Risk Factors Comparison 2025-03-24 to 2024-03-14 Form: 10-K

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You should consider carefully the following risks and uncertainties when reading this Annual Report on Form 10- K, as well as the other information contained herein, including our audited consolidated financial statements and the related notes and the section titled " Management' s Discussion and Analysis of Financial Condition and Results of Operation. " If any of the following risks occur, our business, financial condition and results of operations could be materially and adversely affected. Although we believe that we have identified and discussed below the key risk factors affecting our business, there may be additional risks and uncertainties that are not presently known or that are not currently believed to be significant that may adversely affect our performance or financial condition. In that event, the trading price of our common stock could decline. Risks Related to Our Financial Position and Need for Capital We have incurred significant losses since our inception and anticipate that we will incur continued losses for the foreseeable future. Since our inception we have devoted substantially all of our resources to the development of nomlabofusp. We have incurred significant losses in each year of operation since our inception in 2016. For the years ended December 31, **2024 and** 2023 ~~and 2022~~, we had net losses of $ **80. 6 million and $** 36. 9 million ~~and $ 35. 4 million~~, respectively, and, as of December 31, ~~2023~~ **2024**, we had an accumulated deficit of $ ~~188~~ **269** . ~~6~~ **2** million and we expect to continue to incur significant expenses and net operating losses (" NOLs") for the foreseeable future. We have devoted substantially all of our financial resources and efforts to research and development, including non- clinical studies, our clinical development program, the development of manufacturing processes as well as the manufacture of initial lots of clinical trial material. We expect to incur significant losses for the foreseeable future to further develop and commercialize our lead drug candidate, nomlabofusp. We expect that our expenses will increase substantially if and as we: • continue clinical development efforts for nomlabofusp; • seek regulatory and marketing approvals in the United States and in foreign jurisdictions for our product candidates that successfully complete clinical trials, if any; • establish sales, marketing, distribution and other commercial infrastructure to commercialize various products for which we may obtain marketing approval, if any; • contract for the manufacture of larger quantities of product candidates for clinical development and potentially commercialization; • maintain, expand and protect our intellectual property portfolio; • **expand our operational, financial and management systems and** hire and retain additional personnel, such as clinical, manufacturing, quality control, regulatory and finance personnel; and; • experience any delays or encounter issues with any of the above. We currently have no sales, marketing or medical affairs infrastructure and have no experience in the sales, marketing, or distribution of pharmaceutical products. Assuming nomlabofusp ultimately is approved for marketing in the US and elsewhere, we will need to establish commercial capabilities as well as customer service and support, logistics, and other related functions, or make arrangements with third parties to perform these services. Net losses and negative cash flows have had, and will continue to have, an adverse effect on our liquidity and potentially the ability for us to raise capital due to our unfavorable operating results. We have no commercial revenue and may never become profitable. To date, we have not generated any commercial revenue. Our ability to generate revenue and become profitable depends upon our ability to obtain regulatory approval for, and to successfully commercialize, nomlabofusp or other product candidates that we may develop, in- license or acquire in the future. This will require success in a range of challenging activities, including completing numerous clinical trials of nomlabofusp or any future product candidates, obtaining marketing approval for nomlabofusp and any future product candidates, manufacturing, marketing and selling those products for which we, or any future collaborators or partners, may obtain marketing approval, satisfying any post- marketing requirements and obtaining reimbursement for our products from private insurance and / or government payors. Even if we are able to successfully achieve the above, we do not know what the reimbursement status of nomlabofusp or any other future product candidates will be or when any of these products will generate revenue for us, if at all. We have not generated, and do not expect to generate, any product revenue for the foreseeable future, and expect to continue to incur significant operating losses for the foreseeable future due to the cost of research and development, non- clinical studies and clinical trials and the regulatory approval process for nomlabofusp and any future product candidates. Our ability to generate revenue from nomlabofusp or any future product candidates also depends on a number of additional factors, including our ability to: • successfully complete development activities, including the remaining non- clinical studies and planned clinical trials for our product candidates; • complete and submit BLAs to the FDA and MAAs to the EMA and obtain regulatory approval for indications for which there is a commercial market; • complete and submit applications to, and obtain regulatory approval from, other foreign regulatory authorities; • manufacture or have manufactured any approved products in commercial quantities and on commercially reasonable terms; • develop a commercial organization, or find suitable partners, to market, sell and distribute approved products in the markets in which we have retained commercialization rights; • achieve acceptance among patients, clinicians and advocacy groups for any products we develop; • obtain coverage and adequate reimbursement from third parties, including government payors; and • set a commercially viable price for any products for which we may receive approval. Because of the uncertainties and risks associated with these activities, we are unable to accurately predict the timing and amount of increased expenses, and if or when we might achieve or maintain profitability. We and any future collaborators may never succeed in these activities and, even if we do, or any future collaborators do, we may never generate revenues that are large enough for us to achieve profitability. Even if we are able to complete the processes described above, we anticipate incurring significant costs associated with commercializing nomlabofusp or any of our future product candidates. Even if we achieve profitability in the future, we may not be able to sustain profitability in subsequent periods. Our failure to become and remain profitable could decrease the value of our business and could impair our ability to raise capital, maintain our discovery and

clinical development efforts, expand our business or continue our operations and may require us to raise additional capital that may dilute the ownership interest of shareholders. A decline in the value of our business could also cause shareholders to lose all or part of their investment. We may need to raise additional funding to complete the development and commercialization of nomlabofusp. This funding may not be available on acceptable terms, or at all. Failure to obtain this necessary capital when needed would force us to delay, limit or terminate our product development efforts or other operations. As of December 31, ~~2023~~ **2024**, our existing cash, cash equivalents and marketable securities were $ ~~86~~ **183**. ~~8~~ **5** million, **which** ~~excluding restricted cash of $ 1. 3 million. In February 2024,~~ we **anticipate will** ~~completed an underwritten public offering of 19, 736, 842 shares of our common stock at an offering price of $ 8. 74 per share with net proceeds, after deducting underwriting commissions and offering costs, of approximately $ 161. 6 million. Together with our existing cash, cash equivalents and marketable securities, we have adequate resources to~~ fund our operations into **the second quarter of** 2026. We expect to continue to spend substantial and increasing amounts to conduct clinical trials of nomlabofusp and further research and development activities for nomlabofusp, and for any additional product candidates that we may develop, in- license or acquire in the future. In addition, raising funds in the current economic environment may present substantial challenges, **for example, any sustained disruption in the capital markets from adverse macroeconomic conditions, such as the disruption** and **uncertainty caused by inflationary pressures, rising interest rates, banking instability, monetary policy changes, changes in trade policies, including tariffs (including tariffs that have been or may in the future be imposed by the U. S. or other countries), economic slowdowns or recessions, could negatively impact our ability to raise capital and we cannot predict the extent or duration of such macroeconomic disruptions. Additionally** our expenses will increase as we expand, through development, in- license or acquisition, our pipeline of product candidates. If we obtain marketing approval for any of our product candidates, we will likely incur significant commercialization expenses related to product sales, marketing, manufacturing and distribution to the extent that such sales, marketing, manufacturing and distribution are not the responsibility of a future collaborator. Accordingly, we will need to obtain additional funding in connection with our continuing operations. Our current cash, cash equivalents and marketable securities may not be sufficient to fund all of the efforts that we plan to undertake or to fund the completion of the development and commercialization of nomlabofusp. Accordingly, we may be required to obtain further funding through public or private equity offerings, debt financings, collaborations and licensing arrangements or other sources. The incurrence of indebtedness would result in increased fixed payment obligations and we may be required to agree to certain restrictive covenants, such as limitations on our ability to incur additional debt, limitations on our ability to acquire, sell or license intellectual property rights and other operating restrictions that could adversely impact our ability to conduct our business. Any additional fundraising efforts may divert our management from their day- to- day activities, which may adversely affect our ability to develop and commercialize our product candidates. If additional capital is needed to complete the development and commercialization of nomlabofusp, there can be no assurance that we will be able to raise sufficient additional capital on acceptable terms or at all. If such additional financing is not available on satisfactory terms, or is not available in sufficient amounts, or we do not have sufficient authorized shares, we may be required to delay, limit, or eliminate the development of business opportunities and our ability to achieve our business objectives, our competitiveness, and our business, financial condition, and results of operations will be materially adversely affected. We could also be required to seek funds through arrangements with collaborative partners or otherwise at an earlier stage than otherwise would be desirable and we may be required to relinquish rights to some of our technologies or product candidates or otherwise agree to terms unfavorable to us, any of which may have a material adverse effect on our business, operating results, financial condition and prospects. In addition, geopolitical tension including, for example, the broader impact of the ongoing conflict between Russia and Ukraine and the current conflict in Israel and Gaza (including any escalation or expansion), and the impact of a future pandemic, epidemic or outbreak of an infectious disease ~~, such as COVID- 19,~~ or ~~recent~~ liquidity constraints, failures and instability in U. S. and international financial banking systems on the global financial markets may reduce our ability to access capital, which could negatively affect our liquidity. If additional capital is needed to complete the development and commercialization of nomlabofusp, and if we are unable to obtain funding when needed and / or on acceptable terms, we may be required to significantly curtail, delay or discontinue one or more of our research and development programs, the manufacture of clinical and commercial supplies, product portfolio expansion or pre commercialization efforts, which could adversely affect our business prospects, or we may be unable to continue operations. Raising additional capital may cause dilution to our existing stockholders, restrict our operations or require us to relinquish rights to our technologies, nomlabofusp or other product candidates that we may develop, in- license or acquire in the future. We may seek additional capital through a combination of private or public equity offerings, debt financings, collaborations and licensing arrangements or other sources. To the extent we raise additional capital through the sale of equity or convertible debt securities, existing ownership interests will be diluted and the terms of such financings may include liquidation or other preferences that adversely affect the rights of existing stockholders. Debt or equity financings may be coupled with an additional equity component, such as warrants to purchase shares, which could also result in dilution of our existing ~~stockholder~~ **stockholders '** ~~' s~~ ownership. If we raise additional funds through strategic partnerships and alliances and licensing arrangements with third parties, we may have to relinquish valuable rights, including future revenue streams, to nomlabofusp or other product candidates that we may develop, in- license or acquire in the future, or grant licenses on terms that are not favorable to us. Our ability to use our NOLs and certain other tax attributes may be limited. As of December 31, ~~2023~~ **2024** we had NOL carryforwards that expire for U. S. federal income tax purposes of $ ~~179~~ **$ 198**. ~~0~~ **2** million, a portion of which begin to expire in 2026. Our NOLs could expire unused and be unavailable to offset future income tax liabilities because of their limited duration or because of restrictions under U. S. tax law. NOLs generated in taxable years beginning before January 1, 2018 are permitted to be carried forward for 20 taxable years under applicable U. S. federal income tax law. Under current U. S. federal income tax law, NOLs arising in tax years beginning after December 31, 2020 may not be carried back. Moreover, NOLs generated in taxable years beginning after December 31,

2017 may be carried forward indefinitely. As of December 31, ~~2023~~ **2024**, the Company had federal net operating loss carryforwards that were generated after December 31, 2017 of $ ~~140~~ **159**. ~~3~~ **5** million that do not expire, however these carryforwards are limited to 80 % of the taxable income in any one tax period. In general, under Section 382 of the Internal Revenue Code (the" Code") if a corporation undergoes an " ownership change, " generally defined as a greater than 50 % change (by value) in its equity ownership over a three- year period, the corporation' s ability to use its pre- change NOLs and other pre- change tax attributes (such as capitalized research and development costs and research tax credits) to offset its post- change income may be limited. We believe that as a result of our merger with Zafgen, our ability to utilize NOLs acquired in the transaction and our other NOLs is expected to be severely limited by Section 382 of the Code. Additionally, our July 2021, September 2022 and February 2024 equity transactions could also limit our ability to utilize NOLs in the future. We may also experience ownership changes in the future as a result of subsequent shifts in our stock ownership. As a result, if we earn net taxable income, our ability to use our pre- change NOLs to offset U. S. federal taxable income may be subject to limitations, which could potentially result in increased future tax liability to us. In addition, at the state level, there may be periods during which the use of NOLs is suspended or otherwise limited, which could accelerate or permanently increase state taxes owed and would adversely affect our business, financial condition and results of operations. Changes in tax laws and regulations may have a material adverse effect on our business, financial condition and results of operations. New income, sales, use or other tax laws, statutes, rules, regulations or ordinances could be enacted at any time, which could affect the tax treatment of any of our future domestic and foreign earnings. Any new taxes could adversely affect our domestic and international business operations, and our business and financial performance. Further, existing tax laws, statutes, rules, regulations or ordinances could be interpreted, changed, modified or applied adversely to us. For example, under Section 174 of the code, in taxable years beginning after December 31, 2021, expenses that are incurred for research and development in the U. S. are capitalized and amortized, which may have an adverse effect on our cash flow. In addition, it is unclear how these U. S. federal income tax changes will affect state and local taxation. Generally, future changes in applicable U. S. tax laws and regulations, or their interpretation and application could have an adverse effect on our business, financial conditions and results of operations. Risks Related to Our Product Development and Regulatory Approvals ~~The FDA has placed a partial~~ **Our success is currently dependent upon the success of our lead product candidate, nomlabofusp. We cannot be certain that we will ultimately be successful with our** clinical **development** ~~hold on nomlabofusp and there is uncertainty as to when, or if, the FDA will lift the partial clinical hold on our~~ **or that we** ~~nomlabofusp program, or if it~~ will ever ~~allow further~~ **be able to obtain regulatory approval for nomlabofusp. We currently have no drug products for sale and our business is currently wholly dependent on our successful** clinical development **, regulatory approval and commercialization** of nomlabofusp ~~beyond our currently ongoing OLE study at the 25 mg dose level. To support extended dosing of patients with nomlabofusp, we conducted a 26- week NHP toxicology study. In May 2021, we notified the FDA of certain mortalities which occurred at the two highest dose levels in the then ongoing study. On May 25, 2021, the FDA placed a clinical hold on the nomlabofusp clinical program. In the clinical hold letter, the FDA stated that it needed to review a full study report from the then- ongoing NHP study and that we could not initiate additional interventional clinical trials until we submitted such report and received notification from the FDA that additional clinical trials could commence. At the time of the FDA clinical hold, we had no interventional clinical trials with patients enrolling or enrolled. In July 2021, we completed dosing in the 26- week NHP toxicology study. The study included four~~ **our lead product candidate** ~~dose groups in addition to vehicle. Data from the study were collected throughout the second half of 2021~~ and ~~included in the complete response to the clinical hold submitted to the FDA in January 2022. In February 2022, in response to the complete response to clinical hold, we submitted to the FDA, the FDA stated that it was maintaining the clinical hold and that additional data were needed to resolve the clinical hold. We subsequently submitted a request to the FDA for a Type C meeting, which was granted and held in July 2022. We submitted a complete response to clinical hold incorporating additional information requested by the FDA at the meeting as well as information on the proposed study in August 2022. In September 2022, following the Type C meeting and the submission of the complete response to clinical hold, the FDA allowed the 25 mg cohort of a Phase 2, four~~ **our only product candidate** ~~- week, placebo- controlled, dose exploration trial of nomlabofusp~~ in ~~FA patients discussed above to proceed. In connection with this decision, the FDA lifted its full clinical hold on the nomlabofusp~~ clinical development ~~program and imposed a partial clinical hold. In June 2023~~, **for which** we **have** ~~met with the FDA. Following that meeting, we submitted a complete~~ **completed** ~~response to~~ **two** ~~the FDA' s partial clinical hold that included unblinded safety, PK and frataxin data from the~~ Phase **1 studies** ~~2 trial' s completed 25 mg cohort. In July 2023, following the FDA' s review of our complete response to the partial clinical hold, the FDA cleared initiation of a second cohort (50 mg) in our four- week, placebo- controlled, Phase 2 dose exploration trial~~ and ~~the initiation of our OLE trial with daily dosing of 25 mg. In January 2024, we initiated the OLE trial evaluating daily subcutaneous injections of 25 mg of nomlabofusp self- administered or administered by~~ a ~~caregiver, with the first patient dosed in March 2024. Dose escalation in the OLE trial will be considered based on safety, tolerability, PK, and tissue FXN levels from the Phase 2 trial' s 50 mg cohort as well as available data from the 25 mg dose of nomlabofusp in the OLE trial, and is contingent on FDA review as part of the partial clinical hold. Initial data from the OLE trial is expected in the fourth quarter of 2024. In February 2024, we reported positive top- line data and successful completion of both the 25 mg cohort and the 50 mg cohort of our~~ four- week, placebo- controlled Phase 2 dose exploration study ~~of nomlabofusp in participants with FA. Nomlabofusp was generally well tolerated and demonstrated dose dependent increases in FXN levels in all evaluated tissues (skin and buccal cells) after daily dosing of 14 days followed by every other day dosing until day 28 in the 25 mg and 50 mg cohorts. Our business may be adversely affected if the FDA partial clinical hold does not enable the development program to proceed as planned, if the FDA places a full clinical hold on the development of nomlabofusp, or, if additional non- clinical or clinical studies are required. This may cause significant delays or expense in developing nomlabofusp. Our success is currently dependent upon the success of our lead product candidate, nomlabofusp~~. We **have an ongoing** ~~cannot be certain that data from both cohorts of our~~ Phase 2 ~~dose~~

exploration study augmented by data from the OLE trial will provide the FDA with adequate data to remove the partial clinical hold on nomlabofusp, allow the nomlabofusp development program to proceed as planned in part or in full, or that we will ultimately be successful with our clinical development or that we will be ever able to obtain regulatory approval for nomlabofusp. We currently have no drug products for sale and our business is currently wholly dependent on our successful clinical development, regulatory approval and commercialization of nomlabofusp, our lead product candidate and our only product candidate in clinical development, for which we have completed two Phase 1 studies and a four- week, placebo-controlled Phase 2 dose exploration study in patients with FA **and an ongoing PK run** . In January 2024, as permitted by the FDA, we initiated the OLE trial evaluating daily subcutaneous injections of 25 mg of nomlabofusp self - **in study in adolescent patients** administered or administered by a caregiver, with the first patient dosed in March 2024. Participants who completed treatment in the Phase 2 dose exploration trial, or who previously completed a prior clinical trial of nomlabofusp are potentially eligible for the OLE. Dose escalation in the OLE trial will be considered based on safety, PK, and tissue FXN levels from the 25 mg dose of nomlabofusp in the OLE trial, as well as other data from the Phase 2 trial' s 50 mg cohort, and is contingent on FDA - **FA** review as part of the partial clinical hold. We cannot provide any assurance that the data from our Phase 1 and Phase 2 trials augmented by data from the OLE trial will prove the FDA with adequate data to remove the partial clinical hold, allow us to continue our development of nomlabofusp, or that we will ultimately be successful in our clinical development . In addition to the regulatory and manufacturing hurdles faced by our product candidate, the administration of a protein such as nomlabofusp , may cause an immune response, resulting in the creation of antibodies directed against the protein. These anti- drug antibodies can have no effect or can neutralize the effectiveness of the protein or require that higher doses be used to obtain a therapeutic effect. Neutralizing antibodies may be detected at a later date or upon longer exposure periods and there can be no assurance that neutralizing antibodies will not be detected in the future. **Additionally, while nomlabofusp was selected by the FDA for participation in its Support for clinical Trials Advancing Rare disease Therapeutics (START) Pilot Program, it is unknown whether participation in the program will accelerate the development of nomlabofusp and whether the FDA will continue investing its resources in this program.** If our efforts to develop and commercialize nomlabofusp for the treatment of FA are unsuccessful, or we experience significant delays in doing so, our business could also be substantially harmed. The success of nomlabofusp will depend on several factors, including the following: • maintaining our IND with the FDA in order to continue to conduct clinical trials in the United States , including our ability to expand to higher doses or have the partial clinical hold lifted by the FDA on a timely basis, if at all ; • successfully submitting a BLA for accelerated approval; • successfully recruiting, enrolling and retaining patients in and completing any clinical trials, if allowed to continue, including trials in pediatric patients; • demonstrating long- term chronic daily dosing safety, tolerability and efficacy profiles that are satisfactory to the FDA, EMA and other comparable regulatory authorities for marketing approval; • successfully completing all necessary toxicology studies to support clinical development and regulatory approval for nomlabofusp; • receiving timely marketing approvals from applicable regulatory authorities; • managing the extent and cost of any required post- marketing approval commitments to applicable regulatory authorities; • establishing and maintaining arrangements with third- party manufacturers for nomlabofusp, including developing, validating and maintaining a commercially viable manufacturing process that is compliant with cGMPs; • **developing, validating and implementing a potency control strategy acceptable to the FDA, EMA and other comparable regulatory authorities; •** maintaining and growing an organization of scientists and business people who can develop our product candidates and technology; • obtaining, maintaining and protecting our patents, trade secrets and regulatory exclusivity in the United States and other countries; • successfully launching commercial sales following any marketing approval, including establishing a specialty sales organization, or successfully partnering with another organization, if applicable; • obtaining commercial acceptance of our product candidates, if approved, by patients, the medical community and third- party payors and obtaining and maintaining healthcare coverage and adequate reimbursement; • maintaining an acceptable safety profile following any marketing approval; and • competing with other therapies. Many of these factors are outside of our control, including the clinical development and regulatory approval processes, results of non- clinical and toxicology studies and clinical trials, potential threats to our intellectual property rights and the manufacturing, marketing and sales efforts, respectively. The process of obtaining regulatory approval is expensive and time consuming. The FDA and foreign regulatory authorities may never approve nomlabofusp for sale and marketing, and even if nomlabofusp is ultimately approved, regulatory approval may be delayed or limited in the United States or in other jurisdictions. Even if we are authorized to sell and market nomlabofusp in one or more markets, there is no assurance that we will be able to successfully market nomlabofusp or that nomlabofusp will achieve market acceptance sufficient to generate profits. If we are unable to successfully develop and commercialize nomlabofusp due to failure to obtain regulatory approval for nomlabofusp, to successfully market nomlabofusp, to generate profits from the sale of nomlabofusp, or due to other risk factors outlined in this report, it would have material adverse effects on our business, financial condition, and results of operations as nomlabofusp is currently our sole product candidate. Clinical development is a lengthy and expensive process with an uncertain outcome, and the results of non-clinical studies, toxicology studies or clinical trials may not be predictive of future non- clinical studies, toxicology studies or clinical trial results. Clinical development is expensive and can take many years to complete, and its outcome is inherently uncertain. We cannot guarantee that any non- clinical studies, toxicology studies or clinical trials will be conducted as planned or completed on schedule, if at all, and failure can occur at any time during the non- clinical study, toxicology study or clinical trial process. Despite promising non- clinical, toxicology or clinical results, any product candidate can unexpectedly fail at any stage of non- clinical, toxicology or clinical development. The historical failure rate for product candidates in our industry is high, especially for products in early stages of development. The results from non- clinical studies, toxicology studies or clinical trials of a product candidate may not predict the results of later non- clinical or clinical trials of the product candidate, or in clinical trials with different patient populations such as children and adolescents and interim results of a clinical trial are not necessarily indicative of final results. Product candidates in later stages of clinical trials may fail to show the desired safety and

efficacy characteristics despite having progressed through non- clinical studies and initial clinical trials. It is not uncommon to observe results in clinical trials that are unexpected based on non- clinical studies and early clinical trials, and many product candidates fail in clinical trials despite very promising early results. Favorable safety and efficacy outcomes in adult clinical trials may not be seen in pediatric clinical trials. Moreover, current and future non- clinical and clinical data may be susceptible to varying interpretations and analyses. A number of companies in the pharmaceutical and biotechnology industries have suffered significant setbacks in clinical development even after achieving promising results in earlier studies. Furthermore, we cannot provide assurance that we will be able to successfully progress any future non- clinical programs from candidate identification to Phase 1 clinical development. As is typical in candidate development, we have a program of toxicology studies in animals for nomlabofusp and cannot provide assurance that the findings from such studies or any ongoing or future clinical trials will not adversely affect the clinical development of nomlabofusp. For the foregoing reasons, we cannot be certain that our non- clinical studies and clinical trials will be successful. If non- clinical studies or clinical trials for nomlabofusp or any future product candidates or indications fail to demonstrate safety or efficacy to the satisfaction of the FDA or the equivalent regulatory authorities in other countries, the FDA or equivalent regulatory authority will not approve our product candidates in those and other indications, which could have a material adverse effect on our business, financial condition results of operations and prospects. We do not know whether any ongoing or future clinical trials for nomlabofusp will be completed on schedule, if at all, as the commencement and completion of clinical trials can be delayed, prevented or terminated for a number of reasons, including as a result of safety concerns, or ambiguous or negative interim results. In addition, a clinical trial may be suspended or terminated by us, the FDA, other regulatory authorities, IRBs or ethics committees, an independent data monitoring committee, or safety review committee, overseeing the clinical trial at issue or other regulatory authorities due to a number of factors, including, among others: • failure to conduct the clinical trial in accordance with regulatory requirements or our clinical protocols; • inspection of the clinical trial operations or trial sites by the FDA, the EMA, or other applicable regulatory authorities that reveals deficiencies or violations that require us to undertake corrective action, including the imposition of a partial clinical hold or a full clinical hold; • unforeseen safety issues, including any that could be identified in our prior or future toxicology studies, adverse events or lack of effectiveness; • changes in government regulations or administrative actions; • problems with clinical supply materials; • lack of adequate funding to continue the clinical trial; • challenges in recruiting and enrolling patients to participate in clinical trials, including the size and nature of the patient population, such as pediatric patients, the proximity of patients to clinical trial sites, eligibility criteria for the clinical trial, the nature of the clinical trial protocol, the availability of approved effective treatments for the relevant disease and competition from FDA- approved therapeutics for FA ~~,~~ **and** other clinical trial programs for similar indications ~~, and the resurgence of vaccine resistant, more deadly or more contagious variants of COVID- 19 and the efforts to mitigate those effects~~ ; • difficulties in retaining or recruiting clinical investigators in our ongoing or future clinical trials; • difficulties retaining patients who have enrolled in a clinical trial but may be prone to withdraw due to rigors of the clinical trial, perceived lack of efficacy, side effects, screening and monitoring measures, personal issues or loss of interest; • severe, serious or unexpected drug- related adverse events experienced by patients in clinical trials; • unanticipated negative effects of chronic long- term daily patient dosing; • the FDA, the EMA, or other applicable regulatory authorities may disagree with our clinical trial designs, our interpretation of data from clinical trials, or may change the requirements for approval even after it has reviewed and commented on the design for our clinical trials; • clinical sites and subjects may deviate from trial protocol or drop out of a trial; and • reports from non- clinical studies or clinical testing of other therapies that raise safety or efficacy concerns. Failures or delays in the completion of our clinical trials could result in increased costs and could delay, prevent or limit our ability to generate revenue and continue our business. For our lead product candidate nomlabofusp, we have completed two Phase 1 clinical trials in patients with FA and our four- week, placebo- controlled Phase 2 dose exploration study ~~of nomlabofusp~~ **. We have an ongoing Phase 2 OLE trial** in **patients with** FA **and an ongoing run- in study in adolescent** patients **with** ~~. The FDA~~ **FA. Our** ~~has agreed that we may commence our OLE study in patients who previously participated in one of our previous nomlabofusp~~ clinical trials ~~at the 25 mg level. We cannot be certain that data from both cohorts of our Phase 2 dose exploration study augmented by data from the OLE study will provide the FDA with adequate data to remove the current partial clinical hold on nomlabofusp. Clinical trials~~ may ~~also~~ be delayed or terminated as a result of safety issues in non- clinical or clinical trials, ambiguous or negative interim results or events outside of our control. If future clinical trials of nomlabofusp fail or further delays occur in the United States and or other countries, we may not be able to develop and commercialize nomlabofusp and could fail to realize the potential advantages of doing so, and it could materially adversely affect our business, financial condition and results of operations. In addition, disruptions caused by future pandemic, epidemic or outbreak of an infectious disease, ~~such as the COVID- 19 pandemic~~ may increase the likelihood that we encounter such difficulties or delays in initiating, enrolling, conducting or completing our planned and ongoing clinical trials. Further, FA is a rare disease **and as a result** , there are a limited number of patients in close proximity to clinical trial sites and clinical trial patients may need to travel from other countries to the clinical trial sites in order to participate. In addition, given the limited number of FA patients, the ~~recent~~ approval of a competing therapy for the treatment of FA may make patients less likely to enroll in our clinical trials or less likely to be eligible for our clinical trials. Any inability to successfully initiate or complete clinical trials could result in additional costs to us or impair our ability to generate revenue from product sales. In addition, if we make manufacturing or formulation changes to our product candidates, we may be required to or we may elect to conduct additional studies to bridge our modified product candidates to earlier versions. Clinical trial delays could also shorten any periods during which our products have patent protection and may allow our competitors to bring products to market before we do, which could impair our ability to successfully commercialize our product candidates and may seriously harm our business. We may not be successful in our efforts to identify, discover or acquire additional product candidates. We currently only have one product candidate nomlabofusp in clinical development, although we have other product candidates in pre- clinical development. Therefore, the success of our business largely depends upon our

ability to identify, develop, in- license or acquire and commercialize products targeting rare diseases. We may focus our efforts and resources on potential programs or product candidates that ultimately prove to be unsuccessful. In addition, our research methodology may be unsuccessful in identifying potential product candidates or our potential 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. 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. If any of these events occur, we may be forced to abandon our development efforts for a program or programs, which would have a material adverse effect on our business, financial condition and results of operations. We have no marketed proprietary products and have not yet advanced a product candidate beyond Phase 2 clinical trials, which makes it difficult to assess our ability to develop nomlabofusp or any future product candidates and commercialize any resulting products independently. We have little experience in later stage clinical development, and related regulatory requirements or the commercialization of products. As a result, we have not yet demonstrated our ability to independently and repeatedly conduct clinical development after Phase 1, successfully conduct an international multi- center clinical trial, conduct a pivotal clinical trial, obtain regulatory approval, manufacture drug product on a commercial scale or arrange for a third party to do so on our behalf, and commercialize therapeutic products. We will need to develop such abilities if we are to execute on our business strategy to develop and independently commercialize product candidates for orphan and niche indications. To execute on our business plan for the development of independent programs, we will need to successfully: ~~• obtain the FDA' s permission to continue with the clinical development of nomlabofusp beyond our currently ongoing OLE study at the 25 mg dose level;~~ • execute our clinical development plans for product candidates; • obtain required regulatory approvals in each jurisdiction in which we will seek to commercialize products; • build and maintain appropriate sales, distribution and marketing capabilities; • gain market acceptance, including reimbursement, for our future products, if any; and • manage our spending as costs and expenses increase due to clinical trials, regulatory approvals and commercialization activities. If we are unsuccessful in accomplishing these objectives, we will not be able to develop and commercialize any product candidates independently and could fail to realize the potential advantages of doing so, and it would materially adversely affect our business, financial condition and results of operations. We cannot be certain that we will be able to successfully complete clinical trials for nomlabofusp or any other product candidates. We have advanced only one product candidate into clinical development, nomlabofusp. Our business currently depends primarily on nomlabofusp' s successful clinical development, regulatory approval and commercialization. We submitted our IND and it was accepted, permitting the conduct of clinical trials. We completed two Phase 1 and our four- week, placebo- controlled Phase 2 dose exploration study. ~~As a result of certain mortalities in a non-clinical study of NHPs, the FDA issued a clinical hold in May 2021. In September 2022, following a Type C meeting and the submission of our complete response to the partial clinical hold, the FDA allowed the 25 mg cohort of a Phase 2, four- week, placebo- controlled, dose exploration trial of nomlabofusp in FA patients to proceed. In connection with this decision, the FDA lifted its full clinical hold on the nomlabofusp clinical development program and imposed a partial hold. In July 2023, following the FDA' s review of our complete response to the partial clinical hold, the FDA cleared initiation of a second cohort at 50 mg of our four- week, placebo- controlled, Phase 2 dose exploration trial and to initiate our OLE trial with daily dosing of 25 mg. In February 2024, we reported positive top- line data and successful completion of our four- week, placebo- controlled Phase 2 dose exploration study of nomlabofusp in participants with FA. Nomlabofusp was generally well tolerated and demonstrated dose dependent increases in FXN levels in all evaluated tissues (skin and buccal cells) after daily dosing of 14 days followed by every other day dosing until day 28 in the 25 mg and 50 mg cohorts. Participants in the 25 mg (n = 13) and 50 mg (n = 15) cohorts were randomized 2: 1 to receive subcutaneous injections of nomlabofusp or placebo. The initiation of additional U. S. clinical trials evaluating nomlabofusp are contingent on FDA review of data under the partial clinical hold. The FDA has agreed that we may commence our OLE study in patients who previously participated in one of our previous nomlabofusp clinical trials at the 25 mg level dosed daily. We cannot be certain that data from both cohorts of our Phase 2 dose exploration study augmented by data from the OLE study will provide the FDA with adequate data to remove the current partial clinical hold on nomlabofusp, allow the nomlabofusp development program to proceed as planned in part or in full, or that we will ultimately be successful with our clinical development or that we will be ever able to obtain regulatory approval for.~~ In clinical development of any product candidate, the outcome of toxicology studies and early clinical trials may not be positive and may not be predictive of the success of later non- clinical studies or clinical trials. Adverse toxicology or safety results could lead to clinical holds or other developmental delays. Interim results of clinical trials do not necessarily predict success in those or future clinical trials. Success in adult clinical trials may not predict the outcome of pediatric clinical trials. Published clinical data or case reports from third parties or early clinical trial data of nomlabofusp or any future product candidates may not be predictive of the results of later- stage clinical trials. Interpretation of results from early, usually smaller, studies that suggest a clinically meaningful response in some patients, requires caution. Results from later stages of clinical trials enrolling more patients, or different patient populations, such as pediatric patients, may fail to show the desired safety or efficacy results or otherwise fail to be consistent with the results of earlier trials of the same product candidate. Later clinical trial results may not replicate earlier clinical trials for a variety of reasons, including differences in trial design, different trial endpoints (or lack of trial endpoints in exploratory studies), different patient population, number of patients, patient selection criteria, trial duration, drug dosage and formulation and lack of statistical power. These uncertainties are enhanced where the diseases under study lack established clinical endpoints, validated measures of efficacy, as is often the case with orphan diseases for which no drugs have been developed previously and where the product candidates target novel mechanisms. For example, to our knowledge, nomlabofusp is the only protein replacement therapy being developed for the treatment of FA and therefore non- clinical studies may not be adequate to predict efficacy in a clinical trial due to our novel protein replacement therapy platform. In some instances, there can be significant variability in safety or efficacy results between different clinical trials of the same product candidate due to

numerous factors, including changes in trial procedures set forth in protocols, differences in the size and type of the patient populations, such as pediatric patients, variability of the disease being studied, changes in and adherence to the dosing regimen and other clinical trial protocols and the rate of dropout among clinical trial participants. If we fail to ultimately receive positive results in clinical trials of nomlabofusp, the development timeline and regulatory approval and commercialization prospects for nomlabofusp, and, correspondingly, our business, financial prospects and results of operation would be negatively impacted. Further, nomlabofusp or any future product candidates may not be approved even if they achieve their primary endpoint in clinical trials. The FDA, EMA or foreign regulatory authorities may disagree with our trial design and our interpretation of data from non- clinical studies and clinical trials. In addition, FDA, EMA or foreign regulatory authorities may disagree with the extent of population exposure to assess clinical safety. Any of these regulatory authorities may change its requirements for the approval of a product candidate even after reviewing and providing comments or advice on a protocol for a pivotal clinical trial that, if successful, would potentially form the basis for an application for approval by the FDA, EMA or another regulatory authority. Furthermore, any of these regulatory authorities may also approve nomlabofusp or any future product candidates for a narrower indication than we may request or may grant approval contingent on the performance of costly post- marketing clinical trials. **The FDA and other comparable foreign authorities have substantial discretion in the approval process and determining when or whether regulatory approval will be granted for any product candidate that we develop and may decide that our data are insufficient for approval or require additional non- clinical, clinical, or other data. The U. S. Supreme Court' s July 2024 decision to overturn prior established case law giving deference to regulatory agencies' interpretations of ambiguous statutory language has introduced uncertainty regarding the extent to which FDA' s regulations, policies and decisions may become subject to increasing legal challenges, delays, and / or changes.** We may experience difficulties identifying and enrolling patients in our clinical trials given the limited number of patients who have the disease for which nomlabofusp is being studied or for any other product candidate we may study in the future. Difficulty in enrolling patients could delay or prevent clinical trials of nomlabofusp or any future product candidate. There are also competing FA therapeutics, other competing studies and potentially other FA therapeutics that could be approved that may also limit the availability of prospective participants in nomlabofusp clinical trials. Identifying and qualifying patients to participate in clinical trials of nomlabofusp is critical to our success. The timing of our clinical trials depends in part on the speed at which we can recruit patients to participate in testing nomlabofusp, and we may experience delays in our clinical trials if we encounter difficulties in enrollment, such as difficulties with enrollment in pediatric clinical trials. The conditions for which we are planning to evaluate nomlabofusp and any product candidates we may evaluate in the future, are rare genetic diseases. Accordingly, there are limited patient pools from which to draw for clinical trials. Arranging for investigative sites and recruiting patients for clinical trials in this disease may be very difficult. The recent FDA approval of a product for the treatment of FA may impact our ability to enroll patients in our clinical trials as patients using other FA treatments may be excluded from participation in our nomlabofusp studies or may be less likely to participate in our nomlabofusp clinical trials due to the availability of another FA therapy. If other companies are studying their investigational products in Friedreich' s ataxia and / or if other companies have their products approved for the treatment of FA, it may be more difficult to enroll eligible patients into our clinical trials. Competing priorities at sites and participation of subjects in other studies may limit our ability to execute clinical trials in a timely fashion, if at all. In addition to the rarity of FA and other diseases that we are studying, the eligibility criteria of our clinical trials will further limit the pool of available study participants as it will require patients to have specific characteristics that we can measure to assure their disease is either severe enough or not too advanced to include them in a clinical trial. The process of finding and diagnosing patients may prove costly, especially since the diseases we are studying are rare. We also may not be able to identify, recruit, and enroll a sufficient number of appropriate patients to complete our clinical trials because of demographic criteria for prospective patients, the perceived risks and benefits of the product candidate under study, the proximity and availability of clinical trial sites for prospective patients, and the patient referral practices of physicians. The availability and efficacy of approved and competing therapies and clinical trials can also adversely impact enrollment. Furthermore, our inability to enroll a sufficient number of patients for our clinical trials, including pediatric clinical trials, could result in significant delays or may require us to abandon one or more clinical trials altogether. Enrollment delays in our clinical trials may result in increased development costs for nomlabofusp or any future product candidates, and jeopardize our ability to achieve our clinical development timeline and goals, including the dates by which we will commence, complete and receive results from clinical trials. If patients are unwilling to participate in our trials for any reason, the timeline for recruiting patients, conducting trials, and obtaining regulatory approval of potential products may be delayed. Enrollment delays in our clinical trials may also jeopardize our ability to commence sales of and generate revenues from nomlabofusp, which could cause the value of our company to decline and limit our ability to obtain additional financing, if needed. Any of these occurrences may harm our business, financial condition, and prospects significantly. FA has no FDA- approved therapies that address frataxin deficiency, which is the underlying cause of the disease, and clinical endpoints required to obtain approval are not well defined. There are currently no FDA- approved products to treat FA that are designed to increase frataxin levels. We have concentrated our research and development efforts on developing a novel, FA therapy designed to address frataxin deficiency, which is the underlying cause of the disease, and our future success depends on the success of this therapeutic approach. The clinical trial requirements of the FDA and other comparable regulatory agencies and the criteria these regulators use to determine the safety and efficacy of any product candidate vary substantially according to the type, complexity, novelty and intended use and market of the potential product. **We have** ~~Recently, we~~ had discussions with the FDA regarding the use of tissue FXN levels as a novel surrogate endpoint. The FDA ~~has~~ acknowledged that frataxin deficiency appears to be critical to the pathogenic mechanism of FA, and that there continues to be an unmet need for treatments for FA patients that address the underlying disease pathophysiology. **In March 2025, we announced that FDA stated in written correspondence associated with a meeting through the START pilot program that they are open to considering the use of FXN concentration as a**

reasonably likely surrogate endpoint (" RLSE") and the acceptability of FXN' s use as an RLSE would ultimately be a matter of review of the data in a future marketing application. We intend to pursue an accelerated approval using FXN levels, supportive PD and clinical information, and safety data from the OLE study, along with ~~additional~~ non- clinical pharmacology information needed to support the novel surrogate endpoint approach. We are beginning to plan for a confirmatory study and are targeting a BLA submission ~~in~~ **by** the **end** ~~second half~~ of 2025. The FDA or other regulatory authorities may not agree with this approach. Regulatory authorities in the United States, the United Kingdom and the European Union have not issued definitive guidance as to how to measure and achieve efficacy in treatments for FA. As a result, the design and conduct of clinical trials of nomlabofusp may take longer, be more costly or be less effective as part of the novelty of development in FA. The FDA may not accept that the supportive PD and clinical information, and safety data from the OLE study, along with additional non- clinical pharmacology data we ultimately submit in our BLA, adequately supports the use of FXN levels as a novel surrogate endpoint. Even if the FDA supports the use of FXN levels as a novel surrogate endpoint, the FDA may not agree with the adequacy of the design of clinical trials intended to assess this novel surrogate endpoint. Even if applicable regulatory authorities do not object to our proposed endpoints in an earlier stage clinical trial, such regulatory authorities may require evaluation of additional or different clinical endpoints in later- stage clinical trials. Nomlabofusp, including the effects of long- term daily patient dosing, may cause adverse events or undesirable side effects in clinical trials that could delay or prevent its regulatory approval, limit the commercial profile of approved labeling, or result in significant negative consequences following regulatory approval, if any. Any adverse events or undesirable side effects caused by, or other unexpected properties of, nomlabofusp in non- clinical or clinical studies could cause us, any future collaborators, an IRB or ethics committee or regulatory authorities to interrupt, delay or halt clinical trials of our product candidate and could result in a more restrictive label or the delay or denial of regulatory approval by the FDA or other regulatory authorities. **It** ~~Should the FDA permit us to continue with the clinical development of nomlabofusp, it~~ is possible that as we progress nomlabofusp through clinical trials and toxicology studies, or as the use of nomlabofusp becomes more widespread if it receives regulatory approval, illnesses, injuries, discomforts and other adverse events that were not observed in earlier trials, as well as conditions that did not occur or went undetected in previous trials, may be reported by patients. **For example, recently our Safety Monitoring Team deemed anaphylaxis as an adverse drug reaction likely associated with nomlabofusp. To reduce the risk of allergic reactions, including anaphylaxis, we amended the OLE protocol to administer premedication for the first month of dosing.** If such side effects become known **or their incidence increases** later in development or after approval, such findings may harm our business, financial condition and prospects significantly. Further, if a serious safety issue is identified in connection with the use of nomlabofusp commercially or in third- party clinical trials elsewhere, such issues may adversely affect the development potential of nomlabofusp elsewhere or result in regulatory authorities restricting our ability to develop or commercialize nomlabofusp, if approved. Further, if nomlabofusp were to receive marketing approval and we or others identify undesirable side effects caused by the product (or any other product) after the approval, a number of potentially significant negative consequences could result, including: • regulatory authorities may request that we recall or withdraw the product from the market or may limit the approval of the product through labeling or other means; • regulatory authorities may require the addition of labeling statements, such as a " boxed " warning or a contraindication or a precaution, or labeling restrictions based on patient population; • we may be required to change the way the product is distributed or administered, conduct additional clinical trials or change the labeling of the product; • we may decide to recall or remove the product from the marketplace; • we could be sued or held liable for injury caused to individuals exposed to or taking our product candidates; • we could be required to conduct expensive post- marketing studies; • we could lose our commercial market opportunity and our revenues could decrease substantially; • damage to the public perception of the safety of nomlabofusp; and • our reputation may suffer and physicians or patients might be less likely to use our product or may refer patients to products produced by our competitors. Any of these events could prevent us from achieving or maintaining market acceptance of the affected product candidate and could substantially increase the costs of commercializing our product candidates and significantly impact our ability to successfully commercialize our product candidates and generate revenues, all of which would materially adversely affect our business, financial condition and results of operations. In addition, the patient populations under investigation with nomlabofusp have many co- morbidities that may cause severe illness or death unrelated to our product candidate, which may be attributed to nomlabofusp in a manner that negatively affects the safety profile of our product candidate. Interim, " top- line, " and preliminary data from our clinical trials that we announce or publish from time to time may change as more patient data become available or as additional analyses are conducted, and as the data are subject to audit and verification procedures that could result in material changes in the final data. From time to time, we may publish interim, preliminary or " top- line " data from our clinical trials. Interim data from clinical trials that we may complete are subject to the risk that one or more of the clinical outcomes may materially change as patient enrollment continues and more patient data become available. Preliminary or " top- line " data also remain subject to audit and verification procedures that may result in the final data being materially different from the preliminary data we previously published. As a result, interim and preliminary data should be viewed with caution until the final data are available. Material adverse changes between interim, preliminary or " top- line " data and final data could significantly harm our business, financial condition, results of operations and prospects. Our approach to discover and develop fusion proteins for delivering proteins is novel and may never lead to marketable products. We have concentrated our efforts and research and development activities on delivering proteins (FXN or other) to intracellular targets. Our future success depends on the successful development and manufacturing of such therapeutics and the effectiveness of our platform. The scientific discoveries that form the basis for our research are relatively new. Nomlabofusp uses a novel and unproven approach and mechanism to treat FA and therefore its efficacy and safety are difficult to predict, and there is no guarantee that nomlabofusp will be approved by the FDA, the EMA, or any other regulatory authorities. If nomlabofusp proves to be ineffective, unsafe or commercially unviable, it is possible that our platform and pipeline would have little, if any, value, which

would substantially harm our business, financial condition, results of operations and prospects. In addition, our approach may expose us to additional financial risks and make it more difficult to raise additional capital than other, more advanced proven technologies, which would materially adversely affect our business, financial condition and results of operations. Protein replacement therapies are novel, complex and difficult to manufacture. We could experience manufacturing problems that result in delays in the development or commercialization of our protein replacement therapy platform or product candidates or otherwise harm our business. The manufacture of fusion proteins, such as nomlabofusp and any fusion protein candidates, is technically complex and necessitates substantial expertise and capital investment. Production difficulties caused by unforeseen events may delay the availability of material for clinical trials and commercial products for nomlabofusp or any fusion protein product that may receive regulatory approval in the future. Additionally, because biologic products are complex, the manufacture of such products and product candidates is more difficult and costly. We may not be able to have such products reliably manufactured in accordance with the applicable regulatory requirements in sufficient quantities to support our development programs and, if ultimately approved, commercial supply. There are a limited number of contract manufacturers who specialize in the manufacture of biologic products and those that do may still be developing appropriate processes, controls and facilities for large- scale production. While we believe that there will be sufficient sources of supply that can satisfy our clinical and commercial requirements, we cannot be certain that we will be able to identify and establish additional relationships with such sources, if necessary, in a timely manner or at all, and what the terms and costs of such new arrangements would be, or that such suppliers would be able to supply our potential commercial needs. Furthermore, in the event our primary manufacturer cannot meet our needs, any switch to an alternative manufacturer, if available, would result in a significant delay, would require FDA approval, and cause material additional costs. As further described in these risk factors, the manufacturers of biologic products must comply with strictly enforced cGMP requirements, state and federal regulations, as well as foreign requirements when applicable. Any failure by us or our contract manufacturing organizations to adhere to or document compliance to such regulatory requirements could lead to a delay or interruption in the availability of our program materials for clinical trials or commercial use, among other consequences. If we or our manufacturers fail to comply with the FDA, EMA, or other regulatory authorities, it could result in sanctions being imposed on us, including fines, injunctions, civil penalties, delays, suspension or withdrawal of approvals, clinical holds or termination of clinical trials, Form 483s, warning or untitled letters, regulatory communications warning the public about safety issues with a product, import or export refusals, license revocation, seizures, detentions, or recalls of product candidates or product, operating restrictions, criminal prosecutions or debarment, suits under the civil False Claims Act, corporate integrity agreements, or consent decrees any of which could significantly and adversely affect supplies of our product candidates and our business, financial conditions and results of operations could be materially adversely affected. Our current dependence upon others for the manufacture of our product candidates may also adversely affect our business, results of operations, financial condition, and our ability to commercialize any product candidates that receive regulatory approval on a timely and competitive basis. Fast track designation by the FDA or any future expedited program designations may not lead to a faster development, regulatory review or approval process and it does not increase the likelihood that any of our product candidates will receive marketing approval. We have received fast track therapy designation for nomlabofusp for the treatment of FA. We may, in the future, apply for other expedited program designations from the FDA (such as breakthrough therapy) for nomlabofusp or future product candidates. Designation for these programs is within the discretion of the FDA. Accordingly, even if we believe nomlabofusp or a future product candidate meets the criteria for designation, the FDA may disagree. The receipt of a designation may not result in a faster development process, review or approval compared to products considered for approval without these expedited program designations and, in any event, does not assure ultimate approval by the FDA. In addition, even though nomlabofusp has obtained fast track designation, the FDA may later decide that it no longer meets the criteria for designation and revoke it. Approval of other therapies for the treatment Friedreich' s ataxia could negatively impact our continued fast track therapy designation for nomlabofusp for the treatment of FA. In addition, if we apply to the FDA for other designations for nomlabofusp or future product candidates, the FDA might not grant such designations. If we apply for any similar programs in foreign countries for nomlabofusp or future product candidates, those designations also might not be granted by the regulatory authorities of those countries. Any of the above could adversely affect our business, financial condition and results of operations. We intend to pursue accelerated approval from FDA for nomlabofusp for the treatment of Friedreich' s ataxia, however this may not lead to a faster development or regulatory review or approval process and does not increase the likelihood that we will receive marketing approval. If we are unable to obtain approval under an accelerated pathway, we may be required to conduct additional clinical trials beyond those that we contemplate, which could increase the expense of obtaining, reduce the likelihood of obtaining, and / or delay the timing of obtaining, necessary marketing approvals. We intend to pursue an accelerated approval from FDA for nomlabofusp for the treatment of FA using FXN ~~levels~~ **concentrations as a surrogate endpoint**, supportive pharmacodynamic and clinical information and safety data from the OLE study, along with ~~additional~~ non- clinical pharmacology information needed to support our surrogate biomarker approach. Under the FDA' s accelerated approval program, the FDA may approve a drug or biologic for a serious or life- threatening illness that provides meaningful therapeutic benefit to patients over existing treatments based upon a surrogate endpoint that is reasonably likely to predict clinical benefit, or on a clinical endpoint that can be measured earlier than irreversible morbidity or mortality, that is reasonably likely to predict an effect on irreversible morbidity or mortality or other clinical benefit, taking into account the severity, rarity, or prevalence of the condition and the availability or lack of alternative treatments. ~~Although we have initiated discussions~~ **The FDA stated in written correspondence associated** with ~~FDA~~ **a meeting through the START pilot program that they are open to** ~~concerning~~ **considering** ~~our surrogate biomarker approach,~~ **the use of FXN concentration as an RLSE and the acceptability of FXN' s use as an RLSE would ultimately be a matter of review of the data.** ~~there~~ **There** can be no assurance that data we intend to generate will be successful in establishing that increases in FXN ~~levels~~ **concentrations** are reasonably likely to predict clinical benefit **or that**

increases in FXN concentrations due to treatment with nomlabofusp meet the legal standards to support approval of a marketing application . In addition, ~~future~~ **non-** ~~nonclinical~~ --- **clinical** pharmacology and clinical studies may not provide adequate information or may fail to support the predictive value of increases in FXN and, therefore, its ability to serve as a surrogate endpoint **, or may fail to support that increases in FXN are the result of treatment with nomlabofusp** . Even if these evidentiary requirements are met, we may not be able to accrue adequate exposures to assess clinical safety or expedite the scale up of manufacturing or meet other CMC requirements in a timeframe commensurate with the expedited assessment of clinical efficacy. Approval of other therapies for the treatment of FA, including the approval of omaveloxolone for the treatment of FA, could negatively impact our ability to utilize the accelerated approval pathway, and / or get approval for nomlabofusp. For drugs or biologics granted accelerated approval, post- marketing confirmatory trials are required to describe the anticipated effect on irreversible morbidity or mortality or other clinical benefit. These confirmatory trials must be completed with due diligence and, in some cases, the FDA may require that the trial be designed, initiated and / or fully enrolled prior to approval. Moreover, the FDA may withdraw approval of any product candidate approved under the accelerated approval pathway if, for example: • the trial or trials required to verify the predicted clinical benefit of the product candidate fail to verify such benefit or do not demonstrate sufficient clinical benefit to justify the risks associated with such product; • other evidence demonstrates that the product candidate is not shown to be safe or effective under the conditions of use; • we fail to conduct any required post- approval trial of our product candidate with due diligence; or • we disseminate false or misleading promotional materials relating to the relevant product candidate. In addition, the FDA may terminate the accelerated approval program or change the standards under which accelerated approvals are considered and granted in response to public pressure or other concerns regarding the accelerated approval program. Changes to or termination of the accelerated approval program could prevent or limit our ability to obtain accelerated approval of any of our clinical development programs. Recently, the accelerated approval pathway has come under scrutiny within the FDA and by Congress. The FDA has put increased focus on ensuring that confirmatory studies are conducted with diligence and, ultimately, that such studies confirm the benefit. In addition, the Food and Drug Omnibus Reform Act (“ FDORA ”), included provisions related to the accelerated approval pathway. Pursuant to FDORA, the FDA is authorized to require, as appropriate, that a post- approval study to be underway prior to approval or within a specified time period following approval. FDORA also requires the FDA to specify conditions of any required post- approval study and requires sponsors to submit progress reports for required post- approval studies and any conditions required by the FDA. FDORA also gives the FDA increased authority to withdraw approval of a drug or biologic granted accelerated approval on an expedited basis if the sponsor fails to verify the drug or biologic’ s predicted clinical benefit. FDORA enables the FDA to initiate enforcement action for a sponsor' s failure to conduct with due diligence a required post- approval study, including a failure to meet any required conditions specified by the FDA or to submit timely reports. If we fail to maintain orphan drug designation or other regulatory exclusivity for nomlabofusp or obtain such exclusivity for any of our other product candidates in the future, our competitive position would be harmed. We received orphan drug designation from the FDA for nomlabofusp for the treatment of FA in July 2017. In the United States, orphan drug designation entitles a party to financial incentives such as tax advantages and user- fee waivers. In addition, if a product candidate that has orphan drug designation subsequently receives the first FDA approval for the disease for which it has such designation, the product is entitled to orphan drug exclusivity, which means that the FDA may not approve any other applications, including an NDA or BLA, to market the same drug for the same ~~indication~~ **disease or condition** for seven years, except in limited circumstances, including if the FDA concludes that the later drug is clinically superior to the approved drug. A drug is clinically superior if it is safer, more effective, or makes a major contribution to patient care. In the case of a biological product, whether a drug is the same drug is based on the principal molecular structural features of the product. Orphan drug designation neither shortens the development time or regulatory review time of a drug nor gives the drug any advantage in the regulatory review or approval process. Additionally, we may lose orphan drug exclusivity if we are unable to assure sufficient quantity of the drug to meet the needs of patients with the rare disease or condition. Moreover, orphan drug exclusivity may not effectively protect our product candidates from competition because different drugs can be approved for the same condition. Further, even after an orphan drug is approved, the FDA or comparable foreign regulatory authority can subsequently approve another drug for the same condition if such regulatory authority concludes that the later drug is clinically superior, ~~if~~ **which means that** it is shown to be safer, more effective or makes a major contribution to patient care. We have also received orphan ~~drug~~ designation for nomlabofusp in the European Union. In the European Union, the European **Commission may** ~~Medicines Agency, or EMA’ s Committee for Orphan Medicinal Products grants~~ **grant** orphan ~~drug~~ designation **in respect** ~~to promote the development~~ of products that are intended for the diagnosis, prevention or treatment of **a** life threatening or chronically debilitating ~~conditions~~ **condition** affecting not more than five in 10, 000 persons in the European Union. Additionally, designation is granted for products intended for the diagnosis, prevention or treatment of a life threatening, seriously debilitating or serious and chronic condition and when, without incentives, it is unlikely that sales of the drug in the European Union would be sufficient to justify the necessary investment in developing the drug. In **each case, orphan designation is only available if there is no other satisfactory method approved in the European Union of diagnosing, preventing, or treating the condition, or if such a method exists, the proposed orphan product will be of significant benefit to patients over the existing options. In** the European Union, orphan drug designation also entitles a party to financial incentives such as reduction of fees or fee waivers and ten years of market exclusivity following ~~drug approval~~ **the grant of marketing authorization** . This period may be reduced to six years if the orphan drug designation criteria are no longer met, including where it is shown that the product is sufficiently profitable so that market exclusivity is no longer justified. Loss of orphan drug designation for nomlabofusp or the failure to obtain such designation in other countries or for any future product candidates could adversely affect our business, financial condition and results of operation. If another product has received approval in the indications for which we have received orphan drug designation, we may still receive approval in that indication if we can demonstrate that our product candidate is clinically superior to the existing orphan product.

This demonstration of clinical superiority may be done at the time of initial approval or in post- approval studies, depending on the type of marketing authorization granted. Having to comply with these additional requirements to obtain orphan drug exclusivity could adversely affect our business, financial condition and results of operation. Although we have obtained rare pediatric disease designation for nomlabofusp, we may not be eligible to receive a priority review voucher in the event the FDA determines we no longer meet the criteria for designation, revokes the designation or FDA approval does not occur prior to September 30, 2026. The sponsor of an application for a rare pediatric disease drug product may be eligible for a voucher that can be used or sold to obtain a priority review for a subsequent application submitted under section 505 (b) (1) of the FDCA or section 351 of the PHS Act. The rare pediatric disease priority review voucher program **began to sunset on December 20, 2024, due to failure to pass a continuing resolution package that included its reauthorization. Under the amended statutory sunset provisions, after December 20, 2024, the FDA may award a priority review voucher for an approved rare pediatric disease product application only if the sponsor has rare pediatric disease designation for the drug and if that designation** was **granted** ~~most recently reauthorized~~ by ~~Congress through~~ **December 20, 2024. After** September 30, ~~2024~~ **2026** , ~~with~~ the ~~potential for~~ **FDA may not award any rare pediatric disease** priority review vouchers **. Congress may vote** to **reauthorize this program** ~~be granted through September 30~~ , ~~2026~~ **but its future remains unknown at this time** . We received rare pediatric disease designation from the FDA for nomlabofusp in 2019. We may, in the future, apply for rare pediatric disease designation from the FDA for future product candidates that may qualify for designation **if Congress reauthorizes the program** . Vouchers for rare pediatric disease drugs are awarded for qualifying applications when the drug receives approval. Although nomlabofusp has received rare pediatric disease designation, nomlabofusp may not receive a priority review voucher for a number of reasons: nomlabofusp may not receive approval; nomlabofusp may receive approval in adults, but not pediatric patients; nomlabofusp may not meet the eligibility requirements for a priority voucher at the time we seek approval for nomlabofusp; or we may not meet the current deadline for receiving a priority review voucher (September 30, 2026), in which case we would not be able to obtain a voucher unless Congress further reauthorizes the program. Finally, a rare pediatric disease designation does not necessarily lead to faster development or regulatory review of the product or increase the likelihood that it will receive marketing approval. If **the program is reauthorized and** we apply for designation for future product candidates as drugs for rare pediatric diseases, the FDA may not grant the designation. The failure to maintain rare pediatric disease designation for nomlabofusp or if FDA approval does not occur prior to September 30, 2026 could result in the inability to receive a priority review voucher which could adversely affect our business, financial condition and results of operations. If we fail to maintain PRIME designation in the European Union for nomlabofusp, our competitive position ~~would~~ **could** be harmed. The PRIME scheme is open to medicines under development and for which the applicant intends to apply for an ~~initial MAA~~ **MA** through the centralized procedure. Eligible products must target conditions for which where is an unmet medical need (there is no satisfactory method of diagnosis, prevention or treatment in the European Union or, if there is, the new medicine will bring a major therapeutic advantage **in comparison to existing therapies**) and they must demonstrate the potential to address the unmet medical need by introducing new methods or therapy or improving existing ones. Applicants will typically be at the exploratory clinical trial phase of development and will have preliminary clinical evidence in patients to demonstrate the promising activity of the medicine and its potential to address to a significant extent an unmet medical need. In exceptional cases, applicants from the academic sector or SMEs may submit an eligibility request at an earlier stage of development if compelling non- clinical data in a relevant model provide early evidence of promising activity to establish proof of principle, and first in man studies indicate adequate exposure for the desired pharmacotherapeutic effects and tolerability. If a medicine is selected for the PRIME scheme, the EMA: • appoints a rapporteur from the CHMP or from the CAT to provide continuous support and to build up knowledge of the medicine in advance of the filing of an MMA; • issues guidance on the applicant' s overall development plan and regulatory strategy; • organizes a kick- off meeting with the rapporteur and experts from relevant EMA committees and working groups; • provides a dedicated EMA contact person; and • provides scientific advice at key development milestones, involving additional stakeholders, such as health technology assessment bodies and patients, as needed. For SMEs who enter the scheme based on data showing proof of principle, the appointment of the rapporteur occurs once they have generated data confirming eligibility at proof of concept stage. They are required to submit relevant data and justification as the product development reaches this stage. Medicines that are selected for the PRIME scheme are also expected to benefit from EMA' s accelerated assessment procedure at the time of application for marketing authorization. Where, during the course of development, a medicine no longer meets the eligibility criteria, or if a medicine granted early access to the PRIME scheme cannot later demonstrate proof of concept, support under the PRIME scheme may be withdrawn. Approval of other therapies for the treatment Friedreich' s ataxia, including the ~~recent~~ approval of omaveloxolone could negatively impact our continued access to this and similar programs. Loss of PRIME designation for nomlabofusp or the failure to obtain such designation for any future product candidates could adversely affect our business, financial condition and results of operation. Changes in regulatory requirements, FDA guidance, guidance from other regulatory authorities or unanticipated events during our non- clinical or clinical trials of nomlabofusp or future product candidates may result in changes to clinical trial protocols or additional non- clinical or clinical trial requirements, which could result in increased costs to us and could delay our development timeline. Changes in regulatory requirements, FDA guidance or guidance from EMA or unanticipated events during our non- clinical or clinical trials may force us to terminate or adjust our clinical programs. The FDA, or other applicable regulatory authorities may impose additional clinical trial and / or non- clinical study requirements. Amendments to our clinical trial protocols would require resubmission to the FDA, or the applicable regulatory authorities as well as IRBs and ethics committees for review and approval, which may adversely impact the cost, timing or successful completion of a clinical trial. If we experience delays completing, or if we terminate, any of our clinical trials, or if we are required to conduct additional clinical trials, or non- clinical studies and / or post- market studies, the commercial prospects for nomlabofusp or any other potential product candidates may be harmed and our ability to generate product revenue will be

delayed or eliminated, and it would materially adversely affect our business, financial condition and results of operations. In order to market any product outside of the United States, we must comply with numerous and varying regulatory requirements of other countries regarding drug development and commercialization. The approval processes varies from country to country and can involve additional product testing and additional administrative review periods. The time required to obtain approval in other countries might differ from and be longer than that required to obtain FDA approval. Regulatory approval in one country does not ensure regulatory approval in another, but a failure or delay in obtaining regulatory approval in one country may negatively impact the regulatory process in others. Even if we receive marketing approval for nomlabofusp in the United States, we may never receive regulatory approval to market nomlabofusp outside of the United States. ~~Disruptions at~~ **Changes in** the FDA ~~and ,~~ other government agencies ~~caused by funding shortages~~ or ~~global health concerns~~ **comparable regulatory authorities** could hinder their ability to hire ~~,~~ **and** retain ~~or deploy~~ key leadership and other personnel, ~~or otherwise~~ prevent new ~~or modified~~ products **and services** from being developed, approved or commercialized in a timely manner or ~~at all~~ **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 **current administration is focused on reducing costs of the federal government generally, including significantly reducing the number of government employees. Without appropriation of additional funding to federal agencies, our business operations related to our product development activities for the U. S. market could be impacted. The** ability of the FDA **or comparable foreign regulatory authorities** to review and ~~/ or~~ approve new products can be affected by a variety of factors, including government budget and funding levels, ~~statutory, regulatory, and policy changes, the FDA' s~~ ability to hire and retain key personnel, and accept the payment of user fees, **statutory, regulatory** and **policy changes** ~~other events that may otherwise affect the FDA' s ability to perform routine functions~~ . In addition, government funding of other government agencies **or comparable foreign regulatory authorities 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 **or comparable foreign regulatory authorities** 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. ~~Furthermore~~ **Changes in FDA staffing could result in delays in the FDA' s responsiveness or in its ability to review submissions or applications** , **issue regulations or guidance, or implement or enforce regulatory requirements in a timely fashion or at all. Similar consequences would also result in the event of another significant shutdown of the federal** government **. As** ~~shutdowns could also impact the ability of regulatory authorities and~~ **an** ~~government agencies to function normally and support our operations. For~~ example, **over the last several years,** the U. S. ~~federal~~ government has shut down **several times and** ~~repeatedly since 1980, including for a period of 35 days beginning on December 22, 2018. During a shutdown,~~ certain regulatory ~~authorities and~~ agencies, such as the FDA ~~,~~ **and the SEC** have had to furlough ~~key personnel~~ **critical employees** and stop critical activities. If a prolonged government shutdown occurs, ~~or~~ **including as a result of reaching the debt ceiling,** if ~~global health concerns prevent~~ the FDA or **SEC experiences significant decreases in funding** ~~other regulatory authorities from conducting their regular inspections, reviews,~~ or **personnel** ~~other regulatory activities,~~ it could significantly impact the ability of the FDA or other regulatory authorities to timely review and process our regulatory submissions, which could have a material adverse effect on our business **and our timelines. Further, government shutdowns could impact our ability to access the public markets and obtain additional capital in the future** . Regulatory requirements governing biologic products have changed frequently and may continue to change in the future. Such requirements may lengthen the regulatory review process, require us to perform additional non- clinical studies or clinical trials, and increase our costs, or may force us to delay, limit or terminate certain of our programs. Regulatory requirements governing biologic drug products are evolving and may continue to change in the future. As a result, it is difficult to determine how long it will take or how much it will cost to obtain regulatory approvals for nomlabofusp for the treatment of FA or any other future protein replacement therapy product candidates in any indication, if at all. Regulatory review agencies and the new requirements and guidelines they promulgate may lengthen the regulatory review process, require us to perform additional or larger studies, increase our development costs, lead to changes in regulatory positions and interpretations, delay or prevent approval and commercialization of our product candidates or lead to significant post- approval studies, limitations or restrictions. Legislative and regulatory proposals have been made to expand post- approval requirements and restrict sales and promotional activities for pharmaceutical products. We cannot be sure whether additional legislative changes will be enacted, or whether FDA regulations, guidance or interpretations will be changed, or what the impact of such changes on the marketing approvals of our product candidates, if any, may be. In addition, increased scrutiny by the U. S. Congress of the FDA' s approval process may significantly delay or prevent marketing approval, as well as subject us to more stringent product labeling and post- marketing testing and other requirements. Delays, failure or unexpected costs in obtaining, the regulatory approval necessary to bring our product candidates to market could have a material adverse effect on our business, results of operations, financial condition and prospects. In addition, the clinical trial requirements of the FDA, the EMA and other regulatory authorities and the criteria these regulators use to determine the safety and efficacy of a product candidate vary substantially according to the type, complexity, novelty and intended use and market of such product candidates. The regulatory approval process for novel product candidates such as ours can be more expensive and take longer than for other, better known or more extensively studied product candidates. The clinical trials of nomlabofusp and any future product candidates are, and the manufacturing and marketing of nomlabofusp and any future product candidates, will be subject to extensive and rigorous review and regulation by numerous government authorities in the United States and in other countries, such as within the European Union, where we intend to seek regulatory approval of, and market, any product candidates. Before obtaining regulatory approvals for the commercial sale of any product candidate, we must demonstrate through non- clinical testing and clinical trials that the product candidate is safe and effective for use in each target indication. This process can take many years. In addition, approval policies, regulations, or the type and amount of clinical data necessary to gain approval may change during the course of a product candidate' s clinical development

and may vary among jurisdictions, which may cause delays in the approval of or may result in the decision not to approve our product candidates. We have not obtained regulatory approval for nomlabofusp, and it is possible that this product candidate or any product candidates we may seek to develop in the future will never obtain regulatory approval. If marketing approval is obtained, it will likely include post- marketing studies, and other post- marketing requirements, and surveillance such as REMS which will require the expenditure of substantial resources beyond the proceeds we currently have on hand. Furthermore, we are not permitted to market nomlabofusp in the United States, or the European Union until we receive approval of a BLA from the FDA or ~~a~~ **the grant of an** ~~MAA~~ **MA** from the ~~EMA~~ **European Commission**, or in any other foreign countries until we receive the requisite marketing approval from such countries. The development of drugs for FA or other rare diseases may require initial non- clinical studies, early and usually smaller, clinical trials and randomized, double- blind placebo controlled long- term safety and efficacy trials in order to test the safety and efficacy of the drug. Nomlabofusp requires substantial further clinical development before we can submit a BLA to the FDA. Development and / or regulatory programs for nomlabofusp in any countries other than the United States (such as a MAA to the EMA) are only in very preliminary stages and may require substantial further development in those countries prior to regulatory submissions seeking regulatory approval for marketing. Even after successful completion of clinical trials, there is a risk that the FDA or other regulatory agencies may request further information from us, disagree with our findings or otherwise undertake a lengthy review of our submission. The FDA and certain European regulatory authorities may delay, limit or deny testing or approval of nomlabofusp for many reasons, including, among others: • we may not be able to demonstrate that nomlabofusp is safe and effective to the satisfaction of the FDA or the EMA; • the results of our clinical trials may not meet the level of statistical or clinical significance required by the FDA or the EMA for marketing approval; • the FDA or the EMA may disagree with the number, design, size, duration, conduct or implementation of our clinical trials; • the FDA or the EMA may require that we conduct additional non- clinical studies and / or clinical trials; • the FDA or the EMA may not approve the formulation, manufacturing, labeling or specifications of nomlabofusp; • the CROs that we retain to conduct our clinical trials may take actions outside of our control that materially adversely impact our clinical trials; • the FDA or the EMA may find the data from non- clinical studies and clinical trials insufficient to demonstrate that nomlabofusp' s clinical and other benefits outweigh its safety risks; • the FDA or the EMA may disagree with our interpretation of data from our non- clinical studies or clinical trials; • the FDA or the EMA may not accept data generated at our clinical trial sites; • if and when our BLA is submitted, the FDA could require an FDA advisory committee assessment, or the advisory committee may recommend against approval of our application or may recommend that the FDA require, as a condition of approval, additional non- clinical studies or clinical trials, limitations on approved labeling or distribution and use restrictions; • the FDA or the EMA could require development of a REMS as a condition of approval or post- approval, or may not agree with our proposed REMS, or may impose additional requirements, including requirements that limit the promotion, advertising, distribution, or sales of nomlabofusp; • the FDA or the EMA may find deficiencies with or not approve the manufacturing processes or facilities of third- party manufacturers with which we contract; or • the FDA or the EMA may change their approval policies or adopt new regulations rendering our clinical data insufficient for approval. Any of these factors, many of which are beyond our control, could jeopardize our ability to obtain and / or maintain regulatory approval for and successfully market nomlabofusp. Any delay or failure in obtaining required approvals could have a material adverse effect on our business, financial condition and results of operations. This process can take many years and will likely require the expenditure of substantial resources. Of the large number of drugs in development in the United States, only a small percentage will successfully complete the FDA regulatory approval process and be commercialized. It is possible that the FDA or other regulatory agencies will not approve any application that we submit. It is possible that our product candidates may not obtain appropriate regulatory approvals necessary for us to commence clinical trials for our product candidates. Accordingly, even if we are able to obtain the requisite financing to continue to fund our development and clinical trials, we cannot ensure that nomlabofusp, or any other of our potential product candidates will be successfully developed or commercialized. We are subject to healthcare laws and regulations, and health information privacy and security laws, which could expose us to criminal sanctions, civil penalties, contractual damages, reputational harm and diminished profits and future earnings. Healthcare providers, physicians and others will play a primary role in the recommendation and prescription of nomlabofusp or any potential product candidates, if approved. Our future arrangements with third- party payors will expose us broadly to applicable fraud and abuse and other healthcare laws and regulations that may constrain the business or financial arrangements and relationships through which we market, sell and distribute nomlabofusp or potential product candidates, if we obtain marketing approval. In addition, we may be subject to patient privacy regulation by both the federal government and the states or other countries in which we conduct our business. For more information, see the section of this report titled " Business – Healthcare Laws and Regulations – Other Healthcare Laws. " Ensuring that our future business arrangements with third parties comply with applicable healthcare laws and regulations could be costly. It is possible that governmental authorities will conclude that our business practices do 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, including anticipated activities to be conducted by our sales team, were 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, damages, fines and exclusion from government funded healthcare programs, such as Medicare and Medicaid, any of which could substantially disrupt our operations and would materially adversely affect our business, financial condition and results of operations. If any of the physicians or other 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 government funded healthcare programs, which could have a material adverse effect on our business, results of operations, financial condition and prospects. Healthcare legislation, including potentially unfavorable pricing regulations or other healthcare reform initiatives may increase the difficulty and cost for us to obtain marketing approval of and commercialize our product candidates. The commercial potential for our approved products, if

any, could be affected by changes in healthcare spending and policy in the United States and abroad. We operate in a highly regulated industry. New laws, regulations or judicial decisions or new interpretations of existing laws, regulations or decisions, related to healthcare availability, the method of delivery or payment for healthcare products and services could adversely affect our business, operations and financial condition. The United States and many foreign jurisdictions have enacted or proposed legislative and regulatory changes affecting the healthcare system that may affect our ability to profitably sell our product and product candidates, if approved. The United States government, state legislatures and foreign governments also have shown significant interest in implementing cost- containment programs to limit the growth of government- paid healthcare costs, including price controls, restrictions on reimbursement and requirements for substitution of generic products for branded prescription drugs. The pharmaceutical industry has been a particular focus of these efforts and has been significantly affected by major legislative initiatives. Previously, in March 2010, the ACA was enacted, which was intended to broaden access to health insurance, reduce or constrain the growth of healthcare spending, enhance remedies against fraud and abuse, add new transparency requirements for health care and health insurance industries, impose new taxes and fees on the health industry and impose additional health policy reforms. Healthcare reform initiatives recently culminated in the enactment of the IRA ~~Inflation Reduction Act of 2022 or IRA~~ , which, among other things, allow allows HHS to directly negotiate the ceiling price of a statutorily specified number of drugs and biologic each year that CMS reimburses under Medicare Part B and Part D, requires the payment of rebates on Medicare Part B and Part D drugs whose prices have increased at a rate faster than the rate of inflation, and redesign the Medicare Part D cost sharing structure, including revising manufacturer financial liability for covered products. For more information, see the section of this report titled " Business – Healthcare Laws and Regulations – Healthcare Reform. " **Moreover, the Creating and Restoring Equal Access to Equivalent Samples Act (" CREATES" Act), was enacted in 2019 requiring sponsors of approved new drug applications and biologics license applications to provide sufficient quantities of product samples on commercially reasonable, market- based terms to entities developing generic drugs and biosimilar biological products. The law establishes a private right of action allowing developers to sue application holders that refuse to sell them product samples needed to support their applications. If we are required to provide product samples or allocate additional resources to respond to such requests or any legal challenges under this law, our business could be adversely impacted.** We expect that additional federal, state and foreign healthcare reform measures will be adopted in the future, any of which could limit the amounts that federal and state governments will pay for healthcare products and services, which could result in limited coverage and reimbursement and reduced demand for our products, once approved, or additional pricing pressures. Even if approved, reimbursement policies could limit our ability to sell product candidates that we elect to sell on our own. If approved by regulatory authorities, market acceptance and sales of product candidates that we elect to sell on our own will depend on reimbursement policies and may be affected by healthcare reform measures. Government authorities and third- party payors, such as private health insurers and health maintenance organizations, decide which medications they will pay for and establish reimbursement levels for those medications. Cost containment is a primary concern in the U. S. healthcare industry and elsewhere. Government authorities and these third- party payors have attempted to control costs by limiting coverage and the amount of reimbursement for particular medications. We cannot be sure that reimbursement will be available for nomlabofusp, if approved, or future product candidates that we elect to sell on our own and, if reimbursement is available, the level of such reimbursement. Reimbursement may impact the demand for, or the price of, product candidates that we elect to sell. If reimbursement is not available or is available only at limited levels, we may not be able to successfully commercialize product candidates that we elect to sell. For more information, see the section of this report titled " Business – Healthcare Laws and Regulations – Coverage and Reimbursement. " In some foreign countries, particularly in Canada and European countries, the pricing of prescription pharmaceuticals is subject to strict governmental control. In these countries, pricing negotiations with governmental authorities can take six to 12 months or longer after the receipt of regulatory approval and product launch. To obtain favorable reimbursement for the indications sought or pricing approval in some countries, we may be required to conduct a clinical trial that compares the cost- effectiveness of product candidates that we elect to sell on our own with other available therapies. If reimbursement for product candidates that we elect to sell on our own is unavailable in any country in which we seek reimbursement, if it is limited in scope or amount, if it is conditioned upon our completion of additional clinical trials, or if pricing is set at unsatisfactory levels, our business, financial conditions and results of operations could be materially adversely affected. Even if we obtain marketing approval for a product candidate, our product candidates will remain subject to regulatory oversight. Even if we receive marketing approval for nomlabofusp or a future product candidate, regulatory authorities may still impose significant restrictions on the indicated uses or marketing or impose ongoing requirements for potentially costly post- approval studies. Nomlabofusp or future product candidates will also be subject to ongoing regulatory requirements for manufacturing, labeling, packaging, storage, advertising, promotion, sampling, record- keeping and submission of safety and other post- market information. The FDA has significant post- market authority, including, for example, the authority to require labeling changes based on new safety information and to require post- market studies or clinical trials to evaluate serious safety risks related to the use of a drug. An unsuccessful post-marketing study or failure to complete such a study could result in the withdrawal of marketing approval. Any regulatory approvals that we receive for nomlabofusp may also be subject to a REMS, limitations on the approved indicated uses for which the product may be marketed or to the conditions of approval, or contain requirements for potentially costly post- marketing testing, including post- approval clinical trials, and surveillance to monitor the quality, safety and efficacy of the product, all of which could lead to lower sales volume and revenue. For example, the holder of an approved BLA or NDA is obligated to monitor and report adverse events and any failure of a product to meet the specifications in the BLA or NDA. The holder of an approved BLA or NDA also must 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 federal and state laws. In addition, product

manufacturers and their facilities are subject to payment of user fees and continual review and periodic inspections by the FDA and other regulatory authorities for compliance with cGMP requirements and adherence to commitments made in the BLA or NDA or foreign marketing application. If we, or a regulatory authority, discover (s) 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 or if a regulatory authority disagrees with the promotion, marketing or labeling of that product, a regulatory authority may impose restrictions relative to that product, the manufacturing facility or us, including requiring recall or withdrawal of the product from the market or suspension of manufacturing. If we or our contractors fail to comply with applicable regulatory requirements following approval of nomlabofusp, a regulatory authority may: • issue a warning letter, or untitled letter asserting that we are in violation of the law; • require us to enter into a consent decree, which can include imposition of various fines, reimbursements for inspection costs, required due dates for specific actions and penalties for non-compliance; • request voluntary product recalls; • seek an injunction or impose administrative, civil or criminal penalties or monetary fines; • suspend or withdraw regulatory approval; • suspend any ongoing clinical trials; • refuse to approve a pending BLA or NDA or comparable foreign marketing application (or any supplements thereto) submitted by us; • restrict the marketing or manufacturing of the product; • seize or detain the product or otherwise require the withdrawal of the product from the market; • refuse to permit the import or export of product candidates; or • refuse to allow us to enter into 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 nomlabofusp, if approved, and adversely affect our business, financial condition, results of operations and prospects. In addition, the FDA' s policies, and those of equivalent foreign regulatory agencies, may change and additional government regulations may be enacted that could prevent, limit or delay regulatory approval of nomlabofusp. We cannot predict the likelihood, nature or extent of government regulation that may arise from future legislation or administrative action, either in the United States or abroad. If we are slow or unable to adapt to changes in existing requirements or the adoption of new requirements or policies, or if we are not able to maintain regulatory compliance, we may lose any marketing approval that we may have obtained and we may not achieve or sustain profitability, which would materially and adversely affect our business, financial condition, results of operations and prospects. We may pursue marketing approval for nomlabofusp in the United States, the European Union and in other jurisdictions worldwide. In order to market any product outside of the United States, we must establish and comply with the numerous and varying safety, efficacy and other regulatory requirements of other jurisdictions, including potential additional clinical trials and / or non- clinical studies. Approval procedures vary among jurisdictions and can involve additional testing and additional administrative review periods. The time required to obtain approvals in other jurisdictions might differ from that required to obtain FDA approval. The marketing approval processes in other jurisdictions may implicate all of the risks detailed above regarding FDA approval in the United States as well as other risks. In particular, in many countries outside of the United States, products must receive pricing and reimbursement approval before the product can be commercialized. Obtaining this approval can result in substantial delays in bringing products to market in such countries. Marketing approval in one jurisdiction does not necessarily ensure marketing approval in another, but a failure or delay in obtaining marketing approval in one country may have a negative effect on the regulatory process or commercial activities in others. Failure to obtain marketing approval in other countries or any delay or other setback in obtaining such approval would impair our ability to market a product candidate in such foreign markets. Any such impairment would reduce the size of our potential market, which could have a material adverse impact on our business, financial condition, results of operations and prospects. Our future growth depends, in part, on our ability to penetrate foreign markets, where we would be subject to additional regulatory burdens and other risks and uncertainties. The prevalence of FA is estimated to be approximately three times greater in the European Union than in the United States, and, therefore, represents our largest potential market for nomlabofusp. Our future profitability will depend, in part, on our ability to commercialize nomlabofusp and future product candidates in the European Union and other foreign markets for which we may rely on collaborations with third parties. If we commercialize a product candidate in foreign markets, we would be subject to additional risks and uncertainties, including: • our customers' ability to obtain reimbursement for a product candidate in foreign markets; • compliance with the FCPA; • our inability to directly control commercial activities because we may need to rely on third parties; • the burden of complying with complex and changing foreign regulatory, tax, accounting and legal requirements; • different medical practices and customs in foreign countries affecting acceptance in the marketplace; • import or export licensing requirements; • longer accounts receivable collection times; • longer lead times for shipping; • language barriers for technical training; • reduced protection of intellectual property rights in some foreign countries; • foreign currency exchange rate fluctuations; and • the interpretation of contractual provisions governed by foreign laws in the event of a contract dispute. Sales in the European Union and other foreign markets of a product candidate could also be adversely affected by the imposition of governmental controls, political and economic instability, trade restrictions and changes in tariffs. If we are unable to establish sales and marketing capabilities or enter into agreements with third parties to market and sell nomlabofusp, if approved, we may not be able to generate any revenue. We do not currently have an established infrastructure for the sales, marketing and distribution of biologic or drug products in the United States or foreign countries. In order to market a product candidate, if approved by the FDA or any other regulatory authority, we must build our sales, marketing, managerial and other non- technical capabilities or make arrangements with third parties to perform these services. If we are unable to establish adequate sales, marketing and distribution capabilities, whether independently or with third parties, or if we are unable to do so on commercially reasonable terms, our business, results of operations, financial condition and prospects will be materially adversely affected. Even if we receive marketing approval for nomlabofusp, we may not achieve broad market acceptance, which would limit the revenue that we generate from our sales. The commercial success of nomlabofusp, if developed and approved for marketing by the FDA or EMA or other applicable regulatory authorities, will depend upon the market size for,

and the awareness and acceptance of nomlabofusp among the medical community, including physicians, patients, advocacy groups and healthcare payors. Market acceptance of nomlabofusp, if approved, will depend on a number of factors, including, among others: • if the actual number of patients with FA is lower than we believe; • the relative convenience and ease of subcutaneous injections as the necessary method of administration; • the prevalence and severity of any adverse side effects associated with nomlabofusp; • limitations or warnings contained in the labeling approved for nomlabofusp by the FDA, EMA, or other regulatory authorities, such as a " boxed " warning or if any approval that we obtain is based on a narrower definition of possible patient populations; • availability of alternative treatments, including any competitive FA therapies approved or in development that have been or could be approved or commercially launched prior to approval of nomlabofusp; • the willingness of the target patient population to try new therapies and of physicians to prescribe these therapies; • the strength of marketing and distribution support and timing of market introduction of competitive products; • publicity concerning our products or competing products and treatments; • pricing; • patient acceptance of the cost and inconvenience associated with refrigerated storage for nomlabofusp; • payor acceptance; • increased political pressure on pharmaceutical pricing; • increased pressure on orphan drug pricing for affected patient groups; • the impact of any future changes in U. S. healthcare, including medical financial assistance or a transition to a single- payor system; • the effectiveness of our sales and marketing strategies; • our ability to increase awareness of nomlabofusp through marketing efforts; • our ability to obtain sufficient third- party coverage or reimbursement; • the willingness or ability of patients to pay out- of- pocket in the absence of third- party coverage; and • the likelihood that the FDA may require development of a REMS, as a condition of approval or post- approval or may not agree with our proposed REMS or may impose additional requirements that limit the promotion, advertising, distribution or sales of our product candidates. If nomlabofusp is approved but does not achieve an adequate level of acceptance by patients, advocacy groups, physicians and payors, we may not generate sufficient revenue from nomlabofusp to become or remain profitable and our business, financial condition and results of operations could be materially adversely affected. Our efforts to educate the medical community and third- party payors about the benefits of nomlabofusp may require significant resources and may never be successful. The FDA and other regulatory agencies actively enforce the laws and regulations prohibiting the promotion of off- label uses. If we are found to have improperly promoted off- label uses, we may become subject to significant liability. The FDA and other regulatory agencies strictly regulate the promotional claims that may be made about prescription products, such as nomlabofusp or any potential product candidates, if approved. If we receive marketing approval for nomlabofusp, or any potential product candidates, physicians may prescribe our product candidates to their patients in a manner that is inconsistent with the approved label. If we are found to have promoted such off- label uses, we may become subject to significant liability. The federal government has levied large civil and criminal fines against companies for alleged improper promotion and has enjoined several companies from engaging in off- label promotion and required that they enter into corporate integrity agreements with the Office of Inspector General of the Department of Health and Human Services. The FDA has also requested that companies enter into consent decrees or permanent injunctions under which specified promotional conduct is changed or curtailed. If we cannot successfully manage the promotion of nomlabofusp or any potential product candidates, if approved, we could become subject to significant liability, which would materially adversely affect our business, financial condition and results of operations. Additional competing technologies could emerge, adversely affecting our opportunity to generate revenue from the sale of nomlabofusp, if approved. The biotechnology and pharmaceutical industries are intensely competitive and subject to rapid and significant technological change. For example, omaveloxolone, was approved for the treatment of FA in adults and adolescents aged 16 and older by the FDA and the European Commission in February 2023 and February 2024, respectively. We expect nomlabofusp, if approved, will compete with omaveloxolone and other new, future approved products and may need to demonstrate compelling advantages in efficacy, convenience, tolerability and safety to be commercially successful. Other competitive factors, including biosimilar and gene therapy competition, could force us to lower prices or could result in reduced sales. Many of our current or potential competitors, either alone or with strategic partners, have significantly greater financial resources and expertise in research and development, manufacturing, non- clinical testing, conducting clinical trials, obtaining regulatory approvals, and marketing approved products than we do. New products developed by others could emerge as competitors to nomlabofusp or any other potential product candidates, resulting in nomlabofusp or other product candidates being obsolete before we are able to recover expenses incurred in connection with their development or realize revenues from any commercialized product. The pricing of our current product candidate, if and when approved for marketing, will depend, in part, on the pricing strategies adopted by our competitors. If these or other companies enact pricing strategies that impact the price we can charge for our product candidate, if approved, we may reduce our prices and our revenue and results of operations could be affected. Any new product could also affect our ability to recruit and retain clinical trial patients, to obtain and maintain designations or eligibility for expedited regulatory pathways, and to commercialize current and future product candidates. Given that we are still in a relatively early phase of development for nomlabofusp, the recent approval and commercialization of omaveloxolone and the approval of any future competing technologies could provide competitors with a significant competitive advantage and may create an additional barrier to market acceptance of nomlabofusp. If we are not able to compete effectively against our current and future competitors, our business will not grow and our financial condition and results of operations will be adversely affected. Mergers and acquisitions in the pharmaceutical and biotechnology industries may result in even more resources being concentrated among a smaller number of our competitors. Smaller or early- stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies. These competitors also compete with us in recruiting and retaining qualified scientific and management personnel and establishing clinical trial sites and patient registration for clinical trials, as well as in acquiring technologies complementary to, or necessary for, our programs. Our commercial opportunity could be reduced or eliminated if our competitors develop and commercialize products that are safer, more effective, have fewer or less severe side effects, are more convenient, or are less expensive than any products that we may develop. Our competitors also may obtain FDA, EMA or other

regulatory approval for their products more rapidly than we may obtain approval for ours and may obtain orphan product exclusivity from the FDA for indications our product candidates are targeting, which could result in our competitors establishing a strong market position before we are able to enter the market. Additionally, products or technologies developed by our competitors may render our potential product candidates uneconomical or obsolete, and we may not be successful in marketing any product candidates we may develop against competitors. We may face competition from biosimilars and may face increasing competition over time. We may face competition from biosimilars in both the United States and Europe, and over time we may face increasing biosimilar competition. To the extent that governments adopt more permissive approval frameworks and competitors are able to obtain broader or expedited marketing approval for biosimilars, the rate of increased competition for our biologic drug products could accelerate. Expiration or successful challenge of applicable patent rights could trigger such competition, and we could face more litigation regarding the validity and / or scope of our patents. Our products may also experience greater competition from lower cost biosimilars or generics that come to market when branded products that compete with our products lose their own patent protection. In the European Union, the European Commission has granted marketing authorizations for biosimilars pursuant to a set of general and product class- specific guidelines for biosimilar approvals **originally** issued in 2005 **and updated in 2014** . In addition, in an effort to spur biosimilar utilization and / or increase potential healthcare savings, some countries in the European Union have adopted biosimilar uptake measures such as requiring physician prescribing quotas or promoting switching or pharmacy substitution of biosimilars for the corresponding reference products, and other countries may adopt similar measures. Some countries in the European Union may impose automatic price reductions upon market entry of the second or third biosimilar competitor. In the United States, the ACA authorized the FDA to ~~approve~~ **license** biosimilars via a separate, abbreviated pathway. A growing number of companies have announced that they are in varying stages of development of biosimilar versions of existing biotechnology products. Some companies pursuing development of biosimilars may challenge our patents well in advance of the expiration of our material patents. The U. S. pathway includes the option for biosimilar products meeting certain criteria to be approved as interchangeable with their reference products. Some companies developing biosimilars may seek to ~~register~~ **license** their products as interchangeable biologics, which could make it easier for prescribers or pharmacists to substitute those biosimilars for our products. In addition, critics of the 12- year exclusivity period in the biosimilar pathway ~~law~~ will likely continue to seek to shorten the data exclusivity period and / or to encourage the FDA to interpret narrowly the law' s provisions regarding which new products receive data exclusivity. While we are unable to predict the precise impact of biosimilars, we expect in the future for there to be greater competition in the United States as a result of biosimilars and downward pressure on product prices and sales. These biosimilars or generics may affect the tier designation by third party payors and may require prior authorization for use of nomlabofusp, thereby adding barriers to access. This additional competition could have a material adverse effect on our business, financial condition and results of operations. Risks Related to Our Business If we are unable to manage expected growth in the scale and complexity of our operations, including attracting and hiring additional qualified management, our performance may suffer. We are an early- stage clinical biotechnology company with a small number of employees, and our management systems currently in place are not likely to be adequate to support our future growth plans. As a result, we are highly dependent on our management and scientific personnel. The loss of the services of any of our executive officers, other key employees or consultants and other scientific advisors in the foreseeable future, might impede the achievement of our research, development and commercialization objectives. Competition for qualified personnel in the biopharmaceutical field is intense due to the limited number of individuals who possess the skills and experience required by our industry. We will need to continue to hire additional personnel as we expand our clinical development and if we initiate commercial activities. We may not be able to attract and retain quality personnel on acceptable terms, or at all. In addition, to the extent we hire personnel from competitors, we may be subject to allegations that they have been improperly solicited or that they have divulged proprietary or other confidential information, or that their former employers own their research output. We rely on consultants and advisors, including scientific, non- clinical, manufacturing and clinical advisors, to assist us in formulating our development and commercialization strategy. These consultants and advisors may be employed by other employers and may have commitments under consulting or advisory contracts with other entities that may limit their availability to us. In addition, a possible future pandemic, epidemic or outbreak of an infectious disease may pose a risk to our ability to retain and rely on our executive officers and key employees, including the potential that one or more of such employees or members of their families may contract the virus, which could impact the ability of such employees to perform as expected, which in turn would adversely impact our current and planned operations. Recruiting and retaining qualified scientific, medical clinical, manufacturing, quality assurance, regulatory, legal, public company financial, business, sales, marketing and commercial personnel and implementing and improving our operational, financial and management systems will be critical to our ability to grow and succeed. These demands also will require the hiring of additional executive or management- level personnel or the development of additional expertise by our senior management personnel. Hiring a significant number of additional employees, particularly those at the executive or management level, would increase our expenses significantly. In addition, we may not be able to attract and retain these personnel on acceptable terms given the competition among numerous pharmaceutical companies for similar personnel. We also experience competition for the hiring of scientific personnel from universities and research institutions. Moreover, delays or failures in clinical trials may also make it more challenging to recruit and retain qualified scientific personnel. If we are unable to continue to attract and retain high quality personnel, our ability to pursue our business strategy will be limited and our business, financial condition and results of operations would be adversely affected. Further, if we fail to expand and enhance our operational, financial, management and compliance systems in conjunction with potential future growth, such failure could have a material adverse effect on our business, financial condition and results of operations. We may be unable to successfully implement these tasks on a larger scale and, accordingly, may not achieve our research, development, business and growth goals. We may acquire businesses or products, or form strategic alliances, in the future, and we may not realize the benefits of

such acquisitions or alliances. We may acquire additional businesses or products, form strategic alliances or create joint ventures with third parties that we believe will complement or augment our existing business. If we acquire businesses with promising markets or technologies, we may not be able to realize the benefit of such transactions if we are unable to successfully integrate such businesses with our existing operations and company culture. We may encounter numerous difficulties in developing, manufacturing and marketing any new products resulting from a strategic alliance or acquisition that delays or prevents us from realizing their expected benefits or enhancing our business. We cannot be certain that, following any such transaction, we will achieve the expected synergies to justify the transaction and it could adversely affect our business, financial condition and results of operations. We may seek to establish collaborations and, if we are not able to establish them on commercially reasonable terms, we may have to alter our development and commercialization plans or expand our internal efforts and growth. Our development programs and the potential commercialization of our product candidates will require substantial additional cash to fund expenses. For nomlabofusp, and any future product candidates, we may decide to collaborate with pharmaceutical and / or biotechnology companies for the development and potential commercialization of those product candidates in some or all markets. We face significant competition in seeking appropriate collaborators. Whether we reach a definitive agreement for a collaboration for nomlabofusp or other potential product candidates will depend, among other things, upon our assessment of the collaborator' s resources and expertise, the terms and conditions of the proposed collaboration and the proposed collaborator' s evaluation of a number of factors. Those factors may include the design or results of clinical trials, the likelihood of approval by the FDA or similar regulatory authorities outside the United States, the potential market for the applicable product candidate, the costs and complexities of manufacturing and delivering such product candidate to patients, the potential of competing products, the existence of uncertainty with respect to our ownership of technology, which can exist if there is a challenge to such ownership without regard to the merits of the challenge and industry and market conditions generally. The collaborator may also consider alternative product candidates or technologies for similar indications that may be available to collaborate on and whether such collaboration could be more attractive than the one with us for our product candidate. The terms of any collaboration or other arrangement that we may establish may not be favorable to us. We may also be restricted under existing license agreements from entering into future agreements on certain terms with potential collaborators. Collaborations are complex and time- consuming to negotiate and document. In addition, there have been a significant number of recent business combinations among large pharmaceutical companies that have resulted in a reduced number of potential future collaborators. We may not be able to negotiate collaborations on a timely basis, on acceptable terms, or at all. If we are unable or unwilling to do so, we may have to curtail the development potential product candidates for which we are seeking to collaborate, reduce or delay our development program or one or more of our other development programs, delay potential commercialization in some or all markets or reduce the scope of any sales or marketing activities, or increase our expenditures and undertake development or commercialization activities at our own expense, including potentially increasing our infrastructure and investment outside the United States. If we elect to increase our expenditures to fund development or commercialization activities on our own, we will need to obtain additional capital, which may not be available to us on acceptable terms if at all. If we do not have sufficient funds, we may not be able to further develop our product candidates or bring them to market and generate product revenue. In addition, such efforts may require diversion of a disproportionate amount of our attention away from other day- to- day activities and require devotion of a substantial amount of our time to managing these activities. In addition, any future collaborations that we enter into may not be successful. The success of our collaboration arrangements will depend heavily on the efforts and activities of our collaborators. Collaborators generally have significant discretion in determining the efforts and resources that they will apply to these collaborations. Disagreements between parties to a collaboration arrangement regarding clinical development and commercialization matters can lead to delays in the development process or commercializing the applicable product candidate and, in some cases, termination of the collaboration arrangement. These disagreements can be difficult to resolve if neither of the parties has final decision- making authority. Collaborations with pharmaceutical or biotechnology companies and other third parties often are terminated or allowed to expire by the other party. Any such termination or expiration could adversely affect our business, financial condition, results of operations and could harm our business reputation. We face risks related to health epidemics, and / or other outbreaks of communicable diseases, which could significantly disrupt our operations and may materially and adversely affect our business and financial conditions. Our business could be adversely impacted by the effects of a global pandemic, epidemic or outbreak of an infectious disease ~~, such as, for example, a possible resurgence of vaccine resistant or more highly contagious or deadly variants of COVID- 19 and the efforts to mitigate such outbreaks~~ . Such global outbreaks of communicable diseases globally could materially and adversely impact our operations, including without limitation, our manufacturing and supply chain for nomlabofusp and our planned clinical trials, which could continue to face, enrollment difficulties as hospitals or clinical trials sites experience closures. Because FA is a rare disease, there are a limited number of patients in close proximity to clinical trial sites and clinical trial patients travel from throughout the United States to clinical trial sites to participate. Any travel advisories or infection risks could present increased risks to patients traveling to a clinical trial site for dosing if clinical trials are allowed to continue. These events could delay our clinical trials, increase the cost of completing our clinical trials and negatively impact the integrity, reliability or robustness of the data from our clinical trials. In addition, employee health and availability could be impacted, which may have a material and adverse effect on our business, financial condition and results of operations. Future pandemics could adversely affect global economies and financial markets resulting in an economic downturn that could have a material adverse effect on our business and prospects. We are subject to stringent and evolving U. S. and foreign laws, regulations and rules, contractual obligations, industry standards, policies and other obligations related to data privacy and security. Our actual or perceived failure to comply with such obligations could lead to regulatory investigations or actions; litigation; fines and penalties; disruptions of our business operations; reputational harm; loss of revenue or profits; and other adverse business consequences. In the ordinary course of business, we collect, receive, store, process, generate, use, transfer,

disclose, make accessible, protect, secure, dispose of, transmit, and share (collectively, process) personal data and other sensitive information, including proprietary and confidential business data, trade secrets, intellectual property, data we collect about trial participants in connection with clinical trials, sensitive third- party data, business plans, transactions, financial information and medical information collected by our patient access management team (collectively, sensitive data). Our data processing activities may subject us to numerous data privacy and security obligations, such as various laws, regulations, guidance, industry standards, external and internal privacy and security policies, contractual requirements, and other obligations relating to data privacy and security. In the United States, federal, state, and local governments have enacted numerous data privacy and security laws, including data breach notification laws, personal data privacy laws, consumer protection laws (e. g., Section 5 of the Federal Trade Commission Act), and other similar laws (e. g., wiretapping laws). For example, HIPAA, as amended by HITECH, imposes specific requirements relating to the privacy, security, and transmission of individually identifiable health information. Additionally, in the past few years, numerous U. S. states — including California, Virginia, Colorado, Connecticut, and Utah — have enacted comprehensive privacy laws that impose certain obligations on covered businesses, including providing specific disclosures in privacy notices and affording residents with certain rights concerning their personal data. As applicable, such rights may include the right to access, correct, or delete certain personal data, and to opt- out of certain data processing activities, such as targeted advertising, profiling, and automated decision- making. The exercise of these rights may impact our business and ability to provide our products and services. Certain states also impose stricter requirements for processing certain personal data, including sensitive information, such as conducting data privacy impact assessments. These state laws allow for statutory fines for noncompliance. For example, the California Consumer Privacy Act of 2018, as amended by the California Privacy Rights Act of 2020 (CPRA) (collectively, CCPA) requires businesses to provide specific disclosures in privacy notices and honor requests of California residents to exercise certain privacy rights. The CCPA provides for fines of up to $ 7, 500 per intentional violation and allows private litigants affected by certain data breaches to recover significant statutory damages. Although some U. S. comprehensive privacy laws exempt some data processed in the context of clinical trials, these laws may increase compliance costs and potential liability with respect to other personal data we may maintain about California residents. Similar laws are being considered in several other states, as well as at the federal and local levels, and we expect more jurisdictions to pass similar laws in the future. Outside the United States, an increasing number of laws, regulations, and industry standards may govern data privacy and security. For example, the European Union' s General Data Protection Regulation (EU GDPR), United Kingdom' s GDPR (UK GDPR) (collectively, the GDPR), Brazil' s General Data Protection Law (Lei Geral de Proteção de Dados Pessoais, or LGPD) (Law No. 13, 709 / 2018), and China' s Personal Information Protection Law (PIPL) impose strict requirements for processing personal data. For example, ~~under~~ the GDPR **includes obligations requiring disclosures to individuals regarding data processing activities** , **requiring that safeguards are implemented to protect the security and confidentiality of personal data, limiting retention periods for personal data, creating mandatory data breach notification requirements in certain circumstances, and requiring that certain measures (including contractual requirements) are put in place when engaging third- party processors** ~~companies~~ **Companies that fail to comply with the GDPR** may face temporary or definitive bans on data processing and other corrective actions; fines of up to 20 million Euros under the EU GDPR / 17. 5 million pounds sterling under the UK GDPR or 4 % of annual global revenue, whichever is greater; or private litigation related to processing of personal data brought by classes of data subjects or consumer protection organizations authorized at law to represent their interests. The Swiss Federal Act on Data Protection, or the FADP, also applies to the collection and processing of personal data, including health- related information, by companies located in Switzerland, or in certain circumstances, by companies located outside of Switzerland. The FADP has been revised and adopted by the Swiss Parliament. Companies must comply with the revised version of the FADP and its revised ordinances from September 1, 2023, which may result in an increase of costs of compliance, risks of noncompliance and penalties for noncompliance. In addition, we may be unable to transfer personal data from Europe and other jurisdictions to the United States or other countries due to data localization requirements or limitations on cross- border data flows. Europe and other jurisdictions have enacted laws requiring data to be localized or limiting the transfer of personal data to other countries. In particular, the European Economic Area (EEA) and the UK have significantly restricted the transfer of personal data to ~~the United States and other~~ countries whose privacy laws it believes are inadequate **, including the United States in certain circumstances, unless a derogation exists or adequate international transfer safeguards are put in place (for example, the European Commission approved Standard Contractual Clauses, and the UK International Data Transfer Agreement / Addendum) and transfer impact assessments carried out** . Other jurisdictions may adopt similarly stringent interpretations of their data localization and cross- border data transfer laws. ~~Although there~~ **If we** are **unable** ~~currently various mechanisms that may be used to transfer personal data from the EEA and UK to the United States in compliance with law, such as the EEA standard contractual clauses, the UK' s International Data Transfer Agreement / Addendum, and the EU- U. S. Data Privacy Framework and the UK extension thereto (which allows for transfers to relevant U. S.- based organizations who self- certify compliance and participate in the Framework), these mechanisms are subject to legal challenges, and there is no assurance that we can satisfy or rely on these measures to lawfully transfer personal data to the United States. If there is no lawful manner for us~~ to transfer personal data from the EEA, the UK, or other jurisdictions to the United States, or if the requirements for a legally- compliant transfer are too onerous, we could face significant adverse consequences, including by limiting our ability to conduct clinical trial activities in Europe and elsewhere, the interruption or degradation of our operations, the need to relocate part of or all of our business or data processing activities to other jurisdictions (such as Europe) at significant expense, increased exposure to regulatory actions, substantial fines and penalties, the inability to transfer data and work with partners, vendors and other third parties, and injunctions against our processing or transferring of personal data necessary to operate our business. ~~Some European regulators have ordered certain companies to suspend or permanently cease certain transfers of personal data to recipients outside Europe for allegedly violating the GDPR' s cross- border data transfer limitations. Additionally, companies that transfer~~

~~personal data to recipients outside of the EEA and / or UK to other jurisdictions, particularly to the United States, are subject to increased scrutiny from regulators individual litigants and activist groups.~~ In addition to data privacy and security laws, we may be contractually subject to industry standards adopted by industry groups and may become subject to such obligations in the future. We are also bound by other contractual obligations related to data privacy and security, and our efforts to comply with such obligations may not be successful. We publish privacy policies, marketing materials, and other statements, such as compliance with certain certifications or self- regulatory principles, regarding data privacy and security. If these policies, materials or statements are found to be deficient, lacking in transparency, deceptive, unfair, or misrepresentative of our practices, we may be subject to investigation, enforcement actions by regulators, or other adverse consequences. Additionally, under various privacy laws and other obligations, we may be required to obtain certain consents to process personal data. For example, some of our data processing practices may be challenged under wiretapping laws, if we obtain consumer information from third parties through various methods, including chatbot and session replay providers, or via third- party marketing pixels. These practices may be subject to increased challenges by class action plaintiffs. Our inability or failure to obtain consent for these practices could result in adverse consequences, including class action litigation and mass arbitration demands. Obligations related to data privacy and security (and consumers' data privacy expectations) are quickly changing, becoming increasingly stringent, and creating uncertainty. Additionally, these obligations may be subject to differing applications and interpretations, which may be inconsistent or conflict among jurisdictions. Preparing for and complying with these obligations requires us to devote significant resources and may necessitate changes to our services, information technologies, systems, and practices and to those of any third parties that process personal data on our behalf. We may at times fail (or be perceived to have failed) in our efforts to comply with our data privacy and security obligations. Moreover, despite our efforts, our personnel or third parties on whom we rely ~~on~~ may fail to comply with such obligations, which could negatively impact our business operations. If we or the third parties on which we rely fail, or are perceived to have failed, to address or comply with applicable data privacy and security obligations, we could face significant consequences, including but not limited to: government enforcement actions (e. g., investigations, fines, penalties, audits, inspections, and similar); litigation (including class- action claims) and mass arbitration demands; additional reporting requirements and / or oversight; bans on processing personal data; and orders to destroy or not use personal data. In particular, plaintiffs have become increasingly more active in bringing privacy- related claims against companies, including class claims. Some of these claims allow for the recovery of statutory damages on a per violation basis, and, if viable, carry the potential for monumental statutory damages, depending on the volume of data and the number of violations. Any of these events could have a material adverse effect on our reputation, business, or financial condition, including but not limited to loss of customers; inability to process personal data or to operate in certain jurisdictions; limited ability to develop or commercialize our products; expenditure of time and resources to defend any claim or inquiry; adverse publicity; or substantial changes to our business model or operations. Our internal computer systems, as well as those of CROs, CMOs, vendors, contractors, and consultants, and potential collaborators may experience failures, unauthorized access, or security breaches, potentially causing a material disruption in our product development programs, operations, harm to our brand, significant liabilities, loss of revenue, and additional costs. Despite implementing comprehensive security measures, both our internal computer systems and those of our contracted third - party service providers are susceptible to various threats, including cyber- attacks, computer viruses, unauthorized access, natural disasters, terrorism, war, and telecommunication and electrical failures. The extensive use of mobile devices accessing confidential information further heightens the risks, leading to potential device loss, security incidents, and data breaches that could result in the loss of confidential information and intellectual property. Any system failure, accident, or security breach causing interruptions in our operations may lead to a material disruption in our product development programs and business operations, potentially requiring substantial resources for recovery. For instance, the loss of clinical trial data from completed trials could lead to delays in regulatory approval efforts and significantly increase costs for data recovery or reproduction. We are exposed to risks stemming from misappropriation, misuse, leakage, falsification, or intentional or accidental release or loss of information maintained in our information systems and networks, including personal information of our employees. External parties may attempt to penetrate our systems or induce our employees or those of our vendors to disclose sensitive information. As with other companies, we face threats to our data and systems, including malicious codes, viruses, and cyber- attacks. The increasing number and complexity of these threats over time pose a challenge. A material breach in our security or that of our vendors, contractors, or consultants could harm the market perception of our security measures, resulting in business loss and damage to our reputation and credibility. This could significantly adversely affect our business, financial condition, and results of operations, necessitating substantial amounts of money and resources to repair or replace information systems or networks. Despite our continuous efforts, the possibility of such events occurring cannot be entirely eliminated. In our endeavor to protect systems storing critical information, we have implemented security measures. However, given their size and complexity and the increasing amounts of information maintained, these systems, including our internal information technology systems and those of third- party entities like Contract Research Organizations, Contract Manufacturing Organizations, contractors, and consultants, remain potentially vulnerable to breakdowns, interruptions, and security breaches. Incidents such as service interruptions, system malfunctions, natural disasters, terrorism, war, telecommunication, and electrical failures, as well as security breaches from inadvertent or intentional actions by employees, contractors, consultants, and other third parties, including cyber- attacks by malicious entities, may compromise our system infrastructure. This could lead to the loss, destruction, alteration, or unauthorized access to our data, including trade secrets, confidential information, intellectual property, proprietary business information, and personal information, or data processed or maintained on our behalf. Such incidents could result in financial, legal, business, and reputational harm. The rise in phishing and social engineering attacks and the increase in remote working further elevate security threats. Any disruption or security incident could lead to loss, damage, or unauthorized access to data, potentially exposing us to litigation and governmental investigations, delaying the development and commercialization of our product candidates, and subjecting us to

fines or penalties for noncompliance with privacy and security laws. Notifications and actions following a security incident could impact our reputation and incur significant costs, including legal expenses and remediation. Significant efforts and costs are expected for detection and prevention. Our reliance on third parties for manufacturing introduces an additional layer of risk to our business. Insurance policies, including a specific policy related to cybersecurity losses, may be insufficient, with potential availability issues in the future, high deductibles, and limitations in coverage, posing challenges in mitigating losses from disruptions, failures, or security breaches. If our information technology systems or data, and / or those of third parties upon which we rely, are or were compromised, we could experience adverse consequences resulting from such compromise, including but not limited to regulatory investigations or actions, interruptions to operations or clinical trials, reputational harm, litigation, fines and penalties, disruptions of our business operations, and a loss of customers or sales. In the ordinary course of our business, we, or the third parties upon which we rely, process proprietary, confidential, and sensitive data, including personal data (such as health- related data), intellectual property, and trade secrets. Cyberattacks, malicious internet- based activity, online and offline fraud and other similar activities threaten the confidentiality, integrity, and availability of our sensitive data and information technology systems, and those of the third parties upon which we rely. These threats are prevalent, continue to rise, and are becoming increasingly difficult to detect. These threats come from a variety of sources, including traditional computer " hackers, " hacktivists, threat actors, personnel misconduct or error (such as through theft or misuse), organized criminal threat actors, sophisticated nation- states, and nation- state- supported actors. Some actors now engage and are expected to continue to engage in cyber- attacks, including without limitation nation- state actors for geopolitical reasons and in conjunction with military conflicts and defense activities. During times of war and other major conflicts, we and the third parties upon which we rely may be vulnerable to a heightened risk of these attacks, including retaliatory cyber- attacks, that could materially disrupt our systems and operations, supply chain, and ability to produce, sell and distribute our goods and services. We and the third parties upon which we rely are subject to a variety of evolving threats, including but not limited to, social engineering attacks (including through deep fakes, which may be increasingly more difficult to identify as fake, and phishing attacks), malicious code (such as viruses and worms), malware (including as a result of advanced persistent threat intrusions), denial- of- service attacks, credential stuffing, credential harvesting, personnel misconduct or error, ransomware attacks, supply- chain attacks, software bugs, server malfunction, software or hardware failures, loss of data or other information technology assets, adware, attacks enhanced or facilitated by AI, telecommunications failures, earthquakes, fire, flood, and other similar threats. Ransomware attacks, including by organized criminal threat actors, nation- states, and nation- state- supported actors, are becoming increasingly prevalent and severe and can lead to significant interruptions, delays, or outages in our operations, disruption of clinical trials or otherwise affecting our ability to provide our products or product candidates, loss of sensitive data (including data related to clinical trials) and income, significant extra expenses to restore data or systems, reputational harm and the diversion of funds. Extortion payments may alleviate the negative impact of a ransomware attack, but we may be unwilling or unable to make such payments (including, for example, if applicable laws or regulations prohibit such payments). Remote work has become more common and has increased risks to our information technology systems and data, as more of our employees work from home, utilizing network connections, computers and devices outside our premises, including at home, while in transit or in public locations. Future business transactions (such as acquisitions or integrations) could expose us to additional cybersecurity risks and vulnerabilities, as our systems could be negatively affected by vulnerabilities present in acquired or integrated entities' systems and technologies. Furthermore, we may discover security issues that were not found during due diligence of such acquired or integrated entities, and it may be difficult to integrate companies into our information technology environment and security program. We rely on third- party service providers and technologies to operate critical business systems to process sensitive data in a variety of contexts, including, without limitation, cloud- based infrastructure, drug suppliers, data center facilities, encryption and authentication technology, employee email, content delivery to customers, and other functions. Our ability to monitor these third parties' information security practices and posture (including whether any unremediated vulnerabilities exist or have been exploited) is limited, and these third parties may not have adequate information security measures in place. In addition, supply- chain attacks have increased in frequency and severity, and we cannot guarantee that third parties' infrastructure in our supply chain or our third- party partners' supply chains have not been compromised. While we have implemented security measures designed to protect against security incidents, there can be no assurance that these measures will be effective. We take steps designed to detect, mitigate, and remediate vulnerabilities in our information security systems (such as our hardware and / or software, including that of third parties upon which we rely). We and the third parties upon which may rely may not, however, detect and remediate all such vulnerabilities including on a timely basis. For example, we have identified certain vulnerabilities in our information systems, and we have taken steps to mitigate the risks associated with known vulnerabilities. These steps include implementing compensating controls and other protective measures. Further, we and the third parties upon which we rely may experience delays in developing and deploying remedial measures and patches designed to address identified vulnerabilities. Vulnerabilities could be exploited and result in a security incident. Any of the previously identified or similar threats could cause a security incident or other interruption that could result in unauthorized, unlawful, or accidental acquisition, modification, destruction, loss, alteration, encryption, disclosure of, or access to our sensitive information or our information technology systems, or those of the third parties upon whom we rely. A security incident or other interruption could disrupt our ability (and that of third parties upon whom we rely) to provide our products. We may expend significant resources or fundamentally change our business activities and practices (including our clinical trials) to try to protect against security incidents. Certain data privacy and security obligations may require us to implement and maintain specific security measures or industry- standard or reasonable security measures to protect our information technology systems and sensitive data. Applicable data privacy and security obligations may require us to notify relevant stakeholders, including affected individuals, customers, regulators, and investors, of security incidents. Such disclosures are costly, and the disclosure or the failure to comply with such requirements could lead to adverse consequences. If we (or a third party upon whom we rely)

experience a security incident or are perceived to have experienced a security incident, we may experience adverse consequences. These consequences may include: government enforcement actions (for example, investigations, fines, penalties, audits, and inspections); additional reporting requirements and / or oversight; restrictions on processing sensitive data (including personal data); litigation (including class claims); indemnification obligations; negative publicity; reputational harm; monetary fund diversions; diversion of management attention; interruptions in our operations (including availability of data); financial loss; and other similar harms. Security incidents and attendant consequences may prevent or cause customers to stop using our products, deter new customers from using our products, and negatively impact our ability to grow and operate our business. In addition to experiencing a security incident, third parties may gather, collect, or infer sensitive data about us from public sources, data brokers or other means that reveals competitively sensitive details about our organization and could be used to undermine our competitive advantage or market position. Additionally, sensitive information of the Company could be leaked, disclosed, or revealed as a result of or in connection with the use of generative AI technologies. Our contracts may not contain limitations of liability, and even where they do, there can be no assurance that limitations of liability in our contracts are sufficient to protect us from liabilities, damages, or claims related to our data privacy and security obligations. In addition, our insurance coverage may not be adequate or sufficient in type or amount to protect us from or to mitigate liabilities arising out of our privacy and security practices. The successful assertion of one or more large claims against us that exceeds our available insurance coverage, or results in changes to our insurance policies (including premium increases or the imposition of large deductible or co- insurance requirements), could have an adverse effect on our business. We face **risks related to information technology regulatory and compliance requirements We are subject to an evolving landscape of data privacy and security regulations, including the General Data Protection Regulation (GDPR), Health Insurance Portability and Accountability Act (HIPAA), California Consumer Privacy Act (CCPA), SEC cybersecurity disclosure requirements, among others. Failure to comply with these regulations may result in substantial fines, litigation, regulatory investigations, and reputational harm. The complexity of maintaining compliance across multiple jurisdictions increases our operational burden, requiring continuous updates to security policies, employee training, and IT infrastructure enhancements. Additionally, new or amended privacy laws may impose additional costs and operational challenges, impacting our ability to conduct business effectively. If we or one of our third- party service providers fail to adhere to regulatory requirements, we may be subject to enforcement actions, consent decrees, or restrictions on our ability to process sensitive data, potentially delaying our product development or commercialization efforts. Many of our critical IT systems and data storage rely on cloud service providers. While these providers implement security controls, misconfigurations, lack of visibility into shared responsibility models, or insider threats can lead to data exposure or service disruptions. Failure to comply with evolving global data privacy and security regulations, such as GDPR, HIPAA, CCPA, SEC cybersecurity rules, and industry- specific compliance requirements (e. g., FDA 21 CFR Part 11 for regulated data), could subject us to significant fines, legal liabilities, and reputational harm. We face the risk of shadow IT and unapproved software use by employees Employees or contractors may use unauthorized or unapproved software, cloud services, or personal devices to access, store, or transmit company data, a practice commonly referred to as" Shadow IT". The use of such unapproved technologies increases security risks, as these applications and services may not adhere to our established cybersecurity and compliance standards. Shadow IT may introduce vulnerabilities, expose sensitive data to unauthorized parties, or lead to misconfigurations that may increase the likelihood of cyberattacks or data breaches. Additionally, unvetted third- party applications may not comply with regulatory requirements, exposing us to compliance violations and potential legal liabilities. Despite our ongoing efforts to monitor and control the use of unauthorized software, we may not be able to eliminate all instances of Shadow IT, which could materially impact our security posture, regulatory compliance, and business operations. We face** potential product liability exposure, and, if claims are brought against us, we may incur substantial liability. The use of nomlabofusp and other potential product candidates in clinical trials, if any, and the sale of nomlabofusp and other potential product candidates, if developed and approved, exposes us to the risk of product liability claims. Product liability claims might be brought against us by patients, healthcare providers or others selling or otherwise coming into contact with nomlabofusp or other potential product candidates. For example, we may be sued if any product we develop allegedly causes injury or death or is found to be otherwise unsuitable during product testing, manufacturing, marketing or sale. Physicians and patients may not comply with any warnings that identify known potential adverse effects and patients who should not use our products. If we cannot successfully defend ourselves against product liability claims, we may incur substantial liabilities or be required to stop development or, if approved, limit commercialization of our product candidates. Any such product liability claims may include allegations of defects in manufacturing, defects in design, a failure to warn of dangers inherent in the product, including as a result of interactions with alcohol or other drugs, negligence, strict liability and a breach of warranties. Claims could also be asserted under consumer protection acts in other jurisdictions. If we become subject to product liability claims and cannot successfully defend ourselves against them, we could incur substantial liabilities. In addition, regardless of merit or eventual outcome, product liability claims may result in, among other things: • withdrawal of patients or clinical investigators from our clinical trials; • delay or termination of clinical trials; • substantial monetary awards to patients or other claimants; • decreased demand for nomlabofusp or our other potential product candidates following marketing approval, if obtained; • damage to our reputation and exposure to adverse publicity; • increased FDA warnings on product labels; • initiation of investigations by regulators or ethics committees; • product recalls, withdrawals, or labeling, marketing or promotional restrictions; • litigation costs; • distraction of management' s attention from our primary business; • increased product liability costs; • loss of revenue; and • the inability to successfully commercialize nomlabofusp or other potential product candidates, if approved. We maintain product liability insurance coverage for our clinical trials with a $ 5 million aggregate coverage limit. Nevertheless, our insurance coverage may be insufficient to reimburse us for any expenses or losses we may suffer. Moreover, in the future, we may not be able to maintain

insurance coverage at a reasonable cost or in sufficient amounts to protect us against losses, including if insurance coverage becomes increasingly expensive. If we obtain marketing approval for nomlabofusp or other potential product candidates, we intend to expand our insurance coverage to include the sale of commercial products; however, we may not be able to obtain this product liability insurance on commercially reasonable terms. Large judgments have been awarded in class action lawsuits based on drugs that had unanticipated side effects. The cost of any product liability litigation or other proceedings, even if resolved in our favor, could be substantial, particularly in light of the size of our business and financial resources. A product liability claim or series of claims brought against us could cause our stock price to decline and, if we are unsuccessful in defending such a claim or claims and the resulting judgments exceed our insurance coverage, our financial condition, business, results of operations and prospects could be materially adversely affected. Risks Related to Our Reliance on Third Parties We have limited experience in conducting or supervising clinical trials and must outsource all clinical trials. As a result, many important aspects of our drug development programs are outside of our direct control. We have limited experience in conducting or supervising clinical trials that must be performed to obtain data to submit in concert with applications for approval by the FDA, the EMA or other comparable foreign regulatory authorities. As a result, we expect to continue to rely on CROs, clinical trial sites, clinical data management organizations and consultants to design, conduct, supervise and monitor our non- clinical studies and clinical trials. We, our CROs, and contractors are required to comply with various regulations, including the FDA' s regulations regarding current Good Clinical Practices (" cGCPs") which are enforced by regulatory agencies, including the FDA, and comparable foreign regulatory authorities to ensure the health, safety and rights of patients are protected in clinical development and clinical trials, and that trial data integrity is assured. Regulatory authorities ensure compliance with these requirements through periodic inspections of trial sponsors, principal investigators and clinical trial sites. Our expected reliance on third parties that we do not control does not relieve us of these responsibilities and requirements. If we or any of our CROs, contractors, or clinical trial sites fail to comply with applicable requirements, the clinical data generated in our clinical trials may be deemed unreliable and the FDA, EMA or other comparable foreign regulatory authorities may require us to perform additional clinical trials before approving our marketing applications. We cannot ensure that upon inspection by a given regulatory authority, such regulatory authority will determine that any of our clinical trials comply with such requirements. In addition, our clinical trials must be conducted with products produced under cGMP requirements, which mandate, among other things, the methods, facilities and controls used in manufacturing, processing and packaging of a drug product to ensure its safety and identity. Failure to comply with these regulations may require us to repeat non- clinical studies and / or clinical trials, which would delay the regulatory approval process, and could also subject us to enforcement action, up to and including, civil and criminal penalties, which would materially adversely affect our business, financial condition and results of operations. Our CROs and contractors are not our employees, and except for remedies available to us under our agreements with such CROs and contractors, we cannot control whether or not they devote sufficient time and resources to our ongoing clinical and non- clinical programs. If our CROs and contractors do not successfully carry out their contractual duties or obligations or meet expected deadlines or if the quality or accuracy of the clinical data they obtain is compromised due to the failure to adhere to our clinical protocols, regulatory requirements or for other reasons, our clinical trials may be extended, delayed or terminated and we may not be able to obtain regulatory approval for or successfully commercialize our product candidates. As a result, our operations and the commercial prospects for our product candidates would be harmed, our costs could increase and our ability to generate revenue could be delayed or reduced. In addition, operations of our CROs and contractors could be affected by earthquakes, power shortages, telecommunications failures, water shortages, floods, hurricanes, typhoons, fires, extreme weather conditions, medical epidemics and other natural or man- made disasters or business interruptions. If their facilities are unable to operate because of an accident or incident, even for a short period of time, some or all of our research and development programs may be harmed or delayed, and our operations and financial condition could suffer. We have less direct control over the conduct, timing and completion of these clinical trials and the management of data developed through the clinical trials than would be the case if we were relying entirely upon our own staff. Communicating with outside parties can also be challenging, potentially leading to mistakes as well as difficulties in coordinating activities. These factors may materially adversely affect the willingness or ability of third parties to conduct our clinical trials and may subject us to unexpected cost increases that are beyond our control. Nevertheless, we are responsible for ensuring that each of our clinical trials is conducted in accordance with the applicable protocol, legal and regulatory requirements and scientific standards, and our reliance on CROs and contractors does not relieve us of our regulatory responsibilities. In addition, we must, at times, share confidential information with third parties. 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, intellectual property, data from clinical studies and future development plans. Despite the contractual provisions employed when working with third parties, the need to share confidential information increases the risk that such confidential information become known by our competitors, are inadvertently incorporated into the technology of others, or are disclosed or used in violation of these agreements. Given that our proprietary position is based, in part, on our confidential information, a competitor' s discovery of our confidential information or other unauthorized use or disclosure would impair our competitive position and may have an adverse effect on our business. Moreover, because we have relied on third parties, our internal capacity to perform these functions is limited. Outsourcing these functions involves risk that third parties may not perform to our standards, may not produce results in a timely manner or may fail to perform at all. We currently have a small number of employees, which limits the internal resources we have available to engage new third- party providers, if necessary, and monitor existing third- party providers. To the extent we are unable to engage new third- party providers, if necessary, and successfully manage the performance of third- party service providers in the future, our business may be adversely affected. Though we

carefully manage our relationships with CROs, and contractors there can be no assurance that we will not encounter similar challenges or delays in the future or that these delays or challenges will not have a material adverse impact on our business, financial condition, results of operation and prospects. We rely on third- party supply and manufacturing partners for drug supplies for our research and development, non- clinical activities, and clinical activities, and may do the same for any commercial supplies of our product candidates. We rely on third- party supply and manufacturing partners to supply the materials and components for, and manufacture, our research and development, non- clinical and clinical study drug substance and drug product. We have not yet manufactured or formulated nomlabofusp or any other product candidate on a commercial scale and may not be able to do so for any of our product candidates. We will work to develop and optimize our manufacturing process; however, we cannot be sure that the process will result in therapies that are safe, potent or effective. Given our reliance on third parties and the risk of manufacturing commercial scale quantities, our ability to adequately commercially launch and / or supply nomlabofusp could be adversely affected. We do not own manufacturing facilities or supply sources for such components, non- clinical and clinical study drug substance, product and materials, including devices that may be required for administration, but may develop these capabilities in the future. There can be no assurance that our supply of research and development, non- clinical and clinical development of drugs and other materials will not be limited, interrupted, restricted in certain geographic regions or will be of satisfactory quality or continue to be available at acceptable prices. In particular, replacement of any product formulation manufacturer we may engage could require significant effort and expertise because there may be a limited number of qualified replacements. For example, we rely and expect to continue to rely on a small number of manufacturers to supply us with our requirements for drug substance and formulated drug product related to our nomlabofusp clinical program. The drug substance which is in frozen liquid form for nomlabofusp is currently manufactured for us by a third-party manufacturer, and the frozen liquid form of drug product is made at another manufacturer. We are undertaking a program with a third party manufacturer to begin to produce a lyophilized version of the drug product from the same drug substance, that, once available, we intend to use in certain of our future planned clinical trials. Our research and development programs could be adversely affected by a significant interruption in these manufacturing services or in the supply of drug substance and formulated drugs. In addition, because we rely on multiple manufacturers for our nomlabofusp clinical program, termination of our agreements with any of these manufacturers could significantly adversely impact our current and planned operations. In the event that any of our suppliers or manufacturers fails to perform their obligations to us in relation to quality, timing or otherwise, or if our supply of components or other materials becomes limited or interrupted for other reasons, we may be forced to manufacture the materials ourselves, for which we currently do not have the capabilities or resources, or enter into an agreement with another third party, which we may not be able to do on reasonable terms, if at all. 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 and a feasible alternative may not exist. These factors would increase our reliance on such manufacturer or require us to obtain a license from such 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. The delays associated with the verification of a new manufacturer could negatively affect our ability to develop product candidates in a timely manner or within budget. We also rely on third parties to store master and working cell banks. We currently have one master cell bank and one working cell bank for nomlabofusp and believe we would have adequate backup should any cell bank be lost in a catastrophic event. However, it is possible that we could lose multiple cell banks and have our manufacturing severely impacted by the need to replace the cell banks, which could materially and adversely affect our business, financial condition and results of operations. We may rely on third party manufacturers 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, financial condition and results of operations in a number of ways, including: • an inability to initiate or continue 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, including termination or nonrenewal of the agreement at a time that is costly or inconvenient for us; • delays in manufacturing associated with having to change manufacturers; • 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. We and our contract manufacturers are subject to significant regulation with respect to manufacturing our products. The manufacturing facilities on which we rely may not continue to meet regulatory requirements and have limited capacity. All entities involved in the preparation of therapeutics for clinical trials or commercial sale, including our existing contract manufacturers for nomlabofusp, are subject to extensive regulation. Some components of a finished therapeutic product approved for commercial sale or used in late- stage clinical trials must be manufactured in accordance with cGMP. These regulations govern manufacturing processes and procedures (including record keeping) and the implementation and operation of quality systems to control and assure the quality of investigational products and products approved for sale. Poor control of production processes can lead to the introduction of adventitious agents or other contaminants, or to inadvertent changes in the properties or stability of our product candidates that may not be detectable in final product testing. We or our contract manufacturers must supply all necessary documentation in support of a BLA or NDA on a timely basis and where required, must adhere to the FDA' s or other regulator' s GLPs and cGMP regulations

enforced by the FDA or other regulators through facilities inspection programs. The facilities and quality systems of some or all of our third party contractors must pass a pre- approval inspection for compliance with the applicable regulations as a condition of regulatory approval of nomlabofusp or any of our other potential products. In addition, the regulatory authorities may, at any time, audit or inspect a manufacturing facility involved with the preparation of nomlabofusp or our other potential products or the associated quality systems for compliance with the regulations applicable to the activities being conducted. If these facilities do not pass a pre- approval plant inspection, the FDA or other regulatory approval of the products will not be granted. The regulatory authorities also may, at any time following approval of a product for sale, audit the manufacturing facilities of our third party contractors. If any such inspection or audit identifies a failure to comply with applicable regulations or if a violation of our product specifications or applicable regulations occurs independent of such an inspection or audit, we or the relevant regulatory authority may require remedial measures that may be costly and / or time- consuming for us or a third party to implement and that may include the temporary or permanent suspension of a clinical trial or commercial sales or the temporary or permanent closure of a facility. Any such remedial measures imposed upon us or third parties with whom we contract could materially harm our business. If we or any of our third party manufacturers fail to maintain regulatory compliance, the FDA or other regulators can impose regulatory sanctions including, among other things, refusal to approve a pending application for a biologic product, or revocation of a pre- existing approval. As a result, our business, financial condition and results of operations may be materially harmed. Additionally, if supply from one approved manufacturer is interrupted, there could be a significant disruption in commercial supply. The number of manufacturers with the necessary manufacturing capabilities is limited. In addition, an alternative manufacturer would need to be qualified through a BLA or NDA supplement or similar regulatory submission which could result in further delay. The regulatory agencies may also require additional studies if a new manufacturer is relied upon for commercial production. The delays associated with the verification of a new manufacturer could negatively affect our ability to develop product candidates in a timely manner or within budget. These factors could also cause the delay of manufacturing development, clinical trials, regulatory submissions, required approvals or commercialization of nomlabofusp or any other product candidates, cause us to incur higher costs and prevent us from commercializing our products successfully. Furthermore, if our suppliers fail to meet contractual requirements, and we are unable to secure one or more replacement suppliers capable of production at a substantially equivalent cost, our clinical trials may be delayed, or we could lose potential revenues. Any of the above would materially adversely affect our business, financial condition and results of operations. Changes in methods of product candidate manufacturing may result in additional costs and / or delays. As product candidates progress through clinical to late- stage clinical trials to marketing approval and commercialization, it is common that various aspects of the development program, such as manufacturing methods, are altered along the way in an effort to optimize yield, manufacturing batch size, change drug product dosage form, minimize costs and achieve consistent quality and results. Such changes carry the risk that they will not achieve these intended objectives. Any of these changes could cause our product candidates to perform differently and affect the results of planned clinical trials or other future clinical trials conducted with the altered materials. This could delay completion of clinical trials, require the conduct of bridging clinical trials or the repetition of one or more clinical trials, increase clinical trial costs, delay approval of our product candidates and jeopardize our ability to commercialize our product candidates and generate revenue. We enter into various contracts in the normal course of our business in which we indemnify the other party to the contract. In the event we have to perform under these indemnification provisions, it could materially increase our costs and potential liability. In the normal course of business, we periodically enter into academic, commercial, service, collaboration, licensing, consulting and other agreements that contain indemnification provisions. With respect to our academic and other research agreements, we typically indemnify the institution and related parties from losses arising from claims relating to the products, processes or services made, used, sold or performed pursuant to the agreements for which we have secured licenses, and from claims arising from our or our sublicensees' exercise of rights under the agreement. With respect to our collaboration and contract service agreements, we typically indemnify our collaborators from any third party product liability claims that could result from the production, use or consumption of the product, as well as for alleged infringements of any patent or other intellectual property right by a third party. With respect to consulting agreements, we typically indemnify consultants from claims arising from the good faith performance of their consulting services. Should our obligation under an indemnification provision exceed applicable insurance coverage or should we be denied insurance coverage, our business, financial condition and results of operations could be adversely affected. Similarly, if we are relying on a collaborator to indemnify us and the collaborator is denied insurance coverage or the indemnification obligation exceeds their applicable insurance coverage, and if the collaborator does not have other assets available to indemnify us, our business, financial condition and results of operations could be adversely affected. To the extent we are able to enter into collaborative arrangements or strategic alliances, we may be exposed to risks related to those collaborations and alliances. Biotechnology companies sometimes become dependent upon collaborative arrangements or strategic alliances to complete the development and commercialization of product candidates. In seeking collaborative arrangements and strategic partners, we face significant competition from other companies as well as public and private research institutions. There can be no assurance that we will be able to enter into or maintain strategic alliances on terms favorable to us, or at all. If we elect to enter into collaborative arrangements or strategic alliances, these arrangements may place the development of our product candidates outside our control, may require us to relinquish important rights or may otherwise be on terms unfavorable to us, which could adversely affect our business, financial condition and results of operations. Dependence on collaborative arrangements or strategic alliances would subject us to a number of risks, including the risk that: • we may not be able to control the amount and timing of resources that our collaborators may devote to the relevant product candidates; • our collaborators may experience financial difficulties; • we may be required to relinquish important rights, such as marketing and distribution rights; • business combinations or significant changes in a collaborator' s business strategy may also adversely affect a collaborator' s willingness or ability to complete its obligations under any arrangement; • a collaborator could

independently move forward with a competing drug candidate developed either independently or in collaboration with others, including our competitors; and • collaborative arrangements are often terminated or allowed to expire, which would delay the development and may increase the cost of developing our drug candidates. Risks Related to Our Intellectual Property Rights If, in the United States and other countries, we are unable to adequately protect our proprietary technology or maintain issued patents which are sufficient to protect nomlabofusp or potential product candidates, third parties could compete against us more directly, which would have a material adverse impact on our business, results of operations, financial condition and prospects. Our commercial success will depend in part on our success in obtaining and maintaining issued patents and other intellectual property rights in the United States and elsewhere and protecting our proprietary technology. If we do not adequately protect our intellectual property and proprietary technology, 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. With respect to our patent portfolio, we in- license from WFUHS **a** certain issued U. S. ~~patents~~ **patent** that ~~relate~~ **relates** to **the** ~~nomlabofusp and its~~ use **of the TAT- FXN fusion protein** for treating FA. We also in- license from IU ~~a~~ United States ~~patent~~ **patents** and pending non- provisional applications in the United States and certain foreign countries that relate to the composition of nomlabofusp and methods of use, and certain U. S. patents relating to materials and methods of use relating to the development of nomlabofusp. We also own or co- own pending international PCT, foreign and United States non- provisional applications, ~~a~~ United States ~~Patent~~ **Patents** , and United States provisional applications relating to the development of nomlabofusp, including methods of use of nomlabofusp, biomarkers and to our peptide delivery platform technology. In some cases, we have only filed provisional patent applications on certain aspects of our technologies and each of these provisional patent applications is not eligible to become an issued patent until, among other things, we file a non- provisional patent application within 12 months of the filing date of the applicable provisional patent application. Any failure to file a non- provisional patent application within this timeline could cause us to lose the ability to obtain patent protection for the inventions disclosed in the associated provisional patent applications. With respect to both in- licensed and owned intellectual property, we cannot predict whether the patent applications we and our licensors are currently pursuing will issue as patents in any particular jurisdiction or whether the claims of any issued patents will provide sufficient protection from competitors or other third parties. We cannot provide any assurances that any of our pending patent applications that mature into issued patents will include claims with a scope sufficient to protect nomlabofusp, or other potential product candidates. Other parties have developed technologies that may be related or competitive to our approach and may have filed or may file patent applications and may have received or may receive patents that may overlap or conflict with our patent applications, either by claiming the same methods or formulations or by claiming subject matter that could dominate our patent position. The patent positions of biotechnology and pharmaceutical companies, including our patent position, involve complex legal and factual questions, and, therefore, the issuance, scope, validity and enforceability of any patent claims that we may obtain cannot be predicted with certainty. Patents, if issued, may be challenged, deemed unenforceable, invalidated, or circumvented. U. S. patents and patent applications may also be subject to interference proceedings, ex parte reexamination, or inter partes review proceedings, supplemental examination and challenges in district court. Patents may be subjected to opposition, post- grant review, or comparable proceedings lodged in various foreign, both national and regional, patent offices. These proceedings could result in either loss of the patent or denial of the patent application or loss or reduction in the scope of one or more of the claims of the patent or patent application. In addition, such proceedings may be costly. Thus, any patents that we may own or exclusively license may not provide any protection against competitors. Furthermore, an adverse decision in an interference proceeding can result in a third party receiving the patent right sought by us, which in turn could affect our ability to develop, market or otherwise commercialize nomlabofusp, and other potential product candidates. Furthermore, though an issued patent is presumed valid and enforceable, its issuance is not conclusive as to its validity or its enforceability and it may not provide us with adequate proprietary protection or competitive advantages against competitors with similar products. Competitors may also be able to design around our patents. Other parties may develop and obtain patent protection for more effective technologies, designs or methods. The laws of some foreign countries do not protect our proprietary rights to the same extent as the laws of the United States, and we may encounter significant problems in protecting our proprietary rights in these countries. If these developments were to occur, they could have a material adverse effect on our potential future sales. Our ability to enforce our patent rights depends on our ability to detect infringement. It is difficult to detect infringers who do not advertise the components that are used in their products. Moreover, it may be difficult or impossible to obtain evidence of infringement in a competitor' s or potential competitor' s product. Any litigation to enforce or defend our patent rights, even if we were to prevail, could be costly and time- consuming and would divert the attention of our management and key personnel from our business operations. We may not prevail in any lawsuits that we initiate, and the damages or other remedies awarded if we were to prevail may not be commercially meaningful. In addition, proceedings to enforce or defend our patents could put our patents at risk of being invalidated, held unenforceable, or interpreted narrowly. Such proceedings could also provoke third parties to assert claims against us, including that some or all of the claims in one or more of our patents are invalid or otherwise unenforceable. If any of our patents covering nomlabofusp are invalidated or found unenforceable, our financial position and results of operations would be materially and adversely impacted. In addition, if a court found that valid, enforceable patents held by third parties covered nomlabofusp, our financial position and results of operations would also be materially and adversely impacted. The degree of future protection for our proprietary rights is uncertain, and we cannot ensure that: • any of our patents, or any of our pending patent applications, if issued, will include claims having a scope sufficient to protect nomlabofusp or any other products or product candidates; • any of our pending patent applications will issue as patents; • we will be able to successfully develop and, if approved, commercialize nomlabofusp before our relevant patents expire; • we were the first to make the inventions covered by each of our patents and pending patent applications; • we were the first to file patent applications for these inventions; • others will not develop similar or alternative technologies that do not infringe our patents; • any of our patents will be found to ultimately be valid and enforceable; • any

patents issued to us will provide a basis for an exclusive market for our commercially viable products, will provide us with any competitive advantages or will not be challenged by third parties; • we will develop additional proprietary technologies or product candidates that are separately patentable; or • that our commercial activities or products will not infringe upon the patents of others. We rely upon unpatented trade secrets, unpatented know- how and continuing technological innovation to develop and maintain our competitive position, which we seek to protect, in part, by confidentiality agreements with our employees and consultants. We also have agreements with our employees and selected consultants that obligate them to assign their inventions to us and have non- compete agreements with some, but not all, of our consultants. It is possible that technology relevant to our business will be independently developed by a person that is not a party to such an agreement. Furthermore, if the employees and consultants who are parties to these agreements breach or violate the terms of these agreements, we may not have adequate remedies for any such breach or violation, and we could lose our trade secrets through such breaches or violations. Further, our trade secrets could otherwise become known or be independently discovered by our competitors. If we are unable to adequately protect our proprietary technology or maintain issued patents which are sufficient to protect nomlabofusp or potential future product candidates, third parties could compete against us more directly, which would have a material adverse impact on our business, results of operations, financial condition and prospects. Our key patent **relating to nomlabofusp** , which we license ~~, relates to nomlabofusp~~ will expire in 2040 and we will lose our ability to rely upon this patent to prevent competing products from entering the market, which may impair our ability to generate revenue. We have in- licensed **a** certain ~~patents~~ **patent** relating to nomlabofusp from WFUHS. The U. S. ~~patents~~ **patent** relating to ~~nomlabofusp and its~~ use **of the TAT- FXN fusion protein** for the treatment of FA ~~expire~~ **expires** in ~~2024 and~~ 2025 ~~, respectively~~. When these patents expire, we will be unable to use these patents to try to block others from marketing nomlabofusp in the United States We have also in- licensed an issued United States patent and pending non- provisional patent applications in the United States and certain foreign countries relating to the composition of nomlabofusp and methods of use from IU. This United States patent will expire in 2040 at the earliest. Pending applications if issued as patents, would also expire in 2040 at the earliest. We cannot predict whether these patent applications we and our licensors are currently pursuing will issue as patents in any particular jurisdiction or whether the claims of any issued patents will provide sufficient protection from competitors or other third parties. When these various patents, if issued, expire, we will be unable to use the patents to try to block others from marketing nomlabofusp in the United States. We own a certain United States provisional application and certain United States and foreign non- provisional applications and a United States patent relating to our platform technology. The United States Patent will expire in 2041 at the earliest, and the pending applications, if issued as patents, would be expected to expire in 2041- 2044. The provisional application may not be timely converted into a non- provisional application, and we cannot predict whether these provisional applications and non- provisional patent applications will issue as patents in any particular jurisdiction, or whether the claims of any issued patents will provide sufficient protection from competitors or third parties for potential product candidates. When these various patents expire, we will be unable to use the patents to try to block others from marketing products pertaining to our platform technology. In addition, given the amount of time required for the development, testing, and regulatory review of new product candidates, patents protecting such product candidates might expire before or shortly after such product candidates are commercialized. As a result, our intellectual property may not provide us with sufficient rights to exclude others from commercializing products similar or identical to ours. Once our patents expire, we will be subject to competition from third parties who will be able to use the intellectual property covered by these patents, which could impair our ability to generate revenue and could adversely affect our business, financial condition and results of operations. We may infringe the intellectual property rights of others, which may prevent or delay our product development efforts and stop us from commercializing or may increase the costs of commercializing nomlabofusp or other potential product candidates, if approved. Our success will depend in part on our ability to operate without infringing the intellectual property and proprietary rights of third parties. We cannot ensure that our business, products and methods do not or will not infringe the patents or other intellectual property rights of third parties. The pharmaceutical industry is characterized by extensive litigation regarding patents and other intellectual property rights. Third parties may allege that nomlabofusp or our other potential product candidates or the use of our technologies infringes patent claims or other intellectual property rights held by them or that we are employing their proprietary technology without authorization. Patent and other types of intellectual property litigation can involve complex factual and legal questions, and their outcome is uncertain. Any claim relating to intellectual property infringement that is successfully asserted against us may require us to pay substantial damages, including treble damages and attorneys' fees if we are found to be willfully infringing another party' s patents, for past use of the asserted intellectual property and royalties and other consideration going forward if we are forced to take a license. In addition, we may also be required to indemnify certain of our licensors, vendors or suppliers from any damages they incur related to any infringement of any third party intellectual property by our product candidates. In addition, if any such claim were successfully asserted against us and we could not obtain such a license, we may be forced to stop or delay developing, manufacturing, selling or otherwise commercializing nomlabofusp. Even if we are successful in these proceedings, we may incur substantial costs and divert management time and attention in pursuing these proceedings, which could have a material adverse effect on us. If we are unable to avoid infringing the patent rights of others, we may be required to seek a license, defend an infringement action or challenge the validity of the patents in court, or redesign our products. Patent litigation is costly and time consuming. We may not have sufficient resources to bring these actions to a successful conclusion. In addition, intellectual property litigation or claims could force us to do one or more of the following: • cease developing, selling or otherwise commercializing nomlabofusp; • cease preparations or development of our other potential product candidates; • pay substantial damages for past use of the asserted intellectual property; • obtain a license from the holder of the asserted intellectual property, which license may not be available on reasonable terms, if at all; and • in the case of trademark claims, redesign or rename the trademarks or trade names of our product candidates to avoid infringing the intellectual property rights of third parties, which may not be possible and, even if possible, could be costly and time-

consuming. Any of these risks coming to fruition could have a material adverse effect on our business, results of operations, financial condition and prospects. We may be subject to claims challenging the inventorship or ownership of our patents and other intellectual property. We may also be subject to claims that former employees or other third parties have an ownership interest in our patents or other intellectual property. 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, financial condition and results of operations. 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. We have systems in place to remind us to pay periodic maintenance fees, renewal fees, annuity fees and various other patent and application fees, and we employ an outside law firm to pay these fees. The U. S. Patent and Trademark Office and various foreign governmental patent agencies require compliance with a number of procedural, documentary, fee payment and other similar provisions during the patent application process. We employ an outside law firm and other professionals to help us comply, and in many cases, an inadvertent lapse can be cured by payment of a late fee or by other means in accordance with the applicable rules. However, there are situations in which noncompliance can result in abandonment or lapse of the patent or patent application, resulting in partial or complete loss of patent rights in the relevant jurisdiction. If this occurs, our competitors may be able to enter the market, which would have a material adverse effect on our business. 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. Competitors may infringe on 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 our or our licensors is not valid, is unenforceable and / or is not infringed, or may refuse to stop the other party from using the technology at issue on the 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 which could materially adversely affect our business, financial condition and results of operations. 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 us from the prevailing party. Our business, financial condition and results of operations could be harmed if the prevailing party does not offer us a license on commercially reasonable terms. Our defense of litigation or interference proceedings may fail and, even if successful, may result in substantial costs and distract our management and other employees. We may not be able to prevent, 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, which could adversely affect our business, financial condition and results of operations. 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 investors perceive these results to be negative, it could have a material adverse effect on the price of our common stock. Issued patents covering our product candidates could be found invalid or unenforceable if challenged in court. If we or one of our licensing partners initiated legal proceedings against a third party to enforce a patent covering our product candidate, the defendant could counterclaim that the patent covering our product candidate is invalid and / or unenforceable. In patent litigation in the United States, defendant counterclaims alleging invalidity and / or unenforceability are commonplace. Grounds for a validity challenge include alleged failures to meet any of several statutory requirements, including lack of novelty, obviousness or non-enablement. Grounds for unenforceability assertions include allegations that someone connected with prosecution of the patent withheld relevant information from the U. S. PTO, or made a misleading statement, 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, e. g. opposition proceedings. Such proceedings could result in revocation or amendment of our patents in such a way that they no longer cover our product candidates or competitive products. The outcome following legal assertions of invalidity and unenforceability is unpredictable. With respect to validity, for example, we cannot ensure that there is no invalidating prior act, of which we and the patent examiner were unaware during prosecution. If a defendant were to prevail on a legal assertion of invalidity and / or unenforceability, we would lose at least part, and perhaps all, of the patent protection on our product candidates. Such a loss of patent protection would have a material adverse impact on our business, financial condition, and results of operations. We do not seek to protect our intellectual property rights in all jurisdictions throughout the world and we may not be able to adequately enforce our intellectual property rights even in the jurisdictions where we seek protection. Filing, prosecuting and defending patents on product candidates in all countries and jurisdictions throughout the world would be prohibitively expensive, and our intellectual property rights in some countries outside the United States could be less extensive than those in the United States. In addition, the laws of some foreign countries do not protect intellectual property rights to the same extent as federal and state laws in the United States. Consequently, we may not be able to prevent third parties from practicing our inventions in all countries outside the United States, or from selling or importing products made using our inventions in and into the United States or other jurisdictions. Competitors may use our technologies in jurisdictions where we have not obtained patent protection to develop their own products and further, may export otherwise infringing products to territories where we have patent protection, but enforcement is not as strong as that in the United States. These products may compete with our products and our patents or other

intellectual property rights may not be effective or sufficient to prevent them from competing. Many companies have encountered significant problems in protecting and defending intellectual property rights in foreign jurisdictions. The legal systems of certain countries, particularly certain developing countries, do not favor the enforcement of patents and other intellectual property protection, particularly those relating to biopharmaceuticals, 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. For example, an April 2020 report from the Office of the United States Trade Representative identified a number of countries, including India and China, where challenges to the procurement and enforcement of patent rights have been reported. Several countries, including India and China, have been listed in the report every year since 1989. 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, could put 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, which would materially adversely affect our business, financial condition and results of operations. We are dependent on licensed intellectual property for nomlabofusp. If we were to lose our rights to licensed intellectual property, we may not be able to continue developing or commercializing nomlabofusp, if approved. We have an exclusive license with WFUHS, pursuant to which we exclusively license certain patent rights relating to **use of** the TAT- frataxin fusion protein ~~and its use~~ , on a worldwide basis. We have an exclusive license with IU, pursuant to which we exclusively license certain patent rights relating to nomlabofusp and its use for the treatment of mitochondrial diseases, on a worldwide basis. Our license agreements with WFUHS and IU impose, and we expect our future license agreements will impose, various development, diligence, commercialization, and other obligations on us in order to maintain the licenses. In spite of our efforts, WFUHS, IU, or a future licensor might conclude that we have materially breached our obligations under such license agreements and seek to terminate the license agreements, thereby removing or limiting our ability to develop and commercialize products and technology covered by these license agreements. If these licenses are terminated, or if the underlying patent rights licensed thereunder fail to provide the intended exclusivity, competitors or other third parties would have the freedom to seek regulatory approval of, and to market, products identical to ours and we may be required to cease our development and commercialization of certain of our product candidates or of nomlabofusp. Any of the foregoing could have a material adverse effect on our competitive position, business, financial conditions, results of operations, and prospects. Moreover, 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 the licensor that is not subject to the licensing agreement; • the sublicensing of patent and other rights under our collaborative development relationships; • our diligence obligations under the license agreement and what activities satisfy those diligence obligations; • the inventorship and ownership of inventions and know- how resulting from the joint creation or use of intellectual property by our licensors and us and our partners; • whether and the extent to which inventors are able to contest the assignment of their rights to our licensors; and • the priority of invention of patented technology. The agreements under which we currently license intellectual property or technology from third parties are complex, and certain provisions in such agreements may be susceptible to multiple interpretations. The resolution of any contract interpretation disagreement that may arise could narrow what we believe to be the scope of our rights to the relevant intellectual property or technology or increase what we believe to be our financial or other obligations under the relevant agreement, either of which could have a material adverse effect on our business, financial condition, results of operations, and prospects. Moreover, if disputes over intellectual property that we have licensed prevent or impair our ability to maintain our current licensing arrangements on commercially acceptable terms, we may be unable to successfully develop and commercialize nomlabofusp, which could have a material adverse effect on our business, financial conditions, results of operations, and prospects. Some intellectual property may have been discovered through government funded programs and thus may be subject to federal regulations such as " march- in " rights, certain reporting requirements and a preference for U. S.- based companies. Compliance with such regulations may limit our exclusive rights and limit our ability to contract with non- U. S. manufacturers. Our in- licensed patent rights from WFUHS ~~and IU~~ were funded in part by the U. S. government and are therefore subject to certain federal regulations. When new technologies are developed with U. S. government funding, the U. S. government generally obtains certain rights in any resulting patents, including a non- exclusive license authorizing the U. S. government to use the invention or to have others use the invention on its behalf. The U. S. government' s rights may also permit it to disclose the funded inventions and technology to third parties and to exercise march- in rights to use or allow third parties to use the technology we have licensed that was developed using U. S. government funding. The U. S. government may exercise its march- in rights if it determines that action is necessary because we fail to achieve practical application of the government- funded technology, or because action is necessary to alleviate health or safety needs, to meet requirements of federal regulations, or to give preference to U. S. industry. In addition, our rights in such inventions may be subject to certain requirements to manufacture products embodying such inventions in the United States in certain circumstances and if this requirement is not waived. Any exercise by the U. S. government of such rights or by any third party of its reserved rights could have a material adverse effect on our competitive position, business, financial condition, results of operations, and prospects. We have not yet registered trademarks for a commercial trade name for nomlabofusp or other potential product candidates and failure to secure such registrations could adversely affect our business, financial condition and results of operations. ~~We~~ **In many jurisdictions, we** have not yet registered trademarks for a commercial trade name for nomlabofusp or other potential product candidates. Any future trademark applications may be rejected during trademark registration proceedings. Although we would be given an opportunity to respond to those rejections, we may be unable to overcome such rejections. In addition, the ~~U. S. PTO~~ **USPTO** and comparable agencies

in many foreign jurisdictions give third parties an opportunity to oppose pending trademark applications and to seek to cancel registered trademarks. Opposition or cancellation proceedings may be filed against our trademarks, and our trademarks may not survive such proceedings. Moreover, any name we propose to use with our product candidates in the United States must be approved by the FDA, and similarly in many foreign jurisdictions regardless of whether we have registered it, or applied to register it, as a trademark. The FDA typically conducts a review of proposed product names, including an evaluation of potential for confusion with other product names. If the FDA or a foreign regulatory authority objects to any of our proposed proprietary product names, we may be required to expend significant additional resources in an effort to identify a suitable substitute name that would qualify under applicable trademark laws, not infringe the existing rights of third parties and be acceptable to the FDA or other regulatory authorities. Any trademarks we have obtained or may obtain may be infringed or successfully challenged, resulting in harm to our business. We expect to rely on trademarks as one means to distinguish any of our products that are approved for marketing from the products of our competitors. Once we select new trademarks and apply to register them, our trademark applications may not be approved. Third parties may oppose or attempt to cancel our trademark applications or trademarks, or otherwise challenge our use of the trademarks. In the event that our trademarks are successfully challenged, we could be forced to rebrand our drugs, which could result in loss of brand recognition and could require us to devote resources to advertising and marketing new brands. Our competitors may infringe our trademarks and we may not have adequate resources to enforce our trademarks. If we do not obtain additional protection under the Hatch- Waxman Amendments and similar foreign legislation by extending the patent terms and obtaining data exclusivity for nomlabofusp, our business may be materially harmed. Depending upon the timing, duration and specifics of development and FDA marketing approval of nomlabofusp or our other potential product candidates, one or more of our U. S. patents may be eligible for limited patent term restoration under the Drug Price Competition and Patent Term Restoration Act of 1984, (the" Hatch- Waxman Amendments"). The Hatch- Waxman Amendments permit a patent restoration term of up to five years as compensation for patent term lost during product development and the FDA regulatory review process. However, we may not be granted an extension because of, for example, failing to apply within applicable deadlines, failing to apply prior to expiration of relevant patents or otherwise failing to satisfy applicable requirements. Moreover, the applicable time period or the scope of patent protection afforded could be less than we request. If we are unable to obtain a patent term extension or restoration or the term of any such extension is less than we request, our competitors may obtain approval of competing products following our patent expiration, and our ability to generate revenues, business, financial condition and results of operations could be materially adversely affected. Our proprietary rights may not adequately protect our technologies, which may adversely affect our position in the market, business, financial condition and results of operations. We rely on unpatented trade secrets, know- how, and technology, which are difficult to protect, especially in the pharmaceutical industry, where much of the information about a product must be made public during the regulatory approval process. We seek to protect trade secrets, in part, by entering into confidentiality agreements with employees, consultants and others. These parties may breach or terminate these agreements or may refuse to enter into such agreements with us, and we may not have adequate remedies for such breaches. Furthermore, these agreements may not provide meaningful protection for our trade secrets or other proprietary information or result in the effective assignment to us of intellectual property and may not provide an adequate remedy in the event of unauthorized use or disclosure of confidential information or other breaches of the agreements. Despite our efforts to protect our trade secrets, we or our board members, employees, consultants, contractors or scientific and other advisors may unintentionally or willfully disclose our proprietary information to competitors. If we fail to maintain trade secret protection, our competitive position may be adversely affected. Competitors may also independently discover our trade secrets. Enforcement of claims that a third party has illegally obtained and is using trade secrets is expensive, time consuming and uncertain. If our competitors independently develop equivalent knowledge, methods and know- how, we would not be able to assert our trade secrets against them and our business, financial condition and results of operations could be harmed. Changes in ~~U. S.~~ patent law **in the U. S. and in foreign jurisdictions** could diminish the value of patents in general, thereby impairing our ability to protect our products. As is the case with other biopharmaceutical companies, our success is heavily dependent on intellectual property, particularly patents. Obtaining and enforcing patents in the biopharmaceutical industry involves both technological and legal complexity and is therefore costly, time consuming and inherently uncertain. Patent reform legislation could increase the uncertainties and costs surrounding the prosecution of patent applications and the enforcement or defense of issued patents. The Leahy Smith America Invents Act (" the Leahy Smith Act") enacted in September 2011, brought significant changes to the U. S. patent law system. These include provisions that affect the way patent applications are prosecuted and may affect patent litigation. The United States Patent Office continues to develop and implement new regulations and procedures to govern administration of the Leahy Smith Act, and many of the substantive changes to patent law associated with the Leahy Smith Act became effective on March 16, 2013. The Leahy Smith Act and its implementation could increase the uncertainties and costs surrounding the prosecution of patent applications and the enforcement or defense of our issued patents, all of which could harm our business, results of operations, financial condition and prospects. In addition, the U. S. Supreme Court has ruled on several patent cases in recent years, either narrowing the scope of patent protection available in certain circumstances or weakening the rights of patent owners in certain situations. Additionally, there have been recent proposals for additional changes to the patent laws of the United States and other countries that, if adopted, could impact our ability to enforce our proprietary technology. Depending on future actions by the U. S. Congress, the U. S. courts, the U. S. PTO and the relevant law- making bodies in other countries, 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. We may be subject to damages resulting from claims that we or our employees have wrongfully used or disclosed alleged trade secrets of our employees' former employers. Many of our employees have been previously employed at other biotechnology or pharmaceutical companies, including our competitors or potential competitors. Although we are not aware of any claims currently pending against us, we may be subject to claims that

we or our employees inadvertently or otherwise used or disclosed the trade secrets or other proprietary information of our employees' former employers. Litigation may be necessary to defend against these claims. Even if we are successful in defending against these claims, litigation could result in substantial costs and be a distraction to management. If we fail in defending such claims, in addition to paying monetary claims, we may lose valuable intellectual property rights or personnel. A loss of key personnel or their work product could hamper or prevent our ability to develop and commercialize nomlabofusp or our other potential product candidates, which would materially adversely affect our business, financial condition and results of operations. Risks Related to Our Common Stock Our stock price could be highly volatile, and purchasers of our common stock could incur substantial losses. The market price of our common stock could be subject to significant fluctuations. Market prices for securities of early- stage pharmaceutical, biotechnology, and other life sciences companies have historically been particularly volatile. Some of the factors that may cause the market price of our common stock to fluctuate include: • our ability to obtain regulatory approvals for product candidates, and delays or failures to obtain such approvals; • the results of, and delays in, current, and any future, non- clinical or clinical trials of nomlabofusp or any of our future product candidates, including any delays related to a future pandemic, epidemic or outbreak of an infectious disease; • geopolitical tension, including the potential impact of ongoing conflict between Russia and Ukraine and the current conflict in Israel and Gaza (including any escalation or expansion); • the entry into, or termination of, key agreements, including key licensing or collaboration agreements; • the failure of nomlabofusp or any of our future product candidates, if approved for marketing and commercialization, to achieve commercial success; • issues in manufacturing our approved products, if any, or product candidates; • the initiation of material developments in, or conclusion of, disputes or litigation to enforce or defend any of our intellectual property rights or defend against the intellectual property rights of others; • announcements by us or our commercial partners or competitors of new commercial products, clinical progress (or the lack thereof), significant **business developments,** contracts, commercial relationships, or capital commitments; • adverse publicity relating to our markets, including with respect to other products and potential products in such markets; • the introduction of technological innovations or new therapies competing with our potential products; • the loss of key employees; • general and industry- specific economic conditions potentially affecting our research and development expenditures; • general ~~economic~~ **market** conditions ~~in~~ **and** ~~the~~ **other factors,** ~~United States and abroad (~~ including **factors unrelated** ~~the potential failure of the United States Congress~~ to ~~raise~~ **our operating performance or** the ~~debt ceiling)~~ **operating performance of our competitors, including a global pandemic, or macroeconomic factors such as geopolitical tensions, tariffs, or the outbreak or escalation of hostilities or war** ; • changes in the structure of health care payment systems; • adverse regulatory decisions; • **changes in investor perceptions of us or our industry; •** trading volume of our common stock; and • period- to- period fluctuations in our financial results. There is no guarantee that our common stock will appreciate or even maintain the price at which our holders have purchased it. Therefore, there is a risk that investors may lose all or part of their investment in our securities. Moreover, the stock markets in general have experienced substantial volatility that has often been unrelated to the operating performance of individual companies or the biotechnology sector. These broad market fluctuations may also adversely affect the trading price of our common stock. In the past, following periods of volatility in the market price of a company' s securities, stockholders have often instituted class action securities litigation against those companies. Such litigation, if instituted, could result in substantial costs and diversion of management' s attention and resources, which could significantly impact our profitability and reputation. Any lawsuit to which we are a party, with or without merit, may result in an unfavorable judgment. We also may decide to settle lawsuits on unfavorable terms. Any such negative outcome could result in payments of substantial damages or fines, damage to our reputation or adverse changes to our offerings or business practices. We must maintain effective internal controls over financial reporting, and if we are unable to do so, the accuracy and timeliness of our financial reporting may be adversely affected, which could have a material adverse effect on our business and stock price. We must maintain effective internal control over financial reporting in order to accurately and timely report our results of operations and financial condition. In addition, as a public company, the Sarbanes- Oxley Act requires, among other things, that we assess the effectiveness of our disclosure controls and procedures quarterly and the effectiveness of our internal control over financial reporting at the end of each fiscal year. The rules governing the standards that must be met for our management to assess our internal control over financial reporting pursuant to Section 404 of the Sarbanes- Oxley Act are complex and require significant documentation, testing and possible remediation. These stringent standards require that our audit committee be advised and regularly updated on management' s review of internal control over financial reporting. Our management may not be able to effectively and timely implement controls and procedures which respond to the increased regulatory compliance and reporting requirements that are applicable to us as a public company. If we fail to staff our accounting, finance and information technology functions adequately or maintain internal control over financial reporting adequate to meet the demands that are placed upon us as a public company, including the requirements of the Sarbanes- Oxley Act, or to otherwise prevent material weaknesses in internal control over financial reporting, our business and reputation may be harmed and our stock price may decline. Furthermore, investor perceptions of us may be adversely affected, which could cause a decline in the market price of our common stock. Ownership of our common stock is highly concentrated, and it may prevent other stockholders from influencing significant corporate decisions. Entities affiliated with Deerfield Management Company beneficially own or control approximately ~~38~~ **33** . ~~6~~ **3** % of our outstanding common stock (assuming ~~full exercise of our outstanding pre- funded warrants and~~ no exercise of outstanding options) as of December 31, ~~2023~~ **2024** , on a fully- diluted basis. Accordingly, such entities have substantial influence over the outcome of a corporate action by us requiring stockholder approval, including the election of directors, any merger, consolidation or sale of all or substantially all of our assets or any other significant corporate transaction. These stockholders also may exert influence in delaying or preventing a change in control of the combined company, even if such change in control would benefit our other stockholders. We are a smaller reporting company. We cannot be certain whether the reduced disclosure requirements applicable to smaller reporting companies will make our common shares less attractive to investors or otherwise limit our ability to raise additional funds. We are currently a "

smaller reporting company ” as defined in the Exchange Act of 1934 and have elected to take advantage of certain of the scaled disclosures available to smaller reporting companies, including simplified executive compensation disclosures in our filings, exemption from the provisions of Section 404 (b) of the Sarbanes- Oxley Act requiring that an independent registered accounting firm provide an attestation report on the effectiveness of internal control over financial reporting and certain other decreased disclosure obligations in our SEC filings, including, among other things, only being required to provide two years of audited financial statements in annual reports. Reduced disclosure in our SEC filings due to our status as a smaller reporting company may make it harder for investors to analyze our results of operations and financial prospects. We cannot predict whether investors will find our common stock less attractive because of our reliance on any of these exemptions. If some investors find our common stock less attractive as a result, there may be a less active trading market for our common stock and our stock price may be more volatile. Anti- takeover provisions in our charter documents and under Delaware law could make an acquisition of us more difficult and may prevent attempts by our stockholders to replace or remove our management. Provisions in our certificate of incorporation and bylaws may delay or prevent an acquisition or a change in management. These provisions include a classified board of directors, a prohibition on actions by written consent of our stockholders, and the ability of the board of directors to issue preferred stock without stockholder approval. In addition, because we are in Delaware, we are governed by the provisions of Section 203 of the Delaware General Corporation Law (" DGCL") which prohibits stockholders owning in excess of 15 % of our outstanding voting stock from merging or combining with us. Although we believe these provisions collectively will provide for an opportunity to receive higher bids by requiring potential acquirers to negotiate with our board of directors, they would apply even if the offer may be considered beneficial by some stockholders. In addition, these provisions may frustrate or prevent any attempts by our stockholders to replace or remove then current management by making it more difficult for stockholders to replace members of the board of directors, which is responsible for appointing the members of management. We do not anticipate that we will pay any cash dividends in the foreseeable future. The current expectation is that we will retain our future earnings to fund the development and growth of our business. As a result, capital appreciation, if any, of our common stock will be stockholders’ sole source of gain, if any, for the foreseeable future. Our failure to meet the continued listing requirements of The Nasdaq Stock Market LLC could result in a delisting of our Common Stock. If we fail to satisfy the continued listing requirements of The Nasdaq Stock Market LLC (" Nasdaq") such as the corporate governance requirements or the minimum closing bid price requirement, Nasdaq may take steps to delist our common stock. Such a delisting would likely have a negative effect on the price of our common stock and would impair a stockholders ability to sell or purchase shares of common stock when they wish to do so. In the event of a delisting, we can provide no assurance that any action taken by us to restore compliance with listing requirements would allow the common stock to become listed again, stabilize the market price or improve the liquidity of the common stock, prevent the common stock from dropping below the Nasdaq minimum bid price requirement or prevent future noncompliance with Nasdaq’ s listing requirements. General Risk Factors Financial reporting obligations of being a public company in the United States are expensive and time- consuming, and our management will be required to devote substantial time to new compliance matters. The obligations of being a public company in the United States require significant expenditures and place significant demands on our management and other personnel, including costs resulting from public company reporting obligations under the Securities Exchange Act of 1934, as amended, and the rules and regulations regarding corporate governance practices, including those under the Sarbanes- Oxley Act, the Dodd- Frank Wall Street Reform and Consumer Protection Act of 2010 (the" Dodd- Frank Act") and the listing requirements of Nasdaq on which our securities are listed. These rules require the maintenance of effective disclosure and financial controls and procedures, internal control over financial reporting and strong corporate governance practices, among many other complex rules that are often difficult to implement, monitor and maintain compliance with. Our management and other personnel devote a substantial amount of time to ensure that we comply with all of these requirements and to keep pace with new regulations, otherwise we may fall out of compliance and risk becoming subject to litigation or being delisted, among other potential problems. Our employees may engage in misconduct or other improper activities, including violating applicable regulatory standards and requirements or engaging in insider trading, which could significantly impact our business. We are exposed to the risk of employee fraud or other misconduct. Misconduct by employees could include intentional, reckless or negligent failures to comply with the regulations of the FDA and applicable non- U. S. regulators, provide accurate information to the FDA and applicable non- U. S. 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. Employees may also unintentionally or willfully disclose our proprietary and / or confidential information to competitors. In particular, sales, marketing and business arrangements in the healthcare industry are subject to extensive laws and regulations intended to prevent fraud, misconduct, kickbacks, self- dealing and other abusive practices. These laws and regulations restrict or prohibit a wide range of pricing, discounting, marketing and promotion, sales commission, customer incentive programs and other business arrangements. Employee misconduct could also involve the improper use of, including trading on, information obtained in the course of clinical trials, which could result in regulatory sanctions and serious harm to our reputation. We have adopted a code of conduct, but it is not always possible to identify and deter employee misconduct, and the precautions we take to detect and prevent this activity may be ineffective 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. In addition, we are subject to the risk that a person or government could allege fraud or other misconduct, even if none occurred. If any such actions are instituted against us, and we are not successful in defending ourselves or asserting our rights, those actions could have a significant impact on our business, including the imposition of significant fines or other sanctions. Unfavorable global economic conditions could adversely affect our business, financial condition or results of operations. Our results of operations could be adversely affected by general conditions in the global economy, geopolitical events and general conditions in the global financial markets. A severe or prolonged economic downturn due to geopolitical tension resulting from ongoing conflict

between Russia and Ukraine and the current conflict in Israel and Gaza (including any escalation or expansion) or other factors could result in a variety of risks to our business, including, weakened demand for our product candidates and our ability to raise additional capital when needed on acceptable terms, if at all. A weak or declining economy could also strain our suppliers, possibly resulting in supply disruption, or cause our customers to delay making payments for our products. Any of the foregoing could harm our business and we cannot anticipate all of the ways in which the current economic climate and financial market conditions could adversely impact our business. Adverse developments affecting the financial services industry, including events or concerns involving liquidity, defaults or non- performance by financial institutions or transactional counterparties, could adversely affect our business, financial condition or results of operations. Events involving limited liquidity, defaults, non-performance or other adverse developments that affect financial institutions, transactional counterparties or other companies in the financial services industry or the financial services industry generally, or concerns or rumors about any events of these kinds or other similar risks, have in the past and may in the future lead to market- wide liquidity problems. For example, in early 2023, several financial institutions closed and were taken into receivership by the Federal Deposit Insurance Corporation (“ FDIC ”). Although we assess our banking and customer relationships as we believe necessary or appropriate, our access to funding sources and other credit arrangements in amounts adequate to finance or capitalize our current and projected future business operations could be significantly impaired by factors that affect us, the financial services industry or economy in general. These factors could include, among others, events such as liquidity constraints or failures, the ability to perform obligations under various types of financial, credit or liquidity agreements or arrangements, disruptions or instability in the financial services industry or financial markets, or concerns or negative expectations about the prospects for companies in the financial services industry. In addition, investor concerns regarding the U. S. or international financial systems could result in less favorable commercial financing terms, including higher interest rates or costs and tighter financial and operating covenants, or systemic limitations on access to credit and liquidity sources, thereby making it more difficult for us to acquire financing on acceptable terms or at all. Any decline in available funding or access to our cash and liquidity resources could, among other risks, adversely impact our ability to meet our operating expenses, financial obligations or fulfill our other obligations, result in breaches of our contractual obligations or result in violations of federal or state wage and hour laws. Any of these impacts, or any other impacts resulting from the factors described above or other related or similar factors not described above, could have material adverse impacts on our liquidity and our business, financial condition or results of operations. ~~87~~ **86**