** priority review ~~voucher~~ **program at the end of 2024, and it is unclear whether the Congress under the Trump administration will extend the program** . Where appropriate, we may seek approval from the FDA, EMA or comparable foreign regulatory authorities through the use of accelerated approval pathways. If we are unable to obtain such approval, we may be required to conduct additional preclinical studies or clinical trials beyond those that we contemplate, which could increase the expense of obtaining, and delay the receipt of, necessary marketing approvals. Even if we receive accelerated approval from the FDA, EMA or comparable regulatory authorities, if our confirmatory trials do not verify clinical benefit, or if we do not comply with rigorous post- marketing requirements, the FDA, EMA or such other regulatory authorities may seek to withdraw accelerated approval. Where possible, we may pursue accelerated development strategies in areas of high medical need. We may seek an accelerated approval pathway for one or more of our therapeutic product candidates from the FDA, EMA or comparable foreign regulatory authorities. Under the accelerated approval provisions in the FDCA and the FDA' s implementing regulations, the FDA may grant accelerated approval to a therapeutic candidate that is designed to treat a serious or life- threatening condition, generally

provides a meaningful therapeutic benefit over available therapies, and demonstrates an effect on a surrogate endpoint that is reasonably likely to predict clinical benefit or on a clinical endpoint that can be measured earlier than irreversible morbidity or mortality, or IMM, that is reasonably likely to predict an effect on IMM or other clinical benefit. The FDA considers a clinical benefit to be a positive therapeutic effect that is clinically meaningful in the context of a given disease, such as IMM. For the purposes of accelerated approval, a surrogate endpoint is a marker, such as a laboratory measurement, radiographic image, physical sign, or other measure that is thought to predict clinical benefit, but is not itself a measure of clinical benefit. An intermediate clinical endpoint is a clinical endpoint that can be measured earlier than an effect on IMM that is reasonably likely to predict an effect on IMM or other clinical benefit. The accelerated approval pathway may be used in cases in which the advantage of a new product over available therapy may not be a direct therapeutic advantage, but is a clinically important improvement from a patient and public health perspective. If granted, accelerated approval is usually contingent on the sponsor' s agreement to conduct, in a diligent manner, additional post- approval confirmatory studies to verify and describe the product' s clinical benefit. If such post- approval studies fail to confirm the product' s clinical benefit, the FDA may withdraw its approval of the product. In addition, the FDA currently requires, unless otherwise informed by the agency, pre- approval of promotional materials for products receiving accelerated approval, which could adversely impact the timing of the commercial launch of the product. Prior to seeking accelerated approval, we would seek feedback from the FDA, EMA or comparable foreign regulatory authorities and would otherwise evaluate our ability to seek and receive such accelerated approval. **However,** ~~There~~ **there** can be no assurance that after our evaluation of the feedback and other factors we will decide to pursue or submit a BLA for accelerated approval or any other form of expedited development, review or approval **for LX2006 or any other product candidate** . Similarly, there can be no assurance that after subsequent feedback from the FDA, EMA or comparable foreign regulatory authorities, we will continue to pursue or apply for accelerated approval or any other form of expedited development, review or approval, even if we initially decide to do so. Furthermore, if we decide to submit an application for accelerated approval, there can be no assurance that such application will be accepted or that any approval will be granted on a timely basis, or at all. The FDA, EMA or other comparable foreign regulatory authorities could also require us to conduct further studies prior to considering our application or granting approval of any type, including, for example, if other products are approved via the accelerated pathway and subsequently converted by FDA to full approval. A failure to obtain accelerated approval or any other form of expedited development, review or approval for our therapeutic candidate would result in a longer time period to commercialization of such therapeutic candidate, could increase the cost of development of such therapeutic candidate and could harm our competitive position in the marketplace. Priority review designation by the FDA may not lead to a faster regulatory review or approval process and, in any event, does not assure FDA approval of our product candidates. If the FDA determines that a product candidate is intended to treat a serious disease or condition and, if approved, would provide a significant improvement in the safety or effectiveness of the treatment, prevention, or diagnosis of such disease or condition, the FDA may designate the product candidate for priority review. A priority review designation means that the goal for the FDA to review a marketing application is six months from filing of the application, rather than the standard review period of ten months. We may request priority review for certain of our product candidates. The FDA has broad discretion with respect to whether or not to grant priority review status to a product candidate, so even if we believe a particular product candidate is eligible for such designation or status, the FDA may disagree and decide not to grant it. Moreover, a priority review designation does not necessarily mean a faster regulatory review process or necessarily confer any advantage with respect to approval compared to conventional FDA procedures. Receiving priority review from the FDA does not guarantee approval within the six- month review cycle or thereafter. We may expend our limited resources to pursue a particular product candidate and fail to capitalize on product candidates that may be more profitable or for which there is a greater likelihood of success. Because we have limited financial and management resources, we must focus on development programs and product candidates that we identify for specific diseases. As such, currently we are primarily focused on the development of our current pipeline of product candidates. As a result, we may forego or delay pursuit of opportunities with other product candidates ~~. For example, we are evaluating strategic alternatives to find the appropriate partner to advance our LX1004 program~~ . 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 development programs and product candidates for specific diseases may not yield any commercially viable products. If we do not accurately evaluate the commercial potential or target market for a particular product candidate, we may relinquish valuable rights to that product candidate through collaboration, licensing or other royalty arrangements in cases in which it would have been more advantageous for us to retain sole development and commercialization rights to such product candidate. We may not be successful in our efforts to build a pipeline of additional product candidates. Our business model is centered on developing product candidates targeting patient populations that place significant burden on society and are most amenable to our genetic medicine approach. We are targeting diseases that have seen limited penetration of precision medicine and where we believe there is significant opportunity for gene therapy to play a role as a key therapeutic option. We aim to select, develop and advance product candidates that we believe will have a high probability of technical and regulatory success through development into commercialization. We may not be able to continue to identify and develop new product candidates. Even if we are successful in continuing to build our pipeline, the potential product candidates that we identify may not be suitable for clinical development. For example, they may be shown to have side effects or other characteristics that indicate that they are unlikely to be drugs that will receive marketing approval and achieve market acceptance. If we do not successfully develop and commercialize product candidates based upon our approach, we will not be able to obtain product revenue in future periods, which likely would result in significant harm to our financial position and adversely affect our stock price. If we are unable to successfully validate, develop and obtain regulatory approval for companion diagnostic tests for our product candidates that require or would commercially benefit from such tests, or experience significant delays in doing so, we may not realize the full commercial potential of these product candidates. In connection with the clinical development of our product candidates for

certain indications, we intend to work with collaborators to develop or obtain access to in vitro companion diagnostic tests to identify patient subsets within a disease category who may derive selective and meaningful benefit from our product candidates. To be successful, we or our collaborators will need to address a number of scientific, technical, regulatory and logistical challenges. The FDA and comparable foreign regulatory authorities regulate in vitro companion diagnostics as medical devices and, under that regulatory framework, will likely require the conduct of clinical trials to demonstrate the safety and effectiveness of any diagnostics we may develop, which we expect will require separate regulatory clearance or approval prior to commercialization. The FDA generally will require approval or clearance of the diagnostic at the same time that the FDA approves the therapeutic product if the FDA determines that safe and effective use of a therapeutic product depends on an in vitro companion diagnostic. The clearance or approval of a companion diagnostic as part of the product label will also limit the use of the product candidate to patients who have met the screening criteria tested for by the companion diagnostic. We intend to rely on third parties for the design, development and manufacture of companion diagnostic tests for our therapeutic product candidates that may require such tests. If we enter into such collaborative agreements, we will be dependent on the sustained cooperation and effort of our future collaborators in developing and obtaining approval for these companion diagnostics. It may be necessary to resolve issues such as selectivity / specificity, analytical validation, reproducibility, or clinical validation of companion diagnostics during the development and regulatory approval processes. Moreover, even if data from preclinical studies and early clinical trials appear to support development of a companion diagnostic for a product candidate, data generated in later clinical trials may fail to support the analytical and clinical validation of the companion diagnostic. We and our future collaborators may encounter difficulties in developing, obtaining regulatory approval for, manufacturing and commercializing companion diagnostics similar to those we face with respect to our therapeutic candidates themselves, including issues with achieving regulatory clearance or approval, production of sufficient quantities at commercial scale and with appropriate quality standards, and in gaining market acceptance. If we are unable to successfully develop companion diagnostics for these product candidates, or experience delays in doing so, the development of these product candidates may be adversely affected, these product candidates may not obtain marketing approval, and we may not realize the full commercial potential of any of these product candidates that obtain marketing approval. As a result, our business, results of operations and financial condition could be materially harmed. In addition, a diagnostic company with whom we contract may decide to discontinue selling or manufacturing the companion diagnostic test that we anticipate using in connection with development and commercialization of our product candidates or our relationship with such diagnostic company may otherwise terminate. We may not be able to enter into arrangements with another diagnostic company to obtain supplies of an alternative diagnostic test for use in connection with the development and commercialization of our product candidates or do so on commercially reasonable terms, which could adversely affect and / or delay the development or commercialization of our product candidates. Risks related to the manufacturing of our product candidates We and our contract manufacturers are subject to significant regulation with respect to manufacturing our products. The third- party manufacturing facilities on which we rely, 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 manufacturing of all components of our product candidates. However, if we experience slowdowns or problems with our manufacturing partners and are unable to establish or scale our internal manufacturing capabilities, we will need to continue to contract with manufacturers that can produce the preclinical, clinical and commercial supply of our products. Each supplier may require licenses to manufacture such components 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 in the United States and European Union 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 BLA or an MAA on a timely basis. Our potential manufacturing 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 product candidates or any of our other potential products. In addition, the regulatory authorities may, at any time, audit or inspect a manufacturing facility involved with the preparation of our product candidates or our other potential products 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 **contract manufacturing organizations, or** CMOs do not pass such audit or inspections. If these facilities do not pass a pre-approval plant inspection, FDA approval of the products will not be granted. The 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 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 an 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. Furthermore, if our suppliers fail to meet contractual requirements, and we are unable to secure one or more replacement suppliers capable of production at a substantially equivalent cost, our clinical trials may be delayed, or we could lose potential revenue. 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. We rely on third- party manufacturers to manufacture our product candidates for preclinical studies and clinical trials. The manufacturers of 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 CMOs to adhere to or document compliance to such regulatory requirements could lead to a delay or interruption in the availability of our product candidate materials for clinical trials or enforcement action from the FDA, EMA or foreign regulatory authorities. If we or our manufacturers fail to comply with the requirements of the FDA, EMA or other regulatory authority, sanctions could be 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. There can be no assurances that our third- party manufacturers will be able to meet our timetable and requirements. If any third party with whom we contract fails to perform its obligations, we may be forced to either manufacture the materials ourselves, for which we may not have the capabilities or resources, or enter into an agreement with a different third- party manufacturer, which we may not be able to do on reasonable terms, if at all. In either scenario, our clinical trials and future commercial supply could be delayed significantly as we establish alternative supply sources. In some cases, the technical skills required to manufacture our product candidates may be unique or proprietary to the original third- party manufacturer and we may have difficulty, or there may be contractual restrictions prohibiting us from, transferring such skills to a back- up or alternate supplier, or we may be unable to transfer such skills at all. In addition, if we are required to change manufacturers for any reason, we will be required to verify that the new manufacturer maintains facilities and procedures that comply with quality standards and with all applicable regulations. We will also need to verify, such as through a manufacturing comparability study, that any new manufacturing process will produce our product candidates according to the specifications previously submitted to the FDA or another regulatory authority. The delays associated with the verification of a new manufacturer could negatively affect our ability to develop product candidates or commercialize our products, if approved, in a timely manner or within budget. If we are unable to arrange for alternative third- party manufacturing sources on commercially reasonable terms or in a timely manner, we may be delayed in the development of our product candidates. Our 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. Our product candidates require processing steps that are more complex than those required for most chemical pharmaceuticals. Moreover, unlike chemical pharmaceuticals, the physical and chemical properties of a biologic such as our modified virus generally cannot be fully characterized. As a result, assays of the finished product may not be sufficient to ensure that the product will perform in the intended manner. 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, cell types and reagents, and other production constraints. Our production process also requires a number of highly specific raw materials, cell types and reagents with limited suppliers. Even though we aim to have backup supplies of raw materials, cell types and reagents whenever possible, we cannot be certain they will be sufficient if our primary sources are unavailable. 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 our inability to obtain suitable raw materials for our lead product candidates. 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. In addition, if any of our product candidates obtain approval, the FDA, EMA and other regulatory authorities may require us to submit samples of any lot of any approved product together with the protocols showing the results of applicable tests at any time. Under some circumstances, the FDA, EMA or other regulatory authorities may require that we not distribute a lot until the agency authorizes its release. Slight deviations in the manufacturing process, including those affecting quality attributes and stability, may result in unacceptable changes in the product that could result in lot failures or product recalls. Lot failures or product recalls could cause us to delay product launches or clinical trials, which could be costly to us and otherwise harm our business, financial condition, results of operations and prospects. 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 the 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 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 may get lower priority than our competitors that are larger than we are. We cannot be certain that our suppliers will continue to provide us with the quantities of these raw materials within the timelines that we require or satisfy our anticipated specifications and quality requirements. 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. Any contamination or interruption in our manufacturing process, shortages of raw materials or failure of our suppliers of viruses 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 occurring during the manufacturing process. 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. 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 FDA approval. This could delay the initiation and 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. Regulatory agencies, and in particular the FDA and EMA, have demonstrated increased caution in their regulation of gene therapies, including increased scrutiny related to chemistry, manufacturing and control, or CMC, issues. This increased regulatory scrutiny around gene therapy CMC may result in us being required to conduct additional preclinical studies or clinical trials with respect to any of our product candidates, which may result in delays and increased costs in the development or commercialization of our product candidates and ultimately could lead to the failure to obtain approval for any gene therapy product. Risks related to the commercialization of our product candidates Even if any of our product candidates receive marketing approval, they may fail to achieve the degree of market acceptance by physicians, patients, third- party payors and others in the medical community necessary for commercial success. If any of our product candidates receive marketing approval, they may nonetheless fail to gain sufficient market acceptance by physicians, patients, third- party payors and others in the medical community. If our product candidates do not achieve an adequate level of acceptance, we may not generate significant revenue and we may not become profitable. The degree of market acceptance of our product candidates, if approved for commercial sale, will depend on a number of factors, including: • their efficacy, safety and potential advantages compared to alternative treatments; • our ability to offer our products for sale at competitive prices; • their convenience and ease of administration compared to alternative treatments; • product labeling or product insert requirements of the FDA or foreign regulatory authorities, including any limitations or warnings contained in a product' s approved labeling, including any boxed warning or REMS; • the willingness of the target patient population to try new treatments, such as gene therapy as a novel modality for treatment of our target indications and of physicians to prescribe these treatments; • our ability to hire and retain a sales force in the United States; • the strength of marketing and distribution support; • the availability of coverage and adequate reimbursement for our product candidates, once approved, from third- party payors and government authorities; • the prevalence and severity of any side effects; and • any restrictions on the use of our products together with other medications. Negative public opinion of gene therapy and increased regulatory scrutiny of gene therapy and genetic research may adversely impact the development or commercial success of our current and future product candidates. Our product candidates involve introducing genetic material into a patient' s cells via intrathecal and intravenous administration. The clinical and commercial success of our potential products will depend in part on public acceptance of the use of gene therapy and gene regulation for the prevention or treatment of human diseases. Public attitudes may be influenced by claims that gene therapy and gene regulation are unsafe, unethical or immoral, and consequently, our products may not gain the acceptance of the public or the medical community. Adverse public attitudes may adversely impact our ability to enroll clinical trials. Moreover, our success will depend upon physicians prescribing, and their patients being willing to receive, treatments that involve the use of product candidates we may develop in lieu of, or in addition to, existing treatments with which they are already familiar and for which greater clinical data may be available. More restrictive government regulations or negative public opinion would have a negative effect on our business or financial condition and may delay or impair the development and commercialization of our product candidates or demand for any products once approved. In recent years, sponsors of other clinical trials involving gene therapies have announced imposition of clinical holds by the FDA to evaluate safety issues arising during the trials. Among the risks in any gene therapy product based on viral vectors are the risks of immunogenicity, elevated liver enzymes and insertional oncogenesis.

If any of our vectors demonstrate a similar effect, we may decide or be required to halt or delay further clinical development of any product candidates that utilize that vector. Adverse events in our or others' clinical trials, even if not ultimately attributable to our product candidates, and the resulting publicity could result in increased governmental regulation, unfavorable public perception, potential regulatory delays in the testing or approval of our product candidates, stricter labeling requirements for those product candidates that are approved and a decrease in demand for any such product candidates. The risk of cancer remains a concern for gene therapy, and we cannot assure that it will not occur in any of our planned or future clinical trials or in any clinical trials conducted by other companies. In addition, there is the potential risk of delayed adverse events following exposure to gene therapy products due to persistent biological activity of the genetic material or other components of products used to carry the genetic material. In addition, for our regulated gene replacement therapy product candidates which require that the expression of a therapeutic transgene be tightly regulated, we may inadvertently cause overexpression, which could lead to numerous issues, including safety and toxicity concerns. Furthermore, one of our regulatory gene replacement therapy candidates, LX1020, requires the insertion of miRNA targets into the viral genome, which is a technology that to our knowledge is not present in any approved gene therapy products. If any such adverse events occur, commercialization of our product candidates or further advancement of our clinical trials could be halted or delayed, which would have a negative impact on our business and operations. The affected populations for our other product candidates may be smaller than we or third parties currently project, which may affect the addressable markets for our product candidates. We currently focus our research and product development on several indications that are larger- rare diseases. However, our projections of the number of people who have the diseases we are seeking to treat, as well as the subset of people with these diseases who have the potential to benefit from treatment with our product candidates, are estimates based on our knowledge and understanding of these diseases. These estimates may prove to be incorrect and new studies may further reduce the estimated incidence or prevalence of this disease. The number of patients in the United States, the European Union and elsewhere may turn out to be lower than expected, may not be otherwise amenable to treatment with our product candidate or patients may become increasingly difficult to identify and access, all of which would adversely affect our business, financial condition, results of operations and prospects. The total addressable market opportunity for our product candidates will ultimately depend upon a number of factors, including the diagnosis and treatment criteria included in the final label, if approved for sale in specified indications, acceptance by the medical community, patient access and product pricing and reimbursement. Incidence and prevalence estimates are frequently based on information and assumptions that are not exact and may not be accurate, and the methodology is forward- looking and potentially speculative. The process we have used in developing an estimated incidence and prevalence range for the indications we are targeting has involved collating limited data from multiple sources. Accordingly, the incidence and prevalence estimates included in this Annual Report on Form 10- K should be viewed in that context. Further, the data and statistical information used in this Annual Report, including estimates derived from them, may differ from information and estimates made by our competitors or from current or future studies conducted by independent sources. We face significant competition from other biotechnology and pharmaceutical companies, and our operating results will suffer if we fail to compete effectively. Drug development, particularly in the gene therapy field, is highly competitive and subject to rapid and significant technological advancements. As a significant unmet medical need exists in the cardiovascular disease ~~and Alzheimer's disease areas~~ **area**, there are several large and small pharmaceutical companies focused on delivering therapeutics for the treatment of these diseases, including those that we are initially targeting. It is likely that additional drugs will become available in the future for the treatment of our target diseases. We are aware that our competitors are developing product candidates for the treatment of diseases that our product candidates will target. With respect to LX2006, we are aware of ~~preclinical~~ --- **clinical stage** gene therapy programs in development at **Astellas Pharma Inc. and** Solid Biosciences Inc. ~~and Lacerta Therapeutics~~, ~~Inc.~~ and those being developed in collaborations between Voyager Therapeutics, Inc. and Neurocrine Biosciences, Inc. Additionally, we are aware that Prime Medicine, Inc. and Tune Therapeutics, Inc. have early- stage gene editing discovery efforts. Among other treatment modalities for FA, we are aware that Larimar Therapeutics, Inc. is developing a clinical stage product candidate, CTI-1601, that Design Therapeutics, Inc. is developing a ~~clinical stage~~ product candidate, DT- ~~216~~ **216P2**, and that **PTC Therapeutics, Inc. has submitted a new drug application for vatiquinone to the FDA.** Reata Pharmaceuticals, Inc.' s omaveloxolone (Skyclarys) was approved by the FDA in 2023 **, and** ~~. In 2023~~ Biogen Inc. acquired Reata Pharmaceuticals, Inc. for approximately $ 7. 3 billion **in the same year** and is currently commercializing Skyclarys. With respect to LX2020, both Rocket and Tenaya Therapeutics Inc. are developing an AAV- based gene therapy candidate designed to deliver a functional PKP2 gene to patients with PKP2- ACM ~~. With respect to our portfolio of gene therapy programs for the treatment of homozygous APOE4- associated Alzheimer' s disease, we are aware that uniQure, N. V. is pursuing AMT- 240, a preclinical gene therapy candidate for autosomal dominant Alzheimer' s disease intended to silence the APOE4 variant while expressing the a protective variant, and Novartis has a gene therapy candidate for Alzheimer' s disease that is in the early preclinical stages of development. Many large and small pharmaceutical companies and academic institutions are developing potential treatments for the condition given the significant unmet need and the large population suffering from Alzheimer' s disease. There are multiple FDA- approved treatments for Alzheimer' s disease, including donepezil (Aricept), memantine (Namenda), and in January of 2023, lecanemab was granted accelerated approval by the FDA for the treatment of Alzheimer' s disease based on the observed reduction of amyloid beta plaque and was granted full approval by the FDA in June 2023. In addition, Eli Lilly and Company' s product candidate for the treatment of Alzheimer' s disease, donanemab, has completed a Phase 3 clinical trial. Finally, we are aware that Voyager Therapeutics, Inc. is pursuing Alzheimer' s disease treatments and have early- stage discovery efforts ongoing based on vectorized antibodies~~. Many of our existing or potential competitors have substantially greater financial, technical and human resources than we do and significantly greater experience in the discovery and development of product candidates, as well as in obtaining regulatory approvals of those product candidates in the United States and in foreign countries. Our current and potential future competitors may also have significantly more experience

commercializing drugs, particularly gene therapy and other biologics, that have been approved for marketing. Mergers and acquisitions in the pharmaceutical and biotechnology industries could result in even more resources being concentrated among a small number of our competitors. We will face competition from other drugs or from other non- drug products currently approved or that will be approved in the future in the cardiac and neurology fields, including for the treatment of diseases and diseases in the therapeutic categories we intend to target. Therefore, our ability to compete successfully will depend largely on our ability to: • develop and commercialize drugs that are advantageous as compared to other products in the market; • demonstrate through our clinical trials that our product candidates are differentiated from existing and future therapies; • attract qualified scientific, product development and commercial personnel; • obtain patent or other proprietary protection for our product candidates; • obtain required regulatory approvals; • obtain coverage and adequate reimbursement from, and negotiate competitive pricing with, third- party payors; and • successfully collaborate with other pharmaceutical companies in the discovery, development and commercialization of new medicines. The availability of our competitors' products could limit the demand, and the price we are able to charge, for any product candidate we develop. The inability to compete with existing or subsequently introduced drugs would have an adverse impact on our business, financial condition and prospects. In addition, the reimbursement structure of approved gene therapies by other companies could impact the anticipated reimbursement structure of our gene therapies, if approved, and our business, financial condition, results of operations and prospects. Established pharmaceutical companies may invest heavily to accelerate discovery and development of novel compounds or to in- license novel compounds that could make our product candidates less competitive. In addition, any new product that competes with an approved product must demonstrate compelling advantages in efficacy, convenience, tolerability and safety in order to overcome price competition and to be commercially successful. Accordingly, our competitors may succeed in obtaining patent protection, discovering, developing, receiving regulatory and marketing approval for, or commercializing, drugs before we do, which would have an adverse impact on our business and results of operations. Any product candidates for which we intend to seek approval as biologic products may face competition sooner than anticipated. The ACA includes a subtitle called the Biologics Price Competition and Innovation Act of 2009, or BPCIA, which created an abbreviated approval pathway for biologics that are biosimilar to or interchangeable with an FDA- licensed reference biological product. Under the BPCIA, an application for a biosimilar product may not be submitted to the FDA until four years following the date that the reference product was first licensed 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 licensed. 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. The law is complex and is still being interpreted and implemented by the FDA. As a result, its ultimate impact, implementation and meaning are subject to uncertainty. While it is uncertain when such processes intended to implement BPCIA may be fully adopted by the FDA, any such processes could have an adverse effect on the future commercial prospects for our biologics. There is a risk that any of our product candidates approved as a biological product under a BLA would not qualify for the 12- year period of exclusivity or that this exclusivity could be shortened due to congressional action or otherwise, or that the FDA will not consider our product candidates to be reference products for competing products, potentially creating the opportunity for generic 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 one of our reference products in a way that is similar to traditional generic substitution for non- biologics is not yet clear, and will depend on a number of marketplace and regulatory factors that are still developing. If competitors are able to obtain marketing approval for biosimilars referencing our product candidates, if approved, our products may become subject to competition from such biosimilars, with the attendant competitive pressure and potential adverse consequences. The success of our product candidates will depend significantly on coverage and adequate reimbursement or the willingness of patients, commercial and government payors to pay for these procedures. We believe our success depends on obtaining and maintaining coverage and adequate reimbursement for our product candidates, including LX2006 ~~, LX1001~~ and LX2020, and the extent to which patients will be willing to pay out- of- pocket for such products, in the absence of reimbursement for all or part of the cost. In the United States and in other countries, patients who are provided medical treatment for their conditions generally rely on third- party payors to reimburse all or part of the costs associated with their treatment. The availability of coverage and adequacy of reimbursement for our products by third- party payors, including government health care programs (e. g., Medicare, Medicaid, TRICARE), managed care providers, private health insurers, health maintenance organizations and other organizations is essential for most patients to be able to afford medical services and pharmaceutical products such as our product candidates. Third- party payors often rely upon Medicare coverage policy and payment limitations in setting their own coverage and reimbursement policies. However, decisions regarding the extent of coverage and amount of reimbursement to be provided are made on a payor- by- payor basis. One payor' s determination to provide coverage for a drug product does not assure that other payors will also provide coverage, and adequate reimbursement. For more information, see the section titled " Business – Government Regulation – Coverage and Reimbursement. " The principal decisions about reimbursement for new medicines are typically made by CMS, an agency within HHS. CMS decides whether and to what extent products will be covered and reimbursed under Medicare and private payors tend to follow CMS to a substantial degree **. Most significantly, in August 2022, the Inflation Reduction Act of 2022, or IRA, was signed into law. Among other things, the IRA requires manufacturers of certain drugs to engage in price negotiations with Medicare, with prices that can be negotiated subject to a cap (with resulting prices for the initial ten drugs first effective in 2026); imposes rebates under Medicare Part B and Medicare Part D to penalize price increases that outpace inflation (first due in 2023); redesigns the Medicare Part D benefit (beginning in 2024); and replaces the Part D coverage gap discount program with a new discounting program (beginning in 2025). The IRA permits the Secretary of the HHS to implement**

many of these provisions through guidance, as opposed to regulation, for the initial years. On August 29, 2023, HHS announced the list of the first ten drugs that will be subject to price negotiations. HHS has issued and will continue to issue and update guidance implementing the IRA, although the Medicare drug price negotiation program is currently subject to legal challenges. While the impact of the IRA on the pharmaceutical industry cannot yet be fully determined, it is likely to be significant. Only high- expenditure single- source drugs that have been approved for at least 7 years (11 years for single- source biologics) can qualify for negotiation, with the negotiated price taking effect two years after the selection year. For 2026, the first year in which negotiated prices become effective, CMS selected 10 high- cost Medicare Part D drugs in 2023, negotiations began in 2024, and the negotiated maximum fair price for each drug has been announced. CMS has selected 15 additional Medicare Part D drugs for negotiated maximum fair pricing in 2027. For 2028, up to an additional 15 drugs, which may be covered under either Medicare Part B or Part D, will be selected, and for 2029 and subsequent years, up to 20 additional Part B or Part D drugs will be selected . Third- party payors determine which products and procedures they will cover and establish reimbursement levels. Even if a third- party payor covers a particular product or procedure, the resulting reimbursement payment rates may not be adequate. Patients who are treated in-office for a medical condition generally rely on third- party payors to reimburse all or part of the costs associated with the procedure, including costs associated with products used during the procedure, and may be unwilling to undergo such procedures in the absence of such coverage and adequate reimbursement. Physicians may be unlikely to offer procedures for such treatment if they are not covered by insurance and may be unlikely to purchase and use our product candidates, if approved, for our stated diseases unless coverage is provided and reimbursement is adequate. In addition, for products administered under the supervision of a physician, obtaining coverage and adequate reimbursement may be particularly difficult because of the higher prices often associated with such drugs. Further, coverage policies and third- party reimbursement rates may change at any time. Therefore, even if favorable coverage and reimbursement status is attained, less favorable coverage policies and reimbursement rates may be implemented in the future. In addition, companion diagnostic tests require coverage and reimbursement separate and apart from the coverage and reimbursement for their companion pharmaceutical or biological products. Similar challenges to obtaining coverage and reimbursement, applicable to pharmaceutical or biological products, will apply to companion diagnostics. Reimbursement by a third- party payor may depend upon a number of factors, including the third- party payor' s determination that a procedure is a covered benefit under its health plan; safe, effective and medically necessary; appropriate for the specific patient; cost- effective; supported by peer- reviewed medical journals; included in clinical practice guidelines; and neither cosmetic, experimental, nor investigational. Further, increasing efforts by third- party payors in the United States and abroad to cap or reduce healthcare costs may cause such organizations to limit both coverage and the level of reimbursement for newly approved products and, as a result, they may not cover or provide adequate payment for our product candidates. In order to secure coverage and reimbursement for any product that might be approved for sale, we may need to conduct expensive pharmacoeconomic studies in order to demonstrate the medical necessity and cost- effectiveness of our products, in addition to the costs required to obtain FDA or comparable regulatory approvals. Additionally, we may also need to provide discounts to purchasers, private health plans or government healthcare programs. Our product candidates may nonetheless not be considered medically necessary or cost- effective. If third- party payors do not consider a product to be cost-effective compared to other available therapies, they may not cover the product after approval as a benefit under their plans or, if they do, the level of payment may not be sufficient to allow a company to sell its products at a profit. We expect to experience pricing pressures from third- party payors in connection with the potential sale of any of our product candidates. Foreign governments also have their own healthcare reimbursement systems, which vary significantly by country and region, and we cannot be sure that coverage and adequate reimbursement will be made available with respect to the treatments in which our products are used under any foreign reimbursement system. There can be no assurance that LX2006, ~~LX1001,~~ LX2020 or any other product candidates, if approved for sale in the United States or in other countries, will be considered medically reasonable and necessary, that it will be considered cost- effective by third- party payors, that coverage or an adequate level of reimbursement will be available or that reimbursement policies and practices in the United States and in foreign countries where our products are sold will not adversely affect our ability to sell our product candidates profitably, if they are approved for sale. Product liability lawsuits against us could cause us to incur substantial liabilities and to limit commercialization of any products that we may develop. We face an inherent risk of product liability exposure related to the testing of our product candidates in human clinical trials and will face an even greater risk if we commercially sell any products that we may develop. If we cannot successfully defend ourselves against claims that our product candidates or drugs caused injuries, we will incur substantial liabilities. Regardless of merit or eventual outcome, liability claims may result in: • decreased demand for any product candidates or drugs that we may develop; • injury to our reputation and significant negative media attention; • withdrawal of clinical trial participants; • significant costs to defend the related litigation; • substantial monetary awards paid to trial participants or patients; • loss of revenue; • reduced resources of our management to pursue our business strategy; and • the inability to commercialize any products that we may develop. Although we maintain product liability insurance coverage, such insurance may not be adequate to cover all liabilities that we may incur. We may need to increase our insurance coverage as we expand our clinical trials or if we commence commercialization of our product candidates. Insurance coverage is increasingly expensive. We may not be able to maintain insurance coverage at a reasonable cost or in an amount adequate to satisfy any liability that may arise. Risks related to our dependence on third parties Currently, we rely on our collaborations with Cornell University and **The Regents of** UCSD to conduct research and development for many of our pipeline programs, including conducting preclinical and IND- enabling studies for portions of our near- term future pipeline. Failure or delay of Cornell University or **The Regents of** UCSD to fulfill all or part of their respective obligations to us under our agreements, a breakdown in collaboration between the parties or a complete or partial loss of either of these relationships could materially harm our business. Our collaboration with Cornell University is critical to our business and in May 2020, we entered into two separate

license agreements with Cornell University ~~for preclinical research and development collaborations and non~~ **pursuant to which we obtained rights under certain patents, know** ~~-~~ ~~exclusive license rights~~ **how and data** to **exploit** ~~patents for certain~~ products and technologies **covered by such intellectual property** . As part of our first license agreement, as amended, we assumed oversight for the conduct of the Phase 1 / 2 clinical trial of LX1001 that was initiated by Cornell University at the end of 2019. **As part of our second license agreement with Cornell University, as amended, we obtained certain rights to portfolios for infantile neuronal ceroid lipofuscinosis type 2 (also called CLN2 Batten disease) and FA cardiomyopathy, as well as an assignment of Cornell University' s IND to support the development of our LX1004 program. In February 2021, we further expanded our collaboration and entered into a research collaboration agreement with Cornell University to conduct preclinical research to develop the licensed technology, which expired pursuant to its terms in February 2024. We entered into a third license agreement with Cornell University in April 2024 pursuant to which we obtained certain rights for FA cardiomyopathy, including rights to current and future clinical data from an ongoing Cornell University investigator- initiated Phase 1A trial of a gene therapy candidate AAVrh10. hFXN, known as LX2006 at Lexeo.** Pursuant to these license agreements, we are obligated to diligently proceed with the development, manufacture, and sale of licensed products. If Cornell University delays or fails to perform its obligations under the license agreements ~~, disagrees with our interpretation of their terms, or~~ terminates any of the license agreements **in accordance with its terms, or a dispute otherwise arises between the parties concerning the terms of the agreements or our respective rights to licensed technology** , our pipeline of product candidates would be significantly adversely affected and our prospects may be materially harmed. Our collaboration with UCSD is also highly important to our business, as we have licensed from UCSD intellectual property rights related to our LX2020, LX2021 and LX2022 programs under three separate license agreements, and we have entered into sponsored research agreements with UCSD for preclinical research and development for these programs. If **The Regents of** UCSD delays or fails to perform its obligations under either of the sponsored research agreements **or any of the license agreements** , disagrees with our interpretation of the terms of the sponsored research ~~agreement~~ **agreements , license agreements** or our discovery ~~plan~~ **plans ,** or terminates any of our existing license agreements, our pipeline of product candidates would be significantly adversely affected and our prospects may be materially harmed. We engage CROs to help conduct our ongoing clinical trials. We expect to continue to rely on third parties, including clinical data management organizations, medical institutions and clinical investigators, to conduct those clinical trials and any future 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 to do so on commercially reasonable terms, if at all. Switching or adding a CRO 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 our CROs, there can be no assurance that we will not encounter challenges or delays in the future or that these delays or challenges will not have a material adverse impact on our business, financial condition and prospects. 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 they 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 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. We rely on these parties for execution of our preclinical studies and clinical trials, and generally do not control their activities. Our reliance on these third parties for research and development activities will reduce our control over these activities but will not relieve us of our responsibilities. 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 cGCP regulations, 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, www. clinicaltrials. gov, within specified time frames. 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 cGCPs, or experience material protocol deviations, 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. If **the** FDA or any regulatory authority determines that our clinical data are not reliable for any reason, or if we encounter any data integrity issues, **the** FDA or other regulatory authorities may require us to exclude such data, which may cause the trial to be underpowered and fail to meet the trial endpoints. 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 cGCP 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 LX2006 ~~, LX1001~~ , LX2020 or any other product candidates. We also expect to rely on other third parties to store and distribute product supplies for our clinical trials. Any performance failure on the part of our distributors could delay clinical development or marketing approval of our product candidates or commercialization of our products, producing additional losses and depriving us of potential revenue. We may seek collaborations with non-academic third parties for the development or commercialization of our product candidates. If those collaborations are not successful, we may not be able to capitalize on the market potential of these product candidates. We may seek third- party collaborators for the development and commercialization of our product candidates, including for the commercialization of any of our product candidates that are approved for marketing outside the United States. Our likely collaborators for any such arrangements include regional and national pharmaceutical companies and biotechnology companies. If we enter into any additional such arrangements with any third parties, we will likely have limited control over the amount and timing of resources that our collaborators dedicate to the development or commercialization of our product candidates. Our ability to generate revenue from these arrangements will depend on our collaborators' abilities to successfully perform the functions assigned to them in these arrangements. Collaborations involving our product candidates pose the following risks to us: • collaborators have significant discretion in determining the efforts and resources that they will apply to these collaborations; • collaborators may not perform their obligations as expected; • collaborators may not pursue development and commercialization of any product candidates that achieve regulatory approval or may elect not to continue or renew development or commercialization programs based on clinical trial results, changes in the collaborators' strategic focus or available funding, or external factors, such as an acquisition, that divert resources or create competing priorities; • collaborators may delay clinical trials, provide insufficient funding for a clinical trial program, stop a clinical trial or abandon a product candidate, repeat or conduct new clinical trials or require a new formulation of a product candidate for clinical testing; • collaborators could independently develop, or develop with third parties, products that compete directly or indirectly with our product candidates if the collaborators believe that competitive products are more likely to be successfully developed or can be commercialized under terms that are more economically attractive than ours; • we could grant exclusive rights to our collaborators that would prevent us from collaborating with others; • product candidates discovered in collaboration with us may be viewed by our collaborators as competitive with their own product candidates or drugs, which may cause collaborators to cease to devote resources to the commercialization of our product candidates; • a collaborator with marketing and distribution rights to one or more of our product candidates that achieve regulatory approval may not commit sufficient resources to the marketing and distribution of such products; • disagreements with collaborators, including disagreements over proprietary rights, contract interpretation or the preferred course of development, might cause delays or termination of the research, development or commercialization of product candidates, might lead to additional responsibilities for us with respect to product candidates, or might result in litigation or arbitration, any of which would be time- consuming and expensive; • collaborators may not properly maintain or defend our or their intellectual property rights or may use our or their proprietary information in such a way as to invite litigation that could jeopardize or invalidate such intellectual property or proprietary information or expose us to potential litigation; • collaborators may infringe the intellectual property rights of third parties, which may expose us to litigation and potential liability; and • collaborations may be terminated for the convenience of the collaborator and, if terminated, we could be required to raise additional capital to pursue further development or commercialization of the applicable product candidates. Collaboration agreements may not lead to development or commercialization of product candidates in the most efficient manner or at all. If any future collaborator of ours were to be involved in a business combination, the continued pursuit and emphasis on our product development or commercialization program could be delayed, diminished or terminated. We face significant competition in seeking appropriate collaborators. Whether we reach a definitive agreement for any collaboration will depend, among other things, upon our assessment of the collaborator' s resources and expertise, the terms and conditions of the proposed collaboration and the proposed collaborator' s evaluation of a number of factors. Those factors may include the design or results of clinical trials, the likelihood of approval by the FDA or similar regulatory authorities outside the United States, the potential market for the subject product candidate, the costs and complexities of manufacturing and delivering such product candidate to patients, the potential of competing products, the existence of uncertainty with respect to our ownership of technology, which can exist if there is a challenge to such ownership without regard to the merits of the challenge and industry and market conditions generally. The collaborator may also consider alternative product candidates or technologies for similar diseases that may be available to collaborate on and whether such a collaboration could be more attractive than the one with us for our product candidate. Collaborations are complex and time- consuming to negotiate and document. In addition, there have been a significant number of recent business combinations among large pharmaceutical companies that have resulted in a reduced number of potential future collaborators. We may not be able to negotiate additional collaborations on a timely basis, on acceptable terms, or at all. If we are unable to do so, we may have to curtail the development of such product candidate, reduce or delay its development program or one or more of our other development programs, delay its potential commercialization or reduce the scope of any sales or marketing activities, or increase our expenditures and undertake development or commercialization activities at our own expense. If we elect to increase our expenditures to fund development or commercialization activities on our own, we may need to obtain additional capital, which may not be available to us on acceptable terms or at all. If we do not have sufficient funds, we may not be able to further develop our product candidates or bring them to market and generate revenue. Risks related to intellectual property If we are unable to obtain or protect intellectual property rights related to any of our product candidates, we may not be able to compete effectively in our market. We rely upon a combination of patents, trade secret protection and confidentiality agreements to protect the intellectual property related to our product candidates, including LX2006, LX2020, LX2021, LX2022, LX1001, LX1020, LX1021, and other programs, their respective components, formulations, therapies, methods used to manufacture them and methods **of** treatment ~~. Furthermore, we currently do not have any patents or patent~~

~~applications covering our LX1004 product candidate~~ . Our success depends in large part on our ability to obtain and maintain patent and other intellectual property protection in the United States and in other countries with respect to our proprietary technology and product candidates. We cannot offer any assurances about which of our patent applications will issue, the breadth of any resulting patent or whether any of the issued patents will be found invalid and unenforceable or will be threatened by third parties. We cannot offer any assurances that the breadth of our granted patents will be sufficient to stop a competitor from developing and commercializing a product, including a biosimilar product that would be competitive with one or more of our product candidates. Furthermore, any successful challenge to these patents or any other patents owned by or licensed to us after patent issuance could deprive us of rights necessary for the successful commercialization of any of our product candidates. Further, if we encounter delays in regulatory approvals, the period of time during which we could market a product candidate under patent protection could be reduced. The patent prosecution process is expensive and time- consuming. We may not be able to prepare, file and prosecute all necessary or desirable patent applications at a commercially reasonable cost or in a timely manner or in all jurisdictions. It is also possible that we may fail to identify patentable aspects of inventions made in the course of development and commercialization activities before it is too late to obtain patent protection on them. We may not be able to obtain or maintain patent applications and patents due to the subject matter claimed in such patent applications and patents being in the public domain. In some cases, the work of certain academic researchers in the gene therapy field has entered the public domain, which may preclude our ability to obtain patent protection for certain inventions relating to such work. Although we enter into nondisclosure and confidentiality agreements with parties who have access to confidential or patentable aspects of our research and development output, such as our employees, corporate collaborators, outside scientific collaborators, contract manufacturers, consultants, advisors and other third parties, any of these parties may breach these agreements and disclose such output before a patent application is filed, thereby jeopardizing our ability to seek patent protection. Consequently, we would not be able to prevent any third party from using any technology that is in the public domain to compete with our product candidates **. In addition, as the licensee of patents from Cornell University and UCSD, we have less control over the prosecution and enforcement of those patents than we would if we owned the patents. While we do have typical rights with respect to those patents under university license agreements, our ability to enforce those patents to maintain exclusivity in our markets may be limited by the terms of our agreements with Cornell University and UCSD. In addition, to the extent the federal government provided research funding to the licensor university for any technology licensed under the license agreements, then if the federal government elects to exercise any of its overriding rights which may apply as a result of provision of such funding, we may be subject to changes in market exclusivity or other material business requirements or constraints** . Moreover, depending on the terms of any future in- licenses to which we may become a party, we may not have the right to control the preparation, filing and prosecution of patent applications, or to maintain the patents, covering technology in- licensed from third parties. Therefore, these patents and patent applications may not be prosecuted and enforced in a manner consistent with the best interests of our business. The patent position of biotechnology and pharmaceutical companies generally is highly uncertain, involves complex legal and factual questions and has, in recent years, been the subject of much litigation. As a result, the issuance, scope, validity, enforceability and commercial value of any patent rights are highly uncertain. Our owned and licensed pending and future patent applications may not result in issued patents which protect our technology or product candidates, effectively prevent others from commercializing competitive technologies and product candidates or otherwise provide any competitive advantage. In fact, patent applications may not issue as patents at all. Even if patent applications we license or own currently or in the future issue as patents, they may not issue in a form that will provide us with any meaningful protection, prevent competitors or other third parties from competing with us, or otherwise provide us with any competitive advantage. Any patents that we hold or in- license may be challenged, narrowed, circumvented, or invalidated by third parties. Consequently, we do not know whether any of our technologies and product candidates will be protectable or remain protected by valid and enforceable patents. In addition, our existing patents and any future patents we obtain may not be sufficiently broad to prevent others from using our technology or from developing competing technologies and products, and the coverage claimed in a patent application can be significantly reduced before the patent is issued, and its scope can be reinterpreted after issuance. Any failure to obtain, maintain or defend our patents and other intellectual property could have a material adverse effect on our business, financial conditions, results of operations and prospects. We cannot be certain that we are the first to invent the inventions covered by pending patent applications and, if we are not, we may be subject to priority or entitlement disputes. We may be required to disclaim part or all of the term of certain patents or all of the term of certain patent applications. There may be prior art of which we are not aware that may affect the validity or enforceability of a patent claim. There also may be prior art of which we are aware, but which we do not believe affects the validity or enforceability of a claim, which may, nonetheless, ultimately be found to affect the validity or enforceability of a claim. Since patent applications in the United States and other countries are confidential for a period of time after filing, at any moment in time, we cannot be certain that we were in the past or will be in the future the first to file any patent application related to our product candidates. For example, some patent applications in the United States may be maintained in secrecy until the patents are issued. Further, publications in the scientific literature often lag behind actual discoveries. Consequently, we cannot be certain that others have not filed patent applications for technology covered by our owned and in- licensed issued patents or our pending applications, or that we or, if applicable, a licensor, were the first to invent or first to file an application for the technology. It is possible that defects of form in the preparation or filing of our patents or patent applications may exist, or may arise in the future, for example, with respect to proper priority claims, inventorship, claim scope, or requests for patent term adjustments. If there are material defects in the form, preparation, prosecution, or enforcement of our patents or patent applications, such patents may be invalid and / or unenforceable, and such applications may never result in valid, enforceable patents. Any of these outcomes could impair our ability to prevent competition from third parties, which may have an adverse impact on our business. In addition to the protection provided by our patent estate, we rely on trade secret protection and confidentiality agreements to protect proprietary

know- how that is not amenable to patent protection. Although we generally require all of our employees to assign their inventions to us, and all of our employees, consultants, advisors and any third parties who have access to our proprietary know- how, information, or technology to enter into confidentiality agreements, we cannot provide any assurances that all such agreements have been duly executed, or that our trade secrets and other confidential proprietary information will not be disclosed. In addition, while we have undertaken reasonable efforts to ensure such agreements are enforceable and that employees and third parties comply with their obligations thereunder, these agreements may be found insufficient by a court of law or may be breached, or we may not enter into sufficient agreements with such individuals in the first instance, in either case potentially resulting in the unauthorized use or disclosure of our trade secrets and other intellectual property, including to our competitors, which could cause us to lose any competitive advantage resulting from this intellectual property. Individuals not subject to invention assignment agreements may make adverse ownership claims to our current and future intellectual property. Moreover, our competitors may independently develop knowledge, methods and know- how equivalent to our trade secrets. Competitors could purchase our products, if approved, and replicate some or all of the competitive advantages we derive from our development efforts for technologies on which we do not have patent protection. If any of our trade secrets were to be lawfully obtained or independently developed by a competitor, we would have no right to prevent them, or those to whom they communicate it, from using that technology or information to compete with us. If any of our trade secrets were to be disclosed to or independently developed by a competitor, our competitive position would be harmed. Enforcing a claim that a third- party entity illegally obtained and is using any of our trade secrets is expensive and time- consuming, and the outcome is unpredictable, and we may not be able to obtain adequate remedies for such breaches. We also seek to preserve the integrity and confidentiality of our data and trade secrets by **working to** ~~maintaining~~ --- **maintain the** physical security of our premises and physical and electronic security of our information technology systems. **Our confidentiality** ~~While we have confidence in these individuals, organizations and systems, our~~ agreements ~~or security measures~~ may **, however,** be breached, and we may not have adequate remedies for any breach **. Our and other applicable security measures may also not be able to prevent security breaches and other compromises of the security of our trade secrets or other data** . Also, if the steps taken to maintain our trade secrets are deemed inadequate, we may have insufficient recourse against third parties for misappropriating the trade secret. In addition, others may independently discover our trade secrets and proprietary information. For example, the FDA is considering whether to make additional information publicly available on a routine basis, including information that we may consider to be trade secrets or other proprietary information, and it is not clear at the present time how the FDA' s disclosure policies may change in the future. If we are unable to prevent material disclosure of the non- patented intellectual property related to our technologies to third parties, and there is no guarantee that we will have any such enforceable trade secret protection, we may not be able to establish or maintain a competitive advantage in our market, which could materially adversely affect our business, results of operations and financial condition. Patent terms may be inadequate to protect our competitive position on our products for an adequate amount of time, and if we do not obtain protection under the Hatch- Waxman Amendments and similar non- United States legislation for extending the term of patents covering each of our product candidates, our business may be materially harmed. Given the amount of time required for the development, testing and regulatory review of new product candidates, patents protecting such candidates might expire before or shortly after such product candidates are commercialized. Depending upon the timing, duration and conditions of FDA marketing approval of our product candidates, one or more of our United States patents may be eligible for limited patent term extension under the Drug Price Competition and Patent Term Restoration Act of 1984, referred to as the Hatch- Waxman Amendments, and similar legislation in the European Union. The Hatch- Waxman Amendments permit a patent term extension of up to five years for a patent covering an approved product as compensation for effective patent term lost during product development and the FDA regulatory review process. A patent term extension cannot extend the remaining term of a patent beyond a total of 14 years from the date of product approval. Only one patent may be extended, and only those claims covering the approved drug, a method for using it, or a method for manufacturing it may be extended. However, we may not receive an extension if we fail to apply within applicable deadlines, fail to apply prior to expiration of relevant patents or otherwise fail to satisfy applicable requirements. Moreover, the length of the extension could be less than we request. If we are unable to obtain patent term extension or the term of any such extension is less than we request, the period during which we can enforce our patent rights for that product will be shortened and our competitors may obtain approval to market competing products sooner. As a result, our revenue from applicable products could be reduced and could have a material adverse effect on our business. We in- license key intellectual property necessary for the development of each of our current product candidates. If we fail to comply with our obligations in our current and future intellectual property licenses with third parties, resulting in the termination of such licenses, we could lose rights that are important to our business. We are heavily reliant upon licenses to certain patent rights and proprietary technology for the development of each of our current product candidates. In particular, we in- license key patents and patent applications from Adverum related to LX2006, we in- license patent applications and know- how from Cornell University related to our LX1001, LX1020 and LX1021 product candidates, and we in- license patent applications and know- how from **UCSD** ~~the Regents of the University of California, San Diego~~ , related to our LX2020, LX2021 and LX2022 product candidates. Our license agreements impose diligence and milestone and royalty payment obligations on us, and also contain certain development requirements. If we fail to comply with our obligations, our licensors may have the right to terminate our licenses, in which event we will not be able to develop, manufacture or market any product using the intellectual property under any such terminated agreement and may face other penalties. Such an occurrence would materially adversely affect our business prospects. Certain of our licenses may not provide us with exclusive rights to use the licensed intellectual property and technology, or may not provide us with exclusive rights to use such intellectual property and technology in all relevant fields of use and in all territories in which we may wish to develop or commercialize our technology and product candidates in the future. In addition, the intellectual property rights licensed to us by our licensors, including certain intellectual property licensed by

Cornell University, **UCSD** ~~The Regents of the University of California, San Diego~~, and Adverum, at least in some respects, may be used by such licensors or licensed to third parties, and such third parties may have certain enforcement rights with respect to such intellectual property. Thus, patents licensed to us could be put at risk of being invalidated or interpreted narrowly in litigation filed by or against our licensors or another licensee or in administrative proceedings brought by or against our licensors or another licensee in response to such litigation or for other reasons. As a result, we may not be able to prevent competitors or other third parties from developing and commercializing competitive products, including in territories covered by our licenses. Licenses to additional third- party technology and materials that may be required for our development programs may not be available in the future or may not be available on commercially reasonable terms, or at all, which could have a material adverse effect on our business and financial condition. In such events, we may be required to expend significant time and resources to redesign our technology, product candidates, or the methods for manufacturing them or to develop or license replacement technology, all of which may not be feasible on a technical or commercial basis. If we are unable to do so, we may be unable to develop or commercialize the affected technology or product candidates. Even if we are able to obtain such additional licenses, they may be non- exclusive thereby giving our competitors and other third parties access to the same technology licensed to us. If we or our licensors fail to adequately protect our licensed intellectual property, our ability to commercialize our product candidates and technology could suffer. Although we have oversight rights, Cornell University and **UCSD** ~~The Regents of the University of California, San Diego,~~ generally control the prosecution, maintenance and enforcement of our in- licensed patents and patent applications. Therefore, we cannot be certain that the prosecution, maintenance and enforcement of these patent rights will be in a manner consistent with the best interests of our business, or in compliance with applicable laws and regulations, or will result in valid and enforceable patents and other intellectual property rights. It is possible that our licensors' infringement proceedings or defense activities may be less vigorous than had we conducted them ourselves or may not be conducted in accordance with our best interests. If we or our licensors fail to maintain such patents or patent applications, or if we or our licensor lose rights to those patents or patent applications, the rights we have licensed may be reduced or eliminated and our right to develop and commercialize any of our product candidates that are the subject of such licensed rights could be adversely affected. In addition to the foregoing, the risks associated with patent rights that we license from third parties will also apply to patent rights we may own in the future. Further, if we fail to comply with our development obligations under our license agreements, we may lose our patent rights with respect to such agreement on a territory- by- territory basis, which would affect our patent rights worldwide. In spite of our efforts, our current and future licensors might conclude that we have materially breached our obligations under our license agreements and might therefore terminate such license agreements, thereby removing or limiting our ability to develop and commercialize products and technology covered by these license agreements. 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; • our financial and other obligations under the license agreement; • whether and the extent to which our technology and processes infringe on intellectual property of the licensor that is not subject to the licensing agreement; • our 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 our development and commercialization of our product candidates and what activities satisfy those diligence obligations; • the inventorship or ownership of inventions and know- how resulting from the joint creation or use of intellectual property by our licensors and us and our partners; and • the priority of invention of patented technology. If disputes over intellectual property that we have licensed prevent or impair our ability to maintain our current licensing arrangements on acceptable terms, we may be unable to successfully develop and commercialize the affected technology or product candidates. In addition, if any such disputes result in the termination of our intellectual property licenses, this could result in the loss of our ability to develop and commercialize our lead product candidates, or we could lose other significant rights, experience significant delays in the development and commercialization of our other product candidates, or incur liability for damages, any of which could have a material adverse effect on our business, financial condition, results of operations and prospects. In addition, we may seek to obtain additional licenses from our licensors and, in connection with obtaining such licenses, we may agree to amend our existing licenses in a manner that may be more favorable to the licensors, including by agreeing to terms that could enable third parties, including our competitors, to receive licenses to a portion of the intellectual property that is subject to our existing licenses and to compete with our product candidates. Some of our future agreements with certain of our third- party research partners may provide that improvements developed in the course of our relationship may be owned solely by either us or our third- party research partner. If we determine that rights to such improvements owned solely by a third- party research partner or other third party with whom we collaborate are necessary to commercialize our therapeutic product candidates or maintain our competitive advantage, we may need to obtain a license from such third party in order to use the improvements and continue developing, manufacturing or marketing our drug candidates. We may not be able to obtain such a license on an exclusive basis, on commercially reasonable terms, or at all, which could prevent us from commercializing our product candidates or allow our competitors or others the chance to access technology that is important to our business. Termination of our current or any future license agreements would reduce or eliminate our rights under these agreements and may result in our having to negotiate new or reinstated agreements with less favorable terms or cause us to lose our rights under these agreements, including our rights to important intellectual property or technology. Any of the foregoing could prevent us from commercializing our other product candidates, which could have a material adverse effect on our operating results and overall financial condition. In addition, intellectual property rights that we in- license in the future may be sublicenses under intellectual property owned by third parties, in some cases through multiple tiers. The actions of our licensors may therefore affect our rights to use our sublicensed intellectual property, even if we are in compliance with all of the obligations under our license agreements. Should our licensors or any of the upstream licensors fail to comply with their obligations under the agreements pursuant to which they obtain the rights that are sublicensed to us, or should such agreements

be terminated or amended, our ability to develop and commercialize our product candidates may be materially harmed. In addition, a third party may in the future bring claims that our performance under our license agreements, including our sponsoring of clinical trials, interferes with such third party' s rights under its agreement with one of our licensors. If any such claim were successful, it may adversely affect our rights and ability to advance our product candidates as clinical candidates or subject us to liability for monetary damages, any of which would have an adverse effect on our business, financial condition, results of operations and prospects. We are generally also subject to all of the same risks with respect to protection of intellectual property that we license as we are for intellectual property that we own, which are described above and below. If we or our licensors fail to adequately protect this intellectual property, our ability to commercialize products could suffer. We may not be successful in obtaining or maintaining necessary rights to product components and processes for our development pipeline through acquisitions and in- licenses. Presently, we have obtained rights to certain intellectual property rights through licenses from third parties to develop, manufacture and commercialize our lead product candidates and other potential product candidates in our pipeline. Because the commercialization of our product candidates may require the use of additional intellectual property rights held by third parties, the growth of our business likely will depend, in part, on our ability to acquire or license these intellectual property rights. Our product candidates also require specific formulations and manufacturing processes to work effectively and efficiently, and some of these rights are held by others. 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, important or more expedient to further our business operations. In addition, even if we are able to obtain such licenses, we may fail to obtain any of these licenses at a reasonable cost or on reasonable terms, which would harm our business. Were that to happen, we may need to cease use of the product candidates and technologies covered by those third- party intellectual property rights and may need to seek to develop alternative approaches that do not infringe, misappropriate or violate those intellectual property rights, which may entail additional costs and development delays if we are able to develop such alternatives, or which may not be feasible. Even if we are able to obtain a license, it may be non- exclusive, which means that our competitors may also receive access to the same technologies licensed to us. The licensing and acquisition of third- party intellectual property rights is a competitive practice, and companies that may be more established, or have greater resources than we do, may also be pursuing strategies to license or acquire third- party intellectual property rights that we may consider necessary or attractive in order to commercialize our product candidates. More established companies may have a competitive advantage over us due to their larger size and cash resources or 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. There can be no assurance that we will be able to successfully complete such negotiations and ultimately acquire the rights to the intellectual property surrounding the additional product candidates that we may seek to acquire. We also may be unable to license or acquire third- party intellectual property rights on terms that would allow us to make an appropriate return on our investment. If we are unable to successfully obtain rights to required third- party intellectual property or maintain the existing intellectual property rights we have licensed, we may be required to expend significant time and resources to redesign our product candidates, or to develop or license replacement technology, all of which may not be feasible on a technical or commercial basis, and we may have to abandon development of our product candidates, any of which could have a material adverse effect on our business, financial condition, results of operations and prospects. Our intellectual property licenses with third parties may be subject to disagreements over contract interpretation, which could narrow the scope of our rights to the relevant intellectual property or technology or increase our financial or other obligations to our licensors. We currently depend, and will continue to depend, on our license agreements, including the license agreements with Cornell University and Adverum related to LX2006, with Cornell University related to our LX1001, LX1020 and LX1021 product candidates, and with **UCSD** ~~The Regents of the University of California, San Diego~~ , related to LX2020, LX2021 and LX2022 product candidates. The agreements under which we currently license intellectual property or technology from third parties are complex, and certain provisions in such agreements may be susceptible to multiple interpretations. The resolution of any contract interpretation disagreement that may arise could narrow what we believe to be the scope of our rights to the relevant intellectual property or technology, 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. If any of our licenses or material relationships or any in- licenses upon which our licenses are based are terminated or breached, we may: • lose our rights to develop and market our products; • lose patent protection for our products; • experience significant delays in the development or commercialization of our products; • not be able to obtain any other licenses on acceptable terms, if at all; or • incur liability for damages. These risks apply to any agreements that we may enter into in the future for our products or for any future product candidates. If we experience any of the foregoing, it could have a material adverse effect on our business, financial condition, results or operations and prospects. We cannot be certain that any of our or licensed pending patent applications or our future owned or licensed patent applications will result in issued patent claims covering such aspects of our product candidates. Composition- of- matter patents on the active pharmaceutical ingredient, or API, in prescription drug products are generally considered to be the strongest form of intellectual property protection for drug products because those types of patents provide protection without regard to any particular method of use or manufacture or formulation of the API used. Although we intend to file patent applications in the future that cover these product candidates, we cannot be certain that our future owned or licensed patent applications will cover our current or future product candidates. Method- of- use patents protect the use of a product for the specified method and formulation patents cover formulations of the API. These types of patents do not prevent a competitor or other third party from developing or marketing an identical product for an indication that is outside the scope of the patented method or from developing a different formulation that is outside the scope of the patented formulation. Moreover, with respect to method- of- use patents, even if competitors or other third parties do not actively promote their product for our targeted indications or uses for which we may obtain patents, physicians may recommend that patients use these products off- label, or

patients may do so themselves. Although off- label use may infringe or contribute to the infringement of method- of- use patents, the practice is common, and this type of infringement is difficult to prevent or prosecute. In addition, there are numerous publications and other prior art that may be relevant to our owned or in- licensed method- of- use patents and patent applications and may be used to challenge the validity of these owned or in- licensed patents and patent applications in litigation or other intellectual property- related proceedings. If these types of challenges are successful, our owned or in- licensed patents and patent applications may be narrowed or found to be invalid, and we may lose valuable intellectual property rights. Any of the foregoing could have a material adverse effect on our business, financial conditions, prospects and results of operations. The strength of patents in the biotechnology and pharmaceutical field involves complex legal and scientific questions and can be uncertain. The patent applications that we own or in- license may fail to result in issued patents with claims that cover our product candidates or uses thereof in the United States or in other countries. Even if patents do successfully issue, the issuance of a patent is not conclusive as to its inventorship, scope, validity or enforceability and third parties may challenge the validity, enforceability or scope of our owned and licensed patents in courts or patent offices in the United States and abroad, which may result in those patents being narrowed, invalidated or held unenforceable. Furthermore, even if they are unchallenged, our owned and licensed patents and pending patent applications, if issued, may not adequately protect our intellectual property or prevent competitors or others from designing around our patent claims to circumvent our owned or licensed patents by developing similar or alternative technologies or therapeutics in a non- infringing manner. If the breadth or strength of protection provided by the patents and patent applications we own or license with respect to our product candidates is not sufficient to impede such competition or is otherwise threatened, it could dissuade companies from collaborating with us to develop, and threaten our ability to commercialize, our product candidates. Any of the foregoing could have a material adverse effect on our business, financial condition, results of operations and prospects. We may be involved in lawsuits to protect or enforce our patents, which could be expensive, time- consuming and unsuccessful. Competitors may infringe the patents for which we have applied. To counter infringement or unauthorized use, we may be required to file infringement claims, which can be expensive and time- consuming. If we initiate legal proceedings against a third party to enforce a patent covering one of our product candidates, the defendant could counterclaim that the patent covering our product or product candidate is invalid and / or unenforceable. In patent litigation in the United States, counterclaims alleging invalidity and / or unenforceability are common, and there are numerous grounds upon which a third party can assert invalidity or unenforceability of a patent. In an infringement proceeding, a court may decide that the patent claims we are asserting are invalid and / or unenforceable, or may refuse to stop the other party from using the technology at issue on the grounds that our patent claims do not cover the technology in question. Third parties may also raise similar claims before administrative bodies in the United States or abroad, even outside the context of litigation. Such mechanisms include re- examination, post- grant review, inter partes review and equivalent proceedings in foreign jurisdictions (for example, opposition proceedings). Such proceedings could result in revocation of or amendment to our patents in such a way that they no longer cover our product candidates. The outcome following legal assertions of invalidity and unenforceability is unpredictable. With respect to the validity question, for example, we cannot be certain that there is no invalidating prior art, of which we, our patent counsel and the patent examiner were unaware during prosecution. If a defendant were to prevail on a legal assertion of invalidity and / or unenforceability, we would lose at least part, and perhaps all, of the patent protection on our product candidates. An adverse result in any litigation or defense proceedings could put one or more of our patents at risk of being invalidated or interpreted narrowly, could put our patent applications at risk of not issuing and could have a material adverse impact on our business. Interference proceedings provoked by third parties or brought by us may be necessary to determine the priority of inventions with respect to our patent applications. An unfavorable outcome could require us to cease using the related technology or force us to take a license under the patent rights of the prevailing party, if available. Furthermore, our business could be harmed if the prevailing party does not offer us a license on commercially reasonable terms. Our defense of litigation or interference proceedings may fail and, even if successful, may result in substantial costs and distract our management and other employees. We may not be able to prevent misappropriation of our intellectual property rights, particularly in countries where the laws may not protect those rights as fully as in the United States. Even if we establish infringement of any of our patents by a competitive product, a court may decide not to grant an injunction against further infringing activity, thus allowing the competitive product to continue to be marketed by the competitor. It is difficult to obtain an injunction in U. S. litigation and a court could decide that the competitor should instead pay us a " reasonable royalty " as determined by the court, and / or other monetary damages. A reasonable royalty or other monetary damages may or may not be an adequate remedy. Loss of exclusivity and / or competition from a related product would have a material adverse impact on our business. For certain of our in- licensed patent rights, such as patent rights in- licensed from Cornell University and Adverum, we may not have the right to file a lawsuit for infringement and may have to rely on a licensor to enforce these rights for us. If we are not able to directly assert our licensed patent rights against infringers or if a licensor does not vigorously prosecute any infringement claims on our behalf, we may have difficulty competing in certain markets where such potential infringers conduct their business, and our commercialization efforts may suffer as a result. In addition, we or our licensors, as the case may be, may not be able to detect infringement against our owned or in- licensed patents, which may be especially difficult for manufacturing processes or formulation patents. Even if we or our licensors detect infringement by a third party of our owned or in- licensed patents, we or our licensors, as the case may be, may choose not to pursue litigation against or settlement with the third party. If we or our licensors later sue such third party for patent infringement, the third party may have certain legal defenses available to it that otherwise would not be available but for the delay between when the infringement was first detected and when the suit was brought. These legal defenses may make it impossible for us or our licensors to enforce our owned or in- licensed patents, as the case may be, against that third party. Furthermore, because of the substantial amount of discovery required in connection with intellectual property litigation, there is a risk that some of our confidential information could be compromised by disclosure during this type of litigation. There could also be public announcements of the results of

hearings, motions, or other interim proceedings or developments. If securities analysts or investors perceive these results to be negative, it could have a material adverse effect on the price of our common stock. Third parties may initiate legal proceedings alleging that we are infringing their intellectual property rights, the outcome of which would be uncertain. As our current and future product candidates progress toward commercialization, the possibility of a patent infringement claim against us increases. We cannot provide any assurance that our current and future product candidates do not infringe other parties' patents or other proprietary rights, and competitors or other parties may assert that we infringe their proprietary rights in any event. We may become party to, or threatened with, adversarial proceedings or litigation regarding intellectual property rights with respect to our current and future product candidates, including interference or derivation proceedings before the USPTO, or oppositions and other proceedings in foreign jurisdictions. We may be exposed to, or threatened with, future litigation by third parties having patent or other intellectual property rights alleging that our product candidates, manufacturing methods, formulations, administration methods and / or proprietary technologies infringe, misappropriate or otherwise violate their intellectual property rights. Numerous issued patents and pending patent applications that are owned by third parties exist in the fields in which we are developing our product candidates. As the biotechnology and pharmaceutical industries expand and more patents are issued, the risk increases that our product candidates may give rise to claims of infringement of the patent rights of others. Moreover, it is not always clear to industry participants, including us, the claim scope that may issue from pending patent applications owned by third parties or which patents cover various types of drugs, products or their methods of use or manufacture. Thus, because of the large number of patents issued and patent applications filed in our fields, there may be a risk that third parties, including our competitors, may allege they have patent rights encompassing our product candidates, technologies or methods and that we are employing their proprietary technology without authorization. If we were sued for patent infringement, we would need to demonstrate that the relevant product or methods of using the product either do not infringe the patent claims of the relevant patent or that the patent claims are invalid or unenforceable, and we may not be able to do this. In order to successfully challenge the validity of any such United States patent in federal court, we would need to overcome a presumption of validity. As this burden is high and requires us to present clear and convincing evidence as to the invalidity of any such United States patent claim, there is no assurance that a court of competent jurisdiction would agree with us and invalidate the claims of any such United States patent. Moreover, given the vast number of patents in our field of technology, we cannot be certain that we do not infringe existing patents or that we will not infringe patents that may be granted in the future. While we may decide to initiate proceedings to challenge the validity of these or other patents in the future, we may be unsuccessful, and courts or patent offices in the United States and abroad could uphold the validity of any such patent. Furthermore, because patent applications can take many years to issue and may be confidential for 18 months or more after filing, and because pending patent claims can be revised before issuance, there may be applications now pending which may later result in issued patents that may be infringed by the manufacture, use or sale of our product candidates. Regardless of when filed, we may fail to identify relevant third- party patents or patent applications, or we may incorrectly conclude that a third- party patent is invalid or not infringed by our product candidates or activities. If a patent holder believes that one of our product candidates infringes its patent, the patent holder may sue us even if we have received patent protection for our technology. Moreover, we may face patent infringement claims from non- practicing entities that have no relevant drug revenue and against whom our own patent portfolio may thus have no deterrent effect. If a patent infringement suit were threatened or brought against us, we could be forced to stop or delay research, development, manufacturing or sales of the drug or product candidate that is the subject of the actual or threatened suit. If any third- party patents are held by a court of competent jurisdiction to be valid and enforceable and to cover any of our technology or product candidates, including the manufacturing process of our product candidates, constructs or molecules used in or formed during the manufacturing process, or any final product itself, we could be forced, including by court order, to cease developing, manufacturing or commercializing the infringing product. Alternatively, we may be required to obtain a license from such third party in order to use the infringing technology and continue developing, manufacturing or marketing the infringing product. If we were required to obtain a license to continue to manufacture or market the affected product, we may be required to pay substantial royalties or grant cross- licenses to our patents. We cannot, however, assure that any such license will be available on acceptable terms, if at all. Ultimately, we could be prevented from commercializing a product, or be forced to cease some aspect of our business operations as a result of claims of patent infringement or violation of other intellectual property rights. Further, the outcome of intellectual property litigation is subject to uncertainties that cannot be adequately quantified in advance, including the demeanor and credibility of witnesses and the identity of any adverse party. This is especially true in intellectual property cases that may turn on the testimony of experts as to technical facts upon which experts may reasonably disagree. Furthermore, we may not be able to obtain any required license on commercially reasonable terms or at all. Even if we were able to obtain a license, it could be non- exclusive, thereby giving our competitors access to the same technologies licensed to us; alternatively, or additionally, it could include terms that impede or destroy our ability to compete successfully in the commercial marketplace. In addition, we could be found liable for significant monetary damages, including treble damages and attorneys' fees if we are found to have willfully infringed a patent. A finding of infringement could prevent us from commercializing a product or force us to cease some of our business operations, which could harm our business. Claims that we have misappropriated the confidential information or trade secrets of third parties could have a similar negative impact on our business. Furthermore, because of the substantial amount of discovery required in connection with intellectual property litigation or administrative proceedings, there is a risk that some of our confidential information could be compromised by disclosure. In addition, any uncertainties resulting from the initiation and continuation of any litigation could have material adverse effect on our ability to raise additional funds or otherwise have a material adverse effect on our business, results of operations, financial condition and prospects. The cost to us in defending or initiating any litigation or other proceeding relating to patent or other proprietary rights, even if resolved in our favor, could be substantial, and litigation would divert our management' s attention. Some of our competitors may be able to sustain the costs of complex patent litigation more effectively

than we can because they have substantially greater resources. Uncertainties resulting from the initiation and continuation of patent litigation or other proceedings could delay our research and development efforts and limit our ability to continue our operations. We are currently, and may in the future be, subject to claims that we and our employees, consultants, or independent contractors have wrongfully used or disclosed confidential information or trade secrets of third parties. We employ individuals who were previously employed at other biotechnology or biopharmaceutical companies. Although we ~~try to ensure~~ **require, evidenced by written agreements,** that **all of** our employees, consultants and advisors ~~do~~ not use the proprietary information or know- how of others in their work for us, we may be subject to claims that we or our employees, consultants, or independent contractors have inadvertently or otherwise used or disclosed confidential information of our employees' former employers or other third parties. We may also be subject to claims that former employers or other third parties have an ownership interest in our future patents. Litigation may be necessary to defend against these claims. There is no guarantee of success in defending these claims, and even if we are successful, litigation can have an adverse impact on us due to defense and settlement costs, diversion of management resources, harm to our reputation and other factors. For example, on October 12, 2023, Rocket filed a lawsuit against us and two ~~individuals~~ **former employees** claiming, among other things, misappropriation of confidential information and trade secrets ~~. The individual defendants are a current employee and a former employee of our analytical development team, both of whom were employed at Rocket before joining us in 2021~~. The complaint alleges the individual defendants downloaded confidential Rocket company documents and other proprietary materials prior to leaving Rocket in 2021 and that we used this information to advance our programs **after they became employed by Lexeo**. The complaint seeks unspecified damages and asks the court to enjoin us from competing and working in the market for gene therapy treatments targeting cardiac diseases. ~~We retained legal counsel to assist~~ **In August 2024, we asserted counterclaims against Rocket and Spacecraft Seven LLC, a wholly owned subsidiary of Rocket, for misappropriation of trade secrets, correction of inventorship of certain patents, breach of contract, and tortious interference** with **contract** ~~our ongoing review of the allegations in Rocket's complaint and are confident in our defenses to the allegations~~. **The case** ~~On December 7, 2023, we filed a motion to dismiss the complaint, and the motion~~ is **currently in** ~~fully briefed and pending before~~ the ~~court~~ **discovery phase**. It is not possible to predict the outcome with certainty and an estimate of the possible loss cannot be made. For additional information regarding this litigation, see Item 3: Legal Proceedings. Even if we are successful in defending against these types of claims, litigation or other legal proceedings relating to intellectual property claims may cause us to incur significant expenses and could distract our technical and management personnel from their normal responsibilities. In addition, there could be public announcements of the results of hearings, motions or other interim proceedings or developments, and, if securities analysts or investors perceive these results to be negative, that perception could have a substantial adverse effect on the price of our common stock. This type of litigation or proceeding could substantially increase our operating losses and reduce our resources available for development activities. Some of our competitors may be able to sustain the costs of this type of litigation or proceedings more effectively than we can because of their substantially greater financial resources. Uncertainties resulting from the initiation and continuation of intellectual property litigation or other intellectual property related proceedings could adversely affect our ability to compete in the marketplace. We may be subject to claims challenging the inventorship or ownership of our future patents and other intellectual property. We may also be subject to claims that former employees, collaborators, or other third parties have an ownership interest in our patent applications, our future patents, or other intellectual property. We may be subject to ownership disputes in the future arising, for example, from conflicting obligations of consultants or others who are involved in developing our product candidates and platform discovery. Although it is our policy to require our employees and contractors who may be involved in the conception or development of intellectual property to execute agreements assigning such intellectual property to us, we may be unsuccessful in executing such an agreement with each party who, in fact, conceives or develops intellectual property that we regard as our own, and we cannot be certain that our agreements with such parties will be upheld in the face of a potential challenge, or that they will not be breached, for which we may not have an adequate remedy. The assignment of intellectual property rights may not be self- executing, or the assignment agreements may be breached, and litigation may be necessary to defend against these and other claims challenging inventorship or ownership. 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. Reliance on third parties requires us to share our trade secrets, which increases the possibility that a competitor will discover them or that our trade secrets will be misappropriated or disclosed. If we rely on third parties to manufacture or commercialize our product candidates, or if we collaborate with additional third parties for the development of such product candidates, we must, at times, share trade secrets with them. We may also conduct joint research and development programs that may require us to share trade secrets under the terms of our research and development partnerships or similar agreements. We seek to protect our proprietary technology in part by entering into confidentiality agreements and, if applicable, material transfer agreements, consulting agreements or other similar agreements with our advisors, employees, third- party contractors and consultants prior to beginning research or disclosing proprietary information. These agreements typically limit the rights of the third parties to use or disclose our confidential information, including our trade secrets. Despite the contractual provisions employed when working with third parties, the need to share trade secrets and other confidential information increases the risk that such trade secrets become known by our competitors, are inadvertently incorporated into the technology of others, or are disclosed or used in violation of these agreements. Given that our proprietary position is based, in part, on our know- how and trade secrets, a competitor' s discovery of our trade secrets or other unauthorized use or disclosure could have an adverse effect on our business and results of operations. In addition, these agreements typically restrict the ability of our advisors, employees, third- party contractors and consultants to publish data potentially relating to our trade secrets. Despite our efforts to protect our trade secrets, we may not be

able to prevent the unauthorized disclosure or use of our technical know- how or other trade secrets by the parties to these agreements. Moreover, we cannot guarantee that we have entered into such agreements with each party that may have or have had access to our confidential information or proprietary technology and processes. Monitoring unauthorized uses and disclosures is difficult, and we do not know whether the steps we have taken to protect our proprietary technologies will be effective. If any of the collaborators, scientific advisors, employees, contractors and consultants who are parties to these agreements breaches or violates the terms of any of these agreements, we may not have adequate remedies for any such breach or violation, and we could lose our trade secrets as a result. Moreover, if confidential information that is licensed or disclosed to us by our partners, collaborators, or others is inadvertently disclosed or subject to a breach or violation, we may be exposed to liability to the owner of that confidential information. Enforcing a claim that a third party illegally obtained and is using our trade secrets, like patent litigation, is expensive and time consuming, and the outcome is unpredictable. In addition, courts outside the United States are sometimes less willing to protect trade secrets. Intellectual property rights do not necessarily address all potential threats to our competitive advantage. The degree of future protection afforded by our intellectual property rights is uncertain because intellectual property rights have limitations, and may not adequately protect our business, or permit us to maintain our competitive advantage. The following examples are illustrative: • others may be able to make or use capsids, nucleic acids and vectors that are similar to the biological compositions of our products that are the same as or similar to our product candidates but that are not covered by the claims of owned or in- licensed patents; • others may independently develop similar or alternative technologies or duplicate any of our technologies; • it is possible that others may circumvent our owned or in- licensed patents; • others, including inventors or developers of our owned or in- licensed patented technologies who may become involved with competitors, may independently develop similar technologies that function as alternatives or replacements for any of our technologies without infringing our intellectual property rights; • it is possible that our owned or in-licensed patents or patent applications omit individual (s) who should be listed as inventor (s) or include individual (s) who should not be listed as inventor (s), which may cause these patents or patents issuing from these patent applications to be held invalid or unenforceable; • we or our licensors or our other collaboration partners might not have been the first to conceive and reduce to practice the inventions covered by the patents or patent applications that we own, license or will own or license; • we or our licensors or our other collaboration partners might not have been the first to file patent applications covering certain of the patents or patent applications that we or they own or have obtained a license, or will own or will have obtained a license; • we or our licensors may fail to meet obligations to the U. S. government with respect to in- licensed patents and patent applications funded by U. S. government grants, leading to the loss of patent rights; • it is possible that our pending patent applications will not result in issued patents; • we may not be able to generate sufficient data to support full patent applications that protect the entire breadth of developments in one or more of our programs; • no patent protection may be available with regard to formulation or method of use; • the claims of our owned or in- licensed issued patents or patent applications, if and when issued, may not cover our product candidates; • it is possible that there are prior public disclosures that could invalidate our or our licensors' patents; • it is possible that there are unpublished applications or patent applications maintained in secrecy that may later issue with claims covering our products or technology similar to ours; • issued patents that we own or exclusively license may not provide us with any competitive advantage, or may be held invalid or unenforceable, as a result of legal challenges by our competitors; • we may not exclusively license our patents and, therefore, may not have a competitive advantage if such patents are licensed to others; • our competitors might conduct research and development activities in countries where we do not have patent rights, or in countries where research and development safe harbor laws exist, and then use the information learned from such activities to develop competitive products for sale in our major commercial markets; • the laws of other countries may not protect our or our licensors', as the case may be, proprietary rights to the same extent as the laws of the United States; • there may be significant pressure on the U. S. government and international governmental bodies to limit the scope of patent protection both inside and outside the United States for disease treatments that prove successful, as a matter of public policy regarding worldwide health concerns; • countries other than the United States may, under certain circumstances, force us or our licensors to grant a license under our patents to a competitor, thus allowing the competitor to compete with us in that jurisdiction or forcing us to lower the price of our drug in that jurisdiction; • we have engaged in scientific collaborations in the past and will continue to do so in the future and our collaborators may develop adjacent or competing products that are outside the scope of our patents; • we may not successfully commercialize the product candidates, if approved, before our relevant patents expire; • we may not develop additional proprietary technologies for which we can obtain patent protection; • it is possible that product candidates or technologies we develop may be covered by third parties' patents or other exclusive rights; • ownership, validity or enforceability of our or our licensors' patents or patent applications may be challenged by third parties; and • the patents of third parties or pending or future applications of third parties, if issued, may have an adverse effect on our business. We may have limited geographical protection with respect to certain patents and we may not be able to protect our intellectual property rights throughout the world. Filing and prosecuting patent applications and defending patents covering our product candidates in all countries throughout the world would be prohibitively expensive. Competitors may use our technologies in jurisdictions where we have not obtained patent protection to develop their own products and, further, may export otherwise infringing products to territories where we have patent protection, but enforcement rights are not as strong as that in the United States or Europe. These products may compete with our product candidates, and our future patents or other intellectual property rights may not be effective or sufficient to prevent them from competing. In addition, we may decide to abandon national and regional patent applications before they are granted. The examination of each national or regional patent application is an independent proceeding. As a result, patent applications in the same family may issue as patents in some jurisdictions, such as in the United States, but may issue as patents with claims of different scope or may even be refused in other jurisdictions. It is also quite common that depending on the country, the scope of patent protection may vary for the same product candidate or technology. While we intend to protect our intellectual property rights in our expected significant markets, we cannot ensure that we will be

able to initiate or maintain similar efforts in all jurisdictions in which we may wish to market our product candidates. Accordingly, our efforts to protect our intellectual property rights in such countries may be inadequate, which may have an adverse effect on our ability to successfully commercialize our product candidates in all of our expected significant foreign markets. If we encounter difficulties in protecting, or are otherwise precluded from effectively protecting, the intellectual property rights important for our business in such jurisdictions, the value of these rights may be diminished, and we may face additional competition from others in those jurisdictions. The laws of some jurisdictions do not protect intellectual property rights to the same extent as the laws or rules and regulations in the United States and Europe and many companies have encountered significant difficulties in protecting and defending such rights in such jurisdictions. The legal systems of certain countries, particularly certain developing countries, do not favor the enforcement of patents, trade secrets and other intellectual property rights, which could make it difficult for us to stop the infringement of our future patents or marketing of competing products in violation of our proprietary rights generally. Proceedings to enforce our patent rights in other jurisdictions, whether or not successful, could result in substantial costs and divert our efforts and attention from other aspects of our business, could put our future patents at risk of being invalidated or interpreted narrowly and our patent applications at risk of not issuing as patents, and could provoke third parties to assert claims against us. We may not prevail in any lawsuits that we initiate, and the damages or other remedies awarded, if any, may not be commercially meaningful. Accordingly, our efforts to enforce our intellectual property rights around the world may be inadequate to obtain a significant commercial advantage from the intellectual property that we develop or license. Some countries also have compulsory licensing laws under which a patent owner may be compelled to grant licenses to third parties. In addition, some countries limit the enforceability of patents against government agencies or government contractors. In those countries, the patent owner may have limited remedies, which could materially diminish the value of such patents. If we are forced to grant a license to third parties with respect to any patents relevant to our business, our competitive position may be impaired. Obtaining and maintaining our patent protection depends on compliance with various procedural, document submission, fee payment and other requirements imposed by government patent agencies, and our patent protection could be reduced or eliminated for non- compliance with these requirements. Periodic maintenance fees, renewal fees, annuity fees and various other government fees on patents and / or applications will be due to be paid to the USPTO and various government patent agencies outside of the United States over the lifetime of our patents and / or applications and any patent rights we may obtain in the future. Furthermore, the USPTO and various non- United States government patent agencies require compliance with several procedural, documentary, fee payment and other similar provisions during the patent application process. In many cases, an inadvertent lapse of a patent or patent application can be cured by payment of a late fee or by other means in accordance with the applicable rules. There are situations, however, in which non- compliance can result in abandonment or lapse of the patents or patent applications, resulting in partial or complete loss of patent rights in the relevant jurisdiction. In such an event, potential competitors might be able to enter the market, which could have a material adverse effect on our business. Changes in patent laws or patent jurisprudence could diminish the value of patents in general, thereby impairing our ability to protect our product candidates. As is the case with other biotechnology companies, our success is heavily dependent on intellectual property, particularly patents. Obtaining and enforcing patents in the biotechnology and genetic medicine industries involve both technological and legal complexity. Therefore, obtaining and enforcing biotechnology and genetic medicine patents is costly, time- consuming and inherently uncertain. In addition, the Leahy- Smith America Invents Act ~~(~~**, or the** AIA ~~)~~, which was passed in September 2011, resulted in significant changes to the U. S. patent system. An important change introduced by the AIA is that, as of March 16, 2013, the United States transitioned from a " first- to- invent " to a " first- to- file " system for deciding which party should be granted a patent when two or more patent applications are filed by different parties claiming the same invention. Under a " first- to- file " system, assuming the other requirements for patentability are met, the first inventor to file a patent application generally will be entitled to a patent on the invention regardless of whether another inventor had made the invention earlier. A third party that files a patent application in the USPTO after that date but before us could therefore be awarded a patent covering an invention of ours even if we made the invention before it was made by the third party. This will require us to be cognizant going forward of the time from invention to filing of a patent application and be diligent in filing patent applications, but circumstances could prevent us from promptly filing patent applications on our inventions. Among some of the other changes introduced by the AIA are changes that limit where a patentee may file a patent infringement suit and providing opportunities for third parties to challenge any issued patent in the USPTO. This applies to all of our U. S. patents, even those issued before March 16, 2013. 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. Accordingly, a third party may attempt to use the USPTO procedures to invalidate our patent claims that would not have been invalidated if first challenged by the third party as a defendant in a district court action. It is not clear what, if any, impact the AIA will have on the operation of our business. However, the AIA and its implementation could increase the uncertainties and costs surrounding the prosecution of our or our licensors' patent applications and the enforcement or defense of our or our licensors' issued patents. We may become involved in opposition, interference, derivation, inter partes review or other proceedings challenging our or our licensors' patent rights, and the outcome of any proceedings are highly uncertain. An adverse determination in any such proceeding could reduce the scope of, or invalidate, our owned or in- licensed patent rights, allow third parties to commercialize our technology or products and compete directly with us, without payment to us, or result in our inability to manufacture or commercialize products without infringing third- party patent rights. In addition, the United States federal government retains certain rights in inventions produced with its financial assistance under the Bayh- Dole Act. The federal government retains a " nonexclusive, nontransferable, irrevocable, paid- up license " for its own benefit. The Bayh- Dole Act also provides federal agencies with " march- in rights. " March- in rights ~~allows~~ **allow** the government, in specified

circumstances, to require the contractor or successors in title to the patent to grant a " nonexclusive, partially exclusive, or exclusive license " to a " responsible applicant or applicants. " If the patent owner refuses to do so, the government may grant the license itself. Some of our licensed patents are subject to the provisions of the Bayh- Dole Act. If our licensors fail to comply with the regulations of the Bayh- Dole Act, they could lose title to any patents subject to such regulations, which could affect our license rights under the patents and our ability to stop others from using or commercializing similar or identical technology and products, or limit patent protection for our technology and products. Additionally, the U. S. Supreme Court has ruled on several patent cases in recent years either narrowing the scope of patent protection available in certain circumstances or weakening the rights of patent owners in certain situations, and there are other open questions under patent law that courts have yet to decisively address. In addition to increasing uncertainty with regard to our ability to obtain patents in the future, this combination of events has created uncertainty with respect to the value of patents ~~,~~ once obtained. Depending on decisions by Congress, the federal courts and the USPTO, the laws and regulations governing patents could change in unpredictable ways and could weaken our ability to obtain new patents or to enforce our existing patents and patents that we might obtain in the future. In addition, the European patent system is relatively stringent in the type of amendments that are allowed during prosecution, but the complexity and uncertainty of European patent laws has also increased in recent years. Complying with these laws and regulations could limit our ability to obtain new patents in the future that may be important for our business. If our trademarks and trade names are not adequately protected, then we may not be able to build name recognition in our markets of interest and our business may be adversely affected. Our current or future trademarks or trade names may be challenged, infringed, circumvented or declared generic or descriptive or determined to be infringing on other marks. We may not be able to protect our rights to these trademarks and trade names or may be forced to stop using these names, which we need for name recognition by potential partners or customers in our markets of interest. During trademark registration proceedings, we may receive rejections of our applications by the USPTO or in other foreign jurisdictions. Although we would be given an opportunity to respond to those rejections, we may be unable to overcome such rejections. In addition, in the USPTO and in comparable agencies in many foreign jurisdictions, third parties are given an opportunity to oppose pending trademark applications and to seek to cancel registered trademarks. Opposition or cancellation proceedings may be filed against our trademarks, and our trademarks may not survive such proceedings. If we are unable to establish name recognition based on our trademarks and trade names, we may not be able to compete effectively and our business may be adversely affected. We may license our trademarks and trade names to third parties, such as distributors. Although these license agreements may provide guidelines for how our trademarks and trade names may be used, a breach of these agreements or misuse of our trademarks and tradenames by our licensees may jeopardize our rights in or diminish the goodwill associated with our trademarks and trade names. Moreover, any name we have proposed to use with our product candidate in the United States must be approved by the FDA, regardless of whether we have registered it, or applied to register it, as a trademark. Similar requirements exist in Europe. The FDA typically conducts a review of proposed product names, including an evaluation of potential for confusion with other product names. If the FDA (or an equivalent administrative body in a foreign jurisdiction) objects to any of our proposed proprietary product names, we may be required to expend significant additional resources in an effort to identify a suitable substitute name that would qualify under applicable trademark laws, not infringe the existing rights of third parties and be acceptable to the FDA. Furthermore, in many countries, owning and maintaining a trademark registration may not provide an adequate defense against a subsequent infringement claim asserted by the owner of a senior trademark. At times, competitors or other third parties may adopt trade names or trademarks similar to ours, thereby impeding our ability to build brand identity and possibly leading to market confusion. In addition, there could be potential trade name or trademark infringement claims brought by owners of other registered trademarks or trademarks that incorporate variations of our registered or unregistered trademarks or trade names. If we assert trademark infringement claims, a court may determine that the marks we have asserted are invalid or unenforceable, or that the party against whom we have asserted trademark infringement has superior rights to the marks in question. In this case, we could ultimately be forced to cease use of such trademarks. Risks related to legal and regulatory compliance matters Our current and future relationships with customers, healthcare providers, including physicians, and third-party payors may be subject, directly or indirectly, to federal and state healthcare fraud and abuse laws, false claims laws, health information privacy and security laws and other healthcare laws and regulations. If we are unable to comply, or have not fully complied, with such laws, we could face substantial penalties. We are currently or will in the future be subject to healthcare regulation and enforcement by the U. S. federal government and the states in which we will conduct our business once our product candidates are approved by the FDA and commercialized in the United States. In addition to the FDA' s restrictions on marketing of pharmaceutical products, the U. S. healthcare laws and regulations that may affect our ability to operate include: the federal fraud and abuse laws, including the federal anti- kickback and false claims laws; federal data privacy and security laws; and federal transparency laws related to payments and / or other transfers of value made to physicians and other healthcare professionals and teaching hospitals. For more information, see the section titled " Business – Government Regulation – Other Healthcare Laws and Compliance Requirements. " Many states have similar laws and regulations that may differ from each other and federal law in significant ways, thus complicating compliance efforts. For example, states have anti- kickback and false claims laws that may be broader in scope than analogous federal laws and may apply regardless of payor. In addition, state ~~data~~ **laws regarding the** privacy **and** ~~laws that protect the~~ security of health information may differ from each other and may not be preempted by federal law. Moreover, several states have enacted legislation requiring pharmaceutical manufacturers to, among other things, establish marketing compliance programs, file periodic reports with the state, make periodic public disclosures on sales and marketing activities, report information related to drug pricing, require the registration of sales representatives, and prohibit certain other sales and marketing practices. These laws may adversely affect our sales, marketing and other activities with respect to any product candidate for which we receive approval to market in the United States by imposing administrative and compliance burdens on us. It is possible that governmental authorities will conclude that our

current or future business practices may not comply with current or future statutes, regulations or case law involving applicable fraud and abuse or other healthcare laws and regulations. If our operations are found to be in violation of any of these laws or any other governmental regulations that may apply to us, we may be subject to significant penalties, including, without limitation, civil, criminal and administrative penalties, damages, fines, disgorgement, individual imprisonment, exclusion from participating in federal and state funded healthcare programs, such as Medicare and Medicaid, additional reporting requirements and oversight if we become subject to a corporate integrity agreement or similar agreement to resolve allegations of non-compliance with these laws, contractual damages, diminished profits and future earnings, reputational harm and the curtailment or restructuring of our operations, any of which could harm our business. The risk of being found in violation of these laws is increased by the fact that many of them have not been fully interpreted by the regulatory authorities or the courts, and their provisions are open to a variety of interpretations. Efforts to ensure that our business arrangements with third parties will comply with applicable healthcare laws and regulations will involve substantial costs. Any action against us for violation of these laws, even if we successfully defend against it, could cause us to incur significant legal expenses and divert our management' s attention from the operation of our business. The shifting compliance environment and the need to build and maintain robust and expandable systems to comply with multiple jurisdictions with different compliance and / or reporting requirements increases the possibility that a healthcare company may run afoul of one or more of the requirements. Even if we obtain FDA or EMA approval for any of our product candidates in the United States or European Union, we may never obtain approval for or commercialize any of them in any other jurisdiction, which would limit our ability to realize their full market potential. In order to market any products in any particular jurisdiction, we must establish and comply with numerous and varying regulatory requirements on a country- by- country basis regarding safety and efficacy. Approval by the FDA in the United States or the EMA in the European Union does not ensure approval by regulatory authorities in other countries or jurisdictions. However, the failure to obtain approval in one jurisdiction may negatively impact our ability to obtain approval elsewhere. In addition, clinical trials conducted in one country may not be accepted by regulatory authorities in other countries, and regulatory approval in one country does not guarantee regulatory approval in any other country. Approval processes vary among countries and can involve additional product testing and validation and additional administrative review periods. Seeking foreign regulatory approval could result in difficulties and increased costs for us and require additional preclinical studies or clinical trials which could be costly and time consuming. Regulatory requirements can vary widely from country to country and could delay or prevent the introduction of our products in those countries. We do not have any product candidates approved for sale in any jurisdiction, including in international markets. If we fail to comply with regulatory requirements in international markets or to obtain and maintain required approvals, or if regulatory approvals in international markets are delayed, our target market will be reduced and our ability to realize the full market potential of any product we develop will be unrealized. Even if we receive regulatory approval of our product candidates, we will be subject to 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 product candidate for which we obtain marketing approval will be subject to ongoing regulatory requirements for, among other things, manufacturing processes, submission of post- approval clinical data and safety information, labeling, packaging, distribution, adverse event reporting, storage, recordkeeping, export, import, advertising, promotional activities and product tracking and tracing. These requirements include submissions of safety and other post- marketing information and reports, establishment registration and drug listing requirements, applicable tracking and tracing requirements, continued compliance with cGMP requirements relating to manufacturing, quality control, quality assurance and corresponding maintenance of records and documents, requirements regarding the distribution of samples to physicians and recordkeeping and cGCP requirements for any clinical trials that we conduct post- approval. Any regulatory approvals that we receive for our product candidates or any future product candidates may also be subject to a REMS, limitations on the approved indicated uses for which the product may be marketed or to the conditions of approval, or requirements that we conduct potentially costly post- marketing testing, including Phase 4 trials and surveillance to monitor the quality, safety and efficacy of the product. An unsuccessful post- marketing study or failure to complete such a study could result in the withdrawal of marketing approval. We will further be required to immediately report any serious and unexpected adverse events and certain quality or production problems with our products to regulatory authorities along with other periodic reports. The FDA and EMA closely regulate the post- approval marketing and promotion of genetic therapy medicines to ensure they are marketed only for the approved indications and in accordance with the provisions of the approved labeling. However, companies may share truthful and not misleading information that is otherwise consistent with a product' s FDA approved labeling. The FDA imposes stringent restrictions on manufacturers' communications regarding off-label use and if we market our products for uses beyond their approved diseases, we may be subject to enforcement action for off- label marketing. Violations of the FDCA, relating to the promotion of prescription drugs for unapproved uses may lead to enforcement actions and investigations alleging violations of federal and state health care fraud and abuse laws, as well as state consumer protection laws. The holder of an approved BLA must submit new or supplemental applications and obtain prior approval for certain changes to the approved product, product labeling, or manufacturing process. A company that is found to have improperly promoted off- label uses of their products may be subject to significant civil, criminal and administrative penalties. In addition, later discovery of previously unknown adverse events or other problems with our products, manufacturers or manufacturing processes, including adverse events of unanticipated severity or frequency, or with our third- party manufacturers or manufacturing processes, or failure to comply with regulatory requirements, may yield various results, including: • restrictions on manufacturing such products; • restrictions on the labeling or marketing of a product; • restrictions on product distribution or use; • refusal to allow entry into supply contracts, including government contracts; • requirements to conduct post- marketing studies or clinical trials; • warning or untitled letters, or holds on clinical trials; • withdrawal of the products from the market; • refusal to approve pending applications or supplements to approved applications that we submit; •

recall of products; • fines, restitution or disgorgement of profits or revenues; • suspension or withdrawal of marketing approvals; • refusal to permit the import or export of our products; • product seizure or detention; or • injunctions or the imposition of administrative, civil or criminal penalties or monetary fines. The FDA' s policies, and the policies of foreign regulatory agencies, may change and additional government regulations may be enacted that could prevent, limit or delay regulatory approval of our product candidates. We also cannot predict the likelihood, nature or extent of government regulation that may arise from future legislation or administrative or executive action, either in the United States or abroad. For example, executive orders or other actions could impose significant burdens on, or otherwise materially delay, **the** FDA' s ability to engage in routine oversight activities such as implementing statutes through rulemaking, issuance of guidance, and review and approval of marketing applications. If such executive actions were to impose restrictions on **the** FDA' s ability to engage in oversight and implementation activities in the normal course, our business could be negatively impacted. 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 which would adversely affect our business, prospects and ability to achieve or sustain profitability. Any government investigation of alleged violations of law could require us to expend significant time and resources in response and could generate negative publicity. The occurrence of any event or penalty described above may inhibit our ability to commercialize our current product candidates or any future product candidates and harm our business, financial condition, results of operations and prospects. Enacted and future healthcare legislation may increase the difficulty and cost for us to progress our clinical programs and obtain marketing approval of and commercialize our product candidates and may affect the prices we may set. In the United States, the European Union and other jurisdictions, there have been, and we expect there will continue to be, a number of legislative and regulatory changes and proposed changes to the healthcare system that could affect our future results of operations. In particular, there have been and continue to be a number of initiatives at the U. S. federal and state levels that seek to reduce healthcare costs and improve the quality of healthcare. For more information, see the below section titled " Business — Government Regulation — Healthcare Reform. " The continuing efforts of the government, insurance companies, managed care organizations and other payers of healthcare services to contain or reduce costs of healthcare may adversely affect: • the demand for any of our product candidates, if approved; • the ability to set a price that we believe is fair for any of our product candidates, if approved; • our ability to generate revenues and achieve or maintain profitability; • the level of taxes that we are required to pay; and • the availability of capital. Legislative and regulatory proposals have been made to expand post- approval requirements and restrict sales and promotional activities for pharmaceutical and biologic products. We cannot be sure whether additional legislative changes will be enacted, or whether FDA regulations, guidance or interpretations will be changed, or what the impact of such changes on the marketing approvals of our product candidates, if any, may be. In addition, increased scrutiny by Congress of the FDA' s approval process may significantly delay or prevent marketing approval, as well as subject us to more stringent product labeling and post- marketing testing and other requirements. Moreover, payment methodologies may be subject to changes in healthcare legislation and regulatory initiatives. For example, CMS may develop new payment and delivery models, such as bundled payment models. In addition, recently there has been heightened governmental scrutiny over the manner in which manufacturers set prices for their marketed products, which has resulted in several U. S. presidential executive orders, Congressional inquiries and proposed and enacted federal legislation designed to, among other things, bring more transparency to drug pricing, reduce the cost of prescription drugs under Medicare, and review the relationship between pricing and manufacturer patient programs. We expect that additional U. S. federal healthcare reform measures will be adopted in the future, any of which could limit the amounts that the U. S. federal government will pay for healthcare products and services, which could result in reduced demand for our product candidates or additional pricing pressures and could negatively affect our customers and accordingly, our financial operations. Individual states in the United States have also increasingly passed legislation and implemented regulations designed to control pharmaceutical and biological product pricing, including price or patient reimbursement constraints, discounts, restrictions on certain product access and marketing cost disclosure and transparency measures, and, in some cases, designed to encourage importation from other countries and bulk purchasing. Legally mandated price controls on payment amounts by third- party payors or other restrictions could harm our business, results of operations, financial condition and prospects. In addition, regional healthcare authorities and individual hospitals are increasingly using bidding procedures to determine what pharmaceutical products and which suppliers will be included in their prescription drug and other healthcare programs. This could reduce the ultimate demand for our product candidates or put pressure on our product pricing. In the European Union, similar political, economic and regulatory developments may affect our ability to profitably commercialize our product candidates, if approved. In addition to continuing pressure on prices and cost containment measures, legislative developments at the European Union or member state level may result in significant additional requirements or obstacles that may increase our operating costs. The delivery of healthcare in the European Union, including the establishment and operation of health services and the pricing and reimbursement of medicines, is almost exclusively a matter for national, rather than European Union, law and policy. National governments and health service providers have different priorities and approaches to the delivery of health care and the pricing and reimbursement of products in that context. In general, however, the healthcare budgetary constraints in most ~~EU~~ **European Union** member states have resulted in restrictions on the pricing and reimbursement of medicines by relevant health service providers. Coupled with ever- increasing European Union and national regulatory burdens on those wishing to develop and market products, this could prevent or delay marketing approval of our product candidates, restrict or regulate post- approval activities and affect our ability to commercialize our product candidates, if approved. In markets outside of the United States and the European Union, reimbursement and healthcare payment systems vary significantly by country, and many countries have instituted price ceilings on specific products and therapies. We cannot predict the likelihood, nature or extent of government regulation that may arise from future legislation or administrative action in the United States, the European Union or any other jurisdiction. If we or any third parties we may engage are slow or unable to adapt to changes in

existing requirements or the adoption of new requirements or policies, or if we or such third parties are not able to maintain regulatory compliance, our product candidates may lose any regulatory approval that may have been obtained and we may not achieve or sustain profitability. Changes in funding for the FDA and other government agencies could hinder their ability to hire and retain key leadership and other personnel, or otherwise prevent new products and services from being developed or commercialized in a timely manner, which could negatively impact our business. The ability of the FDA to review and approve new products can be affected by a variety of factors, including government budget and funding levels, ability to hire and retain key personnel, accept the payment of user fees, and statutory, regulatory and policy changes. Average review times at the agency have fluctuated in recent years as a result. In addition, government funding of other government agencies that fund research and development activities is subject to the political process, which is inherently fluid and unpredictable. **Changes in the leadership of the FDA and other federal agencies under the Trump administration, including return- to- office policy, hiring freeze, layoffs, and other policies implemented by the Department of Government Efficiency, may lead to changes in the operations of the FDA, which may have a material impact on the industry and our clinical development plans.** Disruptions at the FDA and other agencies may also slow the time necessary for new drugs to be reviewed and / or approved by necessary government agencies, which would adversely affect our business. **Additionally** ~~For example~~ , ~~over~~ **changes in** the **FDA leadership** ~~last several years~~ , ~~including for 35 days beginning on December 22, 2018, the U. S. government has shut down several times and certain~~ regulatory **actions and** ~~agencies, such as the~~ **other** ~~FDA~~ **actions under the new Trump administration** , **may result in delays in regulatory approval** ~~have had to furlough critical FDA employees and stop critical activities~~ . Our business depends upon the ability of the FDA to accept and review our potential regulatory filings. If a prolonged government shutdown occurs **or if a significant number of federal employees are laid off or leave federal agencies** , it could significantly impact the ability of the FDA to timely review and process our regulatory submissions, which could have a material adverse effect on our ability to advance clinical development of our product candidates. If we are unable to establish sales, marketing and distribution capabilities either on our own or in collaboration with third parties, we may not be successful in commercializing our product candidates or realizing the synergies in the target diseases of our programs, even if they are approved. We do not have any infrastructure for the sales, marketing or distribution of our products, and the cost of establishing and maintaining such an organization may exceed the cost- effectiveness of doing so. We expect to build a focused sales, distribution and marketing infrastructure to market our product candidates in the United States and European Union, if approved. There are significant expenses and risks involved with establishing our own sales, marketing and distribution capabilities, including our ability to hire, retain and appropriately incentivize qualified individuals, generate sufficient sales leads, provide adequate training to sales and marketing personnel, and effectively manage a geographically dispersed sales and marketing team. Any failure or delay in the development of our internal sales, marketing and distribution capabilities could delay any product launch, which would adversely impact the commercialization of our product candidates. Additionally, if the commercial launch of our product candidates for which we recruit a sales force and establish marketing capabilities is delayed or does not occur for any reason, we would have prematurely or unnecessarily incurred these commercialization expenses. This may be costly, and our investment would be lost if we cannot retain or reposition our sales and marketing personnel. We may not have the resources in the foreseeable future to allocate to the sales and marketing of our product candidates in certain international markets. Therefore, our future sales in these markets will largely depend on our ability to enter into and maintain collaborative relationships for such capabilities, the collaborator' s strategic interest in the product and such collaborator' s ability to successfully market and sell the product. We may pursue collaborative arrangements regarding the sale and marketing of LX2006 ~~, LX1001~~ or LX2020, if approved, for certain markets overseas; however, we cannot assure that we will be able to establish or maintain such collaborative arrangements, or if able to do so, that they will have effective sales forces. If we are unable to build our own sales force or negotiate a collaborative relationship for the commercialization of LX2006, ~~LX1001~~ or LX2020, or any of our other product candidates, if approved, we may be forced to delay the potential commercialization of LX2006, ~~LX1001~~ or LX2020 or any of our other product candidates or reduce the scope of our sales or marketing activities for LX2006 ~~, LX1001~~ or LX2020 or any of our other product candidates. If we elect to increase our expenditures to fund commercialization activities internationally, we will need to obtain additional capital, which may not be available to us on acceptable terms, or at all. We could enter into arrangements with collaborative partners at an earlier stage than otherwise would be ideal and we may be required to relinquish rights to LX2006, ~~LX1001~~ or LX2020 or any of our other product candidates or otherwise agree to terms unfavorable to us, any of which may have an adverse effect on our business, operating results and prospects. If we are unable to establish adequate sales, marketing and distribution capabilities, either on our own or in collaboration with third parties, we will not be successful in commercializing LX2006, ~~LX1001~~ or LX2020 or any of our other product candidates, if approved, and may not become profitable and may incur significant additional losses. We will be competing with many companies that currently have extensive and well- funded marketing and sales operations. Without an internal team or the support of a third party to perform marketing and sales functions, we may be unable to compete successfully against these more established companies. If we obtain approval to commercialize any products outside of the United States or the European Union, a variety of risks associated with international operations could adversely affect our business. If LX2006 ~~, LX1001~~ , LX2020 or any of our other product candidates are approved for commercialization, we may seek to enter into agreements with third parties to market them in certain jurisdictions outside the United States and the European Union. We expect that we would be subject to additional risks related to international pharmaceutical operations, including: • different regulatory requirements for drug and biologic approvals and rules governing drug and biologic commercialization in foreign countries; • reduced protection for intellectual property rights; • foreign reimbursement, pricing and insurance regimes; • unexpected changes in tariffs, trade barriers and regulatory requirements; • economic weakness, including inflation, or political instability in particular foreign economies and markets; • foreign currency fluctuations, which could result in increased operating expenses and reduced revenues, and other obligations incident to doing business in another country; • business interruptions

resulting from geopolitical actions, including war and terrorism or natural disasters including earthquakes, typhoons, floods and fires, or from economic or political instability; • greater difficulty with enforcing our contracts; • potential noncompliance with the U. S. Foreign Corrupt Practices Act, or the FCPA, the U. K. Bribery Act 2010 and similar anti- bribery and anticorruption laws in other jurisdictions; and • production shortages resulting from any events affecting raw material supply or manufacturing capabilities abroad. In addition, there are complex regulatory, tax, labor and other legal requirements imposed by individual countries in Europe with which we will need to comply. If we are unable to successfully manage the challenges of international expansion and operations, our business and operating results could be harmed. 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 harm 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. From time to time and in the future, our operations may involve the use of hazardous and flammable materials, including chemicals and biological materials, and may also produce hazardous waste products. Even if we contract with third parties for the disposal of these materials and waste products, we cannot completely eliminate the risk of contamination or injury resulting from these materials. In the event of contamination or injury resulting from the use or disposal of our hazardous materials, we could be held liable for any resulting damages, and any liability could exceed our resources. We also could incur significant costs associated with civil or criminal fines and penalties for failure to comply with such laws and regulations. We maintain workers' compensation insurance to cover us for costs and expenses we may incur due to injuries to our employees, but this insurance may not provide adequate coverage against potential liabilities. However, we do not maintain insurance for environmental liability or toxic tort claims that may be asserted against us. In addition, we may incur substantial costs in order to comply with current or future environmental, health and safety laws and regulations. Environmental laws and regulations may impair our research, development or production efforts. In addition, failure to comply with these laws and regulations may result in substantial fines, penalties or other sanctions. We are subject to a variety of privacy ~~and ,~~ data **protection, and** ~~security~~ **cybersecurity** laws, rules, regulations, policies, industry standards and contractual obligations, and our failure to comply with them could harm our business. We maintain **and otherwise process** a large quantity of sensitive information, including confidential business and personal information in connection with the conduct of our clinical trials and related to our employees **and others** , and we are subject to laws and regulations governing the privacy and security of such information. In the United States, ~~there are~~ numerous federal and state privacy and ~~data security~~ **cybersecurity** laws and regulations ~~governing~~ --- **govern** the collection, use, disclosure and protection of personal information, including federal and state health information privacy laws, federal and state security breach notification laws and federal and state consumer protection laws. The legislative and regulatory landscape for privacy ~~and ,~~ data protection **and cybersecurity** continues to evolve, and there has been an increasing focus on privacy ~~and ,~~ data protection **and cybersecurity** issues, which may affect our business and is expected to increase our compliance costs and exposure to liability. In the United States, numerous federal and state laws and regulations could apply to our operations or the operations of our partners, including state and federal data breach notification laws, state health information privacy **and cybersecurity** laws and federal and state consumer protection laws and regulations that govern the collection, use, disclosure and protection of health- related and other personal information. Among these regulations are: Section 5 of the Federal Trade Commission Act, which prohibits unfair or deceptive commercial practices; new rules adopted by the SEC in July 2023, which require public companies to disclose material cybersecurity incidents they experience and to disclose on an annual basis material information regarding their cybersecurity risk management, strategy, and governance; and **the** HIPAA, as amended by HITECH, and the regulations promulgated thereunder. We may obtain health ~~information~~ **and other data** from third parties, including research institutions from which we obtain clinical trial data, that are subject to privacy and security requirements under HIPAA, and depending on the facts and circumstances, we could be subject to significant penalties if we obtain, use or disclose individually identifiable health information in a manner that is not authorized or permitted by HIPAA. In the European Economic Area, or **the** EEA, and the United Kingdom, or **the** UK, the collection, use, disclosure, transfer or other processing of personal data, including clinical trial data, of individuals is governed by the General Data Protection Regulation, or ~~EU~~ **the European Union** GDPR (with ~~regards~~ **regard** to the EEA) **,** and UK GDPR (with ~~regards~~ **regard** to the UK), as well as applicable national data protection legislation and requirements. In this document, " GDPR " refers to both the ~~EU~~ **European Union** GDPR and the UK GDPR, unless specified otherwise. The GDPR is wide **-** ranging in scope **and** imposes numerous requirements on companies that process personal data, including requirements relating to processing health and other sensitive data, obtaining consent of the individuals to whom the personal data relates, providing information to individuals regarding data processing activities, implementing safeguards to protect the security and confidentiality of personal data, providing notification of data breaches, and taking certain measures when engaging third- party processors. The GDPR imposes substantial fines for breaches and violations (up to the greater of € 20 million (£ 17. 5 million for the UK) or 4 % of our consolidated annual worldwide gross revenue), and confers a private right of action on data subjects and consumer associations to lodge complaints with supervisory authorities, seek judicial remedies and obtain compensation for damages resulting from violations of the GDPR. The GDPR also includes restrictions on cross- border data transfers of personal data to countries outside the EEA and the UK that are not considered by the European Commission ~~and~~ **or** UK government as providing " adequate " protection to personal data, or third countries, including the United States, unless a valid GDPR transfer mechanism (for example, the European Commission **-** approved Standard Contractual Clauses, or SCCs, and the UK International Data Transfer Agreement / Addendum, or UK IDTA) has been put in place. Where relying on the SCCs or UK IDTA for data transfers, we may also be required to carry out transfer impact assessments to assess whether the recipient is subject to local laws which allow public authority access to personal data. The international transfer obligations under the EEA and UK data protection regimes ~~will~~ require significant effort and cost, and may result in us needing to make strategic considerations around where EEA and UK personal data is transferred and which service providers we can utilize for ~~the~~ processing ~~of~~ EEA and UK personal data ~~. Although the UK is regarded as a third~~

~~country under the EU GDPR, the European Commission has issued a decision recognizing the UK as providing adequate protection under the EU GDPR, or Adequacy Decision, and, therefore, transfers of personal data originating in the EEA to the UK remain unrestricted~~ . The UK government has ~~confirmed that personal data transfers from the UK to the EEA remain free flowing. The UK Government has~~ also ~~now~~ introduced a Data Protection and Digital Information Bill, or **the** UK Bill, into the UK legislative process. The aim of the UK Bill is to reform the UK' s data protection regime following Brexit. If passed, the final version of the UK Bill may **reduce** ~~have the effect of further altering~~ the similarities between the UK and EEA data protection regime and threaten the ~~UK Adequacy Decision from the~~ European Commission **' s determination that the UK provides" adequate" protection to personal data** . **Additionally,** ~~This~~ **this adequacy determination is subject to renewal in 2025. Any loss by the UK of its adequacy determination by the European Commission** may lead to additional compliance costs and could increase our overall risk. The respective provisions and enforcement of the ~~EU~~ **European Union** GDPR and UK GDPR may further diverge in the future and create additional regulatory challenges and uncertainties. Compliance with these and any other applicable privacy ~~and~~ **,** data **protection and** ~~security~~ **cybersecurity** laws and regulations is a rigorous and time- intensive process, and we may be required to put in place additional mechanisms ensuring compliance with ~~the~~ new **and evolving laws and regulations. Further, many privacy,** data protection ~~rules~~ **, and cybersecurity regimes are evolving and are inconsistent across jurisdictions** . If we fail **, or are alleged to fail,** to comply with any such laws or regulations, we may face **regulatory inquiries, investigations, and other proceedings, private claims and litigation, and** significant fines and penalties that could adversely affect our business, financial condition and results of operations ~~. Furthermore, the laws are not consistent, and compliance in the event of a widespread data breach is costly~~ . In addition, states are constantly adopting new laws or amending existing laws, requiring attention to frequently changing regulatory requirements. For example, California enacted the California Consumer Privacy Act, or CCPA, which took effect on January 1, 2020, became enforceable ~~by the California Attorney General~~ on July 1, 2020 and has been dubbed the first " GDPR- like " law in the United States. The CCPA gives California residents expanded rights to access and delete their personal information, opt out of certain personal information sharing and receive detailed information about how their personal information is used by requiring covered companies to provide new disclosures to California consumers (as that term is broadly defined) and provide such consumers new ways to opt out of certain sales of personal information. The CCPA provides for civil penalties for violations, as well as a private right of action for data breaches that is expected to increase data breach litigation. Further, the California Privacy Rights Act, or CPRA, which became effective as of January 1, 2023, amended the CCPA and imposes additional **privacy** ~~data protection~~ obligations on companies doing business in California, including additional consumer rights processes, limitations on data uses, new audit requirements for higher risk data and opt **-** outs for certain uses of sensitive data. The amendments introduced by the CPRA also created a new California ~~data~~ **Privacy** ~~protection~~ **Protection** ~~agency~~ **Agency** ~~authorized~~ to ~~issue substantive regulations~~ **implement and enforce this legislation** , and it is anticipated that this development could result in increased privacy and information security enforcement. Although the CCPA, as amended by the CPRA, currently exempts certain health- related information, including clinical trial data, the CCPA may increase our compliance costs and potential liability if we expand our operations into California. Similar broad consumer privacy laws have been enacted in Colorado, Connecticut, **Delaware, Florida, Indiana, Iowa, Kentucky, Maryland, Minnesota, Montana, Nebraska, New Hampshire, New Jersey, Oregon, Rhode Island, Tennessee, Texas, Utah, and** Virginia ~~, Utah, Iowa and Indiana~~ and have been proposed in numerous other states and at the federal level. If passed, these bills may ~~have potentially conflicting~~ **provide for various** requirements that would make compliance challenging **, particularly in light of certain conflicting and otherwise various requirements in the state privacy laws that have been enacted** . In addition to these consumer privacy laws, the state of Washington ~~recently~~ **has** enacted ~~a comprehensive~~ **health- focused** privacy ~~bill~~ **legislation** , ~~called~~ the My Health **,** My Data Act ~~.~~ **, which became** ~~Effective~~ **effective in** March 2024 ~~,~~ **.** ~~this~~ **This** ~~new~~ law ~~will impose~~ **imposes** strict requirements on the collection, use and processing of **certain** health **-** related information that is not subject to HIPAA **. This law also provides for a private right of action** . Other states are considering bills with similar requirements. The Washington law and, if passed, the other state bills, will add additional complexity to our ~~existing~~ compliance obligations. With the GDPR, CCPA and other laws, regulations and other obligations relating to privacy and data protection imposing new and relatively burdensome obligations, and with ~~the~~ substantial uncertainty over the interpretation and application of these and other obligations, we may face challenges in addressing their requirements and making necessary changes to our policies and practices and may incur significant costs and expenses in an effort to do so ~~. We will continue to assess, develop, update, and adapt our practices, procedures, and policies in order to address existing and new requirements under applicable data privacy and protection laws and regulations. However, it is possible that both existing and new laws, regulations, and other obligations to which we are or may be subject, may be interpreted and applied in a manner that is inconsistent with our existing or future privacy and data protection practices. Any failure or perceived failure by us to comply with our obligations may result in governmental investigations or enforcement actions, litigation, claims, or public statements against us and could result in significant liability, cause harm to our brand and reputation, and otherwise materially and adversely affect our reputation and business~~ . We do not currently have ~~any~~ formal data privacy policies and procedures in place and have not completed formal assessments of whether we are in compliance with all applicable data privacy laws and regulations. ~~Additionally~~ **It is possible that both existing and new laws** , ~~if~~ **regulations, and other actual or asserted obligations to which we are or may be subject may be interpreted and applied in manners inconsistent with our existing or future privacy, data protection and cybersecurity practices. Any failure or perceived failure by us to comply with our current or future obligations relating to privacy, data protection or cybersecurity, or any actual or perceived failure by any** third ~~parties~~ **party** with which we work, such as **third- party CMOs, CROs, manufacturers, contractors, consultants, collaborators,** vendors **,** or service providers, **to comply with our applicable policies or other applicable obligations, may result in governmental investigations, enforcement actions, or other proceedings, litigation, claims, or public statements against us and could result in significant liability, cause harm to our**

brand and reputation, and otherwise materially and adversely affect our reputation and business. We may be subject to various governmental export control and trade sanctions laws and regulations that could impair our ability to compete in international markets or subject us to liability if we violate **.We are also subject** to ~~certain countries~~ **export control** and ~~end- users~~ **import laws and regulations** , **including the U.S.Export Administration Regulations,U.S.Customs regulations** and ~~for certain end- uses~~ **various economic and trade sanctions regulations administered by the U** . **S.Treasury Department' s Office** ~~The process for obtaining necessary licenses may be time- consuming or unsuccessful,potentially causing delays in sales or losses~~ of **Foreign Assets Controls** ~~sales opportunities and these licenses may not be issued~~ .Compliance with applicable regulatory requirements regarding the export ~~or import~~ of our ~~product~~ **products** ~~candidates~~ may create delays in the introduction of our ~~product~~ **products** ~~candidates~~ in international markets or,in some cases,prevent the export of our ~~product~~ **products** ~~candidates~~ to some countries altogether.Furthermore,U.S.export control laws and economic sanctions prohibit the shipment of certain ~~product~~ **products** ~~candidates~~ and services to countries ~~,territories~~ ,governments and persons targeted by U.S. **sanctions** applicable laws **and** ~~, rules or~~ regulations **could have negative consequences** ~~or~~ **for us** ~~our policies, such violations may also put our or our clinical trial and employee data~~ , including ~~personal data~~ **reputational harm** , ~~at risk~~ **government investigations** , ~~which could in turn have~~ **penalties, fines, settlements, investigations, criminal sanctions against us, our officers, or our employees, the closing down of facilities, including those of our suppliers** ~~an~~ **and** ~~adverse effect~~ **manufacturers, requirements to obtain export licenses, cessation of business activities in sanctioned countries, implementation of additional compliance programs, and prohibitions** on **the conduct of** our business. We are subject to U. S. and certain foreign anti- corruption laws and regulations ~~, export and import controls, sanctions and embargoes~~ . We could face liability and other serious consequences for violations. We are subject to anti- corruption laws and regulations, including the FCPA, the U. S. domestic bribery statute contained in 18 U. S. C. § 201, the U. S. Travel Act and ~~other state~~ **will be subject to local** and national anti- bribery laws in the countries in which we may conduct activities in the future. Anti- corruption laws are interpreted broadly and generally prohibit companies and their employees, agents, **representatives,** contractors and other third- party ~~collaborators~~ **intermediaries** from offering, promising, giving, or authorizing others to give anything of value, either directly or indirectly through third parties, to any person in the public or private sector to obtain or retain business. The FCPA also requires public companies to make and keep books and records that accurately and fairly reflect the transactions of the corporation and to devise and maintain an adequate system of internal accounting controls. Our business is heavily regulated and therefore involves significant interaction with public officials, including officials of non- United States governments. Additionally, in many other countries, the healthcare providers who prescribe pharmaceuticals are employed by their government, and therefore will be considered foreign officials for purposes of the FCPA. We also expect to rely on third parties for research, preclinical studies and clinical trials and / or to obtain necessary permits, licenses, patent registrations and other marketing approvals **on our behalf outside of the United States** . We can be held liable for the corrupt or other illegal activities of our employees, agents, **representatives,** CROs, contractors and other ~~collaborators and partners~~ **third- party intermediaries** , even if we do not explicitly authorize or have actual knowledge of such activities . We are also subject to export..... targeted by U. S. sanctions . There is no certainty that all of our employees, agents, **representatives** ~~suppliers, manufacturers~~ , contractors or **third- party intermediaries** ~~collaborators, or those of our affiliates,~~ will comply with all applicable anti- corruption ~~, export and import control, and sanctions~~ laws ~~and regulations, particularly given the high level of complexity of these laws~~ . **Allegations concerning or** ~~Violations~~ **violations** of these laws and regulations could result in **whistleblower complaints,** fines, **settlements, investigations,** criminal **or civil** sanctions ~~against us~~ , **adverse media coverage** ~~our~~ **or suspension** ~~officers, or our~~ **or** ~~employees~~ **debarment from government contracts** , **all** ~~the closing down~~ of **which** ~~facilities, including those of our suppliers and manufacturers, requirements to obtain export licenses, cessation of business activities in sanctioned countries, implementation of compliance programs, and prohibitions on the conduct of our business. Any such violations~~ could ~~include prohibitions on our ability to offer our products in one or more countries as well as difficulties in manufacturing or continuing to develop our products, and could materially~~ damage our reputation, our brand, our international expansion efforts, our ability to attract and retain employees, and our business, prospects, operating results and financial condition **. Responding to any investigation or action will likely result in a materially significant diversion of management' s attention and resources and significant defense costs and other professional fees** . Risks related to employee matters and managing our growth Our future success depends on our ability to attract and retain key executives and advisors and to attract, retain and motivate qualified personnel. We are highly dependent on the management, development, clinical, financial and business development expertise of our executive officers, particularly R. Nolan Townsend, our Chief Executive Officer and a member of our board of directors, Eric Adler, M. D., our Chief Medical Officer and Head of Research **, Jose Manuel Otero, our Chief Technical Officer, Kyle Rasbach, our Chief Financial Officer, Jenny R. Robertson, our Chief Legal Officer** , Sandi See Tai, M. D., our Chief Development Officer, as well as on the scientific expertise of our founder, Ronald G. Crystal, M. D., Professor and Chairman of Weill Cornell Medicine' s Department of Genetic Medicine. Each of our executive officers may currently terminate their employment with us at any time and we do not have an employment contract with Dr. Crystal. We do not maintain " key person " insurance for any of our executives or employees. Recruiting and retaining qualified executives, scientists and clinical personnel and, if we progress the development of our product pipeline toward scaling up for commercialization, manufacturing and sales and marketing personnel, will also be critical to our success. The loss of the services of our executive officers or other key employees, or our inability to recruit certain executives, could impede the achievement of our development and commercialization objectives and seriously harm our ability to successfully implement our business strategy. Furthermore, recruiting executive officers, or replacing executive officers and key employees may be difficult and may take an extended period of time because of the limited number of individuals in our industry with the breadth of skills and experience required to successfully develop, gain regulatory approval of and commercialize gene therapy products. Competition to hire from this limited pool is intense, and we have experienced and may continue to experience

challenges filling certain executive roles. We may be unable to hire, train, retain or motivate key personnel on acceptable terms given the competition among numerous pharmaceutical and biotechnology companies for similar personnel. We also experience competition for the hiring of scientific and clinical personnel from universities and research institutions. In addition, we rely on consultants and advisors, including scientific and clinical advisors, to assist us in formulating our development and commercialization strategy. Our consultants and advisors may be employed by employers other than us and may have commitments under consulting or advisory contracts with other entities that may limit their availability to us. If we are unable to continue to attract and retain high quality personnel, our ability to pursue our growth strategy will be limited. We expect to expand our clinical development, manufacturing and regulatory capabilities and potentially implement sales, marketing and distribution capabilities, and as a result, we may encounter difficulties in managing our growth, which could disrupt our operations. As of December 31, ~~2023~~ **2024**, we had ~~58~~ **72** full- time employees. As our development progresses, we expect to experience significant growth in the number of our employees and the scope of our operations, particularly in the areas of clinical product development, regulatory affairs and, if any of our product candidates receives marketing approval, sales, marketing and distribution. To manage our anticipated future growth, we must continue to implement and improve our managerial, operational and financial systems, expand our facilities and continue to recruit and train additional qualified personnel. Due to our limited financial resources and the limited experience of our management team in managing a company with such anticipated growth, we may not be able to effectively manage the expansion of our operations or recruit and train additional qualified personnel. Our choice to focus on multiple therapeutic areas may negatively affect our ability to develop adequately the specialized capability and expertise necessary for operations. The expansion of our operations may lead to significant costs and may divert our management and business development resources. Any inability to manage growth could delay the execution of our business plans or disrupt our operations. Our employees, independent contractors, consultants, collaborators, principal investigators, CROs, suppliers and vendors may be improperly classified and may engage in misconduct or other improper activities, including non- compliance with regulatory standards and requirements. We endeavor to properly classify our employees as exempt or non- exempt with respect to wage and hour laws (including, but not limited to, for purposes of minimum wage, overtime and applicable meal and rest periods), and we monitor and evaluate such classifications. Although there are no current, pending, or threatened claims or investigations against us asserting that any employees have been incorrectly classified as exempt, the possibility nevertheless exists that certain job roles could be deemed to have been incorrectly classified as exempt. In addition, we endeavor to classify our workforce properly, and we monitor and evaluate such classifications. Although there are no current, pending, or threatened claims or investigations against us asserting that any independent contractors have been incorrectly classified, the possibility nevertheless exists that certain contractors could be deemed to be employees. We are exposed to the risk that our employees, independent contractors, consultants, collaborators, principal investigators, CROs, suppliers and vendors may engage in fraudulent conduct or other illegal activity. Misconduct by these parties could include intentional, reckless and / or negligent conduct that violates FDA regulations, including those laws requiring the reporting of true, complete and accurate information to the FDA, manufacturing standards, federal and state healthcare laws and regulations, and laws that require the true, complete and accurate reporting of financial information or data. In particular, sales, marketing and business arrangements in the healthcare industry are subject to extensive laws and regulations intended to prevent fraud, kickbacks, self- dealing and other abusive practices. These laws and regulations may restrict or prohibit a wide range of pricing, discounting, marketing and promotion, sales commission, customer incentive programs and other business arrangements. Misconduct by these parties could also involve the improper use of individually identifiable information, including, without limitation, information obtained in the course of clinical trials, which could result in regulatory sanctions and serious harm to our reputation. Although we have adopted a code of business conduct and ethics, it is not always possible to identify and deter misconduct, and the precautions we take to detect and prevent this activity may not be effective in controlling unknown or unmanaged risks or losses or in protecting us from governmental investigations or other actions or lawsuits stemming from a failure to be in compliance with such laws or regulations. If any such actions are instituted against us, and we are not successful in defending ourselves or asserting our rights, those actions could have a significant impact on our business, including the imposition of significant civil, criminal and administrative penalties, including, without limitation, damages, fines, disgorgement, imprisonment, exclusion from participation in government healthcare programs, such as Medicare and Medicaid, additional reporting requirements and oversight if we become subject to a corporate integrity agreement or similar agreement to resolve allegations of non- compliance with these laws, and the curtailment or restructuring of our operations. The administrator of the 2023 Plan is authorized to exercise its discretion to reprice stock options and stock appreciation rights, and if a repricing occurs, there may be adverse consequences to our business. The administrator of the 2023 Plan, which is our compensation committee, is authorized, subject to the consent of any award holder whose award is materially impaired by such action, to reduce the exercise price of a stock option or stock appreciation right; to cancel a stock option or stock appreciation right in exchange for a different award, cash or other consideration; or to take any other action that is treated as a repricing under generally accepted accounting principles, or each such action, a repricing. We have no current expectation that a repricing will occur. However, if the administrator were to implement a repricing without seeking prior stockholder approval, certain proxy advisory firms and / or institutional investors may express a lack of support for the repricing, and proxy advisory firms may recommend an " against " or " withhold " vote for members of our compensation committee or ~~the~~ **our** board **of directors**. In addition, if we are required to hold an advisory vote on named executive officer compensation (known as a " say on pay " vote) at the time of, or subsequent to, any such repricing, it is likely, based on their current policies, that proxy advisory firms would issue an " against " recommendation on our say on pay proposal. Defending against negative recommendations with respect to our directors and / or say on pay proposal would require management attention, and could be costly and time- consuming. If our stockholders agree with proxy advisory firms' recommendations, we may need to make changes to our compensation and corporate governance practices, and perhaps the composition of our board **of directors** and its

committees, potentially leading to business disruptions and a negative impact on our stock price. Even absent negative reactions from proxy advisory firms and institutional investors, we may be required to recognize a compensation expense and the repricing will require management' s time and attention and the payment of administrative costs and attorney and accounting firm fees. As such, a repricing could cause a negative impact on our stock price, and adverse consequences to our business. Risks related to ownership of our common stock and our status as a public company The trading price of the shares of our common stock may be volatile, and purchasers of our common stock could incur substantial losses. The trading price of our common stock is likely to be highly volatile and could be subject to wide fluctuations in response to various factors, some of which are beyond our control, including limited trading volume. The stock market in general and the market for biopharmaceutical companies in particular have experienced extreme volatility that has often been unrelated to the operating performance of particular companies. As a result of this volatility, investors may not be able to sell their common stock at or above the price paid for the shares. The trading price for our common stock may be influenced by many factors, including those discussed in this " Risk Factors " section and elsewhere in this Annual Report on Form 10- K and: • the reporting of unfavorable preclinical and clinical results; • the commencement, enrollment or results of our clinical trials of LX2006, ~~LX1001,~~ LX2020 or any future clinical trials we may conduct, or changes in the development status of our product candidates; • any delay in our regulatory filings for LX2006 ~~, LX1001~~ , LX2020 or any other product candidate we may develop, and any adverse development or perceived adverse development with respect to the applicable regulatory authority' s review of such filings, including without limitation the FDA' s issuance of a " refusal to file " letter or a request for additional information; • adverse results from, delays in or termination of clinical trials; • adverse regulatory decisions, including failure to receive regulatory approval of our product candidates; • unanticipated serious safety concerns related to the use of LX2006, ~~LX1001 or~~ LX2020 or any other product candidates; • changes in financial estimates by us or by any equity research analysts who might cover our stock; • conditions or trends in our industry; • changes in the market valuations of similar companies; • stock trading price and volume fluctuations of comparable companies and, in particular, those that operate in the biopharmaceutical industry; • publication of research reports about us or our industry or positive or negative recommendations or withdrawal of research coverage by securities analysts; • announcements by us or our competitors of significant acquisitions, strategic partnerships or divestitures; • our relationships with our collaborators; • announcements of investigations or regulatory scrutiny of our operations or lawsuits filed against us; • investors' general perception of our company and our business; • recruitment or departure of key personnel; • overall performance of the equity markets; • trading volume of our common stock; • disputes or other developments relating to proprietary rights, including patents, litigation matters and our ability to obtain patent protection for our technologies; • significant lawsuits, including patent or stockholder litigation or employee or independent contractor litigation; • changes in the structure of healthcare payment systems; • unfavorable geopolitical and economic conditions; and • other events or factors, many of which are beyond our control. The global economy, including credit and financial markets and the banking sector, has experienced extreme volatility and disruptions, including, among other things, severely diminished liquidity and credit availability, declines in consumer confidence, declines in economic growth, increases in unemployment rates, supply chain shortages, increases in inflation rates, bank failures, higher interest rates **, changing foreign trade policies** and uncertainty about economic stability. For example, the ongoing wars in Ukraine and Israel have created volatility in the global capital markets and may have further global economic consequences, including disruptions of the global supply chain and energy markets. Any such volatility and disruptions may have adverse consequences on us or the third parties on whom we rely. If the equity and credit markets continue to deteriorate, it may make any necessary debt or equity financings more difficult to obtain in a timely manner or on favorable terms, more costly or more dilutive. Increased inflation rates can adversely affect us by increasing our costs, including labor and employee benefit costs. In addition, higher inflation and macro turmoil and uncertainty could also adversely affect our buyers and sellers, which could reduce demand for our products. These factors may negatively affect the trading price of our common stock, regardless of our actual operating performance. In addition, in the past, stockholders have initiated class action lawsuits against pharmaceutical and biotechnology companies following periods of volatility in the trading prices of these companies' stock. This risk is especially relevant for us because biopharmaceutical companies have experienced significant stock price volatility in recent years. Such litigation, if instituted against us, could cause us to incur substantial costs and divert management' s attention and resources, which could harm our business. ~~A significant portion of our total outstanding shares are restricted from resale but may be sold into the market in the near future. This could cause the trading price of our common stock to drop significantly, even if our business is doing well. Sales of a substantial number of shares of our common stock in the public market could occur at any time. These sales, or the perception in the market that the holders of a large number of shares intend to sell shares, could reduce the trading price of our common stock. We had 26, 646, 378 shares of common stock outstanding as of December 31, 2023, of which approximately 61. 9 % are subject to a 180- day lock- up period that began on November 2, 2023 and expires on April 30, 2024, provided under lock- up agreements executed in connection with our IPO. All of these shares will, however, be able to be resold after the expiration of the lock- up period, as well as pursuant to customary exceptions thereto or upon the waiver of the lock- up agreement by or on behalf of the underwriters. We registered shares of common stock subject to options or other equity awards issued or reserved for future issuance under our equity compensation plans. Those shares can be freely sold in the public market upon issuance, subject to the lock- up agreements. As restrictions on resale end, the trading price of our stock could decline if the holders of currently- restricted shares sell them or are perceived by the market as intending to sell them.~~ 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 is influenced by the research and reports that equity research analysts publish about us and our business. As a newly public company, we have only limited research coverage by equity research analysts. Equity research analysts may elect not to provide research coverage of our common stock, and such lack of research coverage may adversely affect the trading price of our common stock. While we currently 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 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~~ **cease** coverage of our company or ~~fails~~ **fail** to publish reports on us regularly, demand for our stock could decrease, which in turn could cause our stock price or trading volume to decline ~~. Our executive officers, directors and their affiliates, if they choose to act together, have the ability to significantly influence all matters submitted to stockholders for approval and may prevent new investors from influencing significant corporate decisions. Based on the number of shares of common stock outstanding as of December 31, 2023, our executive officers, directors and stockholders who own more than 5 % of our outstanding common stock and their respective affiliates beneficially hold, in the aggregate, shares representing approximately 48 % of our outstanding common stock. As a result, if these stockholders choose to act together, they would be able to significantly influence all matters submitted to our stockholders for approval, as well as our management and affairs. For example, these persons, if they choose to act together, would significantly influence the election of directors, the composition of our management and approval of any merger, consolidation, sale of all or substantially all of our assets or other business combination that other stockholders may desire. Some of these persons or entities may have interests different than yours. For example, because many of these stockholders purchased their shares at prices substantially below the current trading price of our common stock and have held their shares for a longer period, they may be more interested in selling our company to an acquirer than other investors, or they may want us to pursue strategies that deviate from the interests of other stockholders. Any of these actions could adversely affect the trading price of our common stock~~ . We are an " emerging growth company " and a " smaller reporting company " and, as a result of the reduced disclosure and governance requirements applicable to emerging growth companies and smaller reporting companies, our common stock may be less attractive to investors. We are an " emerging growth company " as defined in the JOBS Act, and we intend to take advantage of some of the exemptions from reporting requirements that are applicable to other public companies that are not emerging growth companies, including: • not being required to comply with the auditor attestation requirements in the assessment of our internal control over financial reporting; • not being required to comply with any requirement that may be adopted by the Public Company Accounting Oversight Board regarding mandatory audit firm rotation or a supplement to the auditor' s report providing additional information about the audit and the financial statements; • reduced disclosure obligations regarding executive compensation in our periodic reports, proxy statements and registration statements; and • not being required to hold a nonbinding advisory vote on executive compensation and stockholder approval of any golden parachute payments not previously approved. We cannot predict if investors will find our common stock less attractive because we will rely on these exemptions. If some investors find our common stock less attractive as a result, there may be a less active trading market for our common stock and our stock price may be more volatile. We may take advantage of these reporting exemptions until we are no longer an emerging growth company. We will remain an emerging growth company until the last day of the fiscal year ending after the fifth anniversary of our IPO, or, if earlier, (i) the last day of the fiscal year in which we have total annual gross revenue of at least $ 1. 235 billion, (ii) the date on which we are deemed to be a large accelerated filer, which means the market value of our common stock that is held by non- affiliates exceeds $ 700 million as of the prior June 30, or (iii) the date on which we have issued more than $ 1. 0 billion in non- convertible debt during the prior three- year period. In addition, we have elected to take advantage of the extended transition period to comply with new or revised accounting standards and to adopt certain of the reduced disclosure requirements available to emerging growth companies. As a result of the accounting standards election, we will not be subject to the same implementation timing for new or revised accounting standards as other public companies that are not emerging growth companies, which may make comparison of our financials to those of other public companies more difficult. As a result of these elections, the information that we provide in this Annual Report on Form 10- K may be different than the information investors may receive from other public companies in which they hold equity interests. In addition, it is possible that some investors will find our common stock less attractive as a result of these elections, which may result in a less active trading market for our common stock and higher volatility in our trading price. Even after we no longer qualify as an emerging growth company, we may, under certain circumstances, still qualify as a " smaller reporting company, " which would allow us to take advantage of many of the same exemptions from disclosure requirements, including reduced disclosure obligations regarding executive compensation in our periodic reports and proxy statements. Because we do not anticipate paying any cash dividends on our common stock in the foreseeable future, capital appreciation, if any, will be your sole source of gains and you may never receive a return on your investment. You should not rely on an investment in our common stock to provide dividend income. We have not declared or paid cash dividends on our common stock to date. We currently intend to retain our future earnings, if any, to fund the development and growth of our business. As a result, capital appreciation, if any, of our common stock will be your sole source of gain for the foreseeable future. Investors seeking cash dividends should not purchase our common stock. Our amended and restated certificate of incorporation provides that the Court of Chancery of the State of Delaware and the federal district courts of the United States of America will be the exclusive forums for substantially all disputes 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 or employees. Our amended and restated certificate of incorporation provides that the Court of Chancery of the State of Delaware is the exclusive forum for the following types of actions or proceedings under Delaware statutory or common law: • any derivative action or proceeding brought on our behalf; • any action asserting a breach of fiduciary duty; • any action asserting a claim against us arising under the Delaware General Corporation Law, or DGCL, our amended and restated certificate of incorporation, or our amended and restated bylaws; • any claim or cause of action seeking to interpret, apply, enforce or determine the validity of our restated certificate or our amended and restated bylaws; • any claim or cause of action as to which the DGCL confers jurisdiction on the Court of Chancery of the state of Delaware; and • any action asserting a claim against us that is governed by the internal- affairs doctrine. This provision would not apply to suits brought to enforce a duty or liability created by the ~~Securities Exchange Act of 1934, as amended, or the~~ Exchange Act. Furthermore,

Section 22 of the Securities Act creates concurrent jurisdiction for federal and state courts over all such Securities Act actions. Accordingly, both state and federal courts have jurisdiction to entertain such claims. To prevent having to litigate claims in multiple jurisdictions and the threat of inconsistent or contrary rulings by different courts, among other considerations, our amended and restated certificate of incorporation further provides that the federal district courts of the United States ~~of America~~ will be the exclusive forum for resolving any complaint asserting a cause of action arising under the Securities Act. While the Delaware courts have determined that such choice of forum provisions are facially valid, a stockholder may nevertheless seek to bring a claim in a venue other than those designated in the exclusive forum provisions. In such instance, we would expect to vigorously assert the validity and enforceability of the exclusive forum provisions of our amended and restated certificate of incorporation. This may require significant additional costs associated with resolving such action in other jurisdictions and there can be no assurance that the provisions will be enforced by a court in those other jurisdictions. These exclusive forum provisions may result in increased costs for investors to bring a claim. Further, these exclusive forum provisions may limit a stockholder' s ability to bring a claim in a judicial forum that it finds favorable for disputes with us or our directors, officers, or other employees, which may discourage lawsuits against us and our directors, officers and other employees. If a court were to find either exclusive forum provision in our amended and restated certificate of incorporation to be inapplicable or unenforceable in an action, we may incur further significant additional costs associated with resolving the dispute in other jurisdictions, all of which could seriously harm our business. Provisions in our corporate charter documents and under Delaware law could make an acquisition of us, which may be beneficial to our stockholders, more difficult and may prevent attempts by our stockholders to replace or remove our current management. Provisions in our certificate of incorporation and our bylaws may discourage, delay, or prevent a merger, acquisition, or other change in control of our company that stockholders may consider favorable, including transactions in which you might otherwise receive a premium for your 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 trading price of our common stock. In addition, because our board of directors is responsible for appointing the members of our management team, these provisions may frustrate or prevent any attempts by our stockholders to replace or remove our 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 only a portion of our directors stand for election at any given annual stockholder meeting; • allow the authorized number of our directors to be changed from time to time by our shareholders or our board of directors; • limit the manner in which stockholders can remove directors from our board of directors; • establish requirements for stockholder proposals that can be acted on at stockholder meetings; • require that stockholder actions must be effected at a duly called stockholder meeting and allow actions by our stockholders by written consent, with certain requirements; • limit who may call stockholder meetings; and • 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. Moreover, because we are incorporated in Delaware, we are governed by the provisions of Section 203 of the DGCL, which prohibits a person who owns in excess of 15 % of our outstanding voting stock from merging or combining with us for a period of three years after the date of the transaction in which the person acquired in excess of 15 % of our outstanding voting stock, unless the merger or combination is approved in a prescribed manner. General risks Unstable market and economic conditions may have serious adverse consequences on our business and financial condition. The global economy, including credit and financial markets, has experienced extreme volatility and disruptions recently, 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 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~~ **conflicts in** ~~between Russia and~~ Ukraine **and the Middle East** and increasing tensions between China and Taiwan 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 political unrest or war, it may make any necessary debt or equity financing more difficult to complete, more costly, and more dilutive. Failure to secure any necessary financing in a timely manner and on favorable terms could have a material adverse effect on our growth strategy, financial performance and share price and could require us to delay or abandon development or commercialization plans. In addition, there is a risk that one or more of our service providers, manufacturers or other partners would not survive or be able to meet their commitments to us under such circumstances, which could directly affect our ability to attain our operating goals on schedule and on budget. We have experienced and 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. 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 of 2002, or the Sarbanes- Oxley Act, and the rules and regulations of ~~the~~ **Nasdaq** ~~stock~~ **Stock** ~~market~~ **Market** ~~on which our common stock is listed~~. The Sarbanes- Oxley Act requires, among other things, that we maintain effective disclosure controls and procedures and internal control over financial reporting. **In addition** ~~Commencing with our fiscal year ending December 31~~, ~~2024, we must perform system~~ **Section 404 of the Sarbanes-Oxley Act requires our management** and ~~process evaluation and testing~~ **independent registered public accounting firm to report on the effectiveness** of our internal control over financial reporting **. We are also required** to ~~allow management~~

disclose changes made in our internal controls and procedures on a quarterly basis. However, for so long as we remain an emerging growth company as defined in the JOBS Act, we intend to **take advantage of certain exemptions from various** ~~report~~ **reporting requirements that are applicable companies that are not emerging growth companies, including, but not limited to, not being required to comply with the auditor attestation requirements of Section 404 of the Sarbanes- Oxley Act. Once we are no longer an emerging growth company or, if prior to such date we opt to no longer take advantage of the applicable exemption, we will be required to include an opinion from our independent registered public accounting firm** on the effectiveness of our internal control over financial reporting ~~in~~ **. An independent assessment of the effectiveness of** our ~~Form 10~~ **internal controls over financial reporting could detect problems that our management' s assessment might not. The requirements of these rules and regulations are difficult, time - consuming and costly** ~~K filing for that year , as required by Section 404 of the Sarbanes- Oxley Act~~ **and place significant strain on our personnel, systems and resources** . ~~This~~ **Effective internal controls over financial reporting are necessary for us to provide reliable financial reports and, together with adequate disclosure controls and procedures, are designed to prevent fraud. In order to maintain and improve the effectiveness of our disclosure controls and procedures and internal control over financial reporting, we have expended and anticipate we** will **continue** ~~require that we incur substantial additional professional fees and internal costs~~ to ~~expand our accounting and finance functions and that we~~ expend significant **resources, including accounting- related costs, and provide significant** management ~~efforts~~ **oversight** . ~~We have never been~~ **Any failure to implement** required **new** ~~to test our~~ **or** ~~internal~~ **improved** ~~control~~ **controls** ~~within a specified period~~ , **or** ~~and, as a result, we may experience difficulty~~ **difficulties encountered** in ~~meeting~~ **their implementation could cause us to fail to meet our reporting obligations. In addition, if we are unable to continue to meet** these ~~reporting~~ requirements ~~in a timely manner~~ **, we may not be able to remain listed on the Nasdaq Global Market** . We may identify 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. If we fail to ~~remediate our identified material weakness, or~~ identify ~~additional~~ material weaknesses, in our internal control over financial reporting, if we are not able to comply with the requirements of Section 404 of the Sarbanes- Oxley Act in a timely manner, 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 trading price of our stock could decline and we could be subject to sanctions or investigations by the stock exchange on which our common stock is listed, the Securities and Exchange Commission, or the SEC, or other regulatory authorities. Our ability to utilize our net operating loss carryforwards and research tax credits to offset future taxable income may be subject to limitations. As of December 31, ~~2023~~ **2024** , we had approximately $ ~~70~~ **106** . ~~2~~ **4** million of U. S. federal net operating loss carryforwards, or NOLs, $ ~~139~~ **212** . ~~3~~ **7** million of U. S. state and local NOLs, and $ **10.** 7 ~~.1~~ million of federal tax credits. U. S. federal NOLs generated in taxable years beginning after December 31, 2017, do not expire and may be carried forward indefinitely, but the deductibility of such NOLs is limited to no more than 80 % of current year taxable income. Our U. S. state and local NOLs begin to expire in 2040 and our federal research tax credits begin to expire in ~~2041~~ **2040** . In addition, under Sections 382 and 383 of the Internal Revenue Code of 1986, as amended, or the Code, if a corporation undergoes an " ownership change, " which is generally defined as a greater than 50 % change, by value, in its equity ownership by certain stockholders over a rolling three- year period, the corporation' s ability to use its pre- change NOLs and certain other pre- change tax attributes (such as research tax credits) to offset its post- change taxable income or taxes may be limited. If we undergo an ownership change, and our ability to use our pre- change NOLs and other pre- change tax attributes (such as tax credits) to offset our post- change income or taxes is limited, it ~~would~~ **may** harm our future results of operations by effectively increasing our future tax obligations. U. S. state and local NOLs may be ~~similar~~ **similarly** limited. In addition, at the U. S. state and local level, there may be periods during which the use of NOLs is suspended or otherwise limited, which could accelerate or permanently increase U. S. state and local taxes owed. Irrespective of the above, our ability to utilize our NOLs and research tax credits to offset future taxable income or taxes is conditioned on our attaining profitability and generating taxable income. We do not know if and when we will generate sufficient taxable income to utilize our NOLs and research tax credits. Changes in tax laws or regulations that are applied adversely to us or our customers may materially harm our business. New tax laws, statutes, rules, regulations, or ordinances could be enacted at any time. Further, existing tax laws, statutes, rules, regulations, or ordinances could be interpreted differently, changed, repealed, or modified at any time. Any such enactment, interpretation, change, repeal, or modification could adversely affect us, possibly with retroactive effect. ~~The~~ **For example, the** IRA enacted a 15 % minimum tax on the adjusted financial statement income of certain large U. S. corporations for taxable years beginning after December 31, 2022, as well as a 1 % excise tax on stock repurchases made by public corporations after December 31, 2022. Further, the Tax Cuts and Jobs Act of 2017, or the Tax Act, enacted many significant changes in U. S. federal tax laws, some of which were further modified by the Coronavirus Aid, Relief, and Economic Security Act, or the CARES Act, and may be modified in the future by the current or a future presidential administration. Among other changes, the Tax Act amended the Code to require that certain research and experimental expenditures be capitalized and amortized over five years if incurred in the United States or fifteen years if incurred in foreign jurisdictions for taxable years beginning after December 31, 2021. **If** ~~Although the U. S. Congress has considered legislation that would defer, modify, or repeal~~ the capitalization and amortization ~~requirement, there is no assurance that such changes will be made. If the~~ requirement is not deferred, repealed, or otherwise modified, it may increase our cash taxes and effective tax rate. In addition, it is uncertain if and to what extent various states will conform to the IRA, the Tax Act, the CARES Act, or any future U. S. federal tax laws. Changes in corporate tax rates, the realization of net deferred tax assets relating to our operations, the taxation of foreign

earnings, and the deductibility of expenses could have a material impact on the value of our deferred tax assets, result in significant one- time charges, and increase our future U. S. tax expenses. Our business and operations would suffer in the event of system failures, cyberattacks or a deficiency in our or our CMOs', CROs', manufacturers', contractors', consultants' or collaborators' cybersecurity. **In the ordinary course of our business, we and third parties with whom we conduct business, including third- party CMOs, CROs, manufacturers, contractors (including sites performing our clinical trials), consultants, and collaborators, collect and store sensitive data, including intellectual property, clinical trial data, proprietary and confidential business information, and personal data and personal information of our clinical trial subjects, employees, and others. The secure maintenance, transmission, and other processing of this information is critical to our operations.** Despite the implementation of security measures, our internal ~~computer~~ systems, as well as those of third parties on which we rely, are vulnerable to damage from, among other things, computer viruses, **ransomware and other forms of** malware, unauthorized access, natural disasters, terrorism, war **,** telecommunication and electrical failures, system malfunctions, cyberattacks or cyber- intrusions ~~over the Internet~~ , **email** ~~attachments~~ **attachment** ~~to emails~~ **compromise, denial of service attacks** , phishing attacks, **and unauthorized or otherwise improper acts or omissions by** persons inside our organization, or persons with access to systems inside our organization. **Any** ~~The risk of a security breach or disruption, particularly through cyberattacks or cyber intrusion, including by computer hackers, foreign governments and cyber terrorists, has generally increased as the number, intensity and sophistication of attempted attacks and intrusions from around the world have increased. If~~ such ~~an~~ event ~~were to occur and cause interruptions in our operations, it~~ could lead to the **prevention of access to,** loss, destruction, alteration, ~~prevention of access to,~~ disclosure, **or** dissemination of, or damage or unauthorized access to **or other processing of** , our data (including trade secrets or other confidential information, intellectual property, proprietary business information and personal data) or **other** data that is processed or maintained on our behalf, and cause ~~interruptions~~ **disruptions** ~~in~~ **to** our operations, which could ~~result in~~ **lead to** a material disruption of our product candidate development programs. For example, the loss **, corruption or unavailability** of preclinical study or clinical trial data from completed, ongoing or planned trials could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data. **More generally** ~~To the extent that any disruption or security breach were to result in a loss of or damage to our data or applications~~ , ~~or inappropriate disclosure of personal, confidential or proprietary information, we could incur liability and the further development of our product candidates could be delayed. In the ordinary course of our business, we collect and store sensitive data, including intellectual property, clinical trial data, proprietary business information, personal data and personally identifiable information of our clinical trial subjects and employees, in our data centers and on our networks. The secure processing, maintenance and transmission of this information is critical to our operations. Despite~~ **despite** ~~our~~ **the implementation of** security measures **in an effort to protect our systems and data** , we cannot ensure that our information technology and infrastructure **or any of our relevant policies or measures** will prevent breakdowns or **disruptions of, or** breaches ~~in our~~ **or incidents impacting,** ~~or our their~~ systems **or those of third parties on which we rely,** or other cybersecurity incidents that ~~cause~~ **lead to** loss, destruction, unavailability, **or unauthorized** alteration, dissemination **or other processing** of, or damage or unauthorized access to, our data, including personal data, **our other** assets ~~and~~ **, or** other data processed or maintained on our behalf, that could **delay clinical development of our product candidates and** have a material adverse effect upon our reputation, business, operations or financial condition. **To** ~~Although, to~~ our knowledge, we have not experienced any ~~such material~~ security breach to date **that has had a material impact on our business or operations** , **but we and the third parties with whom we conduct business have faced, and we anticipate continuing to face, cybersecurity risks, including risks of security breaches and incidents. Risks of security breaches and incidents and other types of system disruptions, particularly through cyberattacks or cyber intrusions, including by hackers, foreign governments, cyber terrorists, and associated actors, have increased generally as the number, intensity, and sophistication of attempted attacks and intrusions from around the world have increased. Geopolitical conflicts and tensions may also increase such risks. Any security breach or incident resulting in** any **loss, destruction, unavailability, alteration, disclosure, dissemination, or other processing of, or damage or unauthorized access to, our data, systems or applications, or inappropriate disclosure or other processing of confidential or proprietary information or personal data,, or any other event resulting in unauthorized access to, or disclosure or processing of,** such ~~breach could compromise our networks and the~~ information ~~stored there could be accessed~~ , ~~publicly disclosed, lost or stolen. Any such access, disclosure or other loss of information could result in legal claims or proceedings, liability under laws that protect the privacy of personal information, significant regulatory penalties, and such an event could disrupt our~~ **or** ~~operations, damage~~ **for it to be believed** ~~our~~ **or reported** ~~reputation, and cause a loss of confidence in us and our ability to conduct clinical trials, which could adversely affect our reputation and delay clinical development of our product candidates. To the extent~~ that any ~~disruption or security breach were to result in a loss~~ of **the foregoing has occurred** ~~or damage to our data or applications~~ , ~~or inappropriate disclosure of confidential or proprietary information or personal data, we~~ could ~~incur~~ **lead to us facing regulatory inquiries and proceedings, incurring** material legal claims and ~~liability~~ **proceedings,** and **incurring material** damage to our reputation ~~, and the further development of our product candidates could be delayed~~ . Any such event could also **disrupt our operations, lead to delays in the further development of our product candidates, cause a loss of confidence in us and our ability to conduct clinical trials,** compel us to comply with federal and state breach notification laws ~~,~~ and foreign law equivalents, subject us to mandatory corrective action **,** and ~~otherwise~~ subject us to ~~substantial~~ **material** liability under **applicable** laws, rules, regulations and standards ~~that protect the privacy and security of personal data~~ , which could result in significant legal and financial exposure and reputational ~~damages~~ **damage** that could ~~potentially~~ have ~~an~~ **a material** adverse effect on our business **, operations, and financial condition** . Notifications and follow- up actions related to a ~~data~~ **security** breach or other security incident could impact our reputation and cause us to incur significant costs, including significant legal expenses and remediation costs. We expect to incur significant costs in an effort to detect and prevent security incidents, and we may face increased costs

and requirements to expend substantial resources in the event of an actual or perceived security incident. However, we cannot guarantee that we will be able to detect or prevent any such incidents, or that we can remediate any such incidents in an effective or timely manner. Our efforts to improve security and protect data from compromise may also identify previously undiscovered instances of data breaches or other cybersecurity incidents. **Any security** ~~To the extent that any data~~ breach ~~, disruption~~ or ~~security~~ incident ~~were to result~~ **resulting** in any loss, destruction, or alteration of, damage ~~,~~ **or** unauthorized access to **,** or **unauthorized or otherwise** inappropriate ~~or unauthorized~~ disclosure ~~or ,~~ dissemination **, or other processing** of, our data, including personal data, or other information that is processed or maintained on our behalf, ~~we~~ **any significant damage to or disruption of our systems, or any perception that any of these has occurred,** could ~~be exposed~~ **expose us** to litigation and governmental investigations and inquiries, ~~the~~ **lead to delays in** further development and commercialization of our product candidates ~~could be delayed~~ , and ~~we could be~~ subject **us** to significant fines ~~or ,~~ penalties **and other liabilities. Our insurance policies may not be adequate to compensate us** for **the potential losses arising from** any ~~noncompliance with applicable state~~ **failure or other disruption of** , ~~federal and foreign privacy and~~ security ~~laws~~ **breach of** , ~~rules~~ **or incident impacting** , ~~regulations~~ **our systems or third- party systems where information important to our business operations is stored or otherwise processed, or any other unauthorized disclosure of information. In addition, such insurance may not be available to us in the future on economically reasonable terms, or at all. Further, our insurance may not cover all claims made against us** and ~~standards~~ **could have high deductibles in any event, and defending a suit, regardless of its merit, could be costly and divert management attention** . We incur increased costs and demands upon management as a result of being a public company. As a public company listed in the United States, we incur significant additional legal, accounting and other expenses that we did not incur as a private company, including the cost of director and officer liability insurance. These additional costs could negatively affect our financial results. In addition, changing laws, regulations and standards relating to corporate governance and public disclosure, including regulations implemented by the SEC and the Nasdaq Stock Market, may increase legal and financial compliance costs and make some activities more time- consuming. These laws, regulations and standards are subject to varying interpretations and, as a result, their application in practice may evolve over time as new guidance is provided by regulatory and governing bodies. We intend to invest resources to comply with evolving laws, regulations and standards, and this investment may result in increased general and administrative expenses and a diversion of management' s time and attention from revenue- generating activities to compliance activities. If notwithstanding our efforts to comply with new laws, regulations and standards, we fail to comply, regulatory authorities may initiate legal proceedings against us and our business may be harmed. Failure to comply with these rules might also make it more difficult for us to obtain some types of insurance, including director and officer liability insurance, and we might be forced to accept reduced policy limits and coverage or incur substantially higher costs to obtain the same or similar coverage. The impact of these events could also make it more difficult for us to attract and retain qualified persons to serve on our board of directors, on committees of our board of directors or as members of senior management. Our disclosure controls and procedures may not prevent or detect all errors or acts of fraud. We are subject to certain reporting requirements of the Exchange Act. Our disclosure controls and procedures are designed to reasonably assure that information required to be disclosed by us in reports we file or submit under the Exchange Act is accumulated and communicated to management, recorded, processed, summarized, and reported within the time periods specified in the rules and forms of the SEC. We believe that any disclosure controls and procedures or internal controls and procedures, no matter how well conceived and operated, can provide only reasonable, not absolute, assurance that the objectives of the control system are met. These inherent limitations include the realities that judgments in decision- making can be faulty, and that breakdowns can occur because of simple error or mistake. Additionally, controls can be circumvented by the individual acts of some persons, by collusion of two or more people, or by an unauthorized override of the controls. Accordingly, because of the inherent limitations in our control system, misstatements or insufficient disclosures due to error or fraud may occur and not be detected.