Risk Factors Comparison 2024-09-26 to 2023-09-21 Form: 10-K

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Investing in our securities involves a high degree of risk. You should carefully consider the risks and uncertainties described below together with all of the other information contained in this Annual Report, including our consolidated financial statements and the related notes, before deciding to invest in our securities. The risks and uncertainties described below and in the documents incorporated by reference herein 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 actually occurs, our business, financial condition, results of operations and prospects could be materially and adversely affected, and the trading price of our common stock could decline. Risk Factor Summary The following is a summary of the risks and uncertainties that could cause our business, financial condition or operating results to be harmed. We encourage you to carefully review the full risk factors contained in this report in their entirety for additional information regarding these risks and uncertainties. • We have incurred significant losses since inception and anticipate that we will continue to incur losses for the foreseeable future. If we are unable to achieve or sustain profitability, the market value of our common stock will likely decline; • We have never generated any revenue from product sales and may never be profitable; • We will need to continue our efforts to raise additional funding, which may not be available on acceptable terms, or at all. Failure to obtain capital when needed may negatively impact our ability to continue as a going concern; • Our product candidates are based on ddRNAi and silence and replace technology. Currently, no product candidates utilizing ddRNAi technology or silence and replace technology have been approved for commercial sale and our approach to the development of ddRNAi technology and silence and replace technology may not result in safe, effective or marketable products; • We are early in our product development efforts and our current product candidate is in early clinical stage. We may not be able to obtain regulatory approvals for the commercialization of our product candidates; • Issues that may impact delivery of our therapeutics to the cell could adversely affect or limit our ability to develop and commercialize product candidates; • We face competition from entities that have developed or may develop product candidates for our target disease indications, including companies developing novel treatments and technology platforms based on modalities and technology similar to ours; and • If we are unable to obtain or protect sufficient intellectual property rights related to our product candidates, we may not be able to obtain exclusivity for our product candidates or prevent others from developing similar competitive products. Risks Related to Our Financial Condition, Capital Requirements We have incurred significant losses since inception and anticipate that we will continue to incur losses for the foreseeable future. If we are unable to achieve or sustain profitability, the market value of our common stock will likely decline. As of June 30, ~~2023~~ **2024**, we had accumulated losses of $ ~~167~~ **190**. ~~9~~ **3** million. We have devoted most of our financial resources to research and development, including our clinical and preclinical development activities. To date, we and our predecessor have financed our operations primarily through the issuance of equity securities, research and development grants from the Australian government and payments from our collaboration partners. We do not expect to generate any significant revenue for the foreseeable future, and we expect to incur significant operating losses for the foreseeable future due to the cost of research and development, preclinical studies and clinical trials and the regulatory approval process for product candidates. The amount of our future net losses is uncertain and will depend, in part, on the rate of our future expenditures. Our ability to continue operations will depend on, among other things, our ability to obtain funding through equity or debt financings, strategic collaborations or additional grants. We expect to continue to incur significant expenses, and we may incur operating losses for the foreseeable future. We anticipate that our expenses will increase substantially if and as we: • continue our research and preclinical development of our product candidates; • expand the scope of our current preclinical studies for our product candidates or initiate clinical, additional preclinical or other studies for product candidates; • seek regulatory and marketing approvals for any of our product candidates that successfully complete clinical trials; • further develop the manufacturing process for our product candidates; • change or add additional manufacturers or suppliers; • seek to identify and validate additional product candidates; • acquire or in- license other product candidates and technologies, which may or may not include those related to our ddRNAi technology and delivery vectors for our therapeutic candidates; • maintain, protect and expand our intellectual property portfolio; • create additional infrastructure to support our operations as a public company in the United States and our product development and future commercialization efforts; and • experience any delays or encounter issues with any of the above. The net losses we incur may fluctuate significantly from quarter to quarter and year to year, such that a period- to- period comparison of our results of operations may not be a good indication of our future performance. In any particular quarter or quarters, our operating results could be below the expectations of securities analysts or investors, which could cause the price of our common stock to decline. We have never generated any revenue from product sales and may never be profitable. Our ability to generate significant revenue and achieve profitability depends on our ability to, alone or with strategic collaboration partners, successfully complete the development of and obtain the regulatory approvals for our product candidates, to manufacture sufficient supply of our product candidates, to establish a sales and marketing organization or suitable third- party alternative for the marketing of any approved products and to successfully commercialize any approved products on commercially reasonable terms. All of these activities will require us to raise sufficient funds to finance business activities. In the past we received milestone payments from our collaborative partners. However, we do not anticipate generating revenue from commercializing product candidates for the foreseeable future, if ever. Our ability to generate future revenues from commercializing product candidates depends heavily on our success in: • establishing proof of concept in preclinical studies and clinical trials for our product candidates; • successfully initiating and completing clinical trials of our product candidates; ~~38~~ **39** • obtaining regulatory and marketing approvals for

product candidates for which we complete clinical trials; • maintaining, protecting and expanding our intellectual property portfolio, and avoiding infringing on intellectual property of third parties; • establishing and maintaining successful licenses, collaborations and alliances with third parties; • developing a sustainable, scalable, reproducible and transferable manufacturing process for our product candidates; • establishing and maintaining supply and manufacturing relationships with third parties that can provide products and services adequate, in amount and quality, to support clinical development and commercialization of our product candidates, if approved; • launching and commercializing any product candidates for which we obtain regulatory and marketing approval, either by collaborating with a partner or, if launched independently, by establishing a sales, marketing and distribution infrastructure; • obtaining market acceptance of any product candidates that receive regulatory approval as viable treatment options; • obtaining favorable coverage and reimbursement rates for our products from third- party payers; • addressing any competing technological and market developments; • identifying and validating new product candidates; and • negotiating favorable terms in any collaboration, licensing or other arrangements into which we may enter. The process of developing product candidates for ddRNAi- based and antisense RNA- based therapeutics contains a number of inherent risks and uncertainties. For example, with regard to ddRNAi, it may not be possible to identify a target region of a disease- associated gene that has not been previously identified and / or patented by others, resulting in restrictions on freedom to operate for that target sequence. Silencing the target gene may not ultimately result in curing the disease as there may be more factors contributing to the development of the disease than the target gene. Silencing the target gene using ddRNAi may lead to short- term or long- term adverse effects that were not predicted or observed in preclinical studies. The delivery of the DNA construct to the target cells may not be possible, or complete or adequate to provide sufficient therapeutic benefit. Even if one or more of our product candidates is approved for commercial sale, we may incur significant costs associated with commercializing any approved product candidate. As one example, our expenses could increase beyond expectations if we are required by the United States Food and Drug Administration (" FDA ") or other regulatory agencies, domestic or foreign, to perform clinical and other studies in addition to those that we currently anticipate. Even if we are able to generate revenues from the sale of any approved products, we may not become profitable and may need to obtain additional funding to continue operations, which could have an adverse effect on our business, financial condition, results of operations and prospects. We will need to continue our efforts to raise additional funding, which may not be available on acceptable terms, or at all. Failure to obtain capital when needed may negatively impact our ability to continue as a going concern. Developing ddRNAi products is expensive, and we expect our research and development expenses to increase substantially in connection with our ongoing activities, particularly as we advance our product candidates in preclinical studies and clinical trials and as we undertake preclinical studies of new product candidates. ~~On August 11, 2023 we closed an underwritten public offering in which we sold 875, 949 shares of common stock, 15, 126, 226 pre- funded warrants to purchase 15, 126, 226 shares of common stock, and 16, 002, 175 common warrants to purchase up to 16, 002, 175 shares of common stock. The combined purchase price for each share of common stock and accompanying common warrant was $ 1. 93, which was allocated as $ 1. 9299 per share of common stock and $ 0. 0001 per common warrant. Each pre- funded warrant was sold together with one common warrant at a combined price of $ 1. 9299, which was allocated as $ 1. 9298 per pre- funded warrant and $ 0. 0001 per common warrant. In addition, the Company granted the underwriter an option to purchase up to 2, 331, 606 additional shares of common stock and / or up to 2, 331, 606 additional common warrants. As of August 15, 2023 the underwriter had partially exercised this option and purchased 458, 134 additional shares of common stock and 458, 134 additional common warrants. Net proceeds from the offering, including the impact of the underwriter' s partial exercise of its option and net of underwriting discounts, commissions, and other offering expenses, totaled $ 28. 6 million.~~ We estimate that our cash and cash equivalents will be sufficient to fund the Company' s operations for at least the next twelve months after the date that this Annual Report is filed. Because the length of time and activities associated with successful development of our product candidates is highly uncertain, we are unable to estimate the actual funds we will require for development and any approved marketing and commercialization activities. In any event, we will require additional capital to obtain regulatory approval for our product candidates and to commercialize any product candidates that receive regulatory approval. Any additional fundraising efforts may divert our management from their day- to- day activities, which may compromise our ability to develop and commercialize our product candidates. In addition, we cannot guarantee that future financing will be available in sufficient amounts or on terms acceptable to us, if at all. Moreover, the terms of any financing may adversely affect the holdings or the rights of our ~~shareholders~~ **stockholders** , and the issuance of additional securities, whether equity or debt, by us, or the possibility of such issuance, may cause the market price of our common stock to decline. If we incur indebtedness we may be required to agree to restrictive covenants, such as limitations on our ability to incur additional debt, limitations on our ability to acquire, sell or license intellectual property rights and other operating restrictions that could compromise our ability to conduct our business. We could also seek financing through arrangements with collaborative partners at an earlier stage than would otherwise be desirable and we may be required to relinquish rights to some or all of our technologies or product candidates or otherwise agree to terms unfavorable to us. If we are unable to obtain funding on a timely basis or on acceptable terms, we may be required to significantly curtail, delay or discontinue one or more of our research or development programs or the commercialization of any approved product candidates. We will continue to seek to raise additional working capital through public equity, private equity or debt financings. If we fail to raise additional working capital, or do so on commercially unfavorable terms, it may materially and adversely affect our business, prospects, financial condition and results of operations, and we may be unable to continue as a going concern. Future reports from our independent registered public accounting firm may also contain statements expressing substantial doubt about our ability to continue as a going concern. If we seek additional financing to fund our business activities in the future and there remains substantial doubt about our ability to continue as a going concern, investors or other financing sources may be unwilling to provide additional funding to us on commercially reasonable terms, if at all. We may be unable to conduct and conclude clinical trials for our product candidate for OPMD if we are unable to raise additional financing. If we are unable to raise additional funds, and thus unable to

continue our studies, our business operations may be adversely affected. ~~40~~ Risks Related to the Product Development and Regulatory Approval of Our Product Candidates Our product candidates are based on ddRNAi and silence and replace technology. Currently, no product candidates utilizing ddRNAi technology or silence and replace technology have been approved for commercial sale and our approach to the development of ddRNAi technology and silence and replace technology may not result in safe, effective or marketable products. We have concentrated our product research and development efforts on our ddRNAi technology and silence and replace technology, and our future success depends on successful clinical development of these technologies. We plan to progress our product candidates using our ddRNAi technology and our silence and replace technology for the treatment of the life- threatening conditions of OPMD. **41** The scientific research that forms the basis of our efforts to develop product candidates is based on the therapeutic use of ddRNAi, and the identification, optimization and delivery of ddRNAi- based product candidates is relatively new. The scientific evidence to support the feasibility of successfully developing therapeutic treatments based on ddRNAi is preliminary and limited. There can be no assurance that any development and technical problems we experience in the future will not cause significant delays or unanticipated costs, or that such development problems can be solved. We may be unable to reach agreement on favorable terms, or at all, with providers of vectors needed to optimize delivery of our product candidates to target disease cells and we may also experience unanticipated problems or delays in expanding our manufacturing capacity or transferring our manufacturing process to commercial partners, any of which may prevent us from completing our preclinical trials, initiating clinical trials or commercializing our products on a timely or profitable basis, if at all. Only a few product candidates based on ddRNAi have been tested in either animals or humans, and a number of clinical trials conducted by other companies using other forms of RNAi technologies have not been successful. We may discover that application of ddRNAi does not possess properties required for a therapeutic benefit, such as the ability to continually express shRNAs for the period of time required to be maximally effective or the ability of viral vectors or other technologies to effectively deliver ddRNAi constructs to target cells in therapeutically relevant concentrations. In addition, application of ddRNAi- based products in humans may result in safety issues. We currently have only limited data, and no conclusive evidence, to suggest that we can effectively produce effective therapeutic treatments using our ddRNAi technology. We are early in our product development efforts and our current product candidates are still in preclinical development. We may not be able to obtain regulatory approvals for the commercialization of our product candidates. The research, testing, manufacturing, labeling, approval, selling, marketing and distribution of biologics is subject to extensive regulation by the FDA and other regulatory authorities, and these regulations differ from country to country. We do not have any products on the market and are early in our development efforts. All of our ddRNAi product candidates and our silence and replace product candidates are in preclinical development. Our current product candidates are subject to the risks of failure typical for development of biologics. The development and approval process is expensive and can take many years to complete, and its outcome is inherently uncertain. In addition, the outcome of preclinical testing and early clinical trials may not be predictive of the success of later clinical trials, and interim results of a clinical trial do not necessarily predict final results. We have not submitted a marketing application, or received marketing approval, for any of our product candidates and will not submit any applications for marketing approval for several years. We have limited experience in conducting and managing clinical trials necessary to obtain regulatory approvals, including marketing approval by the FDA. To receive marketing approval, we must, among other things, demonstrate with evidence from clinical trials that the product candidate is both safe and effective for each indication for which approval is sought, and failure can occur in any stage of development. Satisfaction of the marketing approval requirements typically takes several years and the time needed to satisfy them may vary substantially, based on ~~41~~ the type, complexity and novelty of the biopharmaceutical product. We cannot predict if or when we might receive regulatory approvals for any of our product candidates currently under development. The FDA and foreign regulatory authorities also have substantial discretion in the biopharmaceutical product approval process. The numbers, types and sizes of preclinical studies and clinical trials that will be required for regulatory approval varies depending on the product candidate, the disease or condition that the product candidate is designed to address and the regulations applicable to any particular product candidate. Approval policies, regulations or the type and amount of clinical data necessary to gain marketing approval may change during the course of a product candidate' s clinical development and may vary among jurisdictions, and there may be varying interpretations of data obtained from preclinical studies or clinical trials, any of which may cause **42** delays or limitations in the marketing approval or the decision not to approve an application. Regulatory agencies can delay, limit or deny marketing approval of a product candidate for many reasons, including: • the FDA or comparable foreign regulatory authorities may disagree with the design or implementation of any future clinical trials; • we may be unable to demonstrate to the satisfaction of the FDA or comparable foreign regulatory authorities that a product candidate is safe and effective for its proposed indication; • the results of any future clinical trials may not meet the level of statistical or clinical significance required by the FDA or comparable foreign regulatory authorities for approval; • the patients recruited for a particular clinical program may not be sufficiently broad or representative to assure safety and effectiveness in the full population for which we seek approval; • the results of any future clinical trials may not confirm the positive results from earlier preclinical studies or clinical trials; • we may be unable to demonstrate that a product candidate' s clinical and other benefits outweigh its safety risks; • the FDA or comparable foreign regulatory authorities may disagree with our interpretation of data from preclinical studies or clinical trials; • the data collected from any future clinical trials of our product candidates may not be sufficient to the satisfaction of FDA or comparable foreign regulatory authorities to support the submission of a biologics license application, or BLA, or other comparable submission in foreign jurisdictions or to obtain regulatory approval in the United States or elsewhere; • the FDA or comparable foreign regulatory authorities may only agree to approve a product candidate under conditions that are so restrictive that the product is not commercially viable; • regulatory agencies might not approve or might require changes to our manufacturing processes or facilities; or • regulatory agencies may change their approval policies or adopt new regulations in a manner rendering our clinical data insufficient for approval. Any delay in obtaining or failure to obtain required approvals could

materially and adversely affect our ability to generate revenue from the particular product candidate, which likely would result in significant harm to our financial position and adversely impact the price of our common stock. Furthermore, any regulatory approval to market a product may be subject to limitations on the indicated uses for which we may market the product. These limitations may limit the size of the market for the product. We are not permitted to market our product candidates in the United States or in other countries until we receive approval of a BLA from the FDA or marketing approval from applicable regulatory authorities outside the United States. Obtaining approval of a BLA can be a lengthy, expensive and uncertain process. If we fail to ~~42~~ obtain FDA approval to market our product candidates, we will be unable to sell our product candidates in the United States, which will significantly impair our ability to generate revenues. In addition, failure to comply with FDA and non- U. S. regulatory requirements may, either before or after product approval, if any, subject us to administrative or judicially imposed sanctions, including: • restrictions on our ability to conduct clinical trials, including full or partial clinical holds on ongoing or planned trials; • restrictions on the products, manufacturers, or manufacturing process; • warning letters; **43** • civil and criminal penalties; • injunctions; • suspension or withdrawal of regulatory approvals; • product seizures, detentions or import bans; • voluntary or mandatory product recalls and publicity requirements; • total or partial suspension of production; • imposition of restrictions on operations, including costly new manufacturing requirements; and • refusal to approve pending BLAs or supplements to approved BLAs. Even if we do receive regulatory approval to market a product candidate, any such approval may be subject to limitations on the indicated uses for which we may market the product. It is possible that none of our existing product candidates or any product candidates we may seek to develop in the future will ever obtain the appropriate regulatory approvals necessary for us or our collaborators to commence product sales. Any delay in obtaining, or an inability to obtain, applicable regulatory approvals would prevent us from commercializing our product candidates, generating revenues and achieving and sustaining profitability. Because our ddRNAi and silence and replace product candidates are considered gene therapies, it is difficult to predict the time and cost of product candidate development as well as subsequently obtaining regulatory approval. The clinical trial requirements of the FDA and comparable foreign 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 such product candidates. The regulatory approval process for novel product candidates such as ours can be more expensive and take longer than for other pharmaceutical product candidates. The FDA and comparable foreign regulatory authorities have relatively limited experience with ddRNAi- based and silence and replace- based therapeutics, which 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 or other countries. We and our current collaborators, or any future collaborators, may never receive approval to market and commercialize any product candidate. Even if we or a collaborator obtain regulatory approval, the approval may be for disease indications or patient populations that are not as broad as we intended or desired and may require labeling that includes significant use or distribution restrictions or safety warnings. Regulatory requirements governing gene and cell therapy products have changed frequently and may continue to change in the future. Also, before a clinical trial can begin at any institution, that institution' s institutional review board, or IRB, and its institutional biosafety committee, or IBC, if it has one, have to review the proposed clinical trial to assess the safety of the trial. In addition, adverse developments in clinical trials of gene therapy products conducted by others may cause the FDA or comparable foreign regulatory bodies to change the requirements for approval of any of our product candidates. ~~43~~ These committees and advisory groups and the new guidelines they promulgate and new requirements they may impose may lengthen the clinical development and regulatory review process, require us to perform additional studies, increase our development costs, lead to changes in regulatory positions and interpretations, delay or prevent approval and commercialization of these product candidates or lead to significant post- approval limitations or restrictions. As we advance our product candidates, we will be required to consult with these regulatory committees and advisory groups, and comply with applicable guidelines and requirements as they may change from time to time. If we fail to do so, we may be required to delay or discontinue development of our product candidates. These additional processes may result in a development, review and approval process that is longer than we otherwise would have expected for our product candidates. Delay or failure to obtain, or unexpected costs in obtaining, the regulatory approval necessary to bring a potential product to market would delay or prevent us from commercializing our product candidates, generating revenues and achieving and sustaining profitability. **44** Positive results from preclinical studies of our product candidates are not necessarily predictive of the results of our planned clinical trials of our product candidates. Positive results in preclinical proof of concept and animal studies of our product candidates may not result in positive results in clinical trials in humans. Many companies in the pharmaceutical and biotechnology industries have suffered significant setbacks in clinical trials after achieving positive results in preclinical development or early stage clinical trials, and we cannot be certain that we will not face similar setbacks. These setbacks have been caused by, among other things, preclinical findings made while clinical trials were underway or safety or efficacy observations made in clinical trials, including adverse events. Moreover, preclinical and clinical data are often susceptible to varying interpretations and analyses, and many companies that believed their product candidates performed satisfactorily in preclinical studies and clinical trials nonetheless failed to obtain FDA or other regulatory authority approval. If we fail to produce positive results in our clinical trials of our product candidates, the development timeline and regulatory approval and commercialization prospects for our product candidates, and, correspondingly, our business and financial prospects, would be negatively impacted. Issues that may impact delivery of our therapeutics to the cell could adversely affect or limit our ability to develop and commercialize product candidates. Successful clinical development of ddRNAi- based therapeutics and silence and replace- based therapeutics is largely dependent on using the appropriate delivery methodologies, including viral vectors, to obtain therapeutically relevant concentrations of the DNA constructs that express the shRNAs and engineered transgenes in the appropriate target cells. To develop effective product candidates, we will need to license delivery technologies from third parties or develop delivery technologies with research collaborators. Although delivery technologies, including AAV vectors, have been identified and are well defined for specific tissue types, we continue to seek

vectors with ideal delivery properties for indications we are pursuing, including OPMD. The tissue tropism and other physical properties of AAV vectors are limited and may not be effective for other product candidates or delivery into a wide array of tissues types. AAV vectors can also trigger immune responses in some patients, and those patients will not derive clinical benefit from administration of a product candidate unless steps are taken to clinically address the issue. If we or our collaborators are not successful in identifying effective delivery methodologies to achieve a therapeutically relevant concentration for our product candidates in the target tissues, we may not succeed in developing marketable products. In addition, if we are unable to reach agreement on favorable terms, or at all, with providers of any effective vectors that we do identify, we may not succeed in completing our clinical trials or commercializing our products on a timely or profitable basis, if at all. We use AAV vectors as part of our ddRNAi and silence and replace approaches for several indications. As such, we require licenses and the ability to manufacture large quantities of AAV particles under the FDA' s current good manufacturing practices, or cGMP, requirements and those of comparable foreign regulatory authorities in order to commercialize a product candidate using an AAV vector. ~~44~~ We may find it difficult to enroll patients in any future clinical trials, and patients could discontinue their participation in our current and any future clinical trials, which could delay or prevent our current and any future clinical trials of our product candidates and make those trials more expensive to undertake. Identifying and qualifying patients to participate in current and any future clinical trials of our product candidates is critical to our success. The timing of our clinical trials depends on the speed at which we can recruit patients to participate in testing our product candidates. Patients may be unwilling to participate in any future clinical trials because of negative publicity from adverse events in the biotechnology, RNAi or gene therapy industries. Patients may be unavailable for other reasons, including competitive clinical trials for similar patient populations, and the timeline for recruiting patients, conducting trials and obtaining regulatory approval of potential products may be delayed. If we have difficulty enrolling a sufficient number of patients to conduct any future clinical trials as planned, we may need to delay, limit or discontinue those clinical trials. Clinical trial delays could result in increased costs, slower product development, setbacks in testing the safety and effectiveness of our technology or discontinuation of the clinical trials altogether. **45** We may not be able to identify, recruit and enroll a sufficient number of patients, or those with required or desired characteristics to achieve diversity in a trial, to complete any future clinical trials in a timely manner. Patient enrollment is affected by factors including: • finding and diagnosing patients; • severity of the disease under investigation; • design of the clinical trial protocol; • size and nature of the patient population; • eligibility criteria for the trial in question; • perceived risks and benefits of the product candidate under study; • proximity and availability of clinical trial sites for prospective patients; • availability of competing therapies and clinical trials; • clinicians' and patients' perceptions of the potential advantages of the product being studied in relation to other available therapies, including any new products that may be approved for the indications we are investigating; • patient referral practices of physicians; **and** • ability to monitor patients adequately during and after treatment ~~; and • the COVID- 19 pandemic~~ . We or any potential collaborators plan to seek initial marketing approval for our product candidates in the United States, Canada, Europe and Israel. We may not be able to initiate or continue any future clinical trials in a timely manner if we cannot enroll a sufficient number of eligible patients to participate in the clinical trials required by the FDA or comparable foreign regulatory agencies. Our ability to successfully initiate, enroll and complete a clinical trial in any foreign country is subject to numerous risks unique to conducting business in foreign countries, including: • difficulty in establishing or managing relationships with contract research organizations, or CROs, clinical sites and physicians; • different standards for the conduct of clinical trials; • our inability to locate and engage qualified local consultants, physicians and partners; and • the potential burden of complying with a variety of foreign laws, medical standards and regulatory requirements, including the regulation of biopharmaceutical and biotechnology products and treatments. ~~45~~ In addition, patients enrolled in current and any future clinical trials may discontinue their participation at any time during the trial as a result of a number of factors, including experiencing adverse events, which may or may not be judged related to our product candidates under evaluation. The discontinuation of patients in any one of our trials may cause us to delay or discontinue our clinical trial, or cause the results from that trial not to be positive or sufficient to support either partnering with a pharmaceutical or biotechnology company to further develop and commercialize the product candidate or filing for regulatory approval of the product candidate. We may encounter substantial delays in any future clinical trials or we may fail to demonstrate safety and efficacy to the satisfaction of applicable regulatory authorities. Before obtaining marketing approval from regulatory authorities for the sale of any of our product candidates, we must conduct extensive clinical trials to demonstrate the safety and efficacy of the product candidates in humans. Clinical trials are expensive and time- consuming, and their outcome is uncertain. We ~~may experience delays in initiating and completing any clinical trials that we are conducting or intend to conduct as a result of the COVID- 19 pandemic, and we do not know whether our ongoing or planned clinical trials will begin or progress on schedule, need to be redesigned, enroll patients on time or be completed on schedule, or at all. We~~ cannot guarantee that any **46** clinical trials will be initiated or conducted as planned or completed on schedule, if at all. A failure of one or more clinical trials can occur at any stage of testing. Events that may prevent successful or timely completion of clinical development include: • inability to generate sufficient preclinical, toxicology or other data to support the initiation of human clinical trials; • delays in reaching consensus with regulatory agencies on trial design; • identifying, recruiting and training suitable clinical investigators; • delays in reaching agreement on acceptable terms with prospective CROs and clinical trial sites; • delays in obtaining required IRB or IBC approval at each clinical trial site; • delays in recruiting suitable patients to participate in our clinical trials; • imposition of a clinical hold by regulatory agencies, including after an inspection of our clinical trial operations or trial sites; • failure by our CROs, other third parties or us to adhere to clinical trial requirements; • failure to perform in accordance with the FDA' s good clinical practices, or GCP, or applicable regulatory requirements in other countries; • inability to manufacture, test, release, import or export for use sufficient quantities of our product candidates for use in clinical trials; • failure to manufacture our product candidate in accordance with cGMP requirements or applicable regulatory guidelines in other countries; • delays in the testing, validation and manufacturing of our product candidates; • delays in the delivery of our

product candidates to the clinical trial sites; • delays in having patients complete participation in a trial or return for post-treatment follow- up; • clinical trial sites dropping out of a trial; • occurrence of serious adverse events associated with the product candidate that are viewed to outweigh its potential benefits; • changes in regulatory requirements and guidance that require amending or submitting new clinical trial protocols; 46 • 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 discontinue product development programs ; or • the severity, duration and impact of the COVID- 19 pandemic . Further, a clinical trial may be suspended or discontinued by us, our collaborators, the IRBs or the IBCs at the sites in which such trials are being conducted, the data safety monitoring board, or DSMB, for such trial, or by the FDA or comparable foreign regulatory authorities due to a number of factors, including the imposition of a clinical hold or termination of a trial due to failure to conduct the clinical trial in accordance with regulatory requirements or our clinical protocols, unforeseen safety issues or adverse side effects of our product candidate, or a product candidate from another company that shares similar properties, failure to demonstrate adequate benefit from using a product candidate, changes in governmental regulations or administrative actions or lack of adequate funding to continue the clinical trial. If we experience discontinuation of, or delays in the completion of, any clinical trial of our product candidates, the commercial prospects of our product candidates may be harmed, and our ability to generate product revenues from any of these product candidates may be eliminated or delayed. Furthermore, many of the factors that 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. 47 In addition, if we or our third- party collaborators make significant manufacturing or formulation changes to our product candidates, we or they may need to conduct additional studies to bridge the modified product candidates to earlier versions to ensure comparability, safety and efficacy of the two different product candidates. Any inability to successfully complete preclinical and clinical development could result in additional costs to us or impair our ability to commercialize our programs and product candidates. Clinical trial delays could also shorten any periods during which we may have the exclusive right to commercialize our product candidates or allow our competitors to bring products to market before we do, which could impair our ability to successfully commercialize our product candidates. If the results of our current or any future clinical trials are inconclusive or if there are safety concerns or adverse events associated with our product candidates, we may: • fail to obtain, or be delayed in obtaining, marketing approval for our product candidates; • obtain approval for indications or patient populations that are not as broad as intended or desired; • obtain approval with labeling that includes significant use or distribution restrictions or safety warnings; • need to change the way the product is administered; • be required to perform additional clinical trials to support approval or be subject to additional post- marketing testing requirements; • have regulatory authorities withdraw their marketing approval of the product after granting it; • have regulatory authorities impose restrictions on distribution of the product in the form of a risk evaluation and mitigation strategy, or REMS, or modified REMS, that limit our ability to commercialize the product; • be subject to the addition of labeling statements, such as warnings or contraindications; • be sued and held liable for harm caused to patients; or • experience damage to our reputation. 47 Any of these events could prevent us from achieving or maintaining market acceptance of our product candidates and impair our ability to commercialize our product candidates. In addition, principal investigators for our clinical trials may serve as scientific advisors or consultants to us from time to time and may receive cash or equity compensation in connection with such services. If these relationships and any related compensation result in perceived or actual conflicts of interest, or the FDA concludes that the financial relationship may have affected the interpretation of any particular study, the integrity of the data generated at the applicable clinical trial site may be questioned and the utility of the clinical trial itself may be jeopardized, which could result in the delay or rejection of any BLA we submit to the FDA or any comparable foreign regulating authorities. Any such delay or rejection could prevent us from commercializing our product candidates. Our product candidates and the process for administering our product candidates may cause undesirable side effects or have other properties that could delay or prevent their regulatory approval, limit the commercial profile of an approved label, or result in significant negative consequences following any potential marketing approval. Treatment with our product candidates may produce undesirable side effects or adverse reactions or events. For example, the AAV vector and related capsid protein, which we are currently using to deliver many of our ddRNAi and silence and replace product candidates, could cause adverse immunological side effects due to 48 preexisting and / or recall responses to the naturally occurring virus from which the vector is engineered, or to the DNA construct product itself. These responses may also interfere with therapeutic efficacy if not identified and managed optimally. Preexisting immune responses to AAV manifesting as neutralizing antibodies are common within the general population and may be a limitation to the enrollment of patients in gene therapy clinical trials using AAV vectors, the successful use of AAV vectors in gene therapy clinical trials and the market acceptance of product candidates, if approved, that are delivered using AAV vectors. Patients with neutralizing antibodies to AAV will not derive clinical benefit from administration of such a product candidate unless steps are taken to clinically address the issue and those treatments themselves may cause adverse effects. In previous clinical trials undertaken by other companies involving systemic administration of AAV viral vectors for gene therapy, some subjects experienced adverse events, including the development of a negative T cell response against the AAV capsid protein. If our vectors cause similar adverse events, we may be required to delay or discontinue further clinical development of our product candidates. It is also possible that we may discover new adverse events related to AAV or other vectors, which could potentially enhance the risk to patients who use our product candidates delivered with that vector. Additionally, the procedure used to administer the treatment could result in undesirable health effects. If any such adverse events occur, our current and any future clinical trials could be suspended or discontinued and the FDA or comparable foreign regulatory authorities could order us to cease further development or deny approval of our product candidates for any or all targeted indications. The product- related side effects could affect patient recruitment or the ability of enrolled patients to complete the trial. If we elect or are required to delay, suspend or discontinue any clinical trial of any of our product candidates, the commercial prospects of such product candidates will be harmed and our ability to generate product revenues from any of these product candidates will be delayed or eliminated.

Any of these occurrences may harm our business, financial condition and prospects significantly. Additionally, if any of our biopharmaceutical product candidates receive marketing approval, the FDA could require us to adopt a REMS to ensure that the benefits of the product outweigh its risks, which may include, among other things, a medication guide outlining the risks of gene therapies for distribution to patients and a communication plan to patients and healthcare practitioners. Other elements to assure safe use in a mandated REMS could include, but are not limited to, restrictions upon distribution and prescribing, additional prescriber training, establishment of patient registries and other measures that could limit commercialization of the product. Comparable foreign regulating authorities might require adoption of measures similar to a ~~48~~ REMS. Furthermore, if we or others later identify undesirable side effects caused by our product candidate, a number of potentially significant negative consequences could result, including: • regulatory authorities may withdraw approvals of such product candidate; • regulatory authorities may require additional warnings on the label; • we may be required to create a medication guide outlining the risks of such side effects for distribution to patients; • we may be required to change the way a product candidate is administered or conduct additional clinical trials; • we could be sued and held liable for harm caused to patients; and • our reputation may suffer. Any of these events could prevent us from achieving or maintaining market acceptance of our product candidates and could significantly harm our business, prospects, financial condition and results of operations. **49** If we are unable to successfully develop related diagnostics for our therapeutic product candidates, or experience significant delays in doing so, we may not achieve marketing approval or realize the full commercial potential of our therapeutic product candidates. We may develop related diagnostics for some of our therapeutic product candidates. Such related diagnostics are subject to regulation by the FDA and typically to comparable foreign regulatory authorities as medical devices and typically require separate regulatory approval or clearance prior to commercialization. Marketing approval or clearance of the diagnostic will require sufficient data to support its safety and efficacy. In addition, at least in some cases, the FDA and comparable foreign regulatory authorities may require the development and regulatory approval or clearance of a related diagnostic as a condition to approving our therapeutic product candidates. While we have some limited experience in developing diagnostics, we plan to rely in large part on third parties to perform these functions. We may seek to enter into arrangements with one or more third parties to create a related diagnostic for use with our current or future product candidates. If we or any third parties that we engage to assist us are unable to successfully develop or obtain marketing approval or clearance for related diagnostics for our therapeutic product candidates, or experience delays in doing so: • the development of relevant product candidates may be delayed or impaired altogether if we are unable to appropriately select patients for enrollment in our clinical trials; • our relevant therapeutic product candidate may not receive marketing approval if its effective use depends on a related diagnostic in the regulatory authority' s judgment; and • we may not realize the full commercial potential of any therapeutic product candidates that receive marketing approval if, among other reasons, we are unable to appropriately identify patients with the specific genetic alterations targeted by our therapeutic product candidates. If any of these events were to occur, our business would be harmed. Even if we complete the necessary preclinical studies and clinical trials, we cannot predict when or if we will obtain regulatory approval to commercialize a product candidate or the approval may be for a narrower indication than we expect. We cannot commercialize a product until the appropriate regulatory authorities have reviewed and approved the product candidate. Even if our product candidates demonstrate safety and efficacy in clinical trials, the regulatory ~~49~~ agencies may not complete their review processes in a timely manner, or we may not be able to obtain regulatory approval. Additional delays may result if an FDA Advisory Committee or comparable foreign regulatory authority recommends non- approval or restrictions on approval. In addition, we may experience delays or rejections based upon additional government regulation from future legislation or administrative action, or changes in regulatory agency policy during the period of product development, clinical trials and the review process. Regulatory agencies may also approve a treatment candidate for fewer or more limited indications than requested, may not approve the price we intend to charge for our product candidate, may limit our ability to promote the product, may impose significant limitations upon the approval of the product, including, but not limited to, narrow indications, significant warnings, precautions or contraindications with respect to conditions of use, or may grant approval subject to the performance of post- marketing studies. In addition, regulatory agencies may not approve the labeling claims that are necessary or desirable for the successful commercialization of our product candidates. The FDA or comparable foreign regulatory authorities may impose a REMS or other conditions upon our approval that limit our ability to commercialize the product candidate. Inadequate funding for the FDA and other government agencies could hinder their ability to hire and retain key leadership and other personnel, prevent new products and services from being developed or commercialized in a timely manner or otherwise prevent those agencies from performing normal business functions on which the operation of our business may rely, 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 and accept the payment of user **50** 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 on which our operations may rely, including those that fund research and development activities is subject to the political process, which is inherently fluid and unpredictable. 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. For example, in recent years, including in 2018 and 2019, the U. S. government shut down several times and certain regulatory agencies, such as the FDA, had to furlough critical employees and stop critical activities. Separately, in response to the COVID- 19 pandemic, the FDA postponed most inspections of foreign manufacturing facilities and products, postponed routine surveillance inspections of domestic manufacturing facilities and is conducting only teleconference meetings. Regulatory authorities **in the United States and** outside the United States may adopt similar restrictions or other policy measures in response to **any future similar events** ~~the COVID- 19 pandemic~~ . If a prolonged government shutdown occurs, 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 business. Further, in our operations as a public company, future government shutdowns could

impact our ability to access the public markets and obtain necessary capital in order to properly capitalize and continue our operations. Even if we obtain regulatory approval for a product candidate, our products will remain subject to regulatory scrutiny. Even if we obtain regulatory approval in a jurisdiction, the regulatory authority may still impose significant restrictions on the indicated uses or marketing of our product candidates, or impose ongoing requirements for potentially costly post-approval studies or post- market surveillance. For example, the holder of an approved BLA is obligated to monitor and report to the FDA adverse events and any failure of a product to meet the specifications in the BLA. FDA guidance advises that patients treated with some types of gene therapy undergo follow- up observations for potential adverse events for as long as 15 years. The holder of an approved BLA must also submit new or supplemental applications and obtain FDA approval for certain changes to the approved product, product labeling or manufacturing process. Advertising and promotional materials must comply with FDA rules and are subject to FDA review, in addition to other potentially applicable foreign, federal and state laws. ~~50~~ In addition, product manufacturers and their establishments, products and applications are subject to payment of user fees / or and continual review and periodic inspections by the FDA and comparable foreign regulatory authorities for compliance with cGMP and comparable foreign requirements, and adherence to commitments made in the BLA. If we or a regulatory agency discover previously unknown problems with a product such as adverse events of unanticipated severity or frequency, or problems with the facility where the product is manufactured, a regulatory agency may impose restrictions relative to that product or the manufacturing facility, including requiring recall or withdrawal of the product from the market or suspension of manufacturing. If we fail to comply with applicable regulatory requirements following approval of any of our product candidates, a regulatory agency may: • issue a warning letter asserting that we are in violation of the law; • seek an injunction or impose civil or criminal penalties or monetary fines; • suspend or withdraw regulatory approval; • suspend any ongoing clinical trials; • refuse to permit government reimbursement of our product by government- sponsored third- party payers; **51** • refuse to approve a pending BLA or supplements to a BLA submitted by us for other indications or new product candidates; • seize our product; or • refuse to allow us to enter into or continue supply contracts, including government contracts. 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 product candidates and generate revenues. Even if we obtain and maintain approval for our product candidates from the FDA, we may never obtain approval for our product candidates outside of the United States, which would limit our market opportunities and adversely affect our business. Approval of a product candidate in the United States by the FDA does not ensure approval of such product candidate by regulatory authorities in other countries or jurisdictions, and approval by one foreign regulatory authority does not ensure approval by regulatory authorities in other foreign countries or by the FDA. Sales of our product candidates outside of the United States will be subject to foreign regulatory requirements governing clinical trials and marketing approval. Even if the FDA grants marketing approval for a product candidate, comparable regulatory authorities of foreign countries must also approve the manufacturing and marketing of the product candidates in those countries. Approval procedures vary among jurisdictions and can involve requirements and administrative review periods different from, and greater than, those in the United States, including additional preclinical studies or clinical trials. In many countries outside the United States, a product candidate must be approved for reimbursement before it can be approved for sale in that country. In some cases, the price that we intend to charge for our products, if approved, is also subject to approval. Even if a product candidate is approved, the FDA or comparable regulatory authorities in other countries, as the case may be, may limit the indications for which the product may be marketed, require extensive warnings on the product labeling or require expensive and time-consuming clinical trials or reporting as conditions of approval. Regulatory authorities in countries outside of the United States also have requirements for approval of product candidates with which we must comply prior to marketing in those countries. Obtaining foreign regulatory approvals and compliance with foreign regulatory requirements could result in significant delays, difficulties and costs for us and could delay or prevent the introduction of our product candidates in certain countries. ~~51~~ Further, clinical trials conducted in one country may not be accepted by regulatory authorities in other countries. Regulatory approval of a product candidate in one country does not ensure approval in any other country, but a failure or delay in obtaining regulatory approval in one country may have a negative effect on the regulatory approval process in other countries. Also, regulatory approval for any of our product candidates may be withdrawn based on adverse events reported or regulatory decisions made in other countries. If we fail to comply with the regulatory requirements in international markets and / or fail to receive applicable marketing approvals, our target market will be reduced, our ability to realize the full market potential of our product candidates will be compromised and our business may be adversely affected. Our future prospects may also depend on our collaborators' ability to successfully develop a pipeline of additional product candidates, and we and our collaborators may not be successful in efforts to use our platform technologies to identify or discover additional product candidates. The success of our business depends primarily upon our ability to identify, develop and commercialize products based on our platform technology. We do not have any products on the market and are early in our development efforts. ~~All of our product candidates are in preclinical development.~~ Our product candidates derived from our platform technology may not successfully complete investigational new drug, or IND, enabling studies, and our research programs may fail to identify other potential product candidates for clinical development for a number of reasons. Our and our collaborators' research methodology may be unsuccessful in identifying potential product candidates, our potential product candidates **52** may not demonstrate the necessary preclinical outcomes to progress to clinical studies, or our product candidates may be shown to have harmful side effects or may have other characteristics that may make the products unmarketable or unlikely to receive marketing approval. If any of these events occur, we may be forced to discontinue our development efforts for a program or programs. Research programs to identify new product candidates require substantial technical, financial and human resources. We may focus our efforts and resources on potential programs or product candidates that ultimately prove to be unsuccessful. We may not be able to obtain orphan drug exclusivity, where relevant, in all markets for our product candidates. Of our current pipeline product candidates,

only our silence and replace therapeutic for the treatment of OPMD has been designated with orphan drug status. In January 2018, the FDA granted such designation after our candidate for the treatment of OPMD had been designated an orphan drug in January 2017 by the European Commission. Regulatory authorities in some jurisdictions, including the United States, may designate drugs or biological products for relatively small patient populations as orphan drugs. Under the Orphan Drug Act, the FDA may designate a product as an orphan drug if it is a product intended to treat a " rare disease or condition ", which is generally defined as any disease or condition which affects less than 200, 000 individuals in the United States. The FDA may also designate a product as an orphan drug if it is intended to treat a disease or condition which affects more than 200, 000 individuals in the United States and there is no reasonable expectation that the cost of developing and making a drug or biological product available in the United States for this type of disease or condition will be recovered from sales of the product candidate. Generally, if a product with an orphan drug designation subsequently receives the first marketing approval for the indication for which it has such designation, the product is entitled to a period of marketing exclusivity, which precludes the FDA from approving another marketing application for the same drug for such indication for that time period. The applicable period is seven years in the United States. Orphan drug exclusivity may be lost if the FDA or the European Medicines Agency, or the EMA, determines that the request for designation was materially defective or if the manufacturer is unable to assure sufficient quantity of the drug to meet the needs of patients with the rare disease or condition. While there is no guarantee, FDA orphan drug designation may provide a range of benefits, including a potential fast track process for clinical regulatory approval, potential tax credits for qualified clinical trials and an exemption from FDA application user fees. ~~52~~ Even if we obtain orphan drug exclusivity for a product in the United States or for additional products in the European Union, that exclusivity may not effectively protect the product from competition because different drugs can be approved for the same condition, and the same drug could be approved for a different condition. Even after an orphan drug is approved, the FDA can subsequently approve the same drug, made by a competitor, for the same condition if the FDA concludes that the competitive product is clinically superior in that it is shown to be safer, more effective or makes a major contribution to patient care. In the European Union, the EMA can approve a competitive product if the orphan drug no longer meets the criteria for orphan designation (including sufficient profitability), if the competitive product is safer, more effective or otherwise clinically superior, or if the orphan drug cannot be supplied in sufficient quantities. Risks Related to Our Reliance on Third Parties Our prospects for successful development and commercialization of our products are dependent to varying degrees upon the research, development, commercialization, and marketing efforts of any potential collaborators. We rely on third parties for certain aspects of the research, development, commercialization and marketing of our current and any future product candidates. Other than as provided for in our collaboration agreements, we have no control over the resources, time and effort that our collaborators may devote to the development of product **53** candidates. We are dependent on our collaborators to conduct some aspects of the research and development of each of our product candidates, and expect to need access to them to facilitate and / or to complete the regulatory process. We will likely rely on a pharmaceutical company for the successful marketing and commercialization of any such product candidates for which they / we receive approval, if any. There can be no guarantee at this stage that we will conclude a partnership with such a company on favorable terms, or at all, nor even if we do so, that success will be achieved. Our ability to recognize revenues from successful potential collaborations may be impaired by multiple factors including: • a collaborator may shift its priorities and resources away from our programs due to a change in business strategies, or a merger, acquisition, sale or downsizing of its company or business unit; • a collaborator may cease development in an area that is the subject of a collaboration agreement; • a collaborator may change the success criteria for a particular program or product candidate in development, thereby delaying or ceasing development of such program or product candidate in development; • a collaborator with development or commercialization obligations may not commit sufficient financial or human resources to the development, marketing, distribution or sale of a product, or may otherwise fail in development or commercialization efforts; • a collaborator with manufacturing responsibilities may encounter regulatory, resource or quality issues and be unable to meet demand requirement; • a collaborator could independently develop, or develop with unrelated parties, products that compete directly or indirectly with our product candidates; • a collaborator may exercise its rights under the agreement to discontinue our collaboration; • a dispute may arise between us and a collaborator concerning the development or commercialization of a product candidate, resulting in a delay in milestones, royalty payments, or discontinuation of a program and possibly resulting in costly litigation or arbitration that may divert management attention and resources; • a collaborator may not adequately protect the intellectual property rights associated with a product candidate; ~~53~~ • a collaborator may use our proprietary information or intellectual property in such a way as to expose us actual or threatened litigation from a third party, patent office proceedings or other risks that could jeopardize or invalidate our intellectual property or proprietary information or expose us to potential liability; and • a collaborator may own or co- own, or have a license to use, intellectual property rights associated with a product candidate that results from our collaborating with them, and in such cases, we would not have the exclusive right to commercialize such intellectual property rights. If our potential collaborators do not perform in the manner we expect or fulfill their responsibilities in a timely manner, or at all, the development, regulatory and commercialization process could be delayed or discontinued or otherwise be unsuccessful. Conflicts between us and our collaborators may arise. In the event of discontinuation of one or more of our collaboration agreements, it may become necessary for us to assume the responsibility for any such product candidates at our own expense or seek new collaborators. In that event, we likely would be required to limit the size and scope of one or more of our independent programs or increase our expenditures and seek additional funding, which may not be available on acceptable terms or at all, and our business may be harmed. **54** We rely on third parties to conduct our preclinical studies and clinical trials. We do not have the ability to conduct all aspects of our preclinical testing or any future clinical trials ourselves. We are dependent on third parties to conduct the preclinical studies for our product candidates and will depend on third parties to conduct any future clinical trials for our product candidates, and therefore the timing of the initiation and completion of these trials and studies is reliant on third parties and may

occur at times substantially different from our estimates or expectations. In the case of clinical trials, we expect to rely on CROs and third- party collaborators to conduct any future clinical trials in accordance with our clinical protocols and regulatory requirements. We expect our CROs, investigators and third- party collaborators will play a significant role in the conduct of these trials and subsequent collection and analysis of data. There is no guarantee that any CROs, investigators or the other third-party collaborators on which we rely for administration and conduct of our future clinical trials will devote adequate time and resources to such trials or perform as contractually required. If any of these third parties fails to meet expected deadlines, fails to adhere to our clinical protocols, fails to meet regulatory requirements or otherwise performs in a substandard manner, any future clinical trials may be extended, delayed or terminated. If our current or any of our future clinical trial sites terminates for any reason, we may lose all of the information on subjects enrolled in any such clinical trials. If we cannot contract with acceptable third parties on commercially reasonable terms, or if these third parties do not carry out their contractual duties, satisfy legal and regulatory requirements for the conduct of preclinical studies or clinical trials or meet expected deadlines, our clinical development programs could be delayed or discontinued. In all events, we are responsible for ensuring that each of our preclinical studies, and our future clinical trials are conducted in accordance with the general investigational plan and protocols for the study or trial. The FDA requires clinical trials to be conducted in accordance with current GCP, including 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 and confidentiality of clinical trial participants are protected. Our reliance on third parties that we do not control does not relieve us of these responsibilities and requirements, and any failure to satisfy these responsibilities and requirements, whether caused by us or by third parties upon whom we rely, could have a material adverse effect on our business, financial condition, results of operations and prospects. ~~54~~ Because we rely on third- party manufacturing and supply partners, our supply of research and development, preclinical and clinical development materials may become limited or interrupted or may not be of satisfactory quantity or quality. We rely on third- party supply and manufacturing partners to manufacture and supply the materials for our research and development and preclinical and clinical study supplies. We do not own manufacturing facilities or supply sources for such materials. There can be no assurance that our supply of research and development, preclinical and clinical development biologics and other materials will not be limited, interrupted or restricted in certain geographic regions, be of satisfactory quality or continue to be available at acceptable prices. Replacement of a third- party manufacturer could require significant effort, cost and expertise because there may be a limited number of qualified replacements. The manufacturing process for a product candidate is subject to FDA and foreign regulatory authority review. Suppliers and manufacturers must meet applicable manufacturing requirements and undergo rigorous facility and process validation tests required by regulatory authorities in order to comply with regulatory standards, such as cGMP. In the event that any of our suppliers or manufacturers fails to comply with such requirements or to perform its obligations to us in relation to quality, timing or otherwise, or if our supply of components or other **55** materials becomes limited or interrupted for other reasons, we may be forced to manufacture the materials ourselves or enter into an agreement with another third party, which would be costly and delay any future clinical trials. In some cases, the technical skills or technology required to manufacture our product candidates may be unique or proprietary to the original manufacturer and we may have difficulty, or there may be contractual restrictions prohibiting us from, transferring such skills or technology to another third party. These factors increase our reliance on our manufacturers and may require us to obtain a license from a manufacturer in order to have another third- party manufacture our product candidates. 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 and guidelines of the FDA and comparable foreign regulatory authorities. The delays and costs associated with the verification of a new manufacturer could increase our costs and delay the development of our product candidates. We expect to continue to rely on third- party manufacturers for preclinical and clinical grade product candidates and if we receive regulatory approval for any product candidate. To the extent that we have existing, or enter into future, manufacturing arrangements with third parties, we will depend on these third parties to perform their obligations in a timely manner consistent with contractual and regulatory requirements, including those related to quality control and assurance. If we are unable to obtain or maintain third- party manufacturing for product candidates, or to do so on commercially reasonable terms, we may not be able to develop and commercialize our product candidates successfully. Our or a third party' s failure to execute on our manufacturing requirements could adversely affect our business in a number of ways, including: • an inability to conduct necessary preclinical studies to progress our product candidates to clinical trials; • an inability to initiate or continue any future clinical trials of product candidates under development; • delay in submitting regulatory applications, or receiving regulatory approvals, for product candidates; • loss of the cooperation of a collaborator; • subjecting our product candidates to additional inspections by regulatory authorities; • requirements to cease distribution or to recall batches of our product candidates; and • in the event of approval to market and commercialize a product candidate, an inability to meet commercial demands for our products. ~~55~~ Our third- party manufacturers may be subject to damage or interruption from, among other things, events such as the COVID- 19 pandemic, fire, natural or man- made disaster, power loss, telecommunications failure, unauthorized entry, computer viruses, denial- of-service attacks, acts of terrorism, human error, vandalism or sabotage, financial insolvency, bankruptcy and similar events. We and our licensees or collaborators may disagree over our right to receive payments under any potential collaboration agreements with them, potentially resulting in costly litigation and loss of reputation. Our ability to receive payments under any out- license and collaboration agreements depends on our ability to clearly delineate our rights under those agreements. We have licensed and may license additional portions of our intellectual property to our collaborators with the intent that our collaborators will develop product candidates based on our ddRNAi or other technology to address specific conditions. However, a collaborator may use our intellectual property without our permission, dispute our ownership of intellectual property rights, or argue that our intellectual property does not cover, or add value to, any product candidates they develop. If a dispute arises, it may result in costly patent office procedures and litigation, and our collaborator may refuse to pay us while the dispute is ongoing.

Furthermore, regardless of any resort to legal action, a dispute with a collaborator over intellectual property rights may damage our relationship with that collaborator and may also harm our reputation **56** in the industry. Even if we are entitled to payments from our collaborators, we may not actually receive these payments, or we may experience difficulties in collecting the payments to which we believe we are entitled. After our collaborators launch commercial products containing our licensed traits, we will need to rely on the good faith of our collaborators to report to us the sales they earn from these products and to accurately calculate the payments we are entitled to, a process that will involve complicated and difficult calculations. Although we seek to address these concerns in our license and collaboration agreements by reserving our right to audit financial records, such provisions may not be effective. We have only limited experience in regulatory affairs and intend to rely on consultants and other third parties for regulatory matters, which may affect our ability or the time we require to obtain necessary regulatory approvals. We have limited experience in filing and prosecuting the applications necessary to gain regulatory approvals for gene therapy or ddRNAi product candidates. Moreover, the product candidates that are likely to result from our development programs are based on novel technologies that have not been extensively tested in humans. The regulatory requirements governing these types of product candidates may be less well defined or more rigorous than for conventional products. As a result, we may experience a longer regulatory process in connection with obtaining regulatory approvals of any products that we develop. We intend to rely on independent consultants for purposes of our regulatory compliance and product development and approvals in the United States and elsewhere. Any failure by our consultants to properly advise us regarding, or properly perform tasks related to, regulatory compliance requirements could compromise our ability to develop and seek regulatory approval of our product candidates. Our reliance on third parties requires us to share our trade secrets, which increases the possibility that a competitor will discover them or that our trade secrets will be misappropriated or disclosed. Because we rely on third parties to manufacture our product candidates, and because we collaborate with various organizations and academic institutions on the advancement of our technology and product candidates, we may, at times, share trade secrets with them. We seek to protect our proprietary technology in part by entering into confidentiality agreements and, if applicable, material transfer agreements, collaborative research agreements, consulting agreements or other similar agreements with our collaborators, advisors, employees 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, such as trade secrets. Despite these contractual provisions, the need to share trade secrets and other confidential information increases the risk that such trade secrets become known by potential competitors, are inadvertently incorporated into the technology of others, or ~~56~~ 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, discovery by a third party of our trade secrets or other unauthorized use or disclosure would impair our intellectual property rights and protections in our product candidates. In addition, these agreements typically restrict the ability of our collaborators, advisors, employees and consultants to publish data potentially relating to our trade secrets. Our academic collaborators typically have rights to publish data, provided that we are notified in advance and may delay publication for a specified time in order to secure our intellectual property rights arising from the collaboration. In other cases, publication rights are controlled exclusively by us or we may share these rights with other parties. Despite our efforts to protect our trade secrets, our competitors may discover our trade secrets, either through breach of these agreements, independent development or publication of information including our trade secrets in cases where we do not have proprietary or otherwise protected rights at the time of publication. **57** Risks Related to Commercialization of Our Product Candidates We have not entered into agreements with any third- party manufacturers to support commercialization of our product candidates. We have not yet secured manufacturing capabilities for commercial quantities of our product candidates or established facilities in the desired locations to support commercialization of our product candidates. We intend to rely on third- party manufacturers for commercialization, but have not entered into any agreements with such manufacturers to support our product candidates currently in development. We may be unable to negotiate agreements with third- party manufacturers to support our commercialization activities on commercially reasonable terms. We may encounter technical or scientific issues related to manufacturing or development that we may be unable to resolve in a timely manner or with available funds. Currently, we do not have the capacity to manufacture our product candidates on a commercial scale. In addition, our product candidates are novel, and only one manufacturer currently has experience producing our product candidates on a large scale. If we are unable to engage manufacturing partners to produce our product candidates on a larger scale on reasonable terms, our commercialization efforts will be harmed. Even if we timely develop a manufacturing process and successfully transfer it to the third- party manufacturers of our product candidates, if such third- party manufacturers are unable to produce the necessary quantities of our product candidates, or do so in compliance with cGMP or with pertinent foreign regulatory requirements, and within our planned time frame and cost parameters, the development and sales of our product candidates, if approved, may be impaired. In some jurisdictions, approval of the manufacturer may be required. There is no guarantee such approval can be obtained. If we are unable to enter into agreements with third parties to commercialize our product candidates or establish sales and marketing capabilities to market and sell our product candidates, we may be unable to generate any revenues. We currently have no sales and marketing organization and have no experience selling and marketing pharmaceutical products. To successfully commercialize any product candidates that may be approved, we will need to develop these capabilities, either through our relationships with collaborators or our own. We may seek to enter into collaborations with other entities to utilize their marketing and distribution capabilities, but we may be unable to enter into marketing agreements on favorable terms, if at all. If our future collaborators do not commit sufficient resources to commercialize our future products, if any, and we are unable to develop the necessary marketing capabilities on our own, we will be unable to generate sufficient product revenue to sustain our business. The establishment and development of our own sales force or the establishment of a contract sales force to market any products we may develop will be expensive and time- consuming and could delay any product launch. Moreover, we cannot be certain that we will be able to successfully develop this capability. We ~~57~~ will be competing with many companies that currently have extensive and well- funded marketing and sales operations to recruit, hire, train and

retain marketing and sales personnel. We also face competition in our search for third parties to assist us with the sales and marketing efforts of our product candidates. Without an 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. Physicians, patients, third-party payers or others in the medical community may not be receptive to our product candidates, and we may not generate any future revenue from the sale or licensing of our product candidates. Even if we obtain approval for a product candidate, we may not generate or sustain revenue from sales of the product if the product cannot be sold at a competitive cost or if it fails to achieve market acceptance by physicians, patients, third- party payers or others in the medical community. These market participants may be hesitant to adopt a novel treatment based on ddRNAi or silence and replace technology, and we may not be able **58** to convince the medical community and third- party payers to accept and use, or to provide favorable reimbursement for, any product candidates developed by us or our existing or future collaborators. Market acceptance of our product candidates will depend on, among other factors: • the safety and efficacy of our product candidates; • our ability to offer our products for sale at competitive prices; • the relative convenience and ease of administration of our product candidates; • the prevalence and severity of any adverse side effects associated with our product candidates; • the terms of any approvals and the countries in which approvals are obtained; • limitations or warnings contained in any labeling approved by the FDA or comparable foreign regulatory authorities; • conditions upon the approval imposed by FDA or comparable foreign regulatory authorities, including, but not limited to, a REMS; • the willingness of patients to try new treatments and of physicians to prescribe these treatments; • the availability of government and other third- party payer coverage and adequate reimbursement; and • the availability of alternative effective treatments for the disease indications our product candidates are intended to treat and the relative risks, benefits and costs of those treatments. Since we are focused on the emerging therapeutic modality of ddRNAi, and silence and replace these risks may increase if new competitors are able to market ddRNAi- based therapeutics or silence and replace- based therapeutics or if these treatments become less favored in the commercial marketplace. In addition, we believe that one of the benefits of our ddRNAi and silence and replace technologies is the expected length of time of the effects. If our treatments do not have a long- term effect after administration, such a development would likely significantly and adversely affect market acceptance of our product candidates, if approved. Additional risks apply in relation to any disease indications we pursue which are classified as rare diseases and allow for orphan drug designation by regulatory agencies in major commercial markets, such as the United States or European Union. If pricing is not approved or accepted in the market at an appropriate level for any approved product for which we pursue and receive an orphan drug designation, such product may not generate enough revenue to offset costs of development, manufacturing, marketing and commercialization despite any benefits received from the orphan drug designation, such as market exclusivity, for a period of time. Orphan exclusivity could temporarily delay or block approval of one of our products if a competitor obtains orphan drug designation for its product first. However, even if we obtain orphan exclusivity for one of our products upon approval, our exclusivity may not block the subsequent approval of a competitive product that is shown to be clinically superior to our product. ~~58~~ Market size is also a variable in disease indications not classified as rare. Our estimates regarding potential market size for any indication may be materially different from what we discover to exist at the time we commence commercialization, if any, for a product, which could result in significant changes in our business plan and have a material adverse effect on our business, financial condition, results of operations and prospects ~~. Events such as the COVID- 19 pandemic and other public health crises may materially and adversely affect our business, financial condition and results of operations. The development and commercialization of our products is dependent on the efforts of our third- party collaborators, as we rely on third parties to conduct our preclinical studies and clinical trials, and for our supply of research and development, preclinical and clinical development biologics and other materials. Accordingly, our operations and supply chain could be subject to significant disruptions to the extent that our third- party partners are impacted by the COVID- 19 pandemic or the associated governmental actions. There may be related restrictions or delays with respect to clinical trials and preclinical studies, as well as the export, import or shipment of raw materials, drug substance or drug product that could materially delay our business or clinical trials. Further, certain of our research and development efforts are conducted globally, including our ongoing development of our silence and replace therapeutic for the treatment of Oculopharyngeal Muscular Dystrophy (OPMD), and will be dependent upon our ability to initiate clinical sites and enroll patients despite the ongoing COVID- 19 pandemic. We had also implemented work- from- home measures for the majority of our employees between March 2020 and June 2021, resulting in a reduction of laboratory work and a halt of non- essential business travel. As we transition our employees back to our premises, there is a risk that COVID- 19 infections occur at our offices or laboratory facilities and significantly affect our operations. Additionally, if any of our critical vendors are impacted, our business could be affected if we become unable to timely procure essential equipment, supplies or services in adequate quantities and at acceptable prices. Moreover, the COVID- 19 outbreak has caused significant disruption to the global economy and in the global and U. S. financial markets. This could impact our ability to raise additional funding, which may not be available on acceptable terms, if at all. The extent to which COVID- 19 impacts our results will depend on future developments, which are highly uncertain and cannot be predicted, including new information which may emerge concerning the severity of COVID- 19 and the actions to contain COVID- 19 or treat its impact, among others. In addition, due to the speed with which the COVID- 19 situation is developing and evolving, there is uncertainty around its ultimate impact on public health, business operations and the overall economy; therefore, the negative impact on our financial position, operating results and liquidity cannot be reasonably estimated at this time, but the impact may be material~~ . We face competition from entities that have developed or may develop product candidates for our target disease indications, including companies developing novel treatments and technology platforms based on modalities and technology similar to ours. The development and commercialization of pharmaceutical products is highly competitive. We compete with a variety of multinational pharmaceutical companies and specialized biotechnology companies, as well as technology being developed at universities and other research institutions. Our competitors have developed, are **59** developing or could develop product candidates and processes competitive with our product candidates. Competitive therapeutic

treatments include those that have already been approved and accepted by the medical community, patients and third- party payers, and any new treatments that enter the market. ~~59~~ There may be a significant number of products that are currently under development, and may become commercially available in the future, for the treatment of conditions for which we are developing, and may in the future try to develop, product candidates. This increasingly competitive landscape may compromise the development of our product candidates. We are aware of multiple companies that are working in the field of RNAi therapeutics, including Alnylam, Arbutus, **and** Arrowhead. Some of our current product candidates, if approved, would compete with approved and currently marketed treatments. In addition, our ddRNAi- based product candidates would compete with antisense and other RNA- based pharmaceutical products currently under development. Like RNAi therapeutics, antisense products target mRNA with the objective of suppressing the activity of specific genes. The development of antisense products is more advanced than that of RNAi therapeutics, and antisense technology may become the preferred technology for products that target mRNAs. Many of our competitors have significantly greater financial, technical, manufacturing, marketing, sales and supply resources and experience than we have. If we successfully obtain approval for any product candidate, we will face competition based on many different factors, including the safety and effectiveness of our products, the ease with which our products can be administered and the extent to which patients accept relatively new routes of administration, the timing and scope of regulatory approvals for these products, the availability and cost of manufacturing, marketing and sales capabilities, price, reimbursement coverage and patent position. Competing products could present superior treatment alternatives, including by being more effective, safer, less expensive or marketed and sold more effectively than any products we may develop. Competitive products may make any products we develop obsolete or non- competitive before we recover the expense of developing and commercializing our product candidates. Such competitors could also recruit our employees, which could negatively impact our level of expertise and our ability to execute our business plan. A variety of risks outside of our control associated with international operations could adversely affect our business. If any of our product candidates are approved for commercialization, it is our current intention to market them on a worldwide basis, either alone or in collaboration with others. In addition, we conduct development activities in various jurisdictions throughout the world. We expect that we will be subject to additional risks related to engaging in international operations, including: • different regulatory requirements for approval of biopharmaceutical products in foreign countries; • reduced protection for intellectual property rights; • unexpected changes in tariffs, trade barriers and regulatory requirements; • economic weakness, including inflation, or political instability in particular foreign economies and markets; • compliance with tax, employment, immigration and labor laws for employees living or traveling abroad; • foreign currency fluctuations, which could result in increased operating expenses and reduced revenue, and other obligations incident to doing business in another country; **60** • workforce uncertainty in countries where labor unrest is more common than in Australia or the United States; ~~60~~ • production shortages resulting from any events affecting raw material supply or manufacturing capabilities abroad; and • business interruptions resulting from geopolitical actions, including war and terrorism, natural disasters, including earthquakes, typhoons, floods and fires and disease pandemics and epidemics. The insurance coverage and reimbursement status of newly approved products is uncertain. The availability of coverage and adequate reimbursement by governmental and private payers is essential for most patients to be able to afford expensive treatments. Sales of our product candidates will depend substantially, both in the United States and abroad, on the extent to which the costs of our product candidates will be paid by health maintenance, managed care, pharmacy benefit and similar healthcare management organizations, or reimbursed by third- party payers, and there have been increasing efforts by governmental and other third- party payers, in the United States and abroad, to cap or reduce healthcare costs. If reimbursement is not available, or is available only to limited levels, we may not be able to successfully commercialize our product candidates. Even if coverage is provided, the reimbursement amounts approved by third- party payers may not be high enough to allow us to establish or maintain pricing sufficient to realize a return on our investment. There is significant uncertainty related to the insurance coverage and reimbursement of newly approved products. In the United States, the principal decisions about reimbursement for new medicines are typically made by the Centers for Medicare & Medicaid Services, or CMS, an agency within the U. S. Department of Health and Human Services, as CMS decides whether and to what extent a new medicine will be covered and reimbursed under Medicare. Private payers tend to follow CMS to a substantial degree. It is difficult to predict what CMS will decide with respect to reimbursement for fundamentally novel products such as ours, as there is no body of established practices and precedents for these new products. The intended use of a biopharmaceutical product by a physician can also affect pricing. For example, CMS could initiate a National Coverage Determination administrative procedure, by which the agency determines which uses of a therapeutic product would and would not be reimbursable under Medicare. This determination process can be lengthy, thereby creating a long period during which the future reimbursement for a particular product may be uncertain. Outside the United States, international operations are generally subject to extensive governmental price controls and other market regulations, and we believe the increasing emphasis on cost- containment initiatives in Europe, Canada and other countries is likely to put pressure on the pricing and usage of any of our product candidates that may be approved for marketing in the future. In many countries, the prices of medical products are subject to varying price control mechanisms as part of national health systems. In general, the prices of medicines under such systems can be substantially lower than in the United States. Other countries allow companies to fix their own prices for medicines, but monitor and control company profits. In some countries, we or our collaborators may be required to conduct a clinical trial or other studies that compare the cost- effectiveness of our product candidates to other available therapies in order to obtain or maintain reimbursement or pricing approval. Additional foreign price controls or other changes in pricing regulation could restrict the amount that we are able to charge for our product candidates. Accordingly, in markets outside the United States, the reimbursement for our products may be reduced compared with the United States and may be insufficient to generate commercially reasonable revenue and profits. In the United States, the Patient Protection and Affordable Care Act, as amended by the Health Care Education Reconciliation Act, each enacted in March 2010, as amended, or the ACA, greatly expanded

health insurance coverage in the United States, but has also been the target of litigation and Congressional reform efforts since its adoption, including unsuccessful efforts to seek repeal of the entire ACA. While efforts to strike down the ACA **61** in the courts currently seem stalled, uncertainty regarding the future of the ACA remains, including with respect to legislative efforts. Any significant efforts at the federal or state levels to reform the healthcare system by ~~61~~ changing the way healthcare is provided or funded — including through the expansion of Medicare to larger sections of the population — could have a material impact on our business. In addition, the ACA, and similar laws, may cause such organizations to limit both coverage and level of reimbursement for new products approved and, as a result, they may not cover or provide adequate payment for our product candidates. For example, other legislative changes adopted since the ACA was enacted ACA may cause such organizations to limit both coverage and level of reimbursement for new products approved and, as a result, they may not cover or provide adequate payment for our product candidates. In addition, other legislative changes have been adopted since the ACA was enacted. These changes included aggregate reductions to Medicare payments to providers of up to 2 % per fiscal year effective April 2013 that, due to subsequent legislative amendments to the statute, will stay in effect through 2029 unless additional Congressional action is taken. Note, however, the Coronavirus Aid, Relief, and Economic Security Act (CARES Act), which was signed into law on March 27, 2020, designed to provide financial support and resources to individuals and businesses affected by the COVID- 19 pandemic, suspended the 2 % Medicare sequester from May 1, 2020 through December 31, 2020, and extended the sequester by one year, through 2030. On December 14, 2018, a Texas U. S. District Court Judge ruled that the ACA is unconstitutional in its entirety because the " individual mandate " was repealed by Congress as part of the 2017 Cuts and Jobs Act (the " Tax Act "). Additionally, on December 18, 2019, the U. S. Court of Appeals for the 5th Circuit upheld the District Court ruling that the individual mandate was unconstitutional and remanded the case back to the District Court to determine whether the remaining provisions of the ACA are invalid as well. On March 2, 2020, the United States Supreme Court granted the petitions for writs of certiorari to review this case, although it is unclear when the Supreme Court will make a decision. It is also unclear how such litigation and other efforts to repeal and replace the ACA will impact the ACA and our business. In addition, other legislative changes have been proposed and adopted in the United States since the ACA was enacted. In January 2013, former President Obama signed into law the American Taxpayer Relief Act of 2012, which, among other things, reduced Medicare payments to several providers, and increased the statute of limitations period for the government to recover overpayments to providers from three to five years. This law may result in additional reductions in Medicare and other healthcare funding, which could have a material adverse effect on customers for our product candidates, if approved, and accordingly, our financial operations. We expect to experience pricing pressures in connection with the sale of any of our product candidates due to the trend toward managed healthcare, value and risk- based payment arrangements that put healthcare providers at direct financial risk for the healthcare resources used to care for patient populations and additional legislative changes. The downward pressure on healthcare costs in general, particularly prescription pharmaceutical products and surgical procedures and other treatments, has become very intense. As a result, increasingly high barriers are being erected to the entry of new products. We cannot predict what healthcare reform initiatives may be adopted in the future. ~~It is possible that additional governmental action is taken to address the COVID- 19 pandemic.~~ Further federal, state and foreign legislative and regulatory developments are likely, and we expect ongoing initiatives to increase pressure on biopharmaceutical product pricing. Such reforms could depress pricing for any product candidates that we may successfully develop and for which we may obtain regulatory approval and may negatively affect our overall financial condition and ability to develop additional product candidates. Changes in U. S. tax law may materially adversely affect our financial condition, results of operations and cash flows. The rules dealing with U. S. federal, state, and local income taxation are constantly under review by persons involved in the legislative process and by the Internal Revenue Service and the U. S. Treasury Department. Changes to tax laws (which changes may have retroactive application) could adversely affect us or holders of our **62** common stock, warrants and pre-funded warrants. In recent years, many such changes have been made and ~~62~~ changes are likely to continue to occur in the future. Future changes in U. S. tax laws could have a material adverse effect on our business, cash flow, financial condition or results of operations. We urge our investors to consult with their legal and tax advisors regarding the implications of potential changes in U. S. tax laws on an investment in our common stock, warrants and pre- funded warrants. Our relationships with third- party payers, healthcare professionals and customers in the United States and elsewhere may be subject to anti- kickback, fraud and abuse, false claims, transparency, health information privacy and security and other healthcare laws and regulations, which could expose us to significant penalties. Our relationships with third- party payers, healthcare professionals and customers may expose us to broadly applicable fraud and abuse and other healthcare laws and regulations, including, without limitation, the federal Anti- Kickback Statute and the federal False Claims Act, that may constrain the business or financial arrangements and relationships through which we sell, market and distribute any biopharmaceutical products for which we obtain marketing approval. In addition, we may be subject to transparency laws and patient privacy regulation by the federal government and by the U. S. states and foreign jurisdictions in which we conduct our business. The applicable federal, state and foreign healthcare laws and regulations that may affect our ability to operate include, but are not limited to, the following: • the federal Anti- Kickback Statute, which prohibits, among other things, persons and entities from knowingly and willfully soliciting, offering, receiving or providing remuneration, directly or indirectly, in cash or in kind, to induce or reward, or in return for, either the referral of an individual for, or the purchase, order or recommendation of, any good or service, for which payment may be made under federal and state healthcare programs such as Medicare and Medicaid; • federal civil and criminal false claims laws and civil monetary penalty laws, including the federal False Claims Act, which impose criminal and civil penalties, including civil whistleblower or qui tam actions, against individuals or entities for knowingly presenting, or causing to be presented, to the federal government, including the Medicare and Medicaid programs, claims for payment that are false or fraudulent or making a false statement to avoid, decrease or conceal an obligation to pay money to the federal government; • the federal Health Insurance Portability and Accountability Act of 1996, or HIPAA, which imposes criminal and civil liability for,

among other things, executing a scheme to defraud any healthcare benefit program or making false statements relating to healthcare matters; • HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act of 2009, or HITECH, and their respective implementing regulations, which impose obligations on covered healthcare providers, health plans and healthcare clearinghouses, as well as their business associates that create, receive, maintain or transmit individually identifiable health information for or on behalf of a covered entity, with respect to safeguarding the privacy, security and transmission of individually identifiable health information; • the federal Open Payments program, created under the ACA, and its implementing regulations, which requires certain manufacturers of drugs, devices, biologics and medical supplies for which payment is available under Medicare, Medicaid or the Children' s Health Insurance Program, with specific exceptions, to report annually to the CMS information related to payments or other transfers of value made to physicians, and applicable group purchasing organizations to report annually to CMS ownership and investment interests held by the physicians and their immediate family members by the 90th day of each subsequent calendar year, and disclosure of such information will be made by CMS on a publicly available website; and • analogous state and foreign laws and regulations, such as state anti- kickback and false claims laws, which may apply to sales or marketing arrangements and claims involving healthcare items or services reimbursed by non- governmental third- party payers, including private insurers; state and foreign laws **63** that require biopharmaceutical or biotechnology companies to comply with the industry voluntary ~~63~~ compliance guidelines and the relevant compliance guidance promulgated by the federal government or otherwise restrict payments that may be made to healthcare providers; state and foreign laws that require biopharmaceutical or biotechnology manufacturers to report information related to payments and other transfers of value to physicians and other healthcare providers or marketing expenditures; and state and foreign laws governing the privacy and security of health information in certain circumstances, many of which differ from each other in significant ways and often are not preempted by HIPAA, thus complicating compliance efforts. Efforts to ensure that our business arrangements with third parties will comply with applicable healthcare laws and regulations may involve substantial costs. It is possible that governmental authorities will conclude that our 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 civil, criminal and administrative penalties, including, without limitation, damages, fines, disgorgement, imprisonment, exclusion from participation in government healthcare programs, such as Medicare and Medicaid, and the curtailment or restructuring of our operations, which could have a material adverse effect on our business. If any of the physicians or other healthcare providers or entities with whom we expect to do business is found not to be in compliance with applicable laws, they may be subject to criminal, civil or administrative sanctions, including exclusions from participation in government healthcare programs, which could also materially affect our business. Negative public opinion and increased regulatory scrutiny of gene therapy and genetic research may damage public perception of our product candidates or compromise our ability to conduct our business or obtain regulatory approvals for our product candidates. Gene therapy remains a novel technology and no gene therapy product utilizing ddRNAi or silence and replace has been approved to date in the United States. Public perception may be influenced by claims that gene therapy is unsafe, and gene therapy may not gain the acceptance of the public or the medical community. In particular, our success will depend upon physicians specializing in the treatment of those diseases that our product candidates target prescribing treatments that involve the use of our product candidates in lieu of, or in addition to, existing treatments they are already familiar with 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 we may develop. Our product candidates, including our viral delivery systems, could produce adverse events. Adverse events in our clinical trials or following approval of any of our product candidates, even if not ultimately attributable to our product candidates, 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. Risks Related to Our Business Operations We may not successfully engage in strategic transactions or enter into new collaborations, which could adversely affect our ability to develop and commercialize product candidates, impact our cash position, increase our expenses and present significant distractions to our management. From time to time, we may consider additional strategic transactions, such as collaborations, acquisitions, asset purchases or sales and out- or in- licensing of product candidates or technologies. In particular, we will evaluate and, if strategically attractive, seek to enter into additional collaborations, including with major biotechnology or pharmaceutical companies. The competition for collaborators is significant, and the negotiation process is time- consuming and complex. ~~Any new~~ **Anew** collaboration may be on terms that are not optimal for us ~~,~~ and we may not be ~~able~~ to **64** maintain any new or existing collaboration if, for example, development or approval of a product ~~64~~ candidate is delayed, sales of an approved product candidate do not meet expectations or the collaborator discontinues the collaboration. Any such collaboration, or other strategic transaction, may require us to incur non- recurring or other charges, increase our expenditures, pose significant integration or implementation challenges or disrupt our management or business. These transactions would entail numerous operational and financial risks, including exposure to unknown liabilities, incurrence of substantial debt or dilutive issuances of equity securities to pay transaction consideration or costs, higher than expected collaboration, acquisition or integration costs, write- downs of assets or goodwill or impairment charges, increased amortization expenses, difficulty and cost in facilitating the collaboration or combining the operations and personnel of any acquired business, impairment of relationships with key suppliers, manufacturers or customers of any acquired business due to changes in management and ownership and the inability to retain key employees of any acquired business. Accordingly, although there can be no assurance that we will undertake or successfully complete any additional transactions of the nature described above, any transactions that we do complete may be subject to the foregoing or other risks and have a material adverse effect on our business, results of operations, financial condition and prospects.

Conversely, any failure to enter any collaboration or other strategic transaction that would be beneficial to us could delay and make more expensive the development and potential commercialization of our product candidates and have a negative impact on the competitiveness of any product candidate that reaches market. Any inability to attract and retain qualified key management and technical personnel would impair our ability to implement our business plan. Our success largely depends on the continued service of key management and other specialized personnel, including Dr. Jerel Banks (Chief Executive Officer) and Ms. Megan Boston (Executive Director). The loss of one or more members of our management team or other key employees or advisors, if not adequately replaced, could delay or increase the cost of our research and development programs and materially harm our business, financial condition, results of operations and prospects. The relationships that our key managers have cultivated within our industry make us particularly dependent upon their continued employment with us. We are dependent on the continued service of our technical personnel because of the highly technical nature of our product candidates and the specialized nature of the regulatory approval process for our product candidates. Because our management team and key employees are not obligated to provide us with continued service, they could terminate their employment with us at any time without penalty. We do not maintain key person life insurance policies on any of our management team members or key employees. Our future success will depend in large part on our continued ability to attract and retain other highly qualified scientific, technical and management personnel, as well as personnel with expertise in clinical testing, manufacturing, governmental regulation and commercialization. We face competition for personnel from other companies, universities, public and private research institutions, government entities and other organizations. Our collaborations with outside scientists and consultants may be subject to restriction and change. We work with medical experts, chemists, biologists and other scientists at academic and other institutions, and consultants who assist us in our research, development and regulatory efforts, including the members of our scientific advisory board. In addition, these scientists and consultants have provided, and we expect that they will continue to provide, valuable advice regarding our programs and regulatory approval processes. These scientists and consultants are not our employees and may have other commitments that would limit their future availability to us. If a conflict of interest arises between their work for us and their work for another entity, we may lose their services. In addition, we are limited in our ability to prevent them from establishing competing businesses or developing competing products. For example, if a key scientist acting as a principal investigator in any of our future clinical 65 trials identifies a potential product or compound that is more scientifically interesting to his or her professional interests, his or her availability to remain involved in any future clinical trials could be restricted or eliminated. We may experience difficulties in managing our growth and expanding our operations. We have limited experience in development and commercialization of pharmaceutical products. As our product candidates continue to advance through preclinical studies and any future clinical trials and potentially toward regulatory approval and commercial sale, we will need to expand our development, regulatory, manufacturing and sales capabilities or contract with other organizations to provide these capabilities for us. In the future, we expect to have to manage additional relationships with collaborators or partners, suppliers and other organizations. Our ability to manage our operations and future growth will require us to continue to improve our operational, financial and management controls, reporting systems and procedures. We may not be able to implement improvements to our management information and control systems in an efficient or timely manner and may discover deficiencies in existing systems and controls. Our employees, independent contractors, principal investigators, CROs, consultants and vendors may engage in misconduct or other improper activities, including noncompliance with regulatory standards and insider trading. We are exposed to the risk of fraud or other misconduct by our employees, independent contractors, principal investigators, CROs, consultants, commercial partners and vendors. Misconduct by these parties could include intentional failures to comply with the regulations of the FDA and comparable foreign regulators, provide accurate information to the FDA and comparable foreign regulators, comply with healthcare fraud and abuse laws and regulations in the United States and abroad, report financial information or data accurately or disclose unauthorized activities to us. In particular, sales, marketing and business arrangements in the healthcare industry are subject to extensive laws and regulations intended to prevent fraud, misconduct, kickbacks, self- dealing and other abusive practices. These laws and regulations may restrict or prohibit a wide range of pricing, discounting, marketing and promotion, sales commission, customer incentive programs and other business arrangements. Such misconduct could also involve the improper use of information obtained in the course of any future clinical trials, which could result in regulatory sanctions and cause serious harm to our reputation. We have adopted a code of conduct applicable to all of our employees, but it is not always possible to identify and deter employee misconduct, and the precautions we take to detect and prevent this activity may not be effective in controlling unknown or unmanaged risks or losses or in protecting us from governmental investigations or other actions or lawsuits stemming from a failure to comply with these laws or regulations. If any such actions are instituted against us, and we are not successful in defending ourselves or asserting our rights, those actions could have a significant impact on our business, including the imposition of civil, criminal and administrative penalties, damages, monetary fines, possible exclusion from participation in Medicare, Medicaid and other federal healthcare programs, contractual damages, reputational harm, diminished profits and future earnings, and curtailment of our operations, any of which could adversely affect our ability to operate our business and our results of operations. We could face potential product liability and, if successful claims are brought against us, we may incur substantial liability and costs. The use of our product candidates in clinical trials and the sale of any products for which we may in the future obtain marketing approval exposes us to the risk of product liability claims. Product liability claims might be brought against us by consumers, healthcare providers, pharmaceutical companies or others selling or otherwise coming into contact with our product candidates. There is a risk that our product candidates may induce adverse events. If we cannot successfully defend against product liability claims, we could incur substantial liability and costs. In addition, regardless of merit or eventual outcome, product liability claims may result in: • impairment of our business reputation; • withdrawal of clinical trial participants; 66 • costs due to related litigation; • distraction of management' s attention from our primary business; • substantial monetary awards to patients or other claimants; • the inability to commercialize our product candidates; • decreased

demand for our product candidates, if approved for commercial sale; and • increased cost, or impairment of our ability, to obtain or maintain product liability insurance coverage. We carry combined general public and products liability (including human clinical trials extension) insurance of $ 20 million (per any one occurrence) and $ 10 million (per any one occurrence and in the aggregate), respectively. We believe our product liability insurance coverage is sufficient in light of our current clinical programs. However, we may not be able to maintain insurance coverage at a reasonable cost or in sufficient amounts to protect us against losses due to liability. If we obtain marketing approval for any product candidates, we intend to expand our insurance coverage to include the sale of commercial products, but we may not be able to obtain or maintain product liability insurance on commercially reasonable terms or in adequate amounts. On occasion, large judgments have been awarded against other pharmaceutical companies in class action lawsuits based on pharmaceutical products, or medical treatments that had unanticipated adverse effects. A successful product liability claim or series of claims brought against us could cause the price of our common stock to decline and, if judgments exceed our insurance coverage, could materially and adversely affect our financial position. During the course of treatment, patients may suffer adverse events, including death, for reasons that may be related to our product candidates. Such events could subject us to costly litigation, require us to pay substantial amounts of money to injured patients, delay, negatively impact or end our opportunity to receive or maintain regulatory approval to market our products, or require us to suspend or discontinue our commercialization efforts. Even in a circumstance in which we do not believe that an adverse event is related to our product candidate, the investigation into the circumstance may be time-consuming or inconclusive. These investigations may harm our reputation, delay our regulatory approval process, limit the type of regulatory approvals our product candidates receive or maintain, and compromise the market acceptance of any of our product candidates that may in the future receive regulatory approval. As a result of these factors, a product liability claim, even if successfully defended, could hurt our business and impair our ability to generate revenue. We and our development partners, third- party manufacturers and suppliers use biological materials and may use hazardous materials, and any claims relating to improper handling, storage or disposal of these materials could be time consuming or costly. We and our development partners, third- party manufacturers and suppliers may use hazardous materials, including chemicals and biological agents and compounds that could be dangerous to human health and safety or the environment. Our operations and the operations of our third- party manufacturers and suppliers also produce hazardous waste products. National, state and local laws and regulations in the United States and other countries govern the use, generation, manufacture, storage, handling and disposal of these materials and wastes. Compliance with applicable environmental laws and regulations may be expensive, and current or future environmental laws and regulations may impair our product development and commercialization efforts. In addition, we cannot entirely eliminate the risk of accidental injury or contamination from these materials or wastes. We do not carry specific biological or hazardous waste insurance coverage, and our property, casualty and general liability insurance policies specifically exclude coverage for damages and fines arising from biological or hazardous waste exposure or contamination. Accordingly, in the event of contamination or injury, we could be held liable for damages or be penalized with fines in an amount exceeding our resources, and any future clinical trials, regulatory approvals or product commercialization progress could be suspended. 67 We may use our limited financial and human resources to pursue a particular research program or product candidate and fail to capitalize on programs or product candidates that may be more profitable or for which there is a greater likelihood of success. Because we have limited resources, we may forego or delay pursuit of opportunities with certain programs or product candidates or for indications that later prove to have greater commercial potential. Our resource allocation decisions may cause us to fail to capitalize on viable commercial products or profitable market opportunities. Our spending on current and future research and development programs for product candidates 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 strategic 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, or we may allocate internal resources to a product candidate in a therapeutic area in which it would have been more advantageous to enter into a collaboration arrangement. Our internal computer and information technology systems, or those of our collaborators and other development partners, third- party CROs or other contractors or consultants, may fail or suffer security breaches, which could result in a disruption of our product development programs. Despite the implementation of security measures, our internal computer and information technology systems and those of our current and any future CROs and other contractors, consultants and collaborators are vulnerable to damage from computer viruses, cyber- attacks, unauthorized access, natural disasters, terrorism, war and telecommunication and electrical failures. Such events could cause interruptions of our operations. While we have not experienced any material system failure, accident or security breach to date, if such an event were to occur and cause interruptions in our operations, it could result in a disruption of our development programs and our business operations, whether due to a loss of our trade secrets or other similar disruptions. For example, the loss of clinical trial data from ongoing or future clinical trials or data from preclinical studies could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data. Likewise, we rely on third parties to manufacture our product candidates and will rely on third parties to conduct future clinical trials, and similar events relating to their computer systems could also have similar consequences to our business. To the extent that any disruption or security breach were to result in a loss of, or damage to, our data or applications, or inappropriate disclosure of confidential or proprietary information, we could incur liability and the further development and commercialization of our product candidates could be delayed and become more expensive. **We** ~~As a result of COVID- 19, we~~ may face increased cybersecurity risks due to our reliance on internet technology and the number of our employees that are working remotely, which may create additional opportunities for cybercriminals to exploit vulnerabilities. Cyber- attacks, breaches, interruptions or other data security incidents could result in legal claims or proceedings, liability under federal or state laws that protect the privacy of personal information, regulatory penalties, significant remediation costs, disrupt key business operations and divert attention of management and key information technology

resources. In the United States, notice of breaches must be made to affected individuals, the U. S. Secretary of the Department of Health and Human Services, or HHS, and for extensive breaches, notice may need to be made to the media or U. S. state attorneys general. Such a notice could harm our reputation and our ability to compete. HHS has the discretion to impose penalties without attempting to resolve violations through informal means. In addition, U. S. state attorneys general are authorized to bring civil actions seeking either injunctions or damages in response to violations that threaten the privacy of state residents. There can be no assurance that we, our collaborators, CROs, vendors, and any other business counterparties will be successful in efforts to detect, prevent, protect against or fully recover systems or data from all breakdowns, service interruptions, attacks or breaches of systems. In addition, we do not maintain standalone cyber- security insurance and have limited insurance coverage in the event of any breach or disruption of our or our collaborators', CROs', or vendors' systems, including any unauthorized access or loss of any personal data that we may collect, store or otherwise process. The costs related to significant security breaches or disruptions could be material and exceed 68 the limits of any insurance coverage we may have. To the extent that any disruption or security breach were to result in a loss of, or damage to, our data or systems, or inappropriate disclosure of confidential or proprietary information, including data related to our personnel, we could incur liability and the further development and commercialization of our product candidates could be delayed and our business and operations could be adversely affected and / or could result in the loss or disclosure of critical or sensitive data, which could result in financial, legal, business or reputational harm to us. Our current laboratory operations are concentrated in one location and any events affecting this location may seriously compromise our ability to operate our business and continue the development of our product candidates. Our current laboratory operations are located in our facility situated in Hayward, California. Any unplanned event, such as flood, fire, explosion, earthquake, extreme weather condition, medical epidemics and pandemics, power shortage, telecommunication failure or other natural or manmade accidents or incidents that result in us being unable to fully utilize the facility, may compromise our ability to operate our business, particularly on a daily basis, cause us financial losses and inhibit or delay our continued development of our product candidates. Loss of access to this facility may result in increased costs, delays in the development of our product candidates or interruption of our business operations. As part of our risk management policy, we maintain insurance coverage at levels that we believe are appropriate for our business. However, in the event of an accident or incident at this facility, we cannot assure you that the amounts of insurance will be sufficient to satisfy any damages and losses. If our facility is unable to operate because of an accident or incident or for any other reason, even for a short period of time, any or all of our research and development programs may be harmed. Any business interruption may have a material adverse effect on our business, financial position, results of operations and prospects. The investment of our cash and cash equivalents is subject to risks which may cause losses and affect the liquidity of these investments. As of June 30, ~~2023~~ **2024**, we had $ ~~2~~ **50**. ~~5~~ **9** million in cash and cash equivalents. We historically have invested substantially all of our available cash and cash equivalents in cash deposits meeting the criteria of our investment policy, which is focused on the preservation of our capital. These investments are subject to general credit, liquidity, market and interest rate risks. We may realize losses in the fair value of these investments, which would have a negative effect on our financial results. In addition, should our investments cease paying or reduce the amount of interest paid to us, our interest income would suffer. The market risks associated with our investment portfolio may have an adverse effect on our results of operations, liquidity and financial condition. Risks Related to Our Intellectual Property If we are unable to obtain or protect sufficient intellectual property rights related to our product candidates, we may not be able to obtain exclusivity for our product candidates or prevent others from developing similar competitive products. We rely upon a combination of patents, know- how, trade secret protection and confidentiality agreements — both that we own or possess or that are owned or controlled by our licensors and licensed to us — to protect the intellectual property related to our technology and product candidates. The patent application process, also known as patent prosecution, is expensive and time- consuming, and we and our current or future licensors and licensees may not be able to prepare, file, and prosecute all necessary or desirable patent applications at a reasonable cost or in a timely manner. It is also possible that we or our current licensors or licensees, or any future licensors or licensees, will fail to identify or obtain sufficient protection for patentable aspects of inventions made in the course of development and commercialization activities before it is too late to obtain patent protection on them. Therefore, our patents and patent applications may not be prosecuted ~~69~~ and enforced in a manner consistent with the best interests of our business. 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 **69** with respect to proper priority claims, inventorship, claim scope, patent term adjustments, etc., although we are unaware of any such defects. If we or our current licensors or licensees, or any future licensors or licensees, fail to establish, maintain or protect such patents and other intellectual property rights, such rights may be reduced or eliminated. If our current licensors or licensees, or any future licensors or licensees, are not fully cooperative or disagree with us as to the prosecution, maintenance or enforcement of any patent rights, such patent rights could be compromised. If there are material defects in the form or preparation of our patents or patent applications, such patents or applications may be invalid and unenforceable in circumstances where it is not possible to remedy those material defects. Any of these outcomes could impair our ability to prevent competition from third parties, which may have an adverse impact on our business. 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 in the United States or other jurisdictions. In addition, we cannot guarantee that any patents will issue from any pending or future patent applications owned by or licensed to us. There is no assurance that all of the potentially relevant prior art relating to our patents and patent applications has been found. If such prior art exists, it can invalidate a patent or prevent a patent from issuing from a pending patent application. Even if patents do successfully issue and even if such patents cover our product candidates, third parties may initiate opposition, interference, re-examination, post- grant review, inter parties review, nullification or derivation action in court or before patent offices or similar proceedings challenging the validity, enforceability or scope of such patents, which may result in the patent claims being

narrowed or invalidated. Furthermore, even if our patents and patent applications are unchallenged, they may not adequately protect our intellectual property, provide exclusivity for our product candidates or prevent others from designing around our claims. Any of these outcomes could impair our ability to prevent competition from third parties. If the patent applications we hold or have in- licensed with respect to our programs or product candidates fail to issue, or are revoked, if the breadth or strength of our patent protection is threatened, or if our patent portfolio fails to provide meaningful exclusivity for our product candidates, it could dissuade companies from collaborating with us to develop product candidates and threaten our ability to commercialize future products. Any successful opposition to any patents owned by or licensed to us could deprive us of rights necessary for the successful commercialization of any product candidates that we may develop. 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. Since patent applications in the United States and most other countries are confidential for a period of time (up to 18 months) after filing, we cannot be certain that we or our licensors were the first to file any patent application related to a product candidate. Furthermore, if third parties have filed such patent applications before March 16, 2013, an interference proceeding in the United States can be initiated by such third parties to determine who was the first to invent any of the subject matter covered by the patent claims of our applications. If third parties have filed such applications on or after March 16, 2013, a derivation proceeding in the United States can be initiated by such third parties to determine whether our invention was derived from theirs. Even where we have a valid and enforceable patent, we may not be able to exclude others from practicing our invention where the other party can show that they used the invention in commerce before our filing date or the other party benefits from a compulsory license. In addition, patents have a limited lifespan. In the United States, and in most other jurisdictions with a patent system, the natural expiration of a patent is generally 20 years after its filing date. Various extensions may be available, but the life of a patent, and the protection it affords, is limited. Even if patents covering our product candidates are obtained, once the patent life has expired for a product, we may be open to competition from competitive medications, including biosimilar or generic medications. This risk is material in light of the length of the development process of our products and lifespan of our current patent portfolio. ~~70~~ In addition to the protection afforded by patents, we rely on trade secret protection and confidentiality agreements to protect proprietary know- how that is not patentable or that we elect not to patent, processes for which patents are difficult to enforce and any other elements of our product candidate discovery and **70** development processes that involve proprietary know- how, information or technology that is not covered by patents. However, trade secrets can be difficult to protect. What constitutes a trade secret and what protections are available for trade secrets varies from state to state in the United States and country by country worldwide. We seek to protect our proprietary technology and processes, in part, by entering into confidentiality agreements with our employees, consultants, scientific advisors and contractors. We also seek to preserve the integrity and confidentiality of our data and trade secrets by measures designed to maintain the physical security of our premises and physical and electronic security of our information technology systems. Security measures may be breached, and we may not have adequate remedies for any breach. In addition, our trade secrets may otherwise become known to or be independently discovered by competitors. Although we expect all of our employees and consultants 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, that our trade secrets and other confidential proprietary information will not be disclosed or that competitors will not otherwise gain access to our trade secrets or independently develop substantially equivalent information and techniques. Misappropriation or unauthorized disclosure of our trade secrets can be difficult to detect, could impair our competitive position and may have a material adverse effect on our business. Additionally, 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, as part of its Transparency Initiative, is currently 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 at all. Enforcing a claim that a party illegally disclosed or misappropriated a trade secret is difficult, expensive and time- consuming, and the outcome is unpredictable. Further, the laws of some foreign countries such as India and China do not protect proprietary rights to the same extent or in the same manner as the laws of the United States. As a result, we may encounter significant problems in protecting and defending our intellectual property both in the United States and abroad. If we are unable to prevent 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 markets. We rely on license relationships with a number of third parties for portions of our intellectual property, including platform technology patents relating to our ddRNAi technology. We have in- licensed certain intellectual property from third parties, including technology related to ddRNAi and antisense RNA. In some cases, our licenses to intellectual property are non-exclusive and the licensors may license the technology to our competitors in the same field, which may result is significant competition for us. In other cases, our licenses to intellectual property are exclusive only for a specific field of use (such as human therapeutics), and the licensors retain rights to practice the licensed intellectual property themselves and to grant licenses to third parties in other fields. Such third parties may develop improvements to the licensed intellectual property that are not licensed to us, which could block our ability to continue developing the product candidate covered by the licensed intellectual property, unless we negotiate a license. Such third parties may also disclose competitively sensitive information about the licensed intellectual property that diminishes its value. In other cases, our licenses are for research purposes only. Upon regulatory marketing approval of our product candidates it may be necessary for us to obtain a broader license in order to commercialize. We cannot guarantee the availability of the broader license or that it can be obtained on commercially reasonable terms. ~~71~~ We rely on some of these third party licensors to file and prosecute patent applications and maintain patents

and otherwise protect the intellectual property we license. We have not had and do not have primary control over these activities for certain of our patents or patent applications and other intellectual property rights we license, and therefore cannot guarantee that these patents and applications will be prosecuted or enforced in a manner consistent with the best interests of our business. We cannot be certain that such activities by third parties have 71 been or will be conducted in compliance with applicable laws and regulations or will result in valid and enforceable patents and other intellectual property rights. Additionally, we may not be able to control the publication or other disclosures of research carried out by our licensors relating to technology that could otherwise prove patentable. Pursuant to the terms of some of our license agreements with third parties, some of our third- party licensors have the right, but not the obligation, to control enforcement of our licensed patents or defense of any claims asserting the invalidity of these patents. Even if we are permitted to pursue such enforcement or defense, we will require the cooperation of our licensors, and cannot guarantee that we would receive it and on what terms. We cannot be certain that our licensors will allocate sufficient resources or prioritize their or our prosecution or enforcement of such patents or defense of such claims to protect our interests in the licensed patents. If we cannot obtain patent protection, or enforce existing or future patents against third parties, our competitive position and our financial condition could suffer. If we fail to comply with our obligations in the agreements under which we license intellectual property rights from third parties or otherwise experience disruptions to our business relationships with our licensors, we could lose license rights that are important to our business. We hold rights under license or sublicense agreements with third parties that are important to our business. Under our existing license and sublicense agreements, we are subject to various obligations, including diligence obligations with respect to development and commercialization activities and payment obligations. In spite of our efforts, our licensors may conclude that we have materially breached our obligations under such license agreements and terminate the license agreements, thereby removing or limiting our ability to develop and commercialize product candidates and technology covered by these license agreements. We may need to obtain additional licenses from third parties to advance our research or allow commercialization of our product candidates, such as if we identify new technology that would advance our programs or if an existing license agreement is terminated. We may fail to obtain any of these licenses at a reasonable cost or on reasonable terms, if at all. In that event, we may be required to expend significant time and resources to develop or license replacement technology. If we are unable to do so, we may be unable to develop or commercialize the affected product candidates. In many cases, patent prosecution of our in- licensed technology is controlled solely by the licensor. If our licensors fail to obtain and maintain patent or other protection for the proprietary intellectual property we license from them, we could lose our rights to the intellectual property or our exclusivity with respect to those rights, and our competitors could market competing products using the intellectual property. In some cases, we control the prosecution of patents resulting from licensed technology. In the event we breach any of our obligations related to such prosecution, we may incur significant liability to our licensing partners and the value of the licensed patents may be adversely affected. Licensing of intellectual property is of critical importance to our business and involves complex legal, business and scientific issues and is complicated by the rapid pace of scientific discovery in our industry. Disputes may arise regarding intellectual property subject to a licensing agreement, including: • the scope of rights granted under the license agreement and other interpretation- related issues; • the extent to which our technology and processes infringe on intellectual property of a licensor that is not subject to the licensing agreement; • the sublicensing of patent and other rights under any collaborative relationships we might enter into in the future; ~~72~~ • our diligence obligations under the license agreement and what activities satisfy those diligence obligations; • the ownership of inventions and know- how resulting from the joint creation or use of intellectual property by our licensors and us and our partners; and 72 • the priority of invention of patented technology. If disputes over intellectual property that we have licensed prevent or impair our ability to maintain our current licensing arrangements on acceptable terms, we may be unable to successfully develop and commercialize the affected product candidates. Our results of operations will be affected by the level of royalty payments that we are required to pay to third parties. We are a party to license agreements that require us to remit royalty payments and other payments related to in- licensed intellectual property. Under our in- license agreements, we may pay up- front fees and milestone payments and be subject to future royalties. We cannot precisely predict the amount, if any, of royalties we will owe in the future, and if our calculations of royalty payments are incorrect, we may owe additional royalties, which could negatively affect our results of operations. As our product sales increase, we may, from time to time, disagree with our third- party collaborators as to the appropriate royalties owed, and the resolution of such disputes may be costly, may consume management' s time, and may damage our relationship with our collaborators. Furthermore, we may enter into additional license agreements in the future, which may also include royalty, milestone and other payments. The licenses we may grant to our collaborators and other licensees to use our ddRNAi and other technology may be exclusive to the development of product candidates for certain conditions. Some of the out- licenses we may grant to our collaborators to use our ddRNAi and other technology may be exclusive to the development of product candidates for certain conditions, so long as our collaborators comply with certain requirements. That means that once our ddRNAi technology is licensed to a collaborator for a specified condition, we are generally prohibited from developing product candidates for that condition and from licensing the ddRNAi to any third party for that condition. The limitations imposed by these exclusive licenses could prevent us from expanding our business and increasing our development of product candidates with new collaborators, both of which could adversely affect our business and results of operations. Third- party claims of intellectual property infringement may prevent or delay our development and commercialization efforts. Our commercial success depends in part on our avoiding infringement of the patents and proprietary rights of third parties. There is a substantial amount of litigation, both within and outside the United States, involving patent and other intellectual property rights in the biotechnology and pharmaceutical industries, including patent infringement lawsuits, interferences, oppositions and inter parties review proceedings before the USPTO, and corresponding foreign patent offices. Numerous U. S. and foreign issued patents and pending patent applications, which are owned by third parties, exist in the fields in which we are pursuing development candidates. As the biotechnology and pharmaceutical industries expand and more patents are issued, the risk

increases that our product candidates may be subject to claims of infringement of the patent rights of third parties. Third parties may assert that we are employing their proprietary technology without authorization. There may be third- party patents or patent applications with claims to materials, formulations, methods of manufacture or methods for treatment related to the use or manufacture of our product candidates. Because patent applications can take many years to issue, there may be currently pending patent applications which may later result in issued patents that our product candidates may be accused of infringing. In addition, third parties may obtain patents in ~~73~~ the future and claim that use of our technologies infringes upon these patents. If any third- party patents were held by a court of competent jurisdiction to cover the manufacturing process, methods of use or formulations of any of our product candidates, any DNA constructs formed during the manufacturing process or any final product itself, the holders of any such patents may be able to block our ability to commercialize such product candidate unless we obtained a license under the applicable patents, or until such patents expire. In either case, such a license may not be available on commercially reasonable terms or at all. **73** Parties making claims against us may obtain injunctive or other equitable relief, which could effectively block our ability to further develop and commercialize one or more of our product candidates. Defense of these claims, regardless of their merit, would involve substantial litigation expense and would be a substantial diversion of management and employee resources from our business. In the event of a successful claim of infringement against us, we may have to pay substantial damages, including treble damages and attorneys' fees for willful infringement, pay royalties, redesign our infringing products or obtain one or more licenses from third parties, which may be impossible or require substantial time and monetary expenditure. Even if a license can be obtained on acceptable terms, the rights may be non- exclusive, which could give our competitors access to the same technology or intellectual property rights licensed to us. If we fail to obtain a required license, we may be unable to effectively market product candidates based on our technology, which could limit our ability to generate revenue or achieve profitability and possibly prevent us from generating revenue sufficient to sustain our operations. We may not be successful in obtaining or maintaining necessary rights to gene therapy product components and processes for our development pipeline through acquisitions and in- licenses. Presently, we have certain rights to intellectual property to develop our current gene therapy product candidates. However, our product candidates may require specific formulations to work effectively and efficiently and rights to such formulations may be held by others. In addition, we may need additional intellectual property rights as we develop future product candidates. In particular, we are aware of a third party patent directed to AAV vectors that expires in 2026. In the event we receive regulatory marketing approval before the expiration date it may be necessary for us to obtain a license to the patent in order to commercialize. We cannot guarantee the availability of the license or that it can be obtained on commercially reasonable terms. 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 on terms that we find acceptable, or at all. The licensing and acquisition of third- party intellectual property rights is a competitive area, and a number of more established companies are also pursuing strategies to license or acquire third- party intellectual property rights that we may consider attractive. These established companies may have a competitive advantage over us due to their size, cash resources and greater clinical development and commercialization capabilities. For example, we sometimes collaborate with U. S. and foreign academic institutions to accelerate our preclinical research or development under written agreements with these institutions. Typically, these institutions provide us with an option to negotiate a license to any of the institution' s rights in technology resulting from the collaboration. Regardless of such right of first negotiation for intellectual property, we may be unable to negotiate a license within the specified time frame or under terms that are acceptable to us. If we are unable to do so, the institution may offer the intellectual property rights to other parties, potentially blocking our ability to pursue our program. In addition, companies that perceive us to be a competitor may be unwilling to assign or license rights to us. We also may be unable to license or acquire third- party intellectual property rights on terms that would allow us to make an appropriate return on our investment. If we are unable to successfully obtain rights to required third- party intellectual property rights, our business, financial condition and prospects for growth could suffer. ~~74~~ We may be involved in lawsuits to protect or enforce our patents or the patents of our licensors, which could be expensive, time-consuming and unsuccessful, and issued patents covering our product candidates could be found invalid or unenforceable if challenged in court. Competitors may infringe our patents or the patents of our licensors. To counter infringement or unauthorized use, we may be required to file infringement claims, which can be expensive and time- consuming. In addition, in an infringement proceeding, a court may decide that a patent of ours or our licensors is not valid, is unenforceable or is not infringed, or may refuse to stop the other party from using the technology at issue on the **74** grounds that our patents do not cover the technology in question. 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 and could put our patent applications at risk of not issuing. For example, in patent litigation in the United States, defendant counterclaims alleging invalidity or unenforceability are commonplace. Grounds for a validity challenge could be an alleged failure to meet any of several statutory requirements, for example, lack of novelty, obviousness or non- enablement. Grounds for an unenforceability assertion could be an allegation that someone connected with prosecution of the patent withheld relevant information from the USPTO, or made a misleading statement, during prosecution. The outcome following legal assertions of invalidity and unenforceability during patent litigation is unpredictable. With respect to the validity question, for example, we cannot be certain that there is no invalidating prior art, of which we and the patent examiner were unaware during prosecution. 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, and equivalent proceedings in foreign jurisdictions, such as opposition proceedings. Such proceedings could result in revocation, amendments to our patent claims or statements being made on the record such that our claims may no longer be construed to cover our product candidates. Outcomes or statements on the record in one country could have a disadvantageous effect on prosecution or enforcement of a patent or patent application in another country. The outcome following legal assertions of invalidity and unenforceability is unpredictable. With respect to the validity question, for example, we cannot be certain that no

invalidating prior art exists or that the patent examiner was aware of all material prior art during prosecution. Moreover, in some circumstances, we do not have the right to control the preparation, filing and prosecution of patent applications, or to maintain, enforce or defend the patents, covering technology that we license from third parties. Therefore, these patents and applications may not be prosecuted, enforced and defended in a manner consistent with the best interests of our business. If a defendant were to prevail on a legal assertion of invalidity or unenforceability, we could lose at least part, and perhaps all, of the patent protection on one or more of our products or certain aspects of our platform technology. Such a loss of patent protection could have a material adverse impact on our business. Patents and other intellectual property rights also will not protect our technology if competitors design around our protected technology without legally infringing our patents or other intellectual property rights. Even if resolved in our favor, litigation or other legal proceedings relating to intellectual property claims may cause us to incur significant expenses and could distract our technical and management personnel from their normal responsibilities. In addition, enforcement of a favorable decision by a court can depend on cooperation of a governmental authority which may or may not be available in every jurisdiction. Such litigation or proceedings could substantially increase our operating losses and reduce the resources available for development activities or any future sales, marketing or distribution activities. For our patents and patent applications filed in the United States before March 16, 2013, interference proceedings provoked by third parties or brought by us may be necessary to determine the priority of inventions with respect to our patents or patent applications or those of our licensors. An unfavorable outcome could require us to cease using the related technology or to attempt to license rights to it from the prevailing party. Our business could be harmed if the prevailing party does not offer us a license on commercially reasonable terms. Litigation or interference proceedings may result in a decision adverse to our interests and, even if successful, may result in substantial costs and distract our management and other employees. We may not be able to prevent, alone or with our licensors, misappropriation of our intellectual property rights, particularly in countries where the laws may not protect those rights as fully as in the United States. ~~75~~ 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 cause the trading price of our common stock to fall. **75** We may be subject to claims that our employees, consultants or independent contractors have wrongfully used or disclosed confidential information of third parties or that our employees have wrongfully used or disclosed alleged trade secrets of their former employers. Certain of our key employees and personnel are or were previously employed at universities, medical institutions or other biotechnology or pharmaceutical companies, including our competitors or potential competitors. Although we try to ensure that our employees, consultants and independent contractors 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 intellectual property, including trade secrets or other proprietary information, of any of our employee' s former employer or other third parties. Litigation may be necessary to defend against these claims. Furthermore, universities or medical institutions who employ some of our key employees and personnel in parallel to their engagement by us may claim that intellectual property developed by such person is owned by the respective academic or medical institution under the respective institution intellectual property policy or applicable law. If we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights or personnel. Even if we are successful in defending against such claims, litigation could result in substantial costs, be a distraction to management and other employees, and damage our relationships with the academic and medical institutions. We may be subject to claims challenging the inventorship or ownership of our patents and other intellectual property. We may be subject to claims that former employees, collaborators or other third parties have an ownership interest in our patents or other intellectual property. We may in the future have ownership disputes arising, for example, from conflicting obligations of consultants or others who are involved in developing our product candidates. 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. Obtaining and maintaining our patent protection depends on compliance with various procedural, document submission, fee payment and other requirements imposed by governmental patent agencies, and our patent protection could be reduced or eliminated for non- compliance with these requirements. Periodic maintenance fees, renewal fees, annuity fees and various other governmental fees on patents and applications are required to be paid to the USPTO and various governmental patent agencies outside of the United States in several stages over the lifetime of the patents and applications. The USPTO and various corresponding governmental patent agencies outside of the United States require compliance with a number of procedural, documentary, fee payment and other similar provisions during the patent application process and after a patent has issued. There are situations in which non- compliance can result in abandonment or lapse of the patent or patent application, resulting in partial or complete loss of patent rights in the relevant jurisdiction. ~~76~~ Changes in U. S. patent law 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 industry involve both technological and legal complexity, and it therefore is costly, time-consuming and inherently uncertain. In addition, on September 16, 2011, the Leahy- Smith America Invents Act, or the Leahy-Smith Act, was signed into law. The Leahy- Smith Act includes a number of significant changes to U. S. patent law, including provisions that affect the way patent applications will be prosecuted and may also affect patent litigation. **76** An important change introduced by the Leahy- Smith Act is that, as of March 16, 2013, the United States transitioned 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. 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 had 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. It is not clear what, if any, impact the Leahy- Smith Act will have on the operation of our business. However, the Leahy- Smith Act and its implementation could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents. Among some of the other changes introduced by the Leahy- Smith Act 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 court 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. Recent U. S. Supreme Court rulings such as Association for Molecular Pathology v. Myriad Genetics, Inc. (Myriad I); BRCA1- & BRCA2- Based Hereditary Cancer Test Patent Litig. (Myriad II); and Promega Corp. v. Life Technologies Corp. have also narrowed the scope of patent protection available in certain circumstances and weakened the rights of patent owners in some situations. 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 the U. S. Congress, the federal courts, and the USPTO, the laws and regulations governing patents could change in unpredictable ways that would weaken our ability to obtain new patents or to enforce our existing patents and patents that we might obtain in the future. Our success depends, in part, on our ability to protect our intellectual property and our technologies outside the United States. Our commercial success depends, in part, on our ability to obtain and maintain patent and trade secret protection for our technologies, our traits, and their uses, as well as our ability to operate without infringing upon the proprietary rights of others outside the United States. If we do not adequately protect our intellectual property, competitors may be able to use our technologies and erode or negate any competitive advantage we may have, which could harm our business and ability to achieve profitability. Filing, prosecuting and defending patents on product candidates in all countries around the world would be prohibitively expensive, and our intellectual property rights in some countries outside the United States can be less extensive than those in the United States. In addition, the laws of some foreign countries do not protect intellectual property rights to the same extent as federal and state laws in the United States. In addition, we may ~~77~~ at times in- license third- party technologies for which limited international patent protection exists and for which the time period for filing international patent applications has passed. Consequently, we may not be able to prevent third parties from practicing our inventions in all countries outside the United States, or from selling or importing products made using our inventions in and into the United States or other jurisdictions. Potential competitors may use our technologies in jurisdictions where we have not obtained patent protection to develop their own products and further, may export otherwise infringing products to territories where we have patent protection, but enforcement is not as strong as that in the United States. These products may compete with our product candidates, if approved, and our patents or other intellectual property rights may not be effective or sufficient to prevent them from competing. **77** Many companies have encountered significant problems in protecting and defending intellectual property rights in foreign jurisdictions. The legal systems of certain countries, particularly certain developing countries, do not favor the enforcement of patents, trade secrets and other intellectual property protection, particularly those relating to biotechnology products, which could make it difficult for us to stop the infringement of our patents or marketing of competing products in violation of our proprietary rights generally. Proceedings to enforce our patent rights in foreign jurisdictions could result in substantial costs and divert our efforts and attention from other aspects of our business, could put our patents at risk of being invalidated or interpreted narrowly and our patent applications at risk of not issuing and could provoke third parties to assert claims against us. We may not prevail in any lawsuits that we initiate and the damages or other remedies awarded, if any, may not be commercially meaningful. Accordingly, our efforts to enforce our intellectual property rights around the world may be inadequate to obtain a significant commercial advantage from the intellectual property that we develop or license. Risks Related to an Investment in Our Common Stock **A number of our stockholders hold significant amounts of our common stock and unexercised warrants to acquire common stock, and therefore could exert significant influence over us. While our stockholder base and relative holdings may change over time, a number of institutional investors and similar stockholders currently hold significant ownership positions in our outstanding common stock and outstanding Warrants to acquire Common Stock. In addition, in connection with the April 2024 private placement, we entered into the Board Designation Agreement with Suvretta Capital pursuant to which the Company appointed Kishen Mehta to the Board. The interests of these significant stockholders might not always coincide with the interests of other stockholders, and any influence exerted over our business and affairs by these significant stockholders directly or through an appointee to the Board might not always coincide with the wishes of other stockholders. In addition, the control and influence held by these significant stockholders might have the effect of delaying, deferring, or preventing a transaction or change in control of us, which might involve a premium price for shares of our Common Stock, or which otherwise could have been in your best interests as a stockholder** The market price and trading volume of our common stock may be volatile and may be affected by economic conditions beyond our control. The market price of our common stock may be highly volatile and subject to wide fluctuations. In addition, the trading volume of our common stock may fluctuate and cause significant price variations to occur. If the market price of our common stock declines significantly, you may be unable to resell your shares of our common stock at or above your purchase price, if at all. We cannot assure you that the market price of our common stock will not fluctuate or significantly decline in the future. Some specific factors that could negatively affect the price of our common stock or result in fluctuations in its price and trading volume

include: • results of our clinical trials; • regulatory actions; • actual or expected fluctuations in our operating results; • changes in market valuations of similar companies; • changes in our key personnel; • changes in financial estimates or recommendations by securities analysts; **78** • strategic decisions by us or our competitors, such as acquisitions, collaborations, divestitures, spin- offs, joint ventures, strategic investments or changes in business strategy; • the passage of legislation or other regulatory developments in the United States and other countries affecting us or our industry; • changes in trading volume of our common stock on Nasdaq; ~~78~~ • sales of our common stock by us, our executive officers or our ~~shareholders~~ **stockholders** in the future; and • conditions in the financial markets or changes in general economic conditions. In addition, the stock market has experienced and is currently experiencing significant volatility, particularly with respect to pharmaceutical, biotechnology and other life sciences company stocks. The volatility of pharmaceutical, biotechnology and other life sciences company stocks often does not relate to the operating performance of the companies represented by the stock. An active trading market for our common stock may not continue to develop or may not be liquid enough for you to sell your shares of our common stock quickly or at market price. Although our common stock is listed on Nasdaq, if an active public market in the United States for our common stock does not continue to develop, the market price and liquidity of our common stock may be adversely affected. The price of our common stock may decline, which means you may experience a decrease in the value of your shares of our common stock regardless of our operating performance or prospects. In the past, following periods of volatility in the market price of a company' s securities, ~~shareholders~~ **stockholders** often instituted securities class action litigation against that company. If we were involved in a class action suit, it could divert the attention of senior management and, if adversely determined, could cause us significant financial harm. If securities or industry analysts do not continue to publish research or reports, or if they publish adverse or misleading research or reports, regarding us, our business or our market, our stock price and trading volume could decline. The trading market for our common stock will be influenced by the research and reports that securities or industry analysts publish about us, our business or our market. If no additional securities or industry analysts commence coverage of us, our stock price could be negatively impacted. If any of the analysts who cover us issue adverse or misleading research or reports regarding us, our business model, our intellectual property, our stock performance or our market, or if our operating results fail to meet the expectations of analysts, our stock price would likely decline. If one or more of these analysts cease coverage of us or fail to publish reports on us regularly, we could lose visibility in the financial markets, which in turn could cause our stock price or trading volume to decline. If we fail to establish and maintain proper internal financial reporting controls, our ability to produce accurate financial statements or comply with applicable regulations could be impaired. Section 404 (a) of the Sarbanes- Oxley Act requires that our management assess and report annually on the effectiveness of our internal controls over financial reporting and identify any material weaknesses in our internal controls over financial reporting. Although Section 404 (b) of the Sarbanes- Oxley Act requires our independent registered public accounting firm to issue an annual report that addresses the effectiveness of our internal controls over financial reporting, we rely on the exemption provided for non- accelerated filers, and consequently will not be required to comply with SEC rules that implement Section 404 (b) of the Sarbanes- Oxley Act until such time as we are an accelerated or large accelerated filer. The presence of any material weaknesses in our internal control over financial reporting could result in financial statement errors which, in turn, could lead to errors in our financial reports and / or delays in our financial reporting, which could require us to restate our operating results. We might not identify one or more material **79** weaknesses in our internal controls in connection with evaluating our compliance with Section 404 (a) of the Sarbanes- Oxley Act. In order to maintain and improve the effectiveness of our disclosure controls and procedures and internal controls over financial reporting, we will need to expend significant resources and provide significant management oversight. Implementing any appropriate changes to our internal controls may require specific compliance training of our directors and employees, entail substantial costs in order to modify ~~79~~ our existing accounting systems, take a significant period of time to complete and divert management' s attention from other business concerns. These changes may not, however, be effective in maintaining the adequacy of our internal control. ~~If we are unable to~~ **The report of our management for the fiscal year ended June 30, 2024 is** ~~conclude~~ **included in Item 9A – Controls and Procedures of this Annual Report on Form 10- K. As further outlined in** that ~~we have effective~~ **report, our management has concluded that our** internal ~~controls~~ - **control** over financial reporting **was not effective as of June 30, 2024, due to a material weakness in our internal controls resulting from our accounting personnel not being able to process and account for complex, non- routine transactions in accordance with GAAP. Such finding related to how the exercise price adjustment feature in the Company' s Series 2 warrant agreement was triggered by the Company' s August 11, 2023 public offering. Specifically, the exercise price of the Series 2 Warrants was reset as of that date from $ 11. 22 to $ 1. 9299. The adjustment to the exercise price of such warrants results, solely for accounting purposes, in a deemed dividend totaling $ 618, 987. This amount was not recognized in the Company' s reported results for the quarter ended September 30, 2023, the three- and six- month periods ending December 31, 2023, and the three- and nine- month periods ending March 31, 2024. The impact of this adjustment to the exercise price will be accounted for as an increase in the Company' s accumulated deficit with an offsetting increase in Additional Paid- In Capital. In addition, the deemed dividend will be added to the Company' s net loss, increasing loss attributable to common stockholders for purposes of computing earnings per share (See Note 15 to the Notes to Consolidated Financial Statements titled " Updated quarterly results "). Management evaluated the adjustments and concluded that it was immaterial, both qualitatively and quantitatively. Management concluded that we lack sufficient personnel and outside consultants with technical accounting expertise to process and account for complex and non- routine transactions. A material weakness is a deficiency, or combination of deficiencies, in internal control over financial reporting, such that there is a reasonable possibility that a material misstatement of our annual or interim financial statements will not be prevented or detected on a timely basis. Notwithstanding the material weakness identified above, management has concluded that the particular transaction at issue was not material to the Company In order to remediate this matter, we plan to retain the assistance of additional accounting experts to**

assist in the accounting and reporting of complex, non- routine transactions. Such remedial measures will take time to implement and test and there can be no assurance that such measures will be sufficient to remedy the material weakness identified or that additional material weaknesses or other control or significant deficiencies will not be identified in the future. If we continue to experience material weaknesses in our internal controls or fail to maintain or implement required new or improved controls , investors may lose confidence in our operating results, the price of our common stock could decline and we may be subject to litigation or regulatory enforcement actions. In addition, if we are unable to meet the requirements of Section 404 of the Sarbanes- Oxley Act, our common stock may not be able to remain listed on Nasdaq. We have never declared or paid dividends on our common stock and we do not anticipate paying dividends in the foreseeable future. We have never declared or paid cash dividends on our common stock. For the foreseeable future, we currently intend to retain all available funds and any future earnings to support our operations and to finance the growth and development of our business. Any future determination to declare cash dividends will be made at the discretion of our Board, subject to compliance with applicable laws and covenants under current or future credit facilities, which may restrict or limit our ability to pay dividends, and will depend on our financial condition, operating results, capital requirements, general business conditions and other factors that our Board may deem **80** relevant. We do not anticipate paying any cash dividends on our common stock in the foreseeable future. As a result, a return on your investment in our securities will only occur if the price of our common stock appreciates. Future sales and issuances of our common stock or rights to purchase common stock could result in substantial dilution to the percentage ownership of our stockholders. We expect that significant additional capital will be needed in the future to continue our planned operations. To raise capital, we may sell common stock or other securities convertible into or exchanged for our common stock in one or more transactions, and in a manner we determine from time to time and at prices that may not be the same as the price per share paid by other investors, and dilution to our stockholders could result. The price per share at which we sell additional shares of our common stock, or securities convertible or exchangeable into common stock, in future transactions may be higher or lower than the price per share paid by other investors. New investors could also receive rights, preferences and privileges senior to those of existing holders of our common stock. In addition, in the event of stock dividends, stock splits, reorganizations or similar events affecting our common stock, we may be required to proportionally adjust the conversion price, exercise price or number of shares issuable upon exercise of any outstanding warrants. Our corporate governance structure may prevent our acquisition by another company at a premium over the public trading price of our shares. It is possible that the acquisition of a majority of our outstanding voting stock by another company could result in our stockholders receiving a premium over the public trading price for our shares. Provisions of our restated certificate of incorporation and our amended and restated bylaws, each as amended, and of Delaware corporate law could delay or make more difficult an acquisition of our company by merger, tender offer or proxy contest, even if it would create an immediate benefit to our stockholders. Furthermore, our certificate of incorporation also provides for a classified board of directors with directors divided into three classes serving staggered terms. These provisions may have the effect of delaying or preventing a change in control of us without action by our stockholders and, therefore, could adversely affect the price of our stock or the possibility of sale of shares to an acquiring person. We have a limited number of unreserved, authorized shares. If we seek equity financing we may need to use a significant percentage of our unreserved authorized shares of common stock in such an offering, and would therefore need stockholder approval to implement an increase in ~~80~~ our authorized shares of common stock or a reverse stock split in order to issue additional shares of common stock in the future. Our certificate of incorporation and the Delaware General Corporation Law, or the DGCL, currently require the approval of stockholders holding not less than a majority of all outstanding shares of capital stock entitled to vote in order to approve an increase in our authorized shares of common stock or a reverse stock split. There are no assurances that stockholder approval will be obtained, in which event we will be unable to raise additional capital through the issuance of shares of common stock to fund our future operations. Although we are required to use our reasonable best efforts to have an effective registration statement covering the issuance of shares of common stock underlying certain of our outstanding warrants at the time that holders of our warrants exercise their warrants, we cannot guarantee that a registration statement will be effective, in which case holders of our warrants are entitled to a cashless exercise of their warrants. Pursuant to the terms of certain of our warrants, we are obligated to have an effective registration statement covering the resale of the shares of common stock underlying such warrants. If no registration is effective at the time a warrant holder seeks to exercise their warrants, we would be obligated to issue shares to such warrant holder in a " cashless exercise " in exchange for such holder' s warrants, in which case we would not receive the cash that we would otherwise receive in an exercise of warrants for cash. 81