UNITED STATES
SECURITIES AND EXCHANGE COMMISSION

Washington, D.C. 20549

Form 10-K

(Mark One)

☒ **ANNUAL REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**

For the fiscal year ended December 31, 2024

or

☐ **TRANSITION REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**

For the transition period from to

Commission File Number 001-36510

LARIMAR THERAPEUTICS, INC.

(Exact name of registrant as specified in its charter)

Delaware	**20-3857670**
(State or other jurisdiction of incorporation or organization)	(I.R.S. Employer Identification No.)
Three Bala Plaza East, Suite 506 Bala Cynwyd, Pennsylvania	**19004**
(Address of principal executive offices)	(Zip Code)

(844) 511-9056

(Registrant's telephone number, including area code)

Securities registered pursuant to Section 12(b) of the Act:

Title of each class	Trading Symbol(s)	Name of each exchange on which registered
Common Stock, par value $0.001 per share	LRMR	The Nasdaq Global Market

Indicate by check mark if the registrant is a well-known seasoned issuer, as defined in Rule 405 of the Securities Act. ☐ Yes ☒ No

Indicate by check mark if the registrant is not required to file reports pursuant to Section 13 or Section 15(d) of the Act. ☐ Yes ☒ No

Indicate by check mark whether the registrant (1) has filed all reports required to be filed by Section 13 or 15(d) of the Securities Exchange Act of 1934 during the preceding 12 months (or for such shorter period that the registrant was required to file such reports), and (2) has been subject to such filing requirements for the past 90 days. Yes ☒ No ☐

Indicate by check mark whether the registrant has submitted electronically every Interactive Data File required to be submitted pursuant to Rule 405 of Regulation S-T (§ 232.405 of this chapter) during the preceding 12 months (or for such shorter period that the registrant was required to submit such files). Yes ☒ No ☐

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, a smaller reporting company, or an emerging growth company. See the definitions of "large accelerated filer," "accelerated filer," "smaller reporting company" and "emerging growth company" in Rule 12b-2 of the Exchange Act.

Large accelerated filer	☐	Accelerated filer	☐
Non-accelerated filer	☒	Smaller reporting company	☒
		Emerging growth company	☐

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act. ☐

Indicate by check mark whether the registrant has filed a report on and attestation to its management's assessment of the effectiveness of its internal control over financial reporting under Section 404(b) of the Sarbanes-Oxley Act (15 U.S.C. 7262(b)) by the registered public accounting firm that prepared or issued its audit report. ☐

If securities are registered pursuant to Section 12(b) of the Act, indicate by check mark whether the financial statements of the registrant included in the filing reflect the correction of an error to previously issued financial statements. ☐

Indicate by check mark whether any of those error corrections are restatements that required a recovery analysis of incentive-based compensation received by any of the registrant's executive officers during the relevant recovery period pursuant to §240.10D-1(b). ☐

Indicate by check mark whether the registrant is a shell company (as defined in Rule 12b-2 of the Exchange Act). Yes ☐ No ☒

On the last business day of the most recently completed second fiscal quarter, the aggregate market value (based on the closing sale price of its common stock on that date) of the registrant's Common stock held by non-affiliates of the registrant was $313,608,353.

As of March 20, 2025, the registrant had 64,027,892 shares of Common Stock, $0.001 par value per share, outstanding.

DOCUMENTS INCORPORATED BY REFERENCE

Part III of this Annual Report on Form 10-K incorporates certain information by reference from the registrant's definitive proxy statement for the 2025 annual meeting of stockholders to be filed no later than 120 days after the end of the registrant's fiscal year ended December 31, 2024.

TABLE OF CONTENTS

Item No.		Page No.
	PART I	
ITEM 1.	BUSINESS	6
ITEM 1A.	RISK FACTORS	39
ITEM 1B.	UNRESOLVED STAFF COMMENTS	87
ITEM 1C.	CYBERSECURITY	87
ITEM 2.	PROPERTIES	88
ITEM 3.	LEGAL PROCEEDINGS	88
ITEM 4.	MINE SAFETY DISCLOSURES	88
	PART II	
ITEM 5.	MARKET FOR REGISTRANT'S COMMON EQUITY, RELATED STOCKHOLDER MATTERS AND ISSUER PURCHASES OF EQUITY SECURITIES.	89
ITEM 6.	[RESERVED]	89
ITEM 7.	MANAGEMENT'S DISCUSSION AND ANALYSIS OF FINANCIAL CONDITION AND RESULTS OF OPERATIONS	90
ITEM 7A.	QUANTITATIVE AND QUALITATIVE DISCLOSURES ABOUT MARKET RISK	98
ITEM 8.	FINANCIAL STATEMENTS AND SUPPLEMENTARY DATA	98
ITEM 9.	CHANGES IN AND DISAGREEMENTS WITH ACCOUNTANTS ON ACCOUNTING AND FINANCIAL DISCLOSURE	98
ITEM 9A.	CONTROLS AND PROCEDURES	98
ITEM 9B.	OTHER INFORMATION	99
ITEM 9C	DISCLOSURE REGARDING FOREIGN JURISDICTIONS THAT PREVENT INSPECTIONS	99
	PART III	
ITEM 10.	DIRECTORS, EXECUTIVE OFFICERS AND CORPORATE GOVERNANCE	100
ITEM 11.	EXECUTIVE COMPENSATION	100
ITEM 12.	SECURITY OWNERSHIP OF CERTAIN BENEFICIAL OWNERS AND MANAGEMENT AND RELATED STOCKHOLDER MATTERS	100
ITEM 13.	CERTAIN RELATIONSHIPS AND RELATED TRANSACTIONS, AND DIRECTOR INDEPENDENCE	100
ITEM 14.	PRINCIPAL ACCOUNTING FEES AND SERVICES	100
	PART IV	
ITEM 15.	EXHIBIT AND FINANCIAL STATEMENT SCHEDULES	101
ITEM 16.	FORM 10-K SUMMARY	104
SIGNATURES		105

CAUTIONARY NOTE REGARDING FORWARD-LOOKING STATEMENTS

Statements made in this Annual Report on Form 10-K that are not statements of historical or current facts are "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995. Forward-looking statements discuss our business, operations and financial performance and conditions, as well as our plans, objectives and expectations for our business operations and financial performance and condition. In some cases, you can identify forward-looking statements by terminology such as "aim," "anticipate," "assume," "believe," "contemplate," "continue," "could," "design," "due," "estimate," "expect," "goal," "intend," "may," "objective," "plan," "predict," "positioned," "potential," "seek," "should," "target," "will," "would" and other similar expressions that are predictions of or indicate future events and future trends, or the negative of these terms or other comparable terminology. In addition, statements that "we believe" or similar statements reflect our beliefs and opinions on the relevant subject only. These forward-looking statements, which are subject to risks, uncertainties and assumptions about us, may include projections of our future financial performance, our anticipated growth strategies and anticipated trends in our business.

You should understand that the following important factors could affect our future results and could cause those results or other outcomes to differ materially from those expressed or implied in our forward-looking statements:

- uncertainties in obtaining successful non-clinical or clinical results that reliably and meaningfully demonstrate safety, tolerability and efficacy profiles that are satisfactory to the FDA, European Medicines Agency ("EMA") and/or other comparable regulatory authorities for marketing approval for nomlabofusp (nomlabofusp is the International Nonproprietary Name and the United States Adopted Name for CTI-1601) or any other product candidates that we may develop in the future and unexpected costs that may result therefrom;
- delays in patient recruitment for our clinical trials (including as a result of the impact of FDA approval of competitive products for the treatment of Friedreich's ataxia ("FA"), and/or the impact of other clinical trials of competitive products), delays as a result of clinical and non-clinical results and/or FDA's request for additional information or studies (whether clinical or non-clinical), changes in clinical protocols, adverse events, regulatory restrictions, including additional clinical holds, and milestones for nomlabofusp;
- our ability to successfully execute our ongoing open label extension trial ("OLE") and our pediatric PK run-in trial, including the timing of site initiations and the rate of patient enrollment;
- our ability to benefit from participating in the FDA's Support for Clinical Trials Advancing Rare Disease Therapeutics ("START") pilot program for the development of nomlabofusp;
- uncertainties associated with the clinical development and regulatory approval for nomlabofusp, including potential delays in the commencement, enrollment and completion of clinical trials, the timing of a potential Biologics License Application ("BLA") submission for accelerated approval, including our ability to supply to the FDA all required data for the FDA to review and accept an accelerated application, or any other product candidates that we may develop in the future;
- the difficulties and expenses associated with obtaining and maintaining regulatory approval for nomlabofusp or any other product candidate we may develop in the future, and the indication and labeling under any such approval;
- how long we can continue to fund our operations with our existing cash, cash equivalents and marketable securities and our estimates regarding future results of operations, financial position, research and development costs, capital requirements and our access and needs for additional financing;
- our expectations regarding the use of proceeds from future financings, if any;
- our ability, and the ability of third-party manufacturers we engage, to optimize, scale and validate nomlabofusp or any other product candidate's manufacturing process and to manufacture sufficient quantities of clinical supplies, and, if approved, commercial supplies of nomlabofusp or any other product candidates that we may develop in the future and our ability to maintain our relationships and contracts with our key vendors and to identify and contract with alternate or secondary key vendors;

•our ability to realize any value from nomlabofusp and/or any other product candidates we may develop in the future in light of inherent risks and difficulties involved in successfully bringing product candidates to market and the risk that the product candidates, if approved, will not achieve broad market acceptance;

•our ability to comply with regulatory requirements applicable to our business and other regulatory developments in the United States and other countries;

•the size and growth of the potential markets for nomlabofusp or any other product candidates that we may develop in the future, the rate and degree of market acceptance of nomlabofusp, if approved, or any other product candidate, that we may develop in the future and our ability to serve those markets;

•given both approved and competing therapies and products for the treatment of FA, our ability to obtain and maintain designations or eligibility for expedited regulatory programs, and to commercialize current and future product candidates, if approved, (including the impact of potential barriers to entry if competitors are able to establish a strong market position before we are able to commercialize our products);

•our ability to obtain and maintain patent protection and defend our intellectual property rights against third parties;

•the performance and compliance with the rules and regulations of the FDA (and all other regulatory authorities) of third parties upon which we depend, including third-party contract research organizations ("CROs"), consultants and third-party suppliers, manufacturers, distributors, and logistics providers;

•our ability to recruit and retain key scientific, technical, commercial, and management personnel and to retain our executive officers;

•our ability to maintain proper functionality and security of our internal computer and information systems and prevent or avoid cyber-attacks, malicious intrusion, breakdown, destruction, loss of data privacy or other significant disruption;

•the extent to which geopolitical tensions including regional conflicts around the world, adverse macroeconomic events, including those due to 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, health epidemics, unforeseen emergencies and other outbreaks of communicable diseases could disrupt our operations, the operations of third parties on which we rely or the operations of regulatory agencies we interact with in the development of nomlabofusp and any other product candidates that we may develop;

•the potential impact of regulatory developments in the U.S., including regulatory developments due to changes in the U.S. presidential administration healthcare reform in the United States, including the Inflation Reduction Act of 2022 ("IRA"), and measures being taken worldwide designed to reduce healthcare costs and limit the overall level of government expenditures; and

•the other risks and uncertainties included under the section titled “Risk Factors.”

These forward-looking statements are based on management’s current expectations, estimates, forecasts and projections about our business and the industry in which we operate, and management’s beliefs and assumptions are not guarantees of future performance or development and involve known and unknown risks, uncertainties and other factors that are in some cases beyond our control. In light of the significant uncertainties in these forward-looking statements, you should not rely upon forward-looking statements as predictions of future events. Although we believe the expectations reflected in the forward-looking statements are reasonable, the future results, levels of activity, performance or events and circumstances reflected in the forward-looking statements may not be achieved or occur at all. Except as required by law, we undertake no obligation to publicly update any forward-looking statements, whether as a result of new information, future events or otherwise after the date of this Annual Report on Form 10-K or to reflect the occurrence of any unanticipated events. Comparisons of results for current and any prior periods are not intended to express any future trends or indications of future performance, unless expressed as such, and should only be viewed as historical data.

SUMMARY RISK FACTORS

The risk factors summarized and detailed below could materially harm our business, operating results and/or financial condition, and may impair our future prospects and/or cause the price of our common stock to decline. These are not all of the risks we face, and other factors not presently known to us or that we currently believe are immaterial may also affect our business if they occur. Material risks that may affect our business, operating results and financial condition include, but are not necessarily limited to, those relating to:

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.
- We have no commercial revenue and may never become profitable.
- As of December 31, 2024, we have $183.5 million of cash, cash equivalents and marketable securities which we expect will fund operations into the second quarter of 2026. 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.

Risks Related to Our Product Development and Regulatory Approvals

- 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 or that we will ever be able to obtain regulatory approval for nomlabofusp.
- 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.
- 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 candidates we may study in the future. Difficulty in enrolling patients could delay or prevent clinical trials of nomlabofusp or any future product candidate. There is also a competing approved FA therapeutic, 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.
- 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.
- We intend to pursue accelerated approval from the 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.
- 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.
- Even if we obtain marketing approval for a product candidate, our product candidates will remain subject to regulatory oversight.
- 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.

•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.

•Additional competing technologies could emerge, adversely affecting our opportunity to generate revenue from the sale of nomlabofusp, if approved.

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 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 laws, 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.

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 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 commercial supply of our product 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 key patent relating to nomlabofusp, which we license, 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.

Risks Related to Our Common Stock

•Our stock price could be highly volatile, and purchasers of our common stock could incur substantial losses.

•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.

•Ownership of our common stock is highly concentrated, and it may prevent other stockholders from influencing significant corporate decisions.

PART I

ITEM 1. BUSINESS

Overview

We are a clinical-stage biotechnology company focused on developing treatments for patients suffering from complex rare diseases using our novel cell penetrating peptide ("CPP") technology platform. Our lead product candidate, nomlabofusp (nomlabofusp is the International Nonproprietary Name ("INN") and the United States Adopted Name ("USAN") for CTI-1601), is a subcutaneously administered, recombinant fusion protein intended to deliver tissue frataxin ("FXN"), an essential protein, to the mitochondria of patients with Friedreich's ataxia ("FA"). FA is a rare, progressive, and fatal disease in which patients are unable to produce sufficient FXN due to a genetic abnormality. Currently, there are no treatment options that address the core deficit of FA, low levels of FXN. Nomlabofusp represents the first potential therapy designed to increase FXN levels in patients with FA.

We believe that our CPP platform, which enables a therapeutic molecule to cross a cell membrane in order to reach intracellular targets, has the potential to enable the treatment of other rare and orphan diseases. We intend to use our proprietary platform to target additional orphan indications characterized by deficiencies in or alterations of intracellular content or activity.

Since our inception, we have devoted substantially all of our resources to developing nomlabofusp, building our intellectual property portfolio, developing third-party manufacturing capabilities, business planning, raising capital, and providing general and administrative support for such operations.

As of December 31, 2024, we had cash, cash equivalents and marketable securities of $183.5 million, which we anticipate will fund operations into the second quarter of 2026.

Nomlabofusp Program Update

We have completed two Phase 1 clinical trials and a Phase 2 dose exploration trial and have an ongoing Phase 2 OLE trial in patients with FA and an ongoing pharmacokinetic (PK) run-in study in adolescent patients with FA.

In May 2021, we reported positive top-line data from our Phase 1 FA program after completing dosing of the single ascending dose ("SAD") trial in December 2020 and of the multiple ascending dose ("MAD") trial in March 2021. Data from these trials demonstrated proof-of-concept by showing that daily subcutaneous injections of nomlabofusp for up to 13 days resulted in dose-dependent increases in FXN levels from baseline compared to placebo in all evaluated tissues (buccal cells, skin, and platelets). There were no serious adverse events associated with either the MAD or SAD trials.

In May 2023, we reported preliminary unblinded top-line data from the 25 mg cohort of our Phase 2 four-week, placebo-controlled, dose exploration trial of nomlabofusp in FA patients. Data from the cohort indicated nomlabofusp was generally well tolerated and showed increases in FXN levels from baseline compared to placebo in all evaluated tissues (skin and buccal cells) at day 14.

In July 2023, the FDA cleared initiation of a second cohort (50 mg) of our four-week, placebo-controlled, Phase 2 dose exploration trial of nomlabofusp in patients with FA and the initiation of our OLE trial with daily dosing of 25 mg.

In February 2024, we reported positive top-line data and successful completion of the Phase 2 dose exploration study. Nomlabofusp was generally well tolerated throughout the four-week treatment periods, had a predictable pharmacokinetic profile and led to 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. Patients who completed treatment in our Phase 2 dose exploration study or who previously completed a prior clinical trial of nomlabofusp are eligible to screen and possibly participate in the OLE study.

In March 2024, we dosed the first patient in our OLE trial, evaluating daily subcutaneous injections of 25 mg of nomlabofusp self-administered or administered by a caregiver. The OLE study will evaluate the safety and tolerability, pharmacokinetics, and frataxin levels in peripheral tissues as well as other exploratory pharmacodynamic markers (lipid profiles and gene expression data) following long-term subcutaneous administration of nomlabofusp. In addition, following the completion of enrollment, as well as at least one

participant completing one year of dosing, clinical assessments collected during the study will be compared to data from a matched control arm derived from participants in the Friedreich's Ataxia Clinical Outcome Measures Study ("FACOMS") database.

On May 30, 2024, we announced that the FDA's Center for Drug Evaluation and Research ("CDER") had selected nomlabofusp as one of a few programs for participation in the Support for Clinical Trials Advancing Rare Disease Therapeutics ("START") Pilot Program. The objective of the program is to accelerate the development of drugs for rare diseases that lead to significant disability or death by facilitating frequent advice and regular communication with the FDA staff to expedite the review process of biologics and drugs.

In September 2024, we received access to the Medicines and Healthcare Regulatory Agency ("MHRA") Innovative Licensing and Access Pathway ("ILAP") for the treatment of adults and children with FA. The ILAP is facilitating patient access to novel treatments by accelerating time to market through opportunities for enhanced engagements with UK regulatory authorities and other stakeholders. Along with the receipt of the ILAP designation, nomlabofusp has already been granted orphan drug designations in the U.S. and the European Union (the "EU"), and access to the PRIME scheme in the EU.

In December 2024, we reported positive initial data from our ongoing OLE study. This data included safety, FXN levels, clinical, pharmacokinetic data and dose escalation:

- Nomlabofusp was generally well tolerated for up to 260 days in subjects. The most common adverse events were injection site reactions, with most being mild, brief in duration, and self-limited. Two participants had serious adverse events (one seizure and one anaphylactic reaction) that resolved within 24 hours and withdrew from the study;
- Long-term 25 mg tissue FXN levels showed positive mean change from baseline of 1.32 pg/µg in buccal cells and 9.28 pg/µg in skin cells at Day 90 and that 25 mg of nomlabofusp increased and maintained tissue FXN levels over time, increasing from a mean level of 15% of healthy volunteers ("HV") at baseline to 30% in buccal cells and from 16% to 72% in skin cells at Day 90;
- Tissue FXN levels appear to reach steady-state levels by Day 30 in buccal cells;
- Early clinical data showed trends toward improvement across a number of clinical outcomes for long-term 25 mg daily nomlabofusp including: (1) decreased values indicating early trends towards improvement in the Modified Friedreich Ataxia Rating Scale ("mFARS") and the FARS-Activities of Daily Living ("ADL"), Modified Fatigue Impact Scale and 9-hole peg test at 90 days relative to baseline;
- Key pharmacokinetic data showed rapid absorption after subcutaneous administration with exposure appearing to reach steady state in plasma by day 30 with no further accumulation, which is consistent with data from our Phase 1 and Phase 2 studies.

Also in December 2024, we announced that we were increasing the dose in the OLE to 50 mg of nomlabofusp daily for then currently enrolled and all future OLE study participants. In March 2025, we announced that our Safety Monitoring Team has deemed anaphylaxis as an adverse drug reaction likely associated with nomlabofusp and therefore, we expect to see additional reactions. To reduce the risk of allergic reactions, including anaphylaxis, we amended the OLE protocol to administer premedication for the first month of dosing. The OLE study is ongoing with seven sites activated and participants continuing to enroll. All study participants are currently receiving the 50 mg dose of nomlabofusp. We plan to provide an update on OLE data on at least 30 to 40 study participants, some of whom have been receiving nomlabofusp for more than a year, in September 2025.

In January 2025, we initiated dosing of adolescents (12-17 years old) in a PK run-in study for pediatric patients with FA. Study participants in the PK run-in study are randomized 2:1 to receive either nomlabofusp at a weight-base dose expected to match the PK of adults receiving the 50 mg dose, or placebo, daily for seven days. Following assessment of safety and PK data, participants will be eligible to screen for the OLE study. The data from this cohort is expected to be presented during the nomlabofusp program update in September 2025.

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. FDA recommended focusing on assessments of skin FXN concentrations rather than buccal FXN concentrations due to more consistent sampling and less variability. FDA acknowledged that data we recently submitted appear to support a relationship between increased FXN

concentrations in skin cells and relevant tissues such as the heart, dorsal root ganglion and skeletal muscle. FDA also acknowledged that the nonclinical studies we submitted were performed at relevant human doses. FDA also suggested that we consider exploring the relationship between increases in FXN in skin and changes in pharmacodynamic (PD) markers such as lipid profiles and/or clinical measures to provide additional support for the use of FXN as an RLSE. We plan to continue discussions with FDA regarding the adequacy of the safety data set for a BLA submission seeking accelerated approval targeted for year-end 2025.

Additionally, we have obtained feedback from both FDA and EMA on the global Phase 3 study protocol and we are on track to initiate the study by mid-2025 with potential sites in the U.S., E.U., U.K, Canada, and Australia.

Our Strategy

Our strategy is to become a leader in the treatment of rare diseases by leveraging our technology platform and applying our management team's know-how and expertise to the development of nomlabofusp and other future pipeline programs. Key elements of our strategy include:

•***Advance nomlabofusp through clinical development and regulatory approval in the United States the European Union and other foreign jurisdictions*** We have completed two Phase 1 clinical trials and a Phase 2 dose exploration trial in patients with FA in the United States and have an ongoing OLE study in the U.S with patients age 18 or older with initial dosing of 25 mg of nomlabofusp daily, which, in November 2024, we began increasing to 50 mg daily. In January 2025, we started dosing adolescents (12-17 years old) in a nomlabofusp pediatric PK run-in study. Study participants in the PK run-in study are randomized 2:1 to receive either nomlabofusp or placebo daily for seven days. Adolescents receive a weight-based dose expected to match the PK of adults receiving the 50 mg dose. Adolescents who complete participation in the PK run-in study will be eligible to screen in the ongoing OLE study following assessment of safety and PK data. We are continuing to develop and execute a clinical development plan for regulatory approval of nomlabofusp in the United States, the European Union, the United Kingdom ("UK"), Australia, Canada and potentially other countries. We have obtained feedback from both FDA and EMA on the global Phase 3 study protocol and we are on track to initiate the study by mid-2025 with potential sites in the U.S., E.U., U.K, Canada, and Australia. We are continuing discussions with FDA regarding the adequacy of the safety data set required to support BLA submission and we are targeting BLA submission by the end of 2025 to seek accelerated approval.

•***Intend to pursue the use of tissue frataxin levels as a novel surrogate endpoint to support a potential Biologics License Application ("BLA") submission for accelerated approval***. We have had discussions with the FDA regarding the potential use of tissue FXN levels as a novel surrogate endpoint. The FDA 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 an 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 continuing to plan for a confirmatory study and have received feedback from FDA on the design of this study. We are targeting a BLA submission by the end of 2025.

•***If nomlabofusp receives regulatory approval(s), commercialize nomlabofusp in the United States, the European Union, and other relevant countries independently or with third parties.*** We intend to evaluate commercialization options in the United States, the European Union, the United Kingdom and in other foreign jurisdictions throughout the world where FA patients can benefit, if we are successful in obtaining regulatory approval. We may build our own internal sales force; partner with a contract sales organization, and/or enter into a joint marketing partnership with another pharmaceutical or biotechnology company, whereby we may jointly sell and market nomlabofusp, if approved; or we may seek to out-license nomlabofusp, whereby other pharmaceutical or biotechnology companies sell and market nomlabofusp and pay us milestone and/or royalty payments on sales.

•***Expand our product candidate pipeline to treat a variety of rare diseases.*** We intend to expand our pipeline to treat additional rare diseases. A key component of this strategy is to utilize our novel protein replacement therapy platform technology to deliver FXN or other molecules to intracellular targets. We employ a rational approach to selecting disease targets, and take into account many scientific, business, and indication specific factors before choosing each indication.

•***Continue to improve our novel protein replacement therapy platform.*** We continue to improve the scientific understanding of our platform, including how our technology allows enhanced delivery of cargo proteins, thereby impacting the biological processes associated with the diseases we seek to treat. In addition, with our expertise in the use of a CPP to effectively deliver proteins to intracellular targets, we believe that our scientists are well positioned to design and develop additional therapies that will address unmet medical needs associated with other rare diseases and develop other therapeutics with potentially disease modifying therapeutic action. We also plan to continue to build our intellectual property portfolio to expand our protein replacement therapy platform.

•***Opportunistically evaluate enabling, adjacent or potential competing technologies, and where advantageous, seek licenses or collaborations regarding those technologies, to advance our platform.*** We will continue to evaluate technologies that may enable or enhance our product candidates or our rare disease focus. To facilitate the advancement of our CPP platform, we periodically engage in partnering and licensing discussions with a range of biotechnology or pharmaceutical companies and academic institutions and maintain awareness of complementary technologies, synergistic opportunities and "tuck-in" options.

•***Continue to strengthen key relationships.*** We partner with experts in every aspect of development. We believe this expertise, along with our technology platform, will provide us with the ability to develop and commercialize the drug and biologic candidates we have under development and to maximize the value of our platform. In addition to partnering with experts in drug and biologic development, we collaborate with key opinion leaders, academic institutions, experts in the field of rare diseases and with patient advocacy groups associated with the diseases that are being targeted. We have established a scientific advisory board and we regularly seek advice and input from these experienced thought leaders on matters related to our research and development programs. The members of our scientific advisory board consist of distinguished research scientists, professors and industry experts recognized as key opinion leaders in the fields of rare disease, pediatrics and mitochondrial disease. We build these relationships to enhance our knowledge of the patient's needs and utilize that knowledge to design development programs intended to address unmet medical needs and add value for potential patients.

Platform Technology for Treatment of Rare Genetic Diseases

There are estimated to be over 9,500 rare genetic diseases, which, collectively affect hundreds of millions of people worldwide. Of the hundreds of millions of individuals suffering from these rare genetic diseases, only approximately 5% have therapeutic options available to manage their disease. Many of these diseases result from a deficiency in the amount or the function of a particular target molecule, often a protein. Particularly challenging to treat are those diseases that result from the deficiency of a molecule that is active within a cell or within a cell-based organelle. The challenge to providing treatment of these diseases is the need to improve the amount or function of the therapeutic target by transporting a therapeutic element across the cell membrane and potentially the membrane of the organelle where the target is active in the diseased patients.

The ability to transport therapeutic proteins across biological membranes has, to date, not been reproducibly achieved. The collective population of people with rare diseases stands to benefit from the emergence of a scalable treatment platform that can transport therapeutic proteins across cell membranes to deliver them to the intracellular site of activity. In addition, traditionally, medical treatment for each rare genetic disorder has been approached on a disease-by-disease basis. This approach is inefficient, as there are thousands of diseases, each with a distinct patient population, that cannot be addressed by traditional therapeutic approaches and are in need of treatment options. Our understanding of our therapeutics derived from proprietary gene expression data across several disease models supports the concept that product candidates based on our platform technology could significantly impact common pathological mechanisms in various diseases with comparable etiologies. We are utilizing this approach to identify therapeutic opportunities where our molecules and technology are more likely to be impactful.

Nomlabofusp For the Treatment of Friedreich's ataxia

Friedreich's ataxia

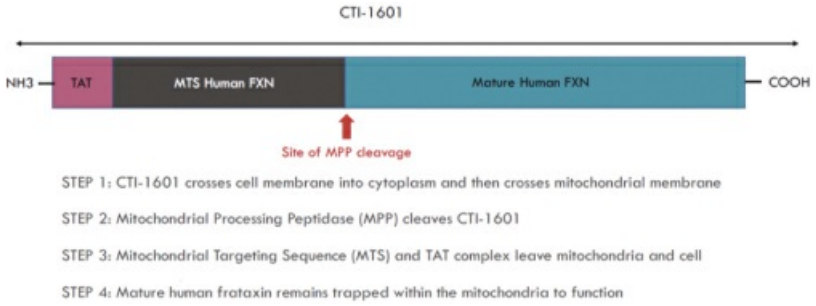
Friedreich's ataxia is a rare genetic disease that is the most commonly inherited ataxia in humans, with approximately 20,000 individuals living with Friedreich's ataxia globally, and of these individuals, approximately 5,000 are in the United States and the majority of the remaining individuals are primarily in Europe. Friedreich's ataxia results from a deficiency of the mitochondrial protein, FXN. FXN is an essential and phylogenetically conserved protein that is found in cells throughout the body, with the highest levels found in the heart, spinal cord, liver, pancreas, and skeletal muscle. FXN is encoded in the nucleus of the cell, expressed in the cytoplasm and transported into the mitochondria, where it is processed to the mature form. As part of this process the mitochondrial targeting sequence is cleaved off in the mitochondria by a naturally occurring enzyme.

Friedreich's ataxia is a progressive multi-symptom disease typically presenting in mid-childhood that affects the functioning of multiple organs and systems. It is a debilitating neurodegenerative disease that results in poor coordination of legs and arms, progressive loss of the ability to walk, generalized weakness, loss of sensation, scoliosis, diabetes and cardiomyopathy as well as impaired vision, hearing and speech. Patients suffer from progressive neurologic and cardiac dysfunction. Key among these is a primary neurodegeneration of the dorsal root ganglia and the dentate nucleus of the cerebellum, which leads to the hallmark clinical findings of progressive limb ataxia and dysarthria. A hypertrophic cardiomyopathy is common and associated with early mortality, typically between 30 and 50 years of age. Omaveloxolone, the first drug approved for the treatment of FA, was approved by the FDA and the European Commission in February 2023 and February 2024, respectively.

Nomlabofusp

Nomlabofusp, an investigational biologic fusion protein that is administered subcutaneously, consists of a CPP genetically fused to human FXN, and includes a mitochondrial targeting sequence. Using our proprietary peptide delivery technology, nomlabofusp is designed to carry the molecule from the intravascular space across the cell membrane and into the mitochondria where the CPP and the mitochondrial targeting sequence are cleaved off to yield mature FXN. See Figure 1.

Figure 1.



We have completed two Phase 1 nomlabofusp clinical trials, and a Phase 2 dose exploration trial and have an ongoing OLE study in the U.S with patients with initial dosing of 25 mg of nomlabofusp daily, which in November 2024 we increased to 50 mg daily. In January 2025, we started dosing of adolescents (12-17 years old) in a nomlabofusp pediatric PK run-in study. Study participants in the PK run-in study are randomized 2:1 to receive either nomlabofusp or placebo daily for seven days. Adolescents receive a weight-based dose equivalent to the 50 mg adult dose. Adolescents who complete participation in the PK run-in study will be eligible to screen in the ongoing OLE study. Following assessment of safety and PK data of each successive cohort, participants will be eligible to screen for the OLE study.

Based on the results of our non-clinical development program as well as results from our Phase 2 clinical trials, we believe that administering nomlabofusp may increase FXN levels in the mitochondria of patients with Friedreich's ataxia and patients could potentially experience:

•improved cellular function;

•a positive impact on Friedreich's ataxia symptoms; and

•a slowing of progression of the disease, potentially prolonging life.

In our Phase 1 clinical trials, nomlabofusp appeared to increase FXN levels in the peripheral tissues that were tested (buccal cells, skin biopsies and platelets) and data from our Phase 2 dose exploration study 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. Based on this, we believe that our technology may allow us to address other rare genetic diseases that either require the replacement of molecules that need to target specific intracellular organelles, or that share similar clinical symptoms that overlap with Friedreich's ataxia. Finally, the use of nomlabofusp to improve mitochondrial function in other rare diseases that demonstrate evidence of mitochondrial dysfunction is also being explored.

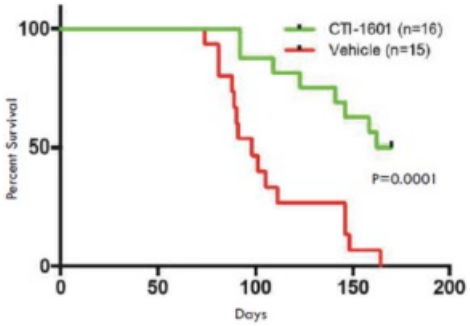
Development of Nomlabofusp

Non-clinical Development

Knock-Out Mice and Other Non-clinical Studies

Nomlabofusp has been demonstrated to prolong the life of knock-out ("KO") mice whose heart and skeletal muscles were deficient in FXN. These mice, when untreated, develop a severe hypertrophic cardiomyopathy similar to patients with Friedreich's ataxia and, like many Friedreich's ataxia patients, die early in life. In non-clinical studies of nomlabofusp, the median survival in animals treated with vehicle of 98 days was extended to a median survival of 166 days in animals treated with nomlabofusp subcutaneously three times per week (p=0.0001). Furthermore, 87.5% of mice treated with nomlabofusp survived beyond the mean age of death in the vehicle treated group (107.5 days) whereas only 33% of vehicle treated animals survived. Results are reflected in Figure 2.

Figure 2.



In a separate study conducted at an independent laboratory, a similar mouse model was studied. In this study doses of 2 mg/kg, 10 mg/kg, 30 mg/kg, 60 mg/kg and 100 mg/kg administered subcutaneously every other day were compared to vehicle. After 2 weeks of dosing, mitochondrial extracts from cardiac tissue were analyzed for the presence of human FXN. In addition, activity of succinate dehydrogenase ("SDH") an enzyme whose activity is dependent on the presence of FXN, was also analyzed. Human FXN was found in the mitochondria of the cardiomyocytes and increased with increasing dose. SDH activity which was suppressed to near zero in vehicle treated animals was also suppressed to near zero in the 2 mg/kg dose group. In the 10 mg/kg dose group activity was increased and in the 30 mg/kg dose group the activity was returned to that of wild type animals. There was no further increase in activity when the animals were dosed with 60 mg/kg or 100 mg/kg but the effect was maintained at levels equivalent to that of wild type animals. See Figures 3 and 4.

Figure 3.

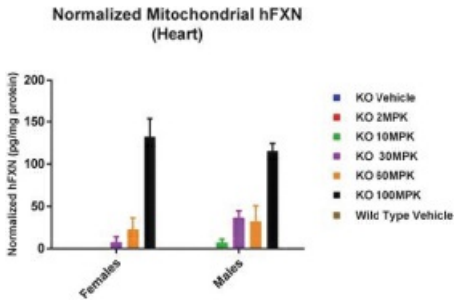
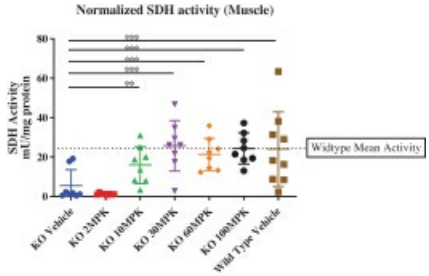


Figure 4.



Another study, also performed at an independent laboratory, demonstrated the maintenance of cardiac function when the same KO mouse model was studied. These mice were treated with nomlabofusp at doses of 10 mg/kg every other day for 6 weeks. Echocardiograms were performed prior to initiating dosing and after 4 weeks of dosing. When compared to vehicle, mice treated with nomlabofusp maintained their left ventricular volume and ejection fraction while vehicle treated mice deteriorated over the same 4-week period. See Figures 5 and 6.

Figure 5.

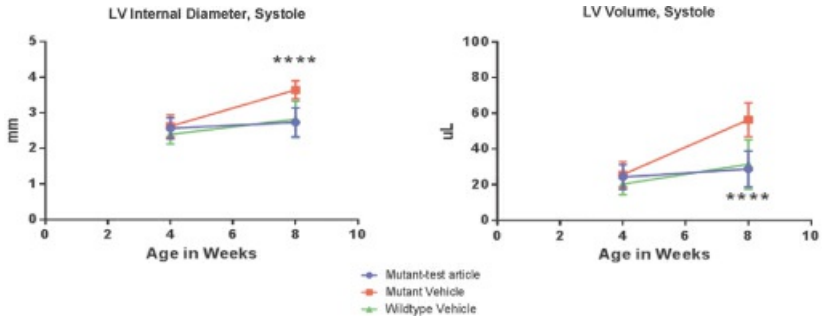
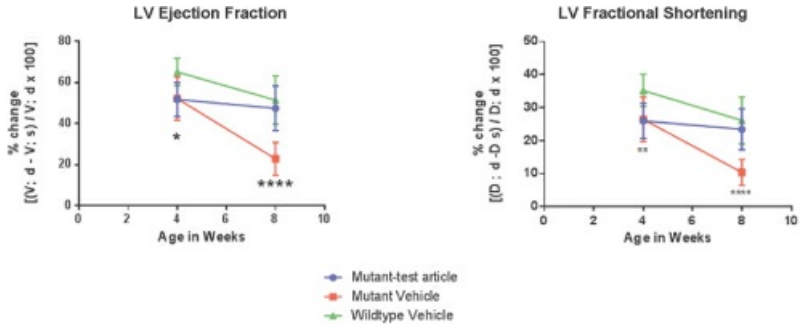


Figure 6.



Using a neurologic mouse model, treatment with nomlabofusp prevented the development of ataxia in mice whose nervous system was deficient in FXN compared to those treated with placebo.

In multiple non-clinical studies in rodents and non-human primates ("NHPs"), human FXN was found to be distributed into all tissues tested following nomlabofusp dosing. See Figure 7.

Figure 7.

Observed hFXN across all tissue and cell types tested:

✓ Brain	✓ Spinal Cord	✓ Skin
✓ Heart	✓ Cardiac Mitochondria	✓ Buccal Cells
✓ Liver	✓ CSF (Cerebrospinal Fluid)	✓ Platelets
✓ Dorsal Root Ganglia	✓ Skeletal Muscle	

Non-Human Primate and Rat Toxicology Studies

We previously conducted 28-day and 13-week GLP toxicology studies of nomlabofusp in two species, rat and NHP. In the rat studies, some injection sites showed edema and erythema with associated histologic changes localized to the injection site. The rat studies showed no significant clinical observations and no significant systemic histopathological findings. In the NHP studies, some injection sites were raised and firm with dose dependent histologic changes localized to the injection sites. The NHP 28-day study showed no systemic toxicity. The NHP 13-week study showed no systemic toxicity in the low and mid-dose groups, and minimal to mild histopathological findings in the high dose group. There were also several episodes of occasional transient muscle rigidity in some animals observed immediately after dosing in the two highest dose groups at very high exposures. These clinical observations resolved with no intervention and all of the NHPs who experienced these clinical observations received all doses and completed the in-life portion of the study.

To support extended dosing of patients with nomlabofusp, we conducted a 26-week NHP toxicology study in 2021. 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 enrolled or enrolling.

In July 2021, we completed dosing in the 26-week NHP toxicology study. The study included four 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. After additional interactions and submission of additional information requested by FDA, in September 2022, the FDA lifted its full clinical hold on the nomlabofusp clinical development program and imposed a partial clinical hold. Following additional interactions

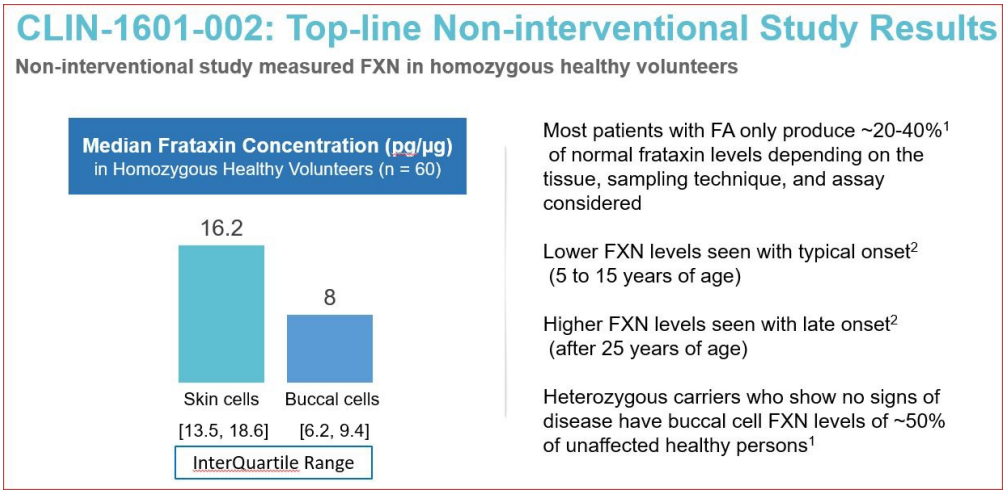
and submission of clinical data, in May 2024, the FDA removed the partial clinical hold on the development of nomlabofusp.

Clinical Development

Non-Interventional Study

In 2021, we conducted a non-interventional study that enrolled 60 healthy adults to examine the range of tissue FXN concentrations in individuals who are homozygous for the normal FXN gene. Tissue samples in these studies were collected using the same sampling techniques and proprietary assay used in the interventional studies. Enrollment in this study was completed in 2022 and data has been used for comparison purposes in the Phase 2 clinical study described below. See Figure 8.

Figure 8.



FXN concentrations were measured in skin and buccal cells from 60 homozygous healthy volunteers utilizing the same sampling technique and assay as clinical trials of nomlabofusp; FXN levels measured via detection of peptide derived from mature FXN; FXN concentrations normalized to total cellular protein content in each sample. 1. E.C. Deutsch et al. Molecular Genetics and Metabolism 101 (2010) 238–245. 2. Friedreich's Ataxia Research Alliance

Clinical Trials

Completed Phase 1 Clinical Studies in Adults with Friedreich's Ataxia

We submitted our Investigational New Drug application ("IND") application for nomlabofusp and began Phase 1 clinical trials in 2019 in patients with FA. We completed two clinical studies of nomlabofusp in adult subjects with FA, a SAD study (completed in 2020) and a MAD study (completed in 2021). The primary objective for the SAD and MAD studies was to assess the tolerability of nomlabofusp at doses ranging from 25 mg to 100 mg administered via subcutaneous injection ("SC"). The secondary objectives were to establish the PK of nomlabofusp administration in humans and to explore the PD of nomlabofusp administration by measuring tissue FXN concentrations in accessible tissues, namely buccal and skin cells, and in platelets. Administration of nomlabofusp via SC injection appeared to be well tolerated up to doses of 100 mg administered daily for up to 13 days. No SAEs were reported in either study. The most common treatment emergent adverse events ("TEAEs") were injection site reactions ("ISRs") that were generally mild, self-limited, and usually resolved within approximately 1 hour. Patients in the 25 mg cohort were dosed once a day for the first 4 days followed by 1 dose every third day through day 13 for a total of 7 doses, while the patients in the 50 mg cohort were dosed once a day for the first 7 days followed by 1 dose every other day through day 13 for a total of 10 doses, and patients in the 100 mg cohort were dosed once a day for 13 days consecutively for a total of 13 doses (see Figures 9 and 10). The study was designed in this fashion to ensure that the PK of nomlabofusp in patients with FA were characterized at the lower doses before the dose was escalated. This is typical of first in human studies such as multiple-ascending dose trials that evaluate a study drug's clinical safety profile.

There was rapid uptake of nomlabofusp into the circulation following subcutaneous injection, and exposure appeared to be proportional to dose.

Figure 9.

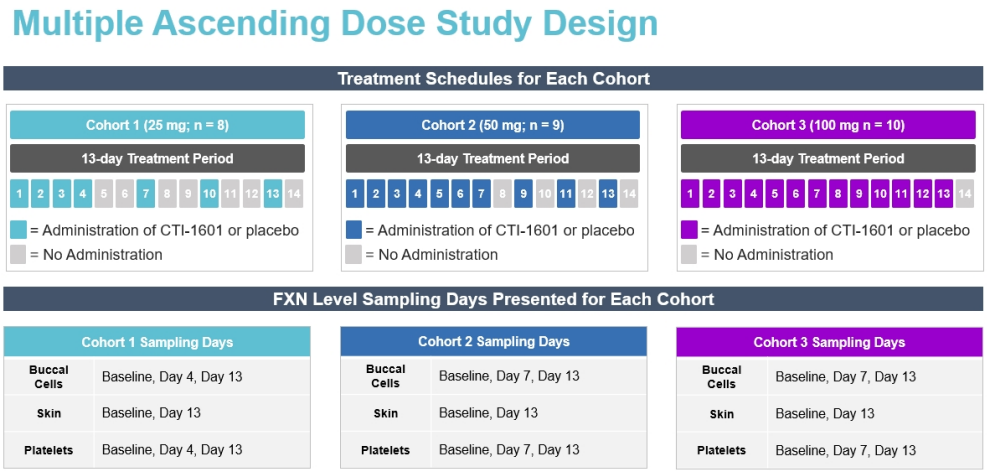
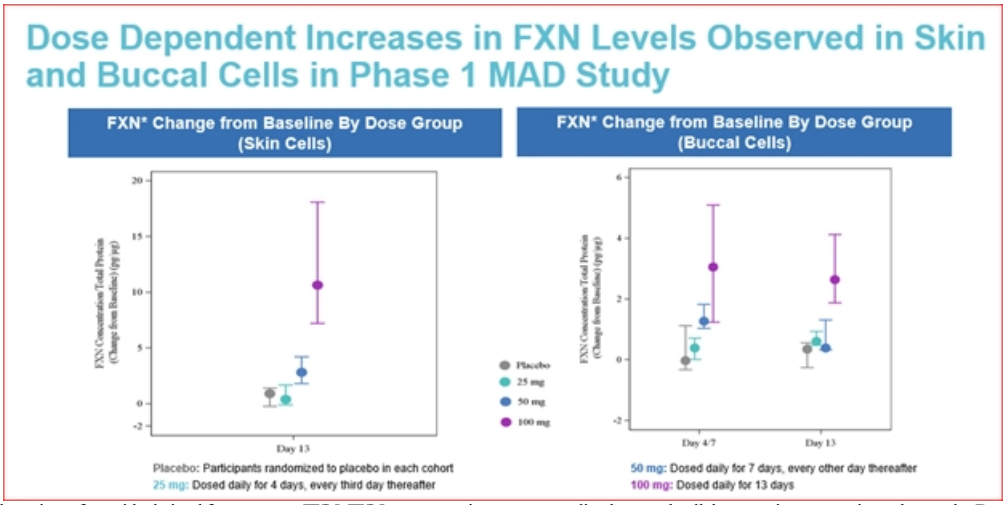


Figure 10.



*FXN levels measured via detection of peptide derived from mature FXN; FXN concentrations are normalized to total cellular protein content in each sample; Data represent median and 25th and 75th percentiles; FXN levels from day 4, & day 13 measurements are shown for data derived from the 25 mg cohort; FXN levels from day 7 & day 13 measurements are shown for data derived from the 50 & 100 mg cohorts.

Completed Phase 2 Dose Exploration Study

The Phase 2 randomized, double-blind, placebo-controlled dose exploration study evaluated the safety, tolerability, PK and PD of two doses of nomlabofusp. Cohort 1 evaluated 25 mg and Cohort 2 evaluated 50 mg. Participants in either the 25 mg or 50 mg cohorts were randomized 2:1 to receive daily subcutaneous injections of either nomlabofusp or placebo for 14 days, and then every other day for an additional 14 days for a total 28-day dosing period.

In May 2023, we reported preliminary unblinded top-line data from the 25 mg cohort of the dose exploration study. Data from the cohort indicated nomlabofusp was generally well tolerated and showed increases in FXN levels from baseline compared to placebo in all evaluated tissues (skin and buccal cells) at day 14. In July 2023, the FDA cleared initiation of the second cohort (50 mg) of the study.

In February 2024, we reported positive top-line data and successful completion of the dose exploration study. Nomlabofusp was generally well tolerated and demonstrated dose response 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.

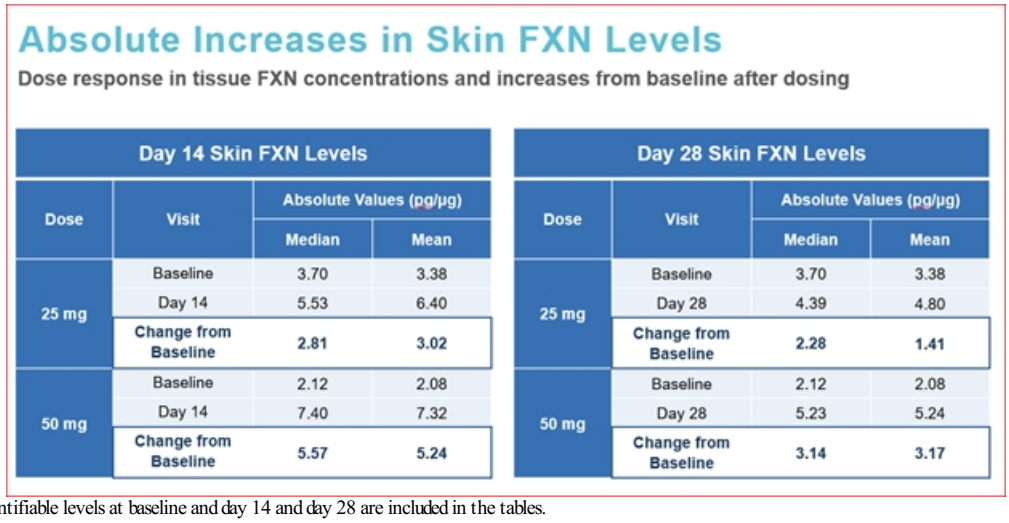
With regard to safety in the Phase 2 dose exploration trial, there were a total of 28 participants (19 received nomlabofusp and 9 received placebo). There were no serious adverse events reported. There was one allergic reaction which was considered to be a severe adverse event in the 25 mg cohort that resolved with standard treatment. This participant withdrew from the trial. The most common adverse events reported were mild and moderate injection site reactions which resolved without any intervention and there were no study discontinuations due to injection site reactions. Consistent with findings in our Phase 1 study, nomlabofusp demonstrated quick absorption after subcutaneous administration and had dose proportional increases in exposure.

Participants treated with 50 mg for 14 days daily had frataxin levels in skin cells increase from less than 17% at baseline relative to healthy volunteers, to 33% to 59% of healthy volunteers after 14 days of treatment. Patients treated with placebo showed no increase in their frataxin levels during this period. Additionally, the majority of treated patients in both cohorts with quantifiable levels of frataxin at baseline and day 14 achieve at least a 100% increase in tissue frataxin in skin cells, and at least a 30% increase in tissue frataxin levels in buccal cells. Figures 11 and 14 show a clear dose response and increase from baseline in skin tissue and buccal tissue frataxin levels, respectively.

The graph on the left in both Figures 12 and 15 shows a dose dependent increase in frataxin levels after 14 days of treatment. With both skin and buccal cells, when dosing is switched to every other day the magnitude of the increase in frataxin levels declines.

The waterfall plots on the right in both Figures 12 and 15 depict the individual changes in frataxin levels from baseline in skin cells and buccal cells at day 14 respectively. Each bar represents an individual patient, with placebo treated participants in gray, the 25 mg cohort in aqua, and the 50 mg cohort in blue. The majority of patients treated with nomlabofusp had significant increases in frataxin levels while placebo patients were basically unchanged. Importantly, there also appears to be a dose response as frataxin levels are notably increased in most of the 50 mg cohort over the 25 mg cohort.

Figure 11.



Day 14 Skin FXN Levels

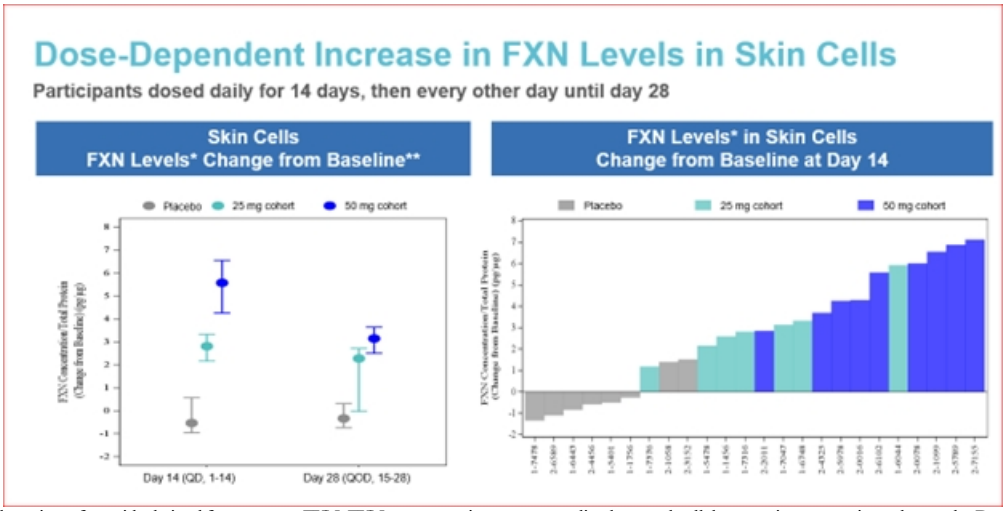
Dose	Visit	Absolute Values (pg/µg)	
		Median	Mean
25 mg	Baseline	3.70	3.38
	Day 14	5.53	6.40
	Change from Baseline	2.81	3.02
50 mg	Baseline	2.12	2.08
	Day 14	7.40	7.32
	Change from Baseline	5.57	5.24

Day 28 Skin FXN Levels

Dose	Visit	Absolute Values (pg/µg)	
		Median	Mean
25 mg	Baseline	3.70	3.38
	Day 28	4.39	4.80
	Change from Baseline	2.28	1.41
50 mg	Baseline	2.12	2.08
	Day 28	5.23	5.24
	Change from Baseline	3.14	3.17

Only participants with quantifiable levels at baseline and day 14 and day 28 are included in the tables.

Figure 12.

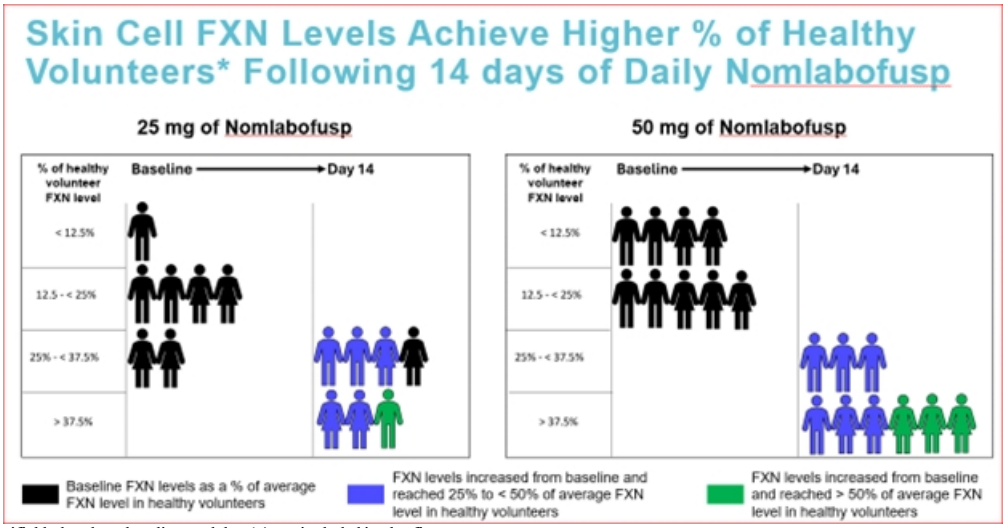


*FXN levels measured via detection of peptide derived from mature FXN; FXN concentrations are normalized to total cellular protein content in each sample. Data represent median and 25th and 75th percentiles. Only participants with quantifiable levels at both baseline and day 14 are included in the figures.
**Median baseline FXN levels in patients were 3.5 pg/µg for the placebo, 3.7 pg/µg for the 25 mg cohort and 2.1 pg/µg for the 50 mg cohort.

Figures 13 and 16 depict how many people had a shift in frataxin levels in skin cells and buccal cells as a percentage of average healthy volunteers after 14 days of treatment. On the left is the 25 mg cohort and on the right is the 50 mg cohort. On the far left of each figure are categories of the percentage of average healthy volunteers. Figures in black to the left of the black vertical line represent patients at baseline. Figures to the right of the black

vertical line represent day 14 frataxin levels as a percentage of average healthy volunteers. In blue are individuals that increase by 25% to 50% and in green are individuals that exceeded 50% of average healthy volunteers.

Figure 13.



Only participants with quantifiable levels at baseline and day 14 are included in the figures.

*% of healthy volunteer FXN level is calculated by dividing each participant's FXN level by the average FXN level (16.34 pg/µg) from the noninterventional healthy volunteer study (N=60).

Figure 14.

Absolute Increases in Buccal FXN Levels

Dose response in tissue FXN concentrations and increases from baseline after dosing

Day 14 Buccal FXN Levels

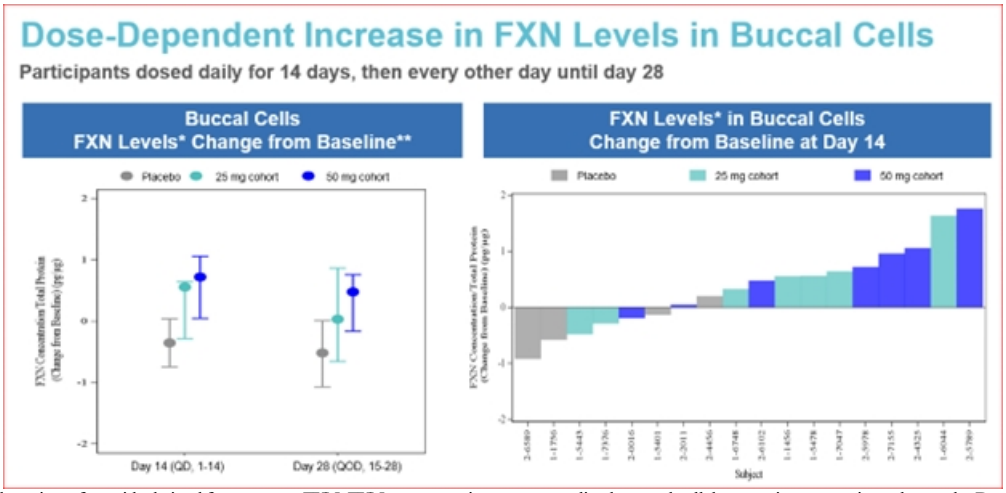
Dose	Visit	Absolute Values (pg/µg)	
		Median	Mean
25 mg	Baseline	1.78	1.80
	Day 14	2.24	2.22
	Change from Baseline	0.56	0.42
50 mg	Baseline	1.61	1.69
	Day 14	2.44	2.38
	Change from Baseline	0.72	0.69

Day 28 Buccal FXN Levels

Dose	Visit	Absolute Values (pg/µg)	
		Median	Mean
25 mg	Baseline	1.70	1.65
	Day 28	1.73	1.76
	Change from Baseline	0.03	0.11
50 mg	Baseline	1.76	1.77
	Day 28	2.15	2.15
	Change from Baseline	0.48	0.38

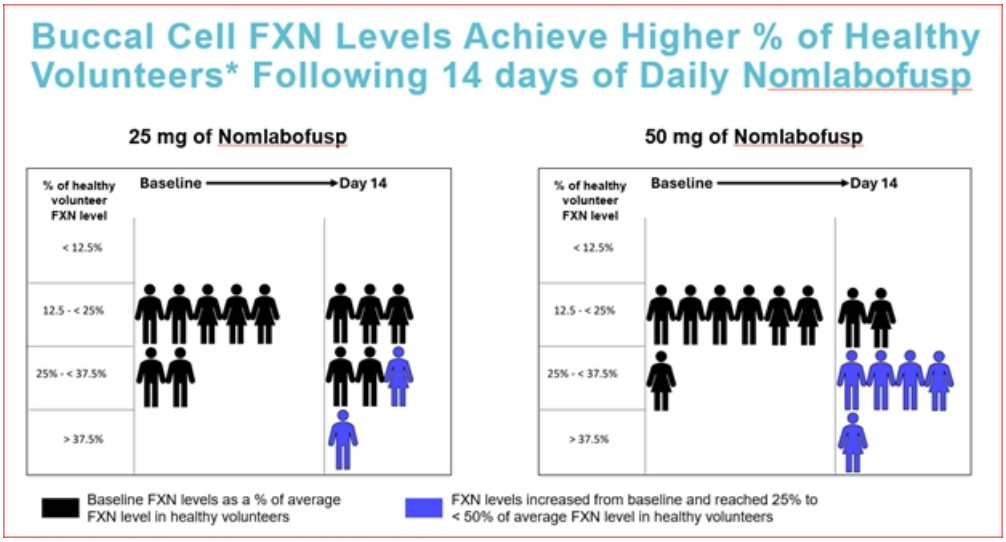
Only participants with quantifiable levels at baseline and day 14 and day 28 are included in the tables.

Figure 15.



*FXN levels measured via detection of peptide derived from mature FXN; FXN concentrations are normalized to total cellular protein content in each sample. Data represent median and 25th and 75th percentiles. Only participants with quantifiable levels at both baseline and Day 14 are included in the figures.
**Median baseline FXN level in patients were 2.1 pg/µg for the placebo, 1.8 pg/µg for the 25 mg cohort and 1.6 pg/µg for the 50 mg cohort.

Figure 16.



Only participants with quantifiable levels at baseline and day 14 are included in the figures.

*% of healthy volunteer FXN level is calculated by dividing each participant's FXN level by the average FXN level (8.24 pg/µg) from the noninterventional healthy volunteer study (N=60).

Open Label Extension

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. Participants who completed treatment in the Phase 2 dose exploration study, or who previously completed a prior clinical trial of nomlabofusp, are potentially eligible to screen for the OLE study. The OLE study will evaluate the safety and tolerability, pharmacokinetics, and frataxin levels in peripheral tissues as well as other exploratory pharmacodynamic markers (lipid profiles and gene expression data) following long-term subcutaneous administration of nomlabofusp. In addition, following the completion of enrollment, as well as at least one participant completing one year of dosing, clinical assessments collected during the study will be compared to data from a matched control arm derived from participants in the Friedreich's Ataxia Clinical Outcome Measures Study ("FACOMS") database.

In December 2024, we reported positive initial data from our ongoing OLE study. This data included safety, FXN levels, clinical, pharmacokinetic data and dose escalation:

- Nomlabofusp was generally well tolerated for up to 260 days in subjects. The most common adverse events were injection site reactions, with most being mild, brief in duration, and self-limited. Two participants had serious adverse events (one seizure and one anaphylactic reaction) that resolved within 24 hours who withdrew from the study;
- Long-term 25 mg tissue FXN levels showed positive mean change from baseline of 1.32 pg/µg in buccal cells and 9.28 pg/µg in skin cells at Day 90 and that 25 mg of nomlabofusp increased and maintained tissue FXN levels over time, increasing from a mean level of 15% of HV at baseline to 30% in buccal cells and from 16% to 72% in skin cells at Day 90;
- Tissue FXN levels appear to reach steady-state levels by Day 30 in buccal cells;
- Early clinical data showed trends toward improvement across a number of clinical outcomes for long-term 25 mg daily nomlabofusp including: (1) decreased values indicating early trends towards improvement in the Modified Friedreich Ataxia Rating Scale (mFARS) and the FARS-Activities of Daily Living ("ADL"), Modified Fatigue Impact Scale and 9 hole peg test at 90 days relative to baseline;
- Key pharmacokinetic data showed rapid absorption after subcutaneous administration with exposure appearing to reach steady state in plasma by day 30 with no further accumulation, which is consistent with data from our Phase 1 and Phase 2 studies.

Also in December 2024, we announced that we were increasing the dose in the OLE to 50 mg of nomlabofusp daily for then currently enrolled and all future OLE study participants. In March 2025, we announced that our Safety Monitoring Team has deemed anaphylaxis as an adverse drug reaction likely associated with nomlabofusp and therefore, we expect to see additional reactions. To reduce the risk of allergic reactions, including anaphylaxis, we amended the OLE protocol to administer premedication for the first month of dosing. The OLE study is ongoing with seven sites activated and participants continuing to enroll, and all study participants are currently receiving the 50 mg dose of nomlabofusp. We plan to provide an update on OLE data on at least 30 to 40 study participants, some of whom have been receiving nomlabofusp for more than a year, in September 2025.

Intellectual Property

Our success depends in large part upon our ability to obtain and maintain proprietary protection for our current and future products and technologies, and to operate without infringing the proprietary rights of others. Our policy is to protect our proprietary position by, among other methods, filing for patent applications on inventions that are important to the development and conduct of our business with the U.S. Patent and Trademark Office and its foreign counterparts. We also intend to rely on regulatory exclusivity (also called data exclusivity), which is separate and distinct from the protection afforded by patents, to protect our products. We further protect our proprietary information by requiring our employees, consultants, contractors and other advisors to execute nondisclosure and assignment of invention agreements upon commencement of their respective employment or engagement. Agreements with our employees also prevent them from bringing the proprietary rights of third parties to us. In addition, we require confidentiality or service agreements from third parties that receive our confidential information or materials.

As of March 17, 2025, our intellectual property portfolio was composed of numerous international Patent Cooperation Treaty ("PCT"), foreign, United States non-provisional patent applications, issued United States patents and United States provisional patent applications that we own or co-own, and five issued United States patents and additional non-provisional patent applications in the United States and in certain foreign jurisdictions that we license from academic institutions. The issued patents in the United States licensed by us, which include issued patents covering the composition of matter for nomlabofusp and methods for treating FA, have expiration dates between 2025 and 2040 without taking potential patent term extension into consideration. The international and the United States non-provisional patent applications licensed by us relate to composition of matter and methods of use for nomlabofusp.

The additional patent applications we own or co-own, which include PCT, foreign and United States non-provisional patent applications, United States patents, and United States provisional patent applications are related to the development of nomlabofusp, including methods of use of nomlabofusp, and to our peptide-delivery platform technology.

A provisional patent application allows for an effective filing date to be established with regard to an invention, but once a provisional patent application is filed, either a corresponding non-provisional patent application or a petition to convert the provisional patent application into a non-provisional patent application must be filed within 12 months or such effective filing date will be lost. If we or our licensor timely files non-provisional patent applications in the United States and in countries outside of the United States with regard to our provisional patent applications and these non-provisional patent applications result in issued patents, such patents are expected to expire in 2045, without taking potential patent term adjustment or patent term extension into consideration.

Nomlabofusp is covered by licensed issued patents (composition of matter and methods of use) in the United States which, if properly maintained, will expire in 2025 and 2040, excluding any patent term extensions that might be available following the grant of marketing authorizations. We also possess an exclusive license to non-provisional applications in the United States and certain countries outside the United States for nomlabofusp (composition of matter and methods of use). If these patent applications in the United States and other countries result in issued patents, those patents would be expected to expire in 2040. This estimated expiration excludes any patent term adjustment that might be available following the grant of the patent and any patent term extensions that might be available following the grant of marketing authorizations. 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.

Patents extend for varying periods according to the date of patent filing or grant and the legal term of patents in various countries where patent protection is obtained. The actual protection afforded by a patent, which can vary from country to country, depends on the type of patent, the scope of its coverage and the availability of legal remedies in the country.

We also use other forms of protection besides patent protection and regulatory exclusivity, such as trademark, copyright, and trade secret protection, to enhance our intellectual property, particularly where we do not believe patent protection is appropriate or obtainable. We aim to take advantage of all of the intellectual property rights that are available to us and believe that this comprehensive approach will provide us with proprietary exclusive positions for our product candidates, such as nomlabofusp, where available.

In-License Agreements

We are party to a License Agreement dated November 30, 2016 with Wake Forest University Health Sciences ("WFUHS") and a License Agreement dated November 30, 2016, as amended, with Indiana University ("IU"). Such agreements provide for a transferable, worldwide license to certain patent rights regarding technology used by us with respect to the development of nomlabofusp.

In partial consideration for the right and license granted under these agreements, we will pay each of WFUHS and IU a royalty of a low single digit percentage of net sales of licensed products depending on whether there is a valid patent covering such products. As additional consideration for these agreements, we are obligated to pay each of WFUHS and IU certain milestone payments of up to $2.6 million in the aggregate upon the achievement of certain developmental milestones, commencing on the enrollment of the first patient in a Phase 1 clinical trial. We will also pay each of WFUHS and IU sublicensing fees ranging up to a low double-digit percentage of sublicense consideration depending on our achievement of certain regulatory milestones as of the time of receipt of the sublicense consideration. We are also obligated to reimburse WFUHS and IU for patent-related expenses. In the

event that we dispute the validity of any of the licensed patents, the royalty rate would be tripled during such dispute. We are also obligated to pay to IU a minimum annual royalty of less than $0.1 million per annum starting in the 2020 calendar year for the term of the agreement.

In the event that we are required to pay IU consideration, then we may deduct 20% of such IU consideration on a dollar-for-dollar basis from the consideration due to WFUHS. In the event that we are required to pay WFUHS consideration, then we may deduct 60% of such WFUHS consideration on a dollar-for-dollar basis from the consideration due to IU.

Competition

The biopharmaceutical industry is characterized by intense and dynamic competition to develop new technologies and proprietary therapies. Any product candidates that we successfully develop into products and commercialize may compete with existing therapies and new therapies that may become available in the future. While we believe that our platform technology, product candidates and scientific expertise in the field of rare diseases provide competitive advantages, we face competition from various sources, including larger and better-funded pharmaceutical companies, specialty pharmaceutical companies and biotechnology companies, as well as from academic institutions, governmental agencies and public and private research institutions. We expect to compete with Biogen's SKYCLARYS™ (omaveloxolone), which 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. Other competitors currently developing therapeutics to treat FA include, but are not limited to Design Therapeutics, Lexeo Therapeutics, Neurocrine Biosciences/Voyager Therapeutics and PTC Therapeutics ("PTC"). The FDA has accepted for filing PTC's NDA for vatiquinone for the treatment of children and adults living with FA with a target action date of August 19, 2025. Many of our competitors have significantly greater name recognition, longer operating histories and financial, technical manufacturing, marketing and human resources than we do. Mergers and acquisitions in the pharmaceutical and biotechnology industries may result in even more resources being concentrated amongst a smaller number of our competitors. Our commercial opportunity could be reduced or eliminated if our competitors develop or market products or other novel therapies that are more effective, safer or less costly than our product candidates or obtain regulatory approval for their products more rapidly than we may obtain approval for our product candidates.

Manufacturing and Supply

Nomlabofusp is a biologic fusion protein that is produced in E. coli. We have worked with contract manufacturers and research organizations to develop, in accordance with current Good Manufacturing Practices ("cGMP") a manufacturing process and analytical methods for drug substance and drug product to support clinical trials. We also use third party manufacturers for the clinical packaging, storage and distribution of nomlabofusp. We rely on third parties to store the nomlabofusp master cell bank and working cell bank, each stored at a different location. We continue to advance the manufacturing of nomlabofusp, obtain stability data, and produce drug product for future clinical trials. We are continually trying to optimize our manufacturing process to increase yields, decrease costs and increase reliability in supply chains. The final process will need to be successfully scaled up to support commercial manufacturing.

The drug substance which is in frozen liquid form for nomlabofusp is currently manufactured for us by KBI Biopharma, Inc. ("KBI"). We are party to a Master Services Agreement, as amended, with KBI, pursuant to which KBI provides biological development and clinical manufacturing services with respect to nomlabofusp. We are no longer producing the frozen liquid form of drug product. We are producing a lyophilized version of the drug product from the same KBI drug substance at Bora Pharmaceutical Services, Inc. ("Bora"). We are party to a Master Services Agreement with Bora, pursuant to which Bora provides biological development and clinical manufacturing services with respect to nomlabofusp. In February 2025, FDA accepted the data supporting the comparability of the lyophilized drug product to the frozen solution and agreed with our plans to introduce the lyophilized product into our clinical development program in mid-2025. The frozen solution is the dosage form currently being used in the OLE. The lyophilized drug product is the formulation that we intend to commercialize.

To supply nomlabofusp drug substance commercially, we intend to scale up the manufacturing process. We have selected Lonza to support this scale-up effort. We are currently tech transferring the process to Lonza and are making adaptations to the process that enable facility fit. We are party to a Master Services Agreement with Lonza, pursuant to which Lonza provides biological development and clinical manufacturing services with respect to nomlabofusp. We intend to demonstrate the comparability of the drug substance manufactured by Lonza and submit

this data to the FDA for their review in 2026. Acceptance of this comparability will allow for the use of drug substance manufactured by Lonza in the clinical development of nomlabofusp.

We have received concurrence from the FDA that our proposed matrixed potency control strategy appears reasonable, pending review of the BLA. We are in the process of implementing this assay at a third-party testing lab and intend to provide a comprehensive data package that supports our ongoing clinical and commercial development. As development of this assay is scientific in nature, efforts may prove to be unsuccessful and will require further discussions with FDA on an appropriate strategy.

Human Capital Resources

Employees and Compensation

In order to achieve the goals and expectations of our Company, it is crucial that we continue to attract and retain top talent. To facilitate talent attraction and retention, we strive to make our company a safe and rewarding workplace, with opportunities for our employees to grow and develop in their careers, supported by strong compensation, benefits, paid time-off and health and wellness programs, including programs that build connections between our employees.

As of December 31, 2024, we employed 65 full-time employees in the United States, of which 48 are directly engaged in research and development with the rest providing administrative, business, commercial and operations support. None of our employees are represented by a labor organization or under any collective-bargaining arrangements. We consider our employee relations to be good.

Our human capital resources objectives include, as applicable, identifying, recruiting, retaining, incentivizing and integrating our existing and new employees, advisors and consultants. The principal purposes of our equity and cash incentive plans are to attract, retain and reward personnel through the granting of stock-based and cash-based compensation awards, in order to increase stockholder value and the success of our company by motivating such individuals to perform to the best of their abilities and achieve our objectives.

Scientific Advisors

We have established a scientific advisory board and we regularly seek advice and input from these experienced thought leaders on matters related to our research and development programs. The members of our scientific advisory board consist of distinguished research scientists, professors and industry experts recognized as key opinion leaders in the fields of rare disease, pediatrics and mitochondrial disease. Their scientific perspectives will be invaluable to determine our strategic scientific pathway and support the development of other potential treatments for complex rare diseases to help fill unmet medical needs in this space. We intend to continue to leverage the broad expertise of our advisors by seeking their counsel on important topics relating to our product development and clinical development programs. Our scientific advisors are not our employees and may have commitments to, or consulting or advisory contracts with, other entities that may limit their availability to us. In addition, our scientific advisors may have arrangements with other companies to assist those companies in developing products or technologies that may compete with ours. All of our scientific advisors are affiliated with other entities and devote only a small portion of their time to us.

Our scientific advisors are set forth in the table below:

Name	Title
Marni Joy Falk, MD	Executive Director, Mitochondrial Medicine Frontier Program at Children's Hospital of Philadelphia; Professor, Department of Human Genetics and Department of Pediatrics, University of Pennsylvania Perelman School of Medicine
Giovanni Manfredi, MD, PhD	Finbar and Marianne Kenny Professor of Clinical and Research Neurology, Weill Cornell Medicine; Professor of Neuroscience, Weil Cornell Medicine
Jill Ostrem, MD	Medical director and division chief of University of California San Francisco (UCSF) Movement Disorders and Neuromodulation Center. Carlin and Ellen Wiegner Endowed Professor of Neurology.
Mark Payne, MD	Professor of Pediatrics, Indiana University School of Medicine

Facilities

We lease office and laboratory space, which consists of approximately 8,000 square feet and 4,000 square feet located in Bala Cynwyd, PA and King of Prussia, PA, respectively. The office lease currently expires on August 31, 2026 and the lab lease expires on May 9, 2028.

As part of the Merger (as defined below) with Zafgen, Inc ("Zafgen"), we acquired a non-cancellable operating lease for approximately 17,705 square feet of office space at 3 Center Plaza, Boston, Massachusetts (the "Boston Lease"). The Boston Lease expires on October 30, 2029. On October 27, 2020, we entered into a sublease agreement whereby we subleased all 17,705 square feet of office space leased under the Boston Lease until October 30, 2029.

Legal Proceedings

From time to time, we may become involved in legal proceedings arising in the ordinary course of our business. We are not presently a party to any legal proceedings that, if determined adversely to us, would individually or taken together have a material adverse effect on us.

Corporate Information

We were founded in 2005 as a Delaware corporation under the name Zafgen, Inc. ("Zafgen"). On May 28, 2020, Zafgen completed a reverse merger with Chondrial Therapeutics, Inc. ("Chondrial") in accordance with the terms of the Agreement and Plan of Merger and Reorganization, dated December 17, 2019, by and among Zafgen, Zordich Merger Sub, Inc., Chondrial and Chondrial Therapeutics Holdings, LLC, pursuant to which Zordich Merger Sub, Inc. merged with and into Chondrial, with Chondrial surviving as our wholly owned subsidiary. Following completion of the merger, Zafgen, Inc. changed its name to Larimar Therapeutics, Inc.

Our principal executive offices are located at Three Bala Plaza East, Suite 506, Bala Cynwyd, PA 19004, and our telephone number is (844) 511-9056. Our website address is www.larimartx.com. The information on, or that can be accessed through, our website is not part of this Annual Report on Form 10-K and is not incorporated by reference herein. We have included our website address as an inactive textual reference only.

Available Information

We file annual, quarterly, and current reports, proxy statements, and other documents with the Securities and Exchange Commission ("the SEC") under the Securities Exchange Act of 1934, as amended ("the Exchange Act"). The SEC maintains an internet website, www.sec.gov, that contains reports, proxy and information statements, and other information regarding issuers, including us, that file electronically with the SEC.

Copies of each of our filings with the SEC on Form 10-K, Form 10-Q and Form 8-K and all amendments to those reports, can be viewed and downloaded free of charge at our website, www.larimartx.com as soon as reasonably practicable after the reports and amendments are electronically filed with or furnished to the SEC.

Our code of ethics, other corporate policies and procedures, and the charters of our Audit Committee, Compensation Committee and Nominating and Corporate Governance Committee are available through our website at www.larimartx.com. We intend to post information regarding any amendments to, or waivers from, our code of ethics on our website.

Government Regulation

U.S. Government Regulation

In the United States, drug and biologic products are subject to extensive regulation by the FDA. The Federal Food, Drug, and Cosmetic Act (“FDCA”) and other federal and state statutes and regulations, govern, among other things, the research, development, testing, manufacture, storage, record keeping, approval, labeling, promotion and marketing, distribution, post-approval monitoring and reporting, sampling, and import and export of pharmaceutical products. Biological products used for the prevention, treatment, or cure of a disease or condition of a human being are subject to regulation under the FDCA, with the exception that the section of the FDCA that governs the approval of drugs via new drug applications (“NDAs”), does not apply to the approval of biologics. In contrast, biologics are approved for marketing under provisions of the Public Health Service Act (“PHS Act”), via a Biologics License Application (“BLA”). However, the application process and requirements for approval of BLAs are very similar to those for NDAs. FDA approval or licensure also must be obtained before the marketing of drug and biological products. The process of obtaining regulatory approvals and the subsequent compliance with appropriate federal, state, local and foreign statutes and regulations require the expenditure of substantial time and financial resources.

Development Process

The process required by the FDA before a drug or biologic product may be marketed in the United States generally involves the following:

•completion of non-clinical laboratory tests and animal studies according to Good Laboratory Practices ("GLPs"), and applicable requirements for the humane use of laboratory animals or other applicable regulations;

•preparation of clinical trial material in accordance with cGMPs;

•submission to the FDA of an IND, which must become effective before human clinical trials may begin;

•approval by an institutional review board ("IRB") reviewing each clinical site before each clinical trial may be initiated;

•performance of adequate and well-controlled human clinical trials according to Good Clinical Practices ("GCPs") and any additional requirements for the protection of human research subjects and their health information, to establish the safety, purity, potency, and efficacy, of the proposed drug or biological product for its intended use;

•submission to the FDA of an NDA or BLA for marketing approval that includes evidence of safety, purity, potency, and efficacy from results of non-clinical testing and clinical trials;

•satisfactory completion of an FDA inspection prior to NDA or BLA approval of the manufacturing facility or facilities where the drug or biological product is produced to assess compliance with cGMPs, to assure that the facilities, methods and controls are adequate to preserve the biological product's identity, strength, quality and purity;

•potential FDA audit of the non-clinical and clinical study sites that generated the data in support of the NDA or BLA;

•potential FDA Advisory Committee meeting to elicit expert input on critical issues and including a vote by external committee members;

•FDA review and approval, or licensure, of the NDA or BLA, and payment of associated user fees, when applicable; and

•compliance with any post approval requirements, including the potential requirement to implement a Risk Evaluation and Mitigation Strategies ("REMS") and the potential requirement to conduct post approval studies.

Before testing any drug or biological product candidate in humans, the product candidate enters the non-clinical testing stage. Non-clinical tests include laboratory evaluations of product chemistry, pharmacology, toxicity and formulation, as well as animal studies to assess the potential safety and activity of the product candidate. The conduct of the non-clinical tests must comply with federal regulations and requirements including GLPs.

The clinical study sponsor must submit the results of the non-clinical tests, together with manufacturing information, analytical data, any available clinical data or literature and a proposed clinical protocol, to the FDA as part of the IND. Some non-clinical testing typically continues after the IND is submitted. An IND is an exemption from the FDCA that allows an unapproved product to be shipped in interstate commerce for use in an investigational clinical trial and a request for FDA authorization to administer an investigational product to humans. The IND automatically becomes effective 30 days after receipt by the FDA, unless the FDA places the clinical trial on a clinical hold within that 30-day time period. In such a case, the IND sponsor must resolve any deficiencies to the satisfaction of the FDA, and the FDA must lift the clinical hold before the clinical trial can begin. The FDA may also impose clinical holds on a drug or biological product candidate at any time before or during clinical trials due to safety concerns or non-compliance. If the FDA imposes a clinical hold, trials may not re-commence without FDA authorization and then only under terms authorized by the FDA.

Clinical trials may involve the administration of the drug or biological product candidate to healthy volunteers or subjects under the supervision of qualified investigators, generally physicians not employed by or under the study sponsor's control. Clinical trials involving some products for certain diseases, including some rare diseases may

begin with testing in patients with the disease. Clinical trials are conducted under protocols detailing, among other things, the objectives of the clinical trial, dosing procedures, subject selection and exclusion criteria, and the parameters to be used to monitor subject safety, including stopping rules that assure a clinical trial will be stopped if certain adverse events should occur. Each protocol and any amendments to the protocol must be submitted to the FDA as part of the IND application. Clinical trials must be conducted and monitored in accordance with the FDA's regulations comprising the GCP requirements, including the requirement that all research subjects or his or her legal representative provide informed consent. Further, each clinical trial must be reviewed and approved by an independent IRB, at or servicing each institution at which the clinical trial will be conducted. An IRB is charged with protecting the welfare and rights of study participants and considers such items as whether the risks to individuals participating in the clinical trials are minimized and are reasonable in relation to anticipated benefits. The IRB also approves the form and content of the informed consent that must be signed by each clinical trial subject or his or her legal representative and must monitor the clinical trial until completed. Additionally, some trials are overseen by an independent group of qualified experts organized by the trial sponsor, known as a data monitoring committee. The data monitoring committee may review safety data and/or efficacy data.

Human clinical trials are typically conducted in three sequential phases that may overlap or be combined:

•***Phase 1***. The drug or biological product is initially introduced into healthy human subjects and tested for safety. In the case of some products for rare diseases, the initial human testing is often conducted in patients.

•***Phase 2***. The drug or biological product is evaluated in a limited patient population to identify possible adverse effects and safety risks, to preliminarily evaluate the efficacy of the product for specific targeted diseases and to determine dosage tolerance, optimal dosage and dosing schedule.

•***Phase 3***. Clinical trials are undertaken to further evaluate dosage, clinical efficacy, potency and safety in an expanded patient population at geographically dispersed clinical trial sites. These clinical trials are intended to establish the overall risk/benefit ratio of the product and provide an adequate basis for product labeling. In most cases, the FDA requires two adequate and well-controlled Phase 3 clinical trials to demonstrate the safety and efficacy of the drug or biologic. In rare instances, a single Phase 3 trial may be sufficient when either (1) the trial is a large, multicenter trial demonstrating internal consistency and a statistically very persuasive finding of a clinically meaningful effect on mortality, irreversible morbidity or prevention of a disease with a potentially serious outcome and confirmation of the result in a second trial would be practically or ethically impossible, or (2) the single trial is supported by confirmatory evidence. In drugs and biologics for rare diseases where patient populations are small, Phase 3 trials might not be required if substantial evidence of effectiveness and an acceptable risk/benefit profile can be demonstrated from Phase 2 trials.

An OLE study may also be conducted. An OLE study typically enrolls participants from previous clinical trials and is designed to gather the long-term safety and tolerability data on a potential new medicine beyond the time period of the original studies.

Post-approval clinical trials, sometimes referred to as Phase 4 clinical trials, may be conducted after initial marketing approval. These clinical trials are used to gain additional experience from the treatment of patients in the intended therapeutic indication in real-world scenarios. They provide additional information regarding risks, benefits and best use, including longer term safety data. In certain instances, the FDA may mandate the performance of Phase 4 clinical trials as a condition of approval of an NDA or BLA.

During all phases of clinical development, regulatory agencies require extensive monitoring and auditing of all clinical activities, clinical data, and clinical trial investigators. Annual progress reports detailing the results of the clinical trials must be submitted to the FDA. Written IND safety reports must be promptly submitted to the FDA and the investigators for serious and unexpected adverse events, any findings from other studies, tests in laboratory animals or in vitro testing that suggest a significant risk for human subjects, or any clinically important increase in the rate of a serious suspected adverse reactions over that listed in the protocol or investigator brochure. The sponsor must submit an IND safety report within 15 calendar days after the sponsor determines that the information qualifies for reporting. The sponsor also must notify the FDA of any unexpected fatal or life-threatening suspected adverse reaction within seven calendar days after the sponsor's initial receipt of the information. The FDA or the sponsor or its data safety monitoring board may suspend a clinical trial at any time on various grounds, including a finding that the research subjects or patients are being exposed to an unacceptable health risk. Similarly, an IRB can suspend or

terminate approval of a clinical trial at its institution if the clinical trial is not being conducted in accordance with the IRB's requirements or if the drug or biological product has been associated with unexpected serious harm to patients.

Concurrent with clinical trials, companies usually complete additional animal studies and must also develop additional information about the physical characteristics of the drug or biological product as well as finalize a process for manufacturing the product in commercial quantities in accordance with cGMP requirements. To help reduce the risk of the introduction of adventitious agents with use of biological products, the PHS Act emphasizes the importance of manufacturing control for products whose attributes cannot be precisely defined. The manufacturing process must be capable of consistently producing quality batches of the product candidate and, among other things, the sponsor must develop methods for testing the identity, strength, quality potency and purity of the final biological product. Additionally, appropriate packaging must be selected and tested, and stability studies must be conducted to demonstrate that the drug or biological product candidate does not undergo unacceptable deterioration over its shelf life.

There are also various laws and regulations regarding laboratory practices, the experimental use of animals, and the use and disposal of hazardous or potentially hazardous substances in connection with the research. In each of these areas, the FDA and other regulatory authorities have broad regulatory and enforcement powers, including the ability to levy fines and civil penalties, suspend or delay issuance of approvals, seize or recall products, and withdraw approvals.

Information about certain clinical trials must be submitted within specific timeframes to the NIH for public dissemination on its clinicaltrials.gov website. Sponsors or distributors of investigational products for the diagnosis, monitoring, or treatment of one or more serious diseases or conditions must also have a publicly available policy on evaluating and responding to requests for expanded access requests.

Review and Approval Processes

After the completion of clinical trials of a drug or biological product, FDA approval of an NDA, or BLA, must be obtained before commercial marketing of the product. The NDA or BLA must include results of product development, laboratory and animal studies, human studies, information on the manufacture and composition of the product, proposed labeling and other relevant information.

Under the Prescription Drug User Fee Act, as amended ("PDUFA"), each NDA or BLA may be accompanied by a significant application fee. The applicant for an approved application is also subject to an annual program fee. Fee waivers or reductions are available in certain circumstances, including a waiver of the application fee for the first application filed by a small business. Additionally, no user fees are assessed on NDAs or BLAs for product candidates designated as orphan drugs, unless the product candidate also includes a non-orphan indication.

Within 60 days following submission of the application, the FDA reviews an NDA or BLA submitted to determine if it is substantially complete before the agency files it. The FDA may refuse to file any NDA or BLA that it deems incomplete or not properly reviewable at the time of submission and may request additional information. In this event, the NDA or BLA must be resubmitted with the additional information. The resubmitted application is also subject to review before the FDA files it. Once the submission is filed, the FDA begins an in-depth substantive review of the NDA or BLA. The FDA reviews the NDA or BLA to determine, among other things, whether the proposed product is safe and effective (for drugs) or safe, potent, and effective (for biologics), for its intended use, and has an acceptable purity profile, and whether the product is being manufactured in accordance with cGMPs to assure and preserve the product's identity, safety, strength, quality, potency and purity. One of the performance goals agreed to by the FDA under the PDUFA is to review standard NDAs or BLAs in ten months from filing and priority NDAs or BLAs in six months from filing, whereupon a review decision is to be made. The review process may be extended by three months if the FDA classifies a response to an FDA request for additional information or clarification as a major amendment.

The FDA may refer applications for novel products or products that present difficult questions of safety or efficacy to an advisory committee, typically a panel that includes clinicians and other experts, for review, evaluation and a recommendation as to whether the application should be approved and under what conditions. The FDA is not bound by the recommendations of an advisory committee, but it considers such recommendations carefully when making decisions. During the review, the FDA will also determine whether a REMS is necessary to assure the safe use of the biological product. If the FDA concludes a REMS is needed, the sponsor of the NDA or BLA must submit a proposed REMS; the FDA will not approve the NDA or BLA without a REMS, if required.

Before approving an NDA or BLA, the FDA may inspect the facilities at which the product is manufactured. The FDA will not approve the product unless it determines that the manufacturing processes and facilities are in compliance with cGMP requirements and adequate to assure consistent production of the product within required specifications. Additionally, before approving an NDA or BLA, the FDA will typically inspect one or more clinical trial sites to assure that the clinical trials were conducted in compliance with IND study requirements and GCP requirements. To assure cGMP and GCP compliance, an applicant must incur significant expenditure of time, money and effort in the areas of training, record keeping, production, and quality control.

Notwithstanding the submission of relevant data and information, the FDA may ultimately decide that the NDA or BLA does not satisfy its regulatory criteria for approval and deny approval. Data obtained from clinical trials are not always conclusive and the FDA may interpret data differently than the sponsor interprets the same data. If the agency decides not to approve the NDA or BLA in its present form, the FDA will issue a complete response letter that usually describes all of the specific deficiencies in the NDA or BLA identified by the FDA. The deficiencies identified may be minor, for example, requiring labeling changes, or major, for example, requiring additional clinical trials. Additionally, the complete response letter may include recommended actions that the applicant might take to place the application in a condition for approval. If a complete response letter is issued, the applicant may either resubmit the NDA or BLA, addressing all of the deficiencies identified in the letter, or withdraw the application.

If a product receives regulatory approval, the approval may be significantly limited to specific diseases and dosages or the indications for use may otherwise be limited, which could restrict the commercial value of the product. Further, the FDA may require that certain contraindications, warnings or precautions be included in the product labeling. The FDA may impose restrictions and conditions on product distribution, prescribing, or dispensing in the form of a risk management plan, or otherwise limit the scope of any approval. In addition, the FDA may require post marketing clinical trials, sometimes referred to as Phase 4 clinical trials, designed to further assess a drug or biological product's safety and effectiveness, and testing and surveillance programs to monitor the safety of approved products that have been commercialized. As a condition for approval, the FDA may also require additional non-clinical testing as a Phase 4 commitment.

Post-Approval Requirements

Maintaining substantial compliance with applicable federal, state, and local statutes and regulations requires the expenditure of substantial time and financial resources. Rigorous and extensive FDA regulation of drug and biological products continues after approval, particularly with respect to cGMP. We will rely, and expect to continue to rely, on third parties for the production of clinical and commercial quantities of any products that we may commercialize. Manufacturers of our products are required to comply with applicable requirements in the cGMP regulations, including quality control and quality assurance and maintenance of records and documentation.

Following approval, the manufacturing facilities are subject to periodic inspections by the FDA and such inspections may result in an issuance of FDA Form 483 deficiency observations, untitled letter, or a warning letter, which can lead to plant shutdown and other more serious penalties and fines. Prior to the institution of any manufacturing changes, a determination needs to be made whether FDA approval is required in advance. If not done in accordance with FDA expectations, the FDA may restrict supply and may take further action. Annual product reports are required to be submitted annually. Other post-approval requirements applicable to drug and biological products, include reporting of cGMP deviations that may affect the identity, potency, purity and overall safety of a distributed product, product tracing requirements, registration and listing, record-keeping requirements, reporting of adverse events, reporting updated safety and efficacy information, and complying with electronic record and signature requirements.

After an NDA or BLA is approved, the product may also be subject to official lot release. As part of the manufacturing process, the manufacturer is required to perform certain tests on each lot of the product before it is released for distribution. If the product is subject to official release by the FDA, the manufacturer submits samples of each lot of product to the FDA together with a release protocol showing a summary of the history of manufacture of the lot and the results of all of the manufacturer's tests performed on the lot. The FDA may also perform certain confirmatory tests on lots of some products, before releasing the lots for distribution by the manufacturer. In addition, the FDA may conduct laboratory research related to the regulatory standards on the safety, purity, potency, and effectiveness of drug and biological products. Systems need to be put in place to record and evaluate adverse events reported by health care providers and patients and to assess product complaints. An increase in severity or new adverse events can result in labeling changes or product recall. Defects in manufacturing of commercial products can result in product recalls.

Manufacturers must also comply with the FDA's advertising and promotion requirements, such as those related to direct-to-consumer advertising, the prohibition on promoting products for uses or in patient populations that are not described in the product's approved labeling (known as "off-label use"), industry-sponsored scientific and educational activities, and promotional activities involving the internet. Discovery of previously unknown problems or the failure to comply with the applicable regulatory requirements may result in restrictions on the marketing of a product or withdrawal of the product from the market as well as possible civil or criminal sanctions. Failure to comply with the applicable U.S. requirements at any time during the product development process, approval process or after approval, may subject an applicant or manufacturer to administrative or judicial civil or criminal sanctions and adverse publicity. FDA sanctions could include refusal to approve pending applications, withdrawal of an approval or license revocation, clinical hold, warning or untitled letters, product recalls, product seizures, total or partial suspension of production or distribution, injunctions, fines, refusals of government contracts, mandated corrective advertising or communications with doctors, debarment, restitution, disgorgement of profits, or civil or criminal penalties. Any agency or judicial enforcement action could have a material adverse effect.

Drug and biologic product manufacturers and other entities involved in the manufacture and distribution of approved drug and biological products are required to register their establishments with the FDA and certain state agencies and are subject to periodic unannounced inspections by the FDA and certain state agencies for compliance with cGMPs and other laws. Accordingly, manufacturers must continue to expend time, money, and effort in the areas of production and quality control to maintain cGMP compliance. Discovery of problems with a product after approval may result in restrictions on a product, manufacturer, or holder of an approved NDA or BLA, including withdrawal of the product from the market. In addition, changes to the manufacturing process or facility generally require prior FDA approval before being implemented and other types of changes to the approved product, such as adding new indications and additional labeling claims, are also subject to further FDA review and approval.

Orphan Drug Designation

Under the Orphan Drug Act, the FDA may grant Orphan Drug Designation ("ODD") to a drug or biologic product intended to treat a rare disease or condition, which is generally a disease or condition that affects fewer than 200,000 individuals in the United States, or more than 200,000 individuals in the United States and for which there is no reasonable expectation that the cost of developing and making a drug or biologic product available in the United States for this type of disease or condition will be recovered from sales of the product. ODD must be requested before submitting an NDA or BLA. After the FDA grants ODD, the identity of the therapeutic agent and its potential orphan use are disclosed publicly by the FDA. ODD does not convey any advantage in or shorten the duration of the regulatory review and approval process.

If a product that has ODD receives the first FDA approval for the disease or condition for which it has such designation, the product is entitled to orphan product exclusivity, which means that the FDA may not approve any other applications to market the same drug or biological product for the same disease or condition for seven years, except in limited circumstances, such as showing clinical superiority to the product with orphan exclusivity by means of greater effectiveness, greater safety or providing a major contribution to patient care or in instances of drug supply issues. Competitors, however, may receive approval of either a different product for the same disease or condition or the same product for a different disease or condition but that could be used off-label in the orphan indication. Orphan drug exclusivity also could block the approval of a product for seven years if a competitor obtains approval first for the same product as defined by the FDA for the same disease or condition, or if the second product is determined to be encompassed within the competitor's scope of product for the same disease. If a company pursues marketing approval for an indication broader than the orphan drug designation it has received, it may not be entitled to orphan drug exclusivity.

Expedited Review and Approval Programs

The FDA has various programs, including fast track designation, priority review, accelerated approval, and breakthrough therapy designation, that are intended to expedite or simplify the process for the development and FDA review of drug and biological products that are intended for the treatment of serious or life-threatening diseases or conditions and demonstrate the potential to address unmet medical needs. The purpose of these programs is to provide important new drug and biological products to patients earlier than under standard FDA review procedures. To be eligible for a fast track designation, the FDA must determine, based on the request of a sponsor, that a drug or biological product is intended to treat a serious or life-threatening disease or condition and demonstrates the potential to address an unmet medical need. The FDA will determine that a product will fill an unmet medical need if it will provide a therapy where none exists or provide a therapy that may be potentially superior to existing therapy based on efficacy or safety factors. In addition to other benefits, such as the ability to have greater

interactions with the FDA, the FDA may initiate review of sections of a fast track NDA or BLA before the application is complete, a process known as rolling review.

The FDA may give a priority review designation to drug or biological products that treat a serious condition and, if approved, would provide a significant improvement in safety or effectiveness. A priority review means that the goal for the FDA to review an application is six months, rather than the standard review of ten months under current PDUFA guidelines. Most products that are eligible for fast track designation may also be considered appropriate to receive a priority review.

In addition, drug and biological products studied for their safety and effectiveness in treating serious or life-threatening illnesses and that provide meaningful therapeutic benefit over existing treatments may be approved on the basis of adequate and well-controlled clinical trials establishing that the drug or biological product has an effect on a surrogate endpoint that is reasonably likely to predict clinical benefit, or on a clinical endpoint that can be measured earlier than irreversible morbidity or mortality, 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. As a condition of approval, the FDA may require a sponsor of a drug or biological product receiving accelerated approval to perform post-marketing studies with due diligence to verify and describe the predicted effect on irreversible morbidity or mortality or other clinical endpoint, and the drug or biological product may be subject to accelerated withdrawal procedures.

The Food and Drug Omnibus Reform Act ("FDORA") was recently enacted, which included provisions related to the accelerated approval pathway. Pursuant to FDORA, the FDA is authorized to require 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, which may include milestones such as a target date of study completion and requires sponsors to submit progress reports for required post-approval studies and any conditions required by the FDA not later than 180 days following approval and not less frequently than every 180 days thereafter until completion or termination of the study. FDORA also provides the FDA increased authority for expedited procedures to withdraw approval of a drug or biologic or indication approved under accelerated approval if, for example, the confirmatory trial fails to verify the predicted clinical benefit of the product. FDORA also enables the FDA to initiate enforcement actions for the 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. In addition, the FDA generally requires, unless otherwise informed by the agency, pre-approval of promotional materials, which could adversely impact the timing of the commercial launch of the product.

Moreover, a sponsor can request designation of a product candidate as a "breakthrough therapy." A breakthrough therapy is defined as a drug or biologic product that is intended, alone or in combination with one or more other drugs, to treat a serious or life-threatening disease or condition, and preliminary clinical evidence indicates that the drug or biologic product may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development. The FDA must take certain actions, such as holding timely meetings and providing advice, intended to expedite the development and review of an application for approval of a breakthrough therapy.

Even if a product qualifies for one or more of these programs, the FDA may later decide that the product no longer meets the conditions for qualification or decide that the time period for FDA review or approval will not be shortened. Furthermore, fast-track designation, priority review, accelerated approval and breakthrough therapy designation, do not change the standards for approval and may not ultimately expedite the development or approval process.

Rare Pediatric Disease Vouchers

The Rare Pediatric Disease Voucher Program is intended to encourage development of new drug and biological products for prevention and treatment of certain rare pediatric diseases. Although there are existing incentive programs to encourage the development and study of drugs and biologics for rare diseases, pediatric populations, and unmet medical needs, this program provides an additional incentive for the development of drugs and biologics for rare pediatric diseases, which may be used alone or in combination with other incentive programs. A rare pediatric disease is defined as a disease that is a serious or life-threatening disease in which the serious or life-threatening manifestations primarily affect individuals aged from birth to 18 years, including age groups often called neonates, infants, children, and adolescents; and is a rare disease or condition as defined in the FDCA, which includes diseases and conditions that affect fewer than 200,000 persons in the United States and diseases and conditions that affect a larger number of persons and for which there is no reasonable expectation that the costs of

developing and making available the product in the United States can be recovered from sales of the product in the United States.

The sponsor of an application for a drug product that obtains rare pediatric disease designation 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. A rare pediatric disease drug product must meet certain eligibility requirements for a priority voucher at the time the sponsor seeks approval. The rare pediatric disease priority review voucher program was most recently re-authorized by Congress through December 31, 2024, with the potential for priority review vouchers to be granted through September 30, 2026. 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 by December 20, 2024. After September 30, 2026, the FDA may not award any rare pediatric disease priority review vouchers unless the program is re-authorized by Congress.

Pediatric Information and Pediatric Exclusivity

Under the Pediatric Research Equity Act ("PREA"), certain NDAs and BLAs and certain supplements to an NDA or BLA must contain data to assess the safety and efficacy of the drug for the claimed indications in all relevant pediatric subpopulations and to support dosing and administration for each pediatric subpopulation for which the product is safe and effective. The FDA may grant deferrals for submission of pediatric data or full or partial waivers. A sponsor who is planning to submit a marketing application for a drug that includes a new active ingredient, new indication, new dosage form, new dosing regimen or new route of administration must submit an initial Pediatric Study Plan ("PSP") within 60 days of an end-of-Phase 2 meeting or, if there is no such meeting, as early as practicable before the initiation of the Phase 3 or Phase 2/3 study. The initial PSP must include an outline of the pediatric study or studies that the sponsor plans to conduct, including study objectives and design, age groups, relevant endpoints and statistical approach, or a justification for not including such detailed information, and any request for a deferral of pediatric assessments or a full or partial waiver of the requirement to provide data from pediatric studies along with supporting information. The FDA and the sponsor must reach an agreement on the PSP. A sponsor can submit amendments to an agreed-upon initial PSP at any time if changes to the pediatric plan need to be considered based on data collected from preclinical studies, early phase clinical trials and/or other clinical development programs.

Under the Best Pharmaceuticals for Children Act ("BPCA"), a drug or biologic product can also obtain pediatric market exclusivity in the United States. Pediatric exclusivity, if granted, adds six months to existing exclusivity periods for all formulations, dosage forms, and indications of the active moiety and to patent terms for drugs; for biologic products, pediatric exclusivity adds six months only to exclusivity periods for all formulations, dosage forms, and indications. This six-month exclusivity, which runs from the end of other exclusivity protection or patent term, may be granted based on the voluntary completion of a pediatric study in accordance with an FDA-issued "Written Request" for such a study, provided that at the time pediatric exclusivity is granted there is not less than nine months of term of the patent or exclusivity remaining at the time of approval.

Biosimilars

The Biologics Price Competition and Innovation Act of 2009 ("BPCIA") created an abbreviated approval pathway for biological products that are demonstrated to be "biosimilar" or "interchangeable" with an FDA-licensed reference biological product. Biosimilarity requires that the proposed biological product be highly similar to the reference product not withstanding minor differences in clinically inactive components; that there be no differences in conditions of use, route of administration, dosage form, and strength; and that no clinically meaningful differences between the product and the reference product in terms of safety, purity, and potency as shown through analytical studies, animal studies and a clinical trial or trials. In order to meet the higher hurdle of interchangeability, a sponsor must demonstrate that the biosimilar product can be expected to produce the same clinical results as the reference product, and for a product that is administered more than once, that the safety and efficacy risk of switching between the reference product and biosimilar product is not greater than the risk of maintaining the patient on the reference product. Complexities associated with the larger, and often more complex, structures of biological products, as well as the process by which such products are manufactured, pose significant hurdles to implementation that are still being evaluated by the FDA.

Under the BPCIA, a reference biologic is granted 12 years of exclusivity from the time of first licensure or BLA approval, of the reference product during which FDA cannot approve an application for a biosimilar or

interchangeable product relying on the reference product. Additionally, no application for a biosimilar can be submitted for four years from the date of licensure of the reference product. During the 12-year period of exclusivity, another company may still market a competing version of the reference product if the FDA approves a BLA for the competing product that does not rely on the reference product; the BLA must be based on the sponsor's own preclinical data and data from adequate and well-controlled clinical trials to demonstrate the safety, purity, and potency of the other company's product. Finally, the first biologic product submitted under the biosimilar abbreviated approval pathway that is determined to be interchangeable with the reference product may obtain exclusivity against a finding of interchangeability for other biologics for the same condition of use for the lesser of (i) one year after first commercial marketing of the first interchangeable biosimilar, (ii) 18 months after the first interchangeable biosimilar is approved if there is no patent challenge, (iii) 18 months after resolution of a lawsuit over the patents of the reference biologic in favor of the first interchangeable biosimilar applicant, or (iv) 42 months after the first interchangeable biosimilar is approved if a patent lawsuit is ongoing within the 42-month period.

Regulation Outside of the United States

In addition to regulations in the United States, we are subject to a variety of regulations in other jurisdictions governing clinical studies, commercial sales, and distribution of our products. Most countries outside of the United States require that clinical trial applications be submitted to and approved by the local regulatory authority for each clinical study. In the European Union, for example, an application must be submitted to the national competent authority and an independent ethics committee in each country in which we intend to conduct clinical trials, much like the FDA and IRB, respectively. Under the Clinical Trials Regulation (EU) No 536/2014, which replaced the Clinical Trials Directive 2001/20/EC on January 31, 2022, a single application is now made through the Clinical Trials Information System ("CTIS"), for clinical trial authorization in up to 30 European Union/European Economic Area countries at the same time and with a single set of documentation.

The assessment of applications for clinical trials is divided into two parts (Part I contains scientific and medicinal product documentation and Part II contains the national and patient-level documentation). Part I is assessed by a coordinated review by the competent authorities of all European Union Member States in which an application for authorization of a clinical trial has been submitted (Member States concerned) of a draft report prepared by a Reference Member State. Part II is assessed separately by each Member State concerned. The role of the relevant ethics committees in the assessment procedure is governed by the national law of the Member State concerned, however overall related timelines are defined by the Clinical Trials Regulation. The Clinical Trials Regulation also provides for simplified reporting procedures for clinical trial sponsors.

In addition, whether or not we obtain FDA approval for a product, we must obtain approval of a product by the comparable regulatory authorities of countries outside the U.S. before we can commence clinical studies or marketing of the product in those countries. The approval process and requirements vary from country to country, so the number and type of non-clinical, clinical, and manufacturing studies needed may differ, and the time may be longer or shorter than that required for FDA approval.

To obtain regulatory approval of a product under the European Union's regulatory system, we are required to submit a marketing authorization application ("MAA"), to be assessed in either the centralized procedure or a national authorization procedure. The centralized procedure allows applicants to obtain a marketing authorization ("MA") that is valid throughout the European Union and the additional Member States of the European Area (Iceland, Liechtenstein and Norway) ("EEA"). It is compulsory for medicinal products manufactured using biotechnological processes, for orphan medicinal products, for advance therapy medicinal products (gene-therapy, somatic cell-therapy or tissue-engineered medicines) and for human products containing a new active substance which are not authorized in the European Union and which are intended for the treatment of HIV, AIDS, cancer, neurodegenerative disorders, auto-immune and other immune dysfunctions, viral diseases or diabetes. The centralized procedure is optional for any other products containing new active substances not authorized in the European Union or for products which constitute a significant therapeutic, scientific or technical innovation or for which a centralized authorization is in the interests of patients in the European Union. When a company wishes to place on the market a medicinal product that is eligible for the centralized procedure, it sends an application directly to the EMA, to be assessed by the Committee for Medicinal Products for Human Use ("CHMP"). The CHMP is responsible for conducting the assessment of whether a medicine meets the required quality, safety and efficacy requirements, and whether the product has a positive risk/benefit/risk profile.

The time limit for the evaluation procedure is 210 days (excluding clock stops, when additional written or oral information is to be provided by the applicant in response to questions asked by the CHMP). The EMA then has

fifteen days to forward its opinion to the European Commission which will make a binding decision on the grant of an MA within 67 days of the receipt of the CHMP.

There are two other procedures in the European Union for the grant of an MA in multiple European Union Member States, where a product does not fall within the mandatory scope of the centralized procedure. If a product has already been authorized for marketing in a European Union Member State, this national MA can be recognized in another European Union Member State through the mutual recognition procedure. If the product has not received a national MA in any European Union Member State at the time of application, it can be approved simultaneously in various Member States through the decentralized procedure.

Regulation (EC) No. 141/2000, provides that a product may be designated as an orphan medicinal product if its sponsor can establish that it is intended for the diagnosis, prevention, or treatment of life-threatening or chronically debilitating condition that affects not more than 5 in 10,000 persons in the European Union when the application is made. In addition, orphan designation can be granted if the product is intended for a life threatening, seriously debilitating, or serious and chronic condition in the European Union where without incentives derived from orphan designation.it is unlikely that sales of the product in the European Union would be sufficient to justify the necessary investment in its development. 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. We have obtained orphan designation in the European Union for nomlabofusp.

Orphan designation provides opportunities for fee reductions, protocol assistance and access to the centralized procedure in the European Union. Fee reductions are not limited to the first year after an MA is granted, except for small and medium enterprises. In addition, if a product which has an orphan designation subsequently receives a centralized MA for the indication for which it has such designation, the product is entitled to orphan market exclusivity, which means the regulatory authorities in the European Union may not approve any other application to market a "similar medicinal product" for the same indication as the authorized orphan product for a period of 10 years. A “similar medicinal product” is defined as a medicinal product containing a similar active substance or substances as contained in an authorized orphan medicinal product, and which is intended for the same therapeutic indication. The exclusivity period may be reduced to six years if, at the end of the fifth year, its is determined that the designation criteria are no longer met, including where it is shown that the product is sufficiently profitable not to justify maintenance of market exclusivity. Additionally, an MA may be granted to a similar medicinal product for the same indication as an authorized orphan product if:

- the second applicant can establish that its product, although similar to the authorized orphan product, is safer, more effective or otherwise clinically superior;
- the MA holder of the authorized orphan product consents to a second orphan medicinal product application; or
- the MA holder of the authorized orphan product cannot supply enough orphan medicinal product.

The EMA offers a scheme to facilitate development of product candidates in indications, often rare, for which few or no therapies currently exist. The PRIority MEdicines, or PRIME, scheme is intended to encourage drug development in areas of unmet medical need and is intended to reinforce early dialogue with, and regulatory support from, the EMA in order to stimulate innovation, optimize development and enable accelerated assessment of such product candidates. It is intended to build upon the scientific advice scheme and accelerated assessment procedure offered by the EMA. The scheme is voluntary and eligibility criteria must be met for a medicine to qualify for PRIME.

The PRIME scheme is open to medicines under development and for which the applicant intends to apply for an MA through the centralized procedure. Eligible products must target conditions for which there 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) 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 (small and medium sized enterprises) 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 Committee for Advanced Therapies ("CAT") to provide continuous support and to build up knowledge of the medicine in advance of the filing of an MAA;

•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, support under the PRIME scheme may be withdrawn.

A Pediatric Investigation Plan ("PIP") in the European Union is aimed at ensuring that the necessary data are obtained to support the authorization of a medicine for children, through studies in children. All applications for marketing authorization for new medicines have to include the results of studies as described in an agreed PIP, unless the study results are deferred, or the medicine is exempt because of a waiver. This requirement also applies when a marketing-authorization holder wants to add a new indication, pharmaceutical form, or route of administration for a medicine that is already authorized. Several rewards and incentives for the development of pediatric medicines for children are available in the EU Medicines authorized across the EU with the results of studies from a PIP included in the product information are eligible for an extension of their supplementary protection certificate ("SPC")by six months(provided an application for such extension is made at the same time as filing the SPC application for the product, or at any point up to two years before the SPC expires). This is the case even when the studies' results are negative. For orphan medicines, the incentive is an additional two years of market exclusivity. Scientific advice and protocol assistance at the EMA are free of charge for questions relating to the development of pediatric medicines.

All of the aforementioned European Union rules are generally applicable in the EEA.

The UK left the European Union on January 31, 2020. As a result of the Northern Ireland protocol following Brexit, the EMA remained responsible for approving novel medicines for supply in Northern Ireland, the European Union regulatory framework continues to apply in Northern Ireland under the EU centralized procedure, and as separate authorization was required to supply the same medicine in Great Britain (England, Wales and Scotland). On February 27, 2023, the UK government and the European Commission announced a political agreement in principle to replace the Northern Ireland Protocol with a new set of arrangements, known as the "Windsor Framework". This new framework fundamentally changes the previous system under the Northern Ireland Protocol, including with respect to the regulation of medicinal products in the UK. In particular, the MHRA is now responsible for approving all medicinal products destined for the UK market (Great Britain and Northern Ireland), and the EMA no longer has any role in approving medicinal products destined for Northern Ireland through the EU centralized procedure. A single UK-wide MA will be granted by the MHRA for all novel medicinal products to be sold in the UK, enabling products to be sold in a single pack and under a single authorization throughout the UK. In addition, the new arrangements require all medicines placed on the UK market to be labeled "UK only" indicating they are not for sale in the EU.

The MHRA has introduced changes to national licensing procedures, including procedures to prioritize access to new medicines that will benefit patients, an accelerated assessment procedure and new routes of evaluation for novel products and biotechnological products. On January 1, 2024, a new international recognition framework came

into effect in the UK under which the MHRA may have regard to decisions on the approval of MAs made by the EMA and certain other regulators when determining an application for a new UK MA.

There is now no pre-marketing authorization orphan designation in the UK. Instead, the MHRA will review applications for orphan designation in parallel to the corresponding MA application. The criteria are essentially the same, but have been tailored for the UK market, i.e. the prevalence of the condition in the UK (rather than the EU) must not be more than 5 in 10,000. Should an orphan designation be granted, the period or market exclusivity will be set from the date of first approval of the product in the UK.

In September 2024, we received access to the UK's Medicines and Healthcare Regulatory Agency ("MHRA") Innovative Licensing and Access Pathway ("ILAP") which for the treatment of adults and children with FA. We may also apply for access to the UK's Innovative Licensing and Access Pathway ("ILAP") for the treatment of adults and children with FA. This Scheme aims to accelerate the time to market and facilitate patient access to certain types of medicinal products in development which target a life-threatening or seriously debilitating condition, or where there is a significant patient or public health need in the UK. To access the ILAP, an applicant applies for an Innovation Passport designation. Once an Innovation Passport designation is granted, the MHRA and its partner agencies (including The All Wales Therapeutics and Toxicology Centre, National Institute for Health and Care Excellence and the Scottish Medicines Consortium) will work with the Innovation Passport designee to define a Target Development Profile, ("TDP"). The TDP sets out a unique product-specific roadmap towards patient access in the UK and provides access to a toolkit to support all stages of the design, development and approvals process, including continuous benefit-risk assessment, increased support for novel development approaches and enhanced patient engagement. However, although the goal of the ILAP is to reduce the time to market and enable earlier patient access, access does not accelerate conduct of clinical trials or mean that the regulatory requirements are less stringent, nor does it ensure that a marketing authorization application will be approved or that any approval will be granted within a particular timeframe or at all.

Healthcare Laws and Regulations

Coverage and Reimbursement

Our ability to successfully commercialize our product candidates will depend in part on the extent to which coverage and adequate reimbursement for these products and related treatments will be available from government health administration authorities, private health insurers and other organizations. Even if coverage is provided, the approved reimbursement amount may not be high enough to allow us to establish or maintain pricing sufficient to realize a sufficient return on our investment. 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.

There is also significant uncertainty related to the insurance coverage and reimbursement of newly approved products and coverage may be more limited than the purposes for which the medicine is approved by the FDA or comparable foreign regulatory authorities. In the United States, the principal decisions about reimbursement for new medicines are typically made by the Centers for Medicare & Medicaid Services ("CMS"), an agency within the U.S. Department of Health and Human Services. CMS decides whether and to what extent a new medicine will be covered and reimbursed under Medicare and private payors tend to follow CMS to a substantial degree. Factors payors consider in determining reimbursement are based on whether the product is a covered benefit under its health plan; safe, effective and medically necessary; appropriate for the specific patient; cost-effective; and neither experimental nor investigational. Increasingly, third-party payors are requiring that drug companies provide them with predetermined discounts from list prices and are challenging the prices charged for medical products.

Outside of the United States, the pricing of pharmaceutical products is subject to governmental control in many countries. For example, in the European Union, pricing and reimbursement schemes vary widely from country to country. Some countries provide that products may be marketed only after a reimbursement price has been agreed. Some countries may require the completion of additional studies that compare the cost-effectiveness of a particular therapy to currently available therapies or so-called health technology assessments, in order to obtain reimbursement or pricing approval. Other countries may allow companies to fix their own prices for products, but monitor and control product volumes and issue guidance to physicians to limit prescriptions. We cannot be sure that reimbursement will be available for any of our product candidates that we commercialize and, if reimbursement is available, the level of reimbursement.

Other Healthcare Laws

Sales of our product candidate, if approved, or any other future product candidate will be subject to healthcare regulation and enforcement by the federal government and the states and foreign governments in which we might conduct our business. The healthcare laws and regulations that may affect our ability to operate include the following:

- the federal Anti-Kickback Statute prohibits, among other things knowingly and willfully, directly or indirectly, soliciting, receiving, offering, or paying any remuneration that is in exchange for or to induce the referral of business, including the purchase, order, lease of any good, facility, item or service for which payment may be made under a federal healthcare program, such as Medicare or Medicaid. The term "remuneration" has been broadly interpreted to include anything of value.

- The Patient Protection and Affordable Care Act as amended by the Health Care and Education Reconciliation Act (collectively, the "ACA"), amended the intent element of the federal Anti-Kickback Statute so that a person or entity no longer needs to have actual knowledge of the statute or specific intent to violate it in order to commit a violation;

- Federal false claims and false statement laws, including the federal civil False Claims Act, prohibits, among other things, any person or entity from knowingly presenting, or causing to be presented, for payment to, or approval by, federal programs, including Medicare and Medicaid, claims for items or services, including drugs, that are false or fraudulent. The ACA amended the federal Anti-Kickback Statute such that a violation of that statute can serve as a basis for liability under the federal civil False Claims Act;

- the federal Health Insurance Portability and Accountability Act of 1996 ("HIPAA") created additional federal criminal statutes that prohibit among other actions, knowingly and willfully executing, or attempting to execute, a scheme to defraud any healthcare benefit program, including private third-party payors or making any false, fictitious or fraudulent statement in connection with the delivery of or payment for healthcare benefits, items or services;

- HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act of 2009 ("HITECH") and their implementing regulations, impose obligations on healthcare providers, health plans, and healthcare clearinghouses, known as covered entities as well as their business associates and their subcontractors that perform certain services involving the storage, use or disclosure of individually identifiable health information; including mandatory contractual terms, with respect to safeguarding the privacy, security, and transmission of individually identifiable health information, and require notification to affected individuals and regulatory authorities of certain breaches of security of individually identifiable health information. HITECH increased the civil and criminal penalties that may be imposed against covered entities, business associates and possibly other persons, and gave state attorneys general new authority to file civil actions for damages or injunctions in federal courts to enforce the federal HIPAA laws and seek attorney's fees and costs associated with pursuing federal civil actions;

- the federal Physician Payments Sunshine Act enacted as part of the ACA, and its implementing regulations requires certain manufacturers of approved drugs, devices, biologics and medical supplies for which payment is available under Medicare, Medicaid or the Children's Health Insurance Program, with specific exceptions, to collect and report annually to the Centers for Medicare and Medicaid Services, the agency that administers the Medicare and Medicaid programs ("CMS") information related to payments or other transfers of value made to physicians (defined to include doctors, dentists, optometrists, podiatrists and chiropractors), physician assistants, nurse practitioners, clinical nurse specialists, certified nurse anesthetists, certified nurse-midwives) and teaching hospitals, as well as ownership and investment interests held by physicians and their immediate family members. The reported data is made available in searchable form on a public website on an annual basis. Failure to submit required information may result in civil monetary penalties; and

- The Foreign Corrupt Practices Act ("FCPA") prohibits U.S. businesses and their representatives from offering to pay, paying, promising to pay or authorizing the payment of money or anything of value to a foreign official in order to influence any act or decision of the foreign official in his or her official capacity or to secure any other improper advantage in order to obtain or retain business.

Many states have similar laws and regulations, such as anti-kickback and false claims laws that may be broader in scope and may apply regardless of payor, in addition to items and services reimbursed under Medicaid and other state programs. Additionally, we may be subject to state laws that require pharmaceutical companies to comply with the federal government's and/or pharmaceutical industry's voluntary compliance guidelines, state laws that require drug manufacturers to report information related to payments and other transfers of value to physicians and other healthcare providers or marketing expenditures. Certain states and local jurisdictions also require the registration of pharmaceutical sales representatives. Further, certain states require the posting of information relating to clinical studies and their outcomes. A growing number of states require the reporting of certain drug pricing information, including information pertaining to and justifying price increases and the price set for newly launched drugs, or to prohibit prescription drug price gouging. We may also be subject to state and foreign laws governing the privacy and security of health information, many of which differ from each other in significant ways and often are not preempted by HIPAA. In the United States, there are numerous federal and state privacy and data security laws and regulations governing the collection, use, disclosure and protection of personal information, including federal and state health information privacy laws, federal and state security breach notification laws, and federal and state consumer protection laws. Each of these laws is subject to varying interpretations and new laws continue to be proposed. At the state level, numerous states have or are in the process of enacting or considering comprehensive state-level data privacy and security laws, rules and regulations while other states have focused on more narrow aspects of privacy. In the state of Washington, for example, the My Health My Data Act will require regulated entities to obtain consent to collect health information, grant consumers certain rights, including to request deletion of their information, and provide for robust enforcement mechanisms, including enforcement by the Washington state attorney-general and a private right of action for consumer claims. Other states have also enacted, proposed, or are considering proposing, data privacy laws, which could further complicate compliance efforts, increase our potential liability and adversely affect our business. Additionally, to the extent that our product is sold in a foreign country, we may be subject to similar foreign laws.

Healthcare Reform

The United States and many foreign jurisdictions have enacted or proposed legislative and regulatory changes affecting the healthcare system. 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. Third-party payors are increasingly challenging the prices charged for medical products and services, examining the medical necessity and reviewing the cost effectiveness of pharmaceutical or biological products, medical devices, and medical services, in addition to questioning safety and efficacy. CMS also has authority to revise reimbursement rates and to implement coverage restrictions for some drugs. Cost reduction initiatives and changes in coverage implemented through legislation or regulation could decrease utilization of and reimbursement for any approved products. While Medicare regulations apply only to drug benefits for Medicare beneficiaries, private payors often follow Medicare coverage policy and payment limitations in setting their own reimbursement rates. Therefore, although private payors have their own methods and approval process apart from Medicare determinations, any reduction in reimbursement that results from federal legislation or regulation may result in a similar reduction in payments from private payors.

The Affordable Care Act substantially changed the way healthcare is financed by both governmental and private insurers, included provisions that affected the pharmaceutical industry. Among other things, the Affordable Care Act expanded manufacturers' rebate liability under the Medicaid Drug Rebate Program, expanded the 340B program, and increased the amount of Medicaid drug rebates manufacturers are required to pay to states. Since that time, there have been multiple healthcare reform measures, including the Inflation Reduction Act of 2022 ("IRA") which, among other things, allows HHS to directly negotiate the selling price of a statutorily specified number of drugs and biologics each year that CMS reimburses under Medicare Part B and Part D. The negotiated price may not exceed a statutory ceiling price. Only high-expenditure single-source biologics that have been approved for at least 11 years (7 years for small molecule drugs) can be selected by CMS for negotiation, with the negotiated price taking effect two years after the selection year. For 2026, the first year in which negotiated prices become effective, CMS selected 10 high-cost Medicare Part D products in 2023, negotiations began in 2024, and the negotiated maximum fair price for each product has been announced. CMS has selected 15 additional Medicare Part D drugs for negotiated maximum fair pricing in 2027. For 2028, an additional 15 drugs, which may be covered under either Medicare Part B or Part D, will be selected, and for 2029 and subsequent years, 20 Part B or Part D drugs will be selected. A drug or biological product that has an orphan drug designation for only one rare disease or condition will be excluded from the IRA's price negotiation requirements, but will lose that exclusion if it receives designations for more than one rare disease or condition, or if is approved for an indication that is not within that single designated

rare disease or condition, unless such additional designation or such disqualifying approvals are withdrawn by the time CMS evaluates the drug for selection for negotiation. The IRA also imposes rebates on Medicare Part B and Part D drugs whose prices have increased at a rate greater than the rate of inflation, and in November 2024, CMS finalized regulations for these inflation rebates. The IRA also extends enhanced subsidies for individuals purchasing health insurance coverage in ACA marketplaces through plan year 2025. The IRA permits the Secretary of HHS to implement many of these provisions through guidance, as opposed to regulation, for the initial years. Manufacturers that fail to comply with the IRA may be subject to various penalties, including significant civil monetary penalties. These provisions have been and may continue to be subject to legal challenges. For example, the provisions related to the negotiation of selling prices of high-expenditure single-source drugs and biologics have been challenged in multiple lawsuits brought by pharmaceutical manufacturers. Thus, while it is unclear how the IRA will be implemented, it will likely have a significant impact on the biopharmaceutical industry and the pricing of prescription drug products.

At the state level, legislatures have increasingly passed legislation and implemented regulations designed to control pharmaceutical product pricing, including price or patient reimbursement constraints, discounts, restrictions on certain product access and marketing cost disclosure and transparency measures, and, in some cases, designed to encourage importation from other countries and bulk purchasing. For example, the FDA released a final rule in September 2020 providing guidance for states to build and submit plans for importing drugs from Canada, and FDA authorized the first such plan in Florida in January 2024. In addition, regional healthcare authorities and individual hospitals are increasingly using bidding procedures to determine what pharmaceutical products and which suppliers will be included in their prescription drug and other healthcare programs.

Additional federal, state and foreign healthcare reform measures may 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 prescription drug products, once approved, or additional pricing pressures.

ITEM 1A. RISK FACTORS

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, we had net losses of $80.6 million and $36.9 million, respectively, and, as of December 31, 2024, we had an accumulated deficit of $269.2 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, 2024, our existing cash, cash equivalents and marketable securities were $183.5 million, which we anticipate will 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 or 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 stockholders' 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, 2024 we had NOL carryforwards that expire for U.S. federal income tax purposes of $ $198.2 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, 2024, the Company

had federal net operating loss carryforwards that were generated after December 31, 2017 of $159.5 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

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 or that we will ever 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, 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. We have an ongoing Phase 2 OLE trial in patients with FA and an ongoing PK run-in study in adolescent patients with FA.

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;

•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;
- 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. We have an ongoing Phase 2 OLE trial in patients with FA and an ongoing run-in study in adolescent patients with FA. Our clinical trials may 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, 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 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:

•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.

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 had discussions with the FDA regarding the use of tissue FXN levels as a novel surrogate endpoint. The FDA 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 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 by the end 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 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 concentrations as a surrogate endpoint, supportive pharmacodynamic and clinical information and safety data from the OLE study, along with 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. The 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 an RLSE and the acceptability of FXN's use as an RLSE would ultimately be a matter of review of the data. There can be no assurance that data we intend to generate will be successful in establishing that increases in FXN 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, non-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 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, which means that it is shown to be safer, more effective or makes a major contribution to patient care.

We have also received orphan designation for nomlabofusp in the European Union. In the European Union, the European Commission may grant orphan designation in respect of products that are intended for the diagnosis, prevention or treatment of a life threatening or chronically debilitating condition affecting not more than five in 10,000 persons in the 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 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 by December 20, 2024. After September 30, 2026, the FDA may not award any rare pediatric disease priority review vouchers. Congress may vote to reauthorize this program, 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 could be harmed.

The PRIME scheme is open to medicines under development and for which the applicant intends to apply for an 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 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.

Changes in the FDA, other government agencies or comparable regulatory authorities could hinder their ability to hire and retain key leadership and other personnel, prevent new products and services from being developed, approved or commercialized in a timely manner or otherwise prevent those agencies from performing normal business functions on which the operation of our business may rely, which could negatively impact our business.

The 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 approve new products can be affected by a variety of factors, including government budget and funding levels, ability to hire and retain key personnel, and accept the payment of user fees, statutory, regulatory and policy changes. 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, 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. 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 an example, over the last several years, the U.S. government has shut down several times and certain regulatory agencies, such as the FDA and the SEC have had to furlough critical employees and stop critical activities.

If a prolonged government shutdown occurs, including as a result of reaching the debt ceiling, if the FDA or SEC experiences significant decreases in funding or personnel 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 the grant of an MA from the 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, 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.

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.

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 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 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 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 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, 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 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 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. If we are unable 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.

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 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. patent that relates to the use of the TAT-FXN fusion protein for treating FA. We also in-license from IU United States 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, United States 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, 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 patent relating to nomlabofusp from WFUHS. The U.S. patent relating to use of the TAT-FXN fusion protein for the treatment of FA expires in 2025. 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, 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 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.

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 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 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 market conditions and other factors, including factors unrelated to our operating performance or the 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 33.3% of our outstanding common stock (assuming no exercise of outstanding options) as of December 31, 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.

ITEM 1B. UNRESOLVED STAFF COMMENTS

None.

ITEM 1C. CYBERSECURITY

Cyber Risk Management and Strategy

We have implemented and maintain various information security processes designed to manage cybersecurity risks relating to our critical computer networks, third party hosted services, communications systems, hardware and software, and our critical data, including intellectual property, confidential information that is proprietary, strategic or competitive in nature, and data related to our clinical trials and products (Information Systems and Data).

Our Director of Information Technology, assisted by our managed information technology service provider and other third party service providers, leads the Company's cybersecurity risk management processes. This group works to identify and assess risks from cybersecurity threats by monitoring and evaluating our threat environment and the Company's risk profile using various methods in certain contexts, including, manual tools, subscribing to reports and services that identify cybersecurity threats, analyzing threat intelligence reports and feeds, conducting scans of certain environments, and other tests.

We implement and maintain various technical, physical, and organizational measures, processes, standards and policies designed to manage risks from cybersecurity threats to our Information Systems and Data, as applicable depending on the environment, including incident detection and response, disaster recovery/business continuity policies, network security controls and data segmentation, access controls, physical security, asset management and tracking, systems monitoring, and employee training.

Our assessment and management of risks from cybersecurity threats are part of the Company's overall risk management processes. Our Director of Information Technology works with management to prioritize our risk management processes and mitigate cybersecurity threats that are more likely to lead to a material impact to our business.

We use third-party service providers to assist us to identify, assess, and manage risks from cybersecurity threats. These third-party service providers may include threat intelligence service providers, cybersecurity consultants, cybersecurity software providers, managed cybersecurity service providers, and dark web monitoring services.

We also have established a process to conduct diligence on certain third parties before engaging with those third parties, such as those that support our GxP processes, which may include a security assessment.

We have not identified any cybersecurity incidents or threats that have materially affected us or are reasonably likely to materially affect us, including our business strategy, results of operations or financial condition; however, like other companies in our industry, we and our third-party vendors may, from time to time, experience threats and security incidents relating to our and our third-party vendors' information systems and infrastructure. For more information, please see our risk factors under Part 1 Item 1A. Risk Factors.

Governance

Our board of directors holds oversight responsibility over the Company's cybersecurity risk management as part of its general oversight function. The audit committee of the board of directors is responsible for overseeing the Company's cybersecurity risk management processes, including oversight of the Company's processes for monitoring and controlling cybersecurity risks.

Our cybersecurity risk assessment and management processes are implemented and maintained by certain Company management, including our Director of Information Technology, who has over 10 years of information technology management experience.

The Director of Information Technology, along with management, is responsible for helping to integrate cybersecurity risk considerations into the Company's overall risk management strategy and communicating key priorities to relevant personnel. Management is responsible for approving budgets, helping prepare for cybersecurity incidents, approving cybersecurity processes, and reviewing security-related reports.

We have processes designed to escalate certain cybersecurity incidents to members of management depending on the circumstances, including our Vice President Legal and Compliance, Vice President Finance and Administration, Vice President - Controller, Chief Financial Officer and Director of Information Technology. In addition, the Company's incident response policy includes a process for reporting to the audit committee of the board of directors for certain cybersecurity incidents.

The audit committee receives reports from members of the Company's management concerning the Company's significant cybersecurity threats and risk and the processes the Company has implemented to address them, as applicable.

ITEM 2. PROPERTIES

We are party to an operating lease for approximately 3,927 square feet of laboratory space in King of Prussia, PA. We believe the lab space is sufficient for our needs for the next several years.

We are party to an operating lease for approximately 8,104 square feet of office space in Bala Cynwyd, Pennsylvania that serves as our corporate office. We expect to increase our office space at our Bala Cynwyd Pennsylvania office in the near term by approximately 7,107 square feet to 15,211 square feet. We believe that appropriate office space will be readily available on commercially reasonable terms.

We are party to an operating lease for approximately 17,705 square feet of office space in Boston, Massachusetts. This lease expires in October 2029. On October 27, 2020, we entered into a sublease agreement whereby we subleased all 17,705 square feet of office space leased under the Boston Lease until October 2029.

ITEM 3. LEGAL PROCEEDINGS

We may be subject to other legal proceedings and claims in the ordinary course of business. We cannot predict the results of any such disputes, and despite the potential outcomes, the existence thereof may have an adverse material impact on us due to diversion of management time and attention as well as the financial costs related to resolving such disputes.

ITEM 4. MINE SAFETY DISCLOSURES

Not applicable.

PART II

ITEM 5. MARKET FOR REGISTRANT'S COMMON EQUITY, RELATED STOCKHOLDER MATTERS AND ISSUER PURCHASES OF EQUITY SECURITIES.

Market Information

Our common stock is publicly traded on the Nasdaq Global Market under the symbol "LRMR."

Holders

As of March 24, 2025, we had approximately 62 record holders of our common stock.

Dividends

We have not declared or paid any dividends since our inception nor do we expect to pay dividends in the foreseeable future.

Securities Authorized for Issuance Under Equity Compensation Plans

Information about our equity compensation plans is incorporated by reference herein to Part III, Item 12 of this Annual Report on Form 10-K.

Recent Sales of Unregistered Securities

There have been no sales of unregistered securities other than as previously disclosed by us in our Current Reports on Form 8-K as filed with the SEC.

Issuer Purchases of Equity Securities

None.

ITEM 6. [Reserved.]

ITEM 7. MANAGEMENT'S DISCUSSION AND ANALYSIS OF FINANCIAL CONDITION AND RESULTS OF OPERATIONS

You should read the following discussion and analysis of our financial condition and results of operations together with the audited consolidated financial statements and the related notes included elsewhere in this Annual Report on Form 10-K. In addition to historical financial information, the following discussion contains forward-looking statements based upon our current plans, expectations and beliefs that involve risks, uncertainties and assumptions. Our actual results may differ materially from those described in or implied by these forward-looking statements as a result of many factors, including those set forth under the section titled "Risk Factors" and in other parts of this Annual Report on Form 10-K.

Overview

We are a clinical-stage biotechnology company focused on developing treatments for patients suffering from complex rare diseases using our novel cell penetrating peptide ("CPP") technology platform. Our lead product candidate, nomlabofusp (nomlabofusp is the International Nonproprietary Name and the United States Adopted Name for CTI-1601), is a subcutaneously administered, recombinant fusion protein intended to deliver tissue frataxin ("FXN"), an essential protein, to the mitochondria of patients with Friedreich's ataxia ("FA"). FA is a rare, progressive, and fatal disease in which patients are unable to produce sufficient FXN due to a genetic abnormality. Currently, there are no treatment options that address the core deficit of FA, low levels of FXN. Nomlabofusp represents the first potential therapy designed to systemically increase FXN levels in patients with FA.

We believe that our CPP platform, which enables a therapeutic molecule to cross a cell membrane in order to reach intracellular targets, has the potential to enable the treatment of other rare and orphan diseases. We intend to use our proprietary platform to target additional orphan indications characterized by deficiencies in or alterations of intracellular content or activity.

Since our inception, we have devoted substantially all of our resources to developing nomlabofusp, building our intellectual property portfolio, developing third-party manufacturing capabilities, business planning, raising capital, developing sales and marketing capacities, and providing general and administrative support for such operations.

Nomlabofusp Program Update

We have completed two Phase 1 clinical trials and a Phase 2 dose exploration trial, and have an ongoing Phase 2 OLE trial in patients with FA and an ongoing PK run-in study in adolescent patients with FA.

In May 2021, we reported positive top-line data from our Phase 1 FA program after completing dosing of the single ascending dose ("SAD") trial in December 2020 and of the multiple ascending dose ("MAD") trial in March 2021. Data from these trials demonstrated proof-of-concept by showing that daily subcutaneous injections of nomlabofusp for up to 13 days resulted in dose-dependent increases in FXN levels from baseline compared to placebo in all evaluated tissues (buccal cells, skin, and platelets). There were no serious adverse events associated with either the MAD or SAD trials.

In May 2023, we reported preliminary unblinded top-line data from the 25 mg cohort of our Phase 2 four-week, placebo-controlled, dose exploration trial of nomlabofusp in FA patients. Data from the cohort indicated nomlabofusp was generally well tolerated and showed increases in FXN levels from baseline compared to placebo in all evaluated tissues (skin and buccal cells) at day 14.

In July 2023, the FDA cleared initiation of a second cohort (50 mg) of our four-week, placebo-controlled, Phase 2 dose exploration trial of nomlabofusp in patients with FA and the initiation of our OLE trial with daily dosing of 25 mg.

In February 2024, we reported positive top-line data and successful completion of the Phase 2 dose exploration study. Nomlabofusp was generally well tolerated throughout the four-week treatment periods, had a predictable pharmacokinetic profile and led to 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. Patients who completed treatment in our Phase 2 dose exploration study or who previously completed a prior clinical trial of nomlabofusp are eligible to screen and possibly participate in the OLE study.

In March 2024, we dosed the first patient in our OLE trial, evaluating daily subcutaneous injections of 25 mg of nomlabofusp self-administered or administered by a caregiver. The OLE study will evaluate the safety and tolerability, pharmacokinetics, and frataxin levels in peripheral tissues as well as other exploratory

pharmacodynamic markers (lipid profiles and gene expression data) following long-term subcutaneous administration of nomlabofusp. In addition, following the completion of enrollment, as well as at least one participant completing one year of dosing, clinical assessments collected during the study will be compared to data from a matched control arm derived from participants in the Friedreich's Ataxia Clinical Outcome Measures Study ("FACOMS") database.

On May 30, 2024, we announced that the FDA's Center for Drug Evaluation and Research ("CDER") had selected nomlabofusp as one of a few programs for participation in the Support for Clinical Trials Advancing Rare Disease Therapeutics ("START") Pilot Program. The objective of the program is to accelerate the development of drugs for rare diseases that lead to significant disability or death by facilitating frequent advice and regular communication with the FDA staff to expedite the review process of biologics and drugs.

In September 2024, we received access to the Medicines and Healthcare Regulatory Agency ("MHRA") Innovative Licensing and Access Pathway ("ILAP") for the treatment of adults and children with FA. The ILAP is facilitating patient access to novel treatments by accelerating time to market through opportunities for enhanced engagements with UK regulatory authorities and other stakeholders. Along with the receipt of the ILAP designation, nomlabofusp has already been granted orphan drug designations in the U.S. and the European Union (the "EU"), and access to the PRIME scheme in the EU.

In December 2024, we reported positive initial data from our ongoing OLE study. This data included safety, FXN levels, clinical, pharmacokinetic data and dose escalation:

- Nomlabofusp was generally well tolerated for up to 260 days in subjects. The most common adverse events were injection site reactions, with most being mild, brief in duration, and self-limited. Two participants had serious adverse events (one seizure and one anaphylactic reaction) that resolved within 24 hours and withdrew from the study;
- Long-term 25 mg tissue FXN levels showed positive mean change from baseline of 1.32 pg/µg in buccal cells and 9.28 pg/µg in skin cells at Day 90 and that 25 mg of nomlabofusp increased and maintained tissue FXN levels over time, increasing from a mean level of 15% of HV at baseline to 30% in buccal cells and from 16% to 72% in skin cells at Day 90;
- Tissue FXN levels appear to reach steady-state levels by Day 30 in buccal cells;
- Early clinical data showed trends toward improvement across a number of clinical outcomes for long-term 25 mg daily nomlabofusp including: (1) decreased values indicating early trends towards improvement in the Modified Friedreich Ataxia Rating Scale (mFARS) and the FARS-Activities of Daily Living ("ADL"), Modified Fatigue Impact Scale and 9 hole peg test at 90 days relative to baseline;
- Key pharmacokinetic data showed rapid absorption after subcutaneous administration with exposure appearing to reach steady state in plasma by day 30 with no further accumulation, which is consistent with data from our Phase 1 and Phase 2 studies.

Also in December 2024, we announced that we were increasing the dose in the OLE to 50 mg of nomlabofusp daily for then currently enrolled and all future OLE study participants. In March 2025, we announced that our Safety Monitoring Team has deemed anaphylaxis as an adverse drug reaction likely associated with nomlabofusp and therefore, we expect to see additional reactions. To reduce the risk of allergic reactions, including anaphylaxis, we amended the OLE protocol to administer premedication for the first month of dosing. The OLE study is ongoing with seven sites activated and participants continuing to enroll. All study participants are currently receiving the 50 mg dose of nomlabofusp. We plan to provide an update on OLE data on at least 30 to 40 study participants, some of whom have been receiving nomlabofusp for more than a year, in September 2025.

In January 2025, we initiated dosing of adolescents (12-17 years old) in a PK run-in study for pediatric patients with FA. Study participants in the PK run-in study are randomized 2:1 to receive either nomlabofusp at a weight-base dose expected to match the PK of adults receiving the 50 mg dose, or placebo, daily for seven days. Following assessment of safety and PK data, participants will be eligible to screen for the OLE study. The data from this cohort is expected to be presented during the nomlabofusp program update in September 2025.

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. FDA recommended focusing on assessments of skin FXN concentrations rather than buccal FXN concentrations due to more consistent sampling and less variability. FDA acknowledged that data we recently submitted appear to support a relationship between increased FXN concentrations in skin cells and relevant tissues such as the heart, dorsal root ganglion and skeletal muscle. FDA

also acknowledged that the nonclinical studies we submitted were performed at relevant human doses. FDA also suggested that we consider exploring the relationship between increases in FXN in skin and changes in pharmacodynamic (PD) markers such as lipid profiles and/or clinical measures to provide additional support for the use of FXN as an RLSE. We plan to continue discussions with FDA regarding the adequacy of the safety data set for a BLA submission seeking accelerated approval targeted for year-end 2025.

Additionally, we have obtained feedback from both FDA and EMA on the global Phase 3 study protocol and we are on track to initiate the study by mid-2025 with potential sites in the U.S., E.U., U.K, Canada, and Australia.

Financing Activities, Including Recent Material Financings

We have funded our operations to date primarily with proceeds from sales of common stock, proceeds from the sale of prefunded warrants for the purchase of common stock, the acquisition in 2020 of cash, cash equivalents, marketable securities and restricted cash upon the merger with Zafgen, Inc. ("Zafgen") and, prior to the 2020 merger with Zafgen, capital contributions from Chondrial Holdings, LLC.

In February 2024, we completed an underwritten public offering in which we issued and sold 19,736,842 shares of our common stock at a public offering price of $8.74 per share. We received net proceeds of approximately $161.8 million after deducting underwriting discounts, commissions and other offering expenses.

In May 2024, we entered into a Sales Agreement (the "ATM Agreement") with Guggenheim Securities, LLC in connection with the establishment of an “at-the-market” offering program providing for the sale of up to an aggregate of $100 million of shares of our common stock from time to time. To date, we have made no sales under the ATM agreement.

Critical Accounting Policies and Significant Judgments and Estimates

Our consolidated financial statements are prepared in accordance with Generally Accepted Accounting Principles ("GAAP"). The preparation of our consolidated financial statements and related disclosures requires us to make estimates and assumptions that affect the reported amount of assets, liabilities, costs and expenses, and related disclosures. We believe that the estimates and assumptions involved in the accounting policies described below may have the greatest potential impact on our consolidated financial statements and, therefore, consider these to be our critical accounting policies. We evaluate these estimates and assumptions on an ongoing basis. Our actual results may differ from these estimates under different assumptions and conditions.

Research and Development Expense

Costs for certain research and development activities, such as manufacturing, non-clinical studies and clinical trials are generally recognized based on the evaluation of the progress of completion of specific tasks using information and data provided by our vendors and collaborators, and accordingly, are considered an area of significant judgment and management’s review of manufacturing, non-clinical and clinical expenses. This process involves reviewing open contracts and purchase orders, communicating with our personnel and outside vendors to identify services that have been performed on our behalf and estimating the level of service performed and the associated costs incurred for the services when we have not yet been invoiced or otherwise notified of the actual costs. We work with vendors and suppliers to ensure that our estimates of our research and development expenses are reasonable. We expect to increase our investment in research and development in order to advance nomlabofusp through additional clinical trials. As a result, we expect that our research and development expenses will continue to increase in the foreseeable future as we pursue clinical development of nomlabofusp and/or any other product candidates we develop.

Stock Compensation Expense

We measure all stock-based awards granted to employees and directors based on the fair value on the date of grant using the Black-Scholes option-pricing model. The Black-Scholes option-pricing model requires the use of highly subjective assumptions which determine the fair value of stock-based awards. The assumptions used in our option-pricing model represent management’s best estimates. These estimates are complex, involve a number of variables, uncertainties and assumptions and the application of management’s judgment, and thus are inherently subjective. If factors change and different assumptions are used, our stock-based compensation expense could be materially different in the future.

Prior to May 28, 2020, we were a private company and lacked company-specific historical and implied volatility information for our common stock. Prior to January 1, 2023, the Company estimated its expected common stock price volatility solely based on the historical volatility of publicly traded peer companies with comparable

characteristics including enterprise value, risk profiles and position within the industry. Beginning on January 1, 2023, the Company began blending its historical data starting in June 2020 (following its merger with Zafgen in 2020) with its historical peer group. We regularly evaluate our peer group to assess changes in circumstances where identified companies may no longer be similar to us, in which case, more suitable companies whose share prices are publicly available would be utilized in the calculation. We expect to continue to do so until we have full historical data regarding the volatility of our own traded stock price.

The expected term of our stock options has been determined utilizing the "simplified" method for awards that qualify as "plain-vanilla" options. The risk-free interest rate is determined by reference to the U.S. Treasury yield curve in effect at the time of grant of the award for time periods approximately equal to the expected term of the award. The expected dividend yield considers the fact that we have never paid cash dividends on common stock and do not expect to pay any cash dividends in the foreseeable future.

Compensation expense of those awards is recognized over the requisite service period, which is generally the vesting period of the respective award. Typically, we issue awards with only service-based vesting conditions and record the expense for these awards using the straight-line method. We account for forfeitures as they occur.

We classify stock-based compensation expense in our consolidated statements of operations and comprehensive loss in the same manner in which the award recipient's payroll costs are classified or in which the award recipient's service payments are classified.

Financial Operations Overview

Revenue

To date, we have not generated any revenue from product sales, and do not expect to generate any revenue from the sale of products in the foreseeable future. If our development efforts result in clinical success and regulatory approval or collaboration agreements with third parties for our product candidates, we may generate revenue from those product candidates or collaborations.

Operating Expenses

The majority of our operating expenses since inception have consisted primarily of research and development activities, and general and administrative costs.

Research and Development Expenses

Research and development expenses, which consist primarily of costs associated with our product research and development efforts, are expensed as incurred. Research and development expenses consist primarily of:

- third-party contract costs relating to research, formulation, manufacturing, non-clinical studies and clinical trial activities;
- employee related costs, including salaries, benefits and stock-based compensation expenses for employees engaged in scientific research and development functions;
- external costs of outside consultants and vendors;
- payments made under our third-party licensing agreements;
- sponsored research agreements;
- laboratory consumables; and
- allocated facility-related costs.

At this time, we cannot reasonably estimate or know the nature, timing and estimated costs of the efforts that will be necessary to complete the clinical and commercial development of nomlabofusp, or any other product candidates we develop. We are also unable to predict when, if ever, material net cash inflows will commence from sales of our product candidates. The duration, costs, and timing of clinical trials and development of nomlabofusp or any other product candidates we develop will depend on a variety of factors, including:

- the scope, rate of progress and expense of clinical trials and other research and development activities;
- clinical trial results;

•uncertainties in clinical trial enrollment rate or design;

•significant and changing government regulation;

•the timing and receipt of any regulatory approvals;

•the influence of the FDA or other regulatory authorities on our clinical trial design and timing;

•establishing manufacturing capabilities or making arrangements with third-party manufacturers and risk involved with development of manufacturing processes, FDA pre-approval inspection practices and successful completion of manufacturing batches for clinical development and other regulatory purposes;

•our ability to obtain and maintain patent and trade secret protection and regulatory exclusivity for our product candidates; and

•our ability to recruit and retain key research and development personnel.

A change in the outcome of one or more of these variables with respect to the development of a product candidate could significantly change the costs, timing and viability associated with the development of that product candidate. For example, if the FDA or another regulatory authority were to require us to conduct additional non-clinical or clinical trials beyond those that we currently anticipate will be required for the completion of clinical development of a product candidate, or if we experience significant delays in enrollment in any of our clinical trials, we could be required to expend significant additional financial resources and time on the completion of clinical development.

General and Administrative Expenses

General and administrative expenses consist primarily of personnel costs, consisting of salaries, related benefits and stock-based compensation, costs related to our executive, finance, information technology, and costs related to other administrative functions. General and administrative expenses also include insurance expenses and professional fees for auditing, tax, and legal services, including legal expenses to pursue patent protection for our intellectual property. We expect that our general and administrative expenses will increase in the foreseeable future as we hire additional employees to implement, improve and scale our operational, financial, commercial and management systems.

Results of Operations

The following commentary is a discussion and analysis of our financial condition as of December 31, 2024 and results of operations and cash flows for the year ended December 31, 2024 compared to the year ended December 31, 2023 and should be read in conjunction with the consolidated financial statements and accompanying notes.

Comparison of the years ended December 31, 2024 and 2023

The following table summarizes our results of operations for the years ended December 31, 2024 and 2023:

	Year Ended December 31,		
	2024	2023	Increase (Decrease)
		(in thousands)	
Statement of Operations Data:			
Operating expenses:			
Research and development	$ 73,278	$ 27,670	$ 45,608
General and administrative	17,612	14,088	3,524
Total operating expenses	90,890	41,758	49,132
Loss from operations	(90,890)	(41,758)	(49,132)
Other income, net	10,286	4,809	5,477
Net loss	$ (80,604)	$ (36,949)	$ (43,655)

Research and development expenses

Research and development expenses for the twelve months ended December 31, 2024 increased $45.6 million compared to the twelve months ended December 31, 2023 due to continued and increased clinical development of

nomlabofusp and related regulatory and bioanalysis. The increase in research and development expenses was primarily driven by an increase of $36.1 million in nomlabofusp manufacturing costs including production scale-up, costs and manufacturing costs related to producing drug product doses to be used in ongoing and planned clinical trials, an increase of $4.5 million in personnel expense due to increased clinical, regulatory, quality and manufacturing headcount, an increase of $1.3 million of professional fees related to regulatory, quality, medical, and clinical consulting costs, an increase of $1.2 million in clinical costs primarily associated with the OLE study which began dosing patients in the first quarter of 2024, an increase of $1.0 million in stock compensation costs associated with the incremental 2024 grants for existing and new employees, an increase of $0.9 million related to our participation in the Friedreich's Ataxia Research Alliance's ("FARA's") TRACK-FA Neuroimaging Consortium conducting a natural history study designed to establish disease-specific neuroimaging biomarkers to track disease progression in the brain and spinal cord and provide a basis for utilizing these biomarkers in clinical trials, an increase of $0.3 million in internal lab costs related primarily to gene expression and lipid studies and an increase of $0.2 million in facility costs associated with the new lab space.

General and administrative expenses

General and administrative expenses for the twelve months ended December 31, 2024 increased $3.5 million compared to the twelve months ended December 31, 2023. The increase in general and administrative expenses was primarily driven by an increase of $1.5 million in professional fees primarily related to consulting costs related to commercial activity and other public company related expenses, an increase of $1.4 million in personnel expense due to increased headcount, an increase of $0.4 million of other expense related to computer software, information technology services and recruiting and an increase of $0.2 million in stock compensation costs associated with 2024 grants to existing and new employee grants.

Other income, net

Other income, net was $10.3 million of income in the twelve months ended December 31, 2024 compared to $4.8 million in the twelve months ended December 31, 2023. The increase primarily relates to interest income on a higher investment base and higher investment yields on that base during the current period.

Liquidity and Capital Resources

Since our inception, we have not generated any revenue from any sources, including from product sales, and have incurred significant operating losses and negative cash flows from our operations. We have devoted substantially all of our resources to developing nomlabofusp, building our intellectual property portfolio, developing third-party manufacturing capabilities, business planning, capital raising, and providing general and administrative support for such operations.

The following table summarizes our sources and uses of cash for each of the periods presented below:

	Year Ended December 31,	
	2024	2023
	(in thousands)	
Net cash used in operating activities	$ (70,760)	$ (33,459)
Net cash provided by (used in) investing activities	(85,387)	33,353
Net cash provided by financing activities	161,883	30
Net increase (decrease) in cash, cash equivalents and restricted cash	$ 5,736	$ (76)

Net cash used in operating activities

During the year ended December 31, 2024, net operating activities used $70.8 million of cash, resulting from our net loss of $80.6 million, adjusted for noncash expenses of $3.7 million, and changes in our operating assets and liabilities provided cash of $6.2 million. Our net loss was attributed to operating expenses of $90.9 million, offset by other income (net) of $10.3 million. The change in operating assets and liabilities was primarily due to an increase in accounts payable, an increase in accrued expenses and offset by an increase in prepaid assets, all associated with increasing expenditures and development activity.

During the year ended December 31, 2023, operating activities used $33.5 million of cash, resulting from our net loss of $36.9 million, adjusted for noncash expenses of $6.0 million, and changes in our operating assets and liabilities resulting in a use of cash of $2.5 million. Our net loss was attributed to operating expenses of $41.8

million, offset by other income (net) of $4.8 million. The change in operating assets and liabilities was primarily due to a decrease in accounts payable and accrued expenses and an increase in prepaid assets.

Net cash provided by (used in) investing activities

During the year ended December 31, 2024, investing activities used $85.4 million of net cash, resulting from purchases of $227.9 million in marketable securities, which was offset by $143.0 million of maturities and sales of marketable securities.

During the year ended December 31, 2023, investing activities provided $33.4 million of net cash, resulting from $134.8 million of maturities and sales of marketable securities, which was offset by purchases of $101.2 million in marketable securities.

Net cash provided by financing activities

During the year ended December 31, 2024, financing activities provided $161.9 million of cash primarily from an offering of common stock.

During the year ended December 31, 2023, financing activities provided less than $0.1 million of cash from the exercise of stock options and warrants.

Operating Capital Requirements

We have not yet commercialized any products and do not expect to generate revenue from the commercial sale of any products for several years, if at all.

We have to date incurred net losses. We incurred net losses of approximately $80.6 million and $36.9 million for the twelve months ended December 31, 2024 and 2023, respectively. As of December 31, 2024, we had an accumulated deficit of $269.2 million and a cash, cash equivalents and marketable securities balance of $183.5 million, excluding restricted cash of $0.6 million.

Losses have resulted principally from costs incurred in connection with research and development activities, and general and administrative costs associated with the development of nomlabofusp and our operations. We expect to incur significant expenses and operating losses for the foreseeable future as we expect to continue to incur expenses in connection with our ongoing activities, if and as we:

- continue to advance the development of nomlabofusp through additional clinical trials, including related manufacturing costs;
- seek to identify and advance development of additional product candidates into clinical development and identify additional indications for our product candidates;
- seek to obtain regulatory approvals for nomlabofusp and other potential product candidates;
- identify, acquire or in-license other product candidates and technologies;
- maintain, leverage and expand our intellectual property portfolio; and
- expand our operational, financial, commercial and management systems and personnel, including personnel to support our clinical development and future commercialization efforts and our operations as a public company.

In February 2024, we completed an underwritten public offering in which we issued and sold 19,736,842 shares of our common stock and received net proceeds of approximately $161.8 million after deducting underwriting discounts, commissions and other offering expenses. We anticipate that our current cash, cash equivalents and marketable securities will fund operations into the second quarter of 2026. If we encounter unexpected delays in our clinical trials or if there are other unanticipated changes to our operating plan from our current assumptions that negatively impact our operations, we may reduce expenditures in order to further extend our existing cash resources. Until we can generate substantial revenue, if ever, we expect to seek additional funding through a combination of public or private equity offerings, debt/royalty financings, collaborations, strategic alliances 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, minimum cash balances, 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.

There can be no assurance that we will be able to raise sufficient additional capital on acceptable terms, if 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, strategic alliances 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 and prospects. In addition, geopolitical tensions, volatility of capital markets, and other adverse macroeconomic events, including those due to inflationary pressures, rising interest rates, bank instability and the ability of the U.S. government to manage federal debt limits, as well as the potential impact of health crises on the global financial markets may reduce our ability to access capital, which could negatively affect our liquidity and ability to continue as a going concern.

If we are unable to obtain sufficient 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 and/or pre commercialization efforts, which could adversely affect our business prospects, or we may be unable to continue operations. 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.

Recently Issued Accounting Pronouncements

Please read Note 2 to our audited consolidated financial statements included in Part IV, Item 15, of this Annual Report for a description of recent accounting pronouncements applicable to our business.

Other Company Information

None.

ITEM 7A. QUANTITATIVE AND QUALITATIVE DISCLOSURES ABOUT MARKET RISK

We are a smaller reporting company as defined by Rule 12b-2 of the Exchange Act and are not required to provide the information required under this item.

ITEM 8. FINANCIAL STATEMENTS AND SUPPLEMENTARY DATA

Our audited consolidated financial statements and the report of our independent registered public accounting firm are included in this Annual Report on Form 10-K on the pages indicated in Part IV, Item 15.

ITEM 9. CHANGES IN AND DISAGREEMENTS WITH ACCOUNTANTS ON ACCOUNTING AND FINANCIAL DISCLOSURE

None.

ITEM 9A. CONTROLS AND PROCEDURES

Evaluation of Disclosure Controls and Procedures

We maintain "disclosure controls and procedures," as such term is defined in Rules 13a-15(e) and 15d-15(e) under the Exchange Act, that are designed to ensure that information required to be disclosed by us in reports that we file or submit under the Exchange Act is recorded, processed, summarized and reported within the time periods specified in SEC rules and forms, and that such information is accumulated and communicated to our management, including our principal executive and our principal financial officers, as appropriate, to allow timely decisions regarding required disclosure.

With respect to the year ended December 31, 2024, under the supervision and with the participation of our management, we conducted an evaluation of the effectiveness of the design and operations of our disclosure controls and procedures. Based upon this evaluation, our Chief Executive Officer and Chief Financial Officer have concluded that our disclosure controls and procedures were effective as of December 31, 2024 to provide reasonable assurance that the information required to be disclosed by us in this Annual Report was (a) reported within the time periods specified by SEC rules and regulations and (b) communicated to our management, including our Chief Executive Officer and Chief Financial Officer, to allow timely decisions regarding any required disclosure.

Management's Annual Report on Internal Control Over Financial Reporting

Our management is responsible for establishing and maintaining adequate internal control over financial reporting as defined in Rules 13a-15(f) and 15d-15(f) under the Exchange Act. Our internal control over financial reporting is a process designed to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles. Because of its inherent limitations, internal control over financial reporting may not prevent or detect misstatements. Projections of any evaluation of effectiveness to future periods are subject to the risk that controls may become inadequate because of changes in conditions, or that the degree of compliance with the policies or procedures may deteriorate. Under the supervision and with the participation of our management, including our principal executive officer and principal financial officer, we conducted an evaluation of the effectiveness of our internal control over financial reporting based on the framework in Internal Control – Integrated Framework (2013) issued by the Committee of Sponsoring Organizations of the Treadway Commission. Based on our evaluation under that framework, our management concluded that our internal control over financial reporting was effective as of December 31, 2024.

Changes in Internal Control Over Financial Reporting

There were no changes in our internal control over financial reporting identified in connection with the evaluation required by Rule 13a-15(d) and 15d-15(d) of the Exchange Act that occurred during the quarter ended December 31, 2024, that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

ITEM 9B. OTHER INFORMATION

(a) None.

(b) During the fourth quarter of the fiscal year ended December 31, 2024, no director or "officer" as defined in Rule 16a-1(f) under the Exchange Act adopted or terminated any Rule 10b5-1 trading plan or arrangements or any non-Rule 10b5-1 trading plan or arrangements, in both cases as defined in Item 408(a) of Regulation S-K.

ITEM 9C. DISCLOSURE REGARDING FOREIGN JURISDICTIONS THAT PREVENT INSPECTIONS

Not applicable

PART III

ITEM 10. DIRECTORS, EXECUTIVE OFFICERS AND CORPORATE GOVERNANCE

The information required by Item 10 of Form 10-K is incorporated by reference to the information contained in our definitive proxy statement for the 2025 annual meeting of stockholders.

We have adopted insider trading policies and procedures governing the purchase, sale, and other dispositions of our securities by directors, officers, and employees that we believe are reasonably designed to promote compliance with insider trading laws, rules and regulations, and applicable Nasdaq listing standards. Our insider trading policy states, among other things, that our directors, officers, and employees are prohibited from trading in such securities while in possession of material, nonpublic information. The foregoing summary of our insider trading policies and procedures does not purport to be complete and is qualified by reference to our Insider Trading Policy filed as an exhibit to this Annual Report on Form 10-K. In addition, with regard to trading in our own securities, it is our policy to comply with the federal securities laws and the applicable exchange listing requirements.

ITEM 11. EXECUTIVE COMPENSATION

The information required by Item 11 of Form 10-K is incorporated by reference to the information contained in our definitive proxy statement for the 2025 annual meeting of stockholders.

ITEM 12. SECURITY OWNERSHIP OF CERTAIN BENEFICIAL OWNERS AND MANAGEMENT AND RELATED STOCKHOLDER MATTERS

The information required by Item 12 of Form 10-K is incorporated by reference to the information contained in our definitive proxy statement for the 2025 annual meeting of stockholders.

ITEM 13. CERTAIN RELATIONSHIPS AND RELATED TRANSACTIONS, AND DIRECTOR INDEPENDENCE

The information required by Item 13 of Form 10-K is incorporated by reference to the information contained in our definitive proxy statement for the 2025 annual meeting of stockholders.

ITEM 14. PRINCIPAL ACCOUNTING FEES AND SERVICES

Our independent accounting firm is PricewaterhouseCoopers LLP, Philadelphia, Pennsylvania, USA, PCAOB Auditor ID: 238.

The information required by Item 14 of Form 10-K is incorporated by reference to the information contained in our definitive proxy statement for the 2025 annual meeting of stockholders.

PART IV

ITEM 15. EXHIBITS, FINANCIAL STATEMENT SCHEDULES

1. Financial Statements

See Index to the Consolidated Financial Statements on page F-1 of this Annual Report.

2. Financial Statement Schedules

None, as all information required in these schedules is included in the Notes to the Consolidated Financial Statements.

3. Exhibits

Reference is made to the Exhibit Index below for a list of exhibits required by Item 601 of Regulation S-K to be filed as part of this Annual Report.

The following exhibits are being filed herewith:

EXHIBIT INDEX

* Filed Herewith
+ Certain confidential portions (indicated by brackets and asterisks) have been omitted from this exhibit
# Certain information in this document has been excluded pursuant to Regulation S-K, Item 601(b)(10).
^ Certain schedules, annexes or exhibits have been omitted pursuant to Item 601(a)(5) of Regulation S-K, but will be furnished supplementally to the SEC upon request.
** Furnished herewith. This certification will not be deemed "filed" for purposes of Section 18 of the Securities Exchange Act of 1934, as amended, or otherwise subject to the liability of that section. Such certification will not be deemed to be incorporated by reference into any filing under the Securities Act of 1933, as amended, except to the extent specifically incorporated by reference into such filing.

Exhibit No.	Exhibit
2.1	Agreement and Plan of Merger, dated as of December 17, 2019, by and among Zafgen, Inc., Chondrial Therapeutics, Inc., Chondrial Therapeutics Holdings, LLC, and Zordich Merger Sub, Inc. (incorporated by reference to Exhibit 2.1 of the Company's Current Report on Form 8-K filed on December 18, 2019).
2.2	Amendment No. 1 to Agreement and Plan of Merger, dated as of March 6, 2020, by and among Zafgen, Inc., Chondrial Therapeutics, Inc., Chondrial Therapeutics Holdings, LLC, and Zordich Merger Sub, Inc. (incorporated by reference to Exhibit 2.1 of the Company's Current Report on Form 8-K filed on March 6, 2020).
3.1	Ninth Amended and Restated Certificate of Incorporation of Larimar Therapeutics, Inc. (formerly known as Zafgen, Inc.) (incorporated by reference to Exhibit 3.1 of the Company's Current Report on Form 8-K filed on June 24, 2014).
3.2	Certificate of Amendment of Ninth Amended and Restated Certificate of Incorporation of Zafgen, Inc. related to the Reverse Stock Split, dated May 28, 2020 (incorporated by reference to Exhibit 3.1 of the Company's Current Report on Form 8-K filed on June 2, 2020).
3.3	Certificate of Amendment of Ninth Amended and Restated Certificate of Incorporation of Zafgen, Inc. related to the Name Change, dated May 28, 2020 (incorporated by reference to Exhibit 3.1 of the Company's Current Report on Form 8-K filed on June 2, 2020).
3.4	Certificate of Amendment of Ninth Amended and Restated Certificate of Incorporation of Larimar Therapeutics, Inc., dated May 31, 2024 (incorporated by reference to Exhibit 3.1 of the Company's Quarterly Report on Form 10-Q filed on August 8, 2024).
3.5	Amended and Restated By-laws of Larimar Therapeutics, Inc. (formerly known as Zafgen, Inc.) (incorporated by reference to Exhibit 3.2 of the Company's Current Report on Form 8-K filed on June 24, 2014).
4.1	Description of the Company's Securities Registered Pursuant to Section 12 of the Securities Exchange Act of 1934 (incorporated by reference to Exhibit 4.2 of the Company's Annual Report on Form 10-K filed March 4, 2021).
10.1	Larimar Therapeutics, Inc. 2020 Equity Incentive Plan (incorporated by reference to Exhibit 10.1 of the Company's Current Report on Form 8-K filed on September 29, 2020).
10.2	Larimar Therapeutics, Inc. Form of Stock Option Grant Notice and Award Agreement under its 2020 Equity Incentive Plan (incorporated by reference to Exhibit 10.2 of the Company's Current Report on Form 8-K filed on September 29, 2020).
10.3*	Restricted Stock Award Agreement for Company Employees under the Larimar Therapeutics, Inc. 2020 Equity Incentive Plan (incorporated by reference to Exhibit 10.3 to the Company's Annual Report on Form 10-K filed on March 14, 2024).
10.4*	Non-Qualified Stock Option Grant Notice and Award Agreement (incorporated by reference to Exhibit 10.4 to the Company's Annual Report on Form 10-K filed on March 14, 2024).
10.5	Form of Performance-Based Restricted Stock Unit Award Agreement under the Larimar Therapeutics, Inc. 2020 Equity Incentive Plan (incorporated by reference to Exhibit 10.1 to the Company's Current Report on Form 8-K filed on January 27, 2025).
10.6	Registration Rights Agreement, dated as of June 1, 2020, by and among the Company and certain Investors (incorporated by reference to Exhibit 10.2 of the Company's Current Report on Form 8-K filed on June 2, 2020).
10.7	Registration Rights Agreement, dated as of June 8, 2020, by and among the Company and certain Investors (incorporated by reference to Exhibit 10.3 of the Company's Registration Statement on Form S-3 filed on June 26, 2020).

10.8	Form of Indemnification Agreement between the Company and its directors (incorporated by reference to Exhibit 10.3 of the Company's Current Report on Form 8-K filed on June 2, 2020).
10.9	Employment Agreement, dated July 31, 2020, by and between the Company and Carole Ben-Maimon (incorporated by reference to Exhibit 10.1 of the Company's Current Report on Form 8-K filed on August 6, 2020).
10.10	Employment Agreement, dated June 1, 2020, by and between the Company and Michael Celano (incorporated by reference to Exhibit 10.4 of the Company's Current Report on Form 8-K filed on June 2, 2020).
10.11	Employment Agreement, dated February 7, 2023, by and between the Company and Gopi Shankar (incorporated by reference to Exhibit 10.10 of the Company's Annual Report on Form 10-K filed on March 14, 2023).
10.12	Employment Agreement dated May 23, 2023, by and between the Company and Russell Clayton (incorporated by reference to Exhibit 10.1 of the Company's Quarterly Report on Form 10-Q filed on August 10, 2023).
10.13#	License Agreement, by and between the Company and Wake Forest University Health Sciences, effective as of November 30, 2016. (incorporated by reference to Exhibit 10.6 of the Company's Quarterly Report on Form 10-Q filed on August 14, 2020).
10.14#	Amendment 1 to License Agreement, by and between the Company and Wake Forest University Health Sciences, effective as of November 28, 2017 (incorporated by reference to Exhibit 10.7 of the Company's Quarterly Report on Form 10-Q filed on August 14, 2020).
10.15#	Amendment 2 to License Agreement, by and between the Company and Wake Forest University Health Sciences, effective as of March 29, 2019 (incorporated by reference to Exhibit 10.8 of the Company's Quarterly Report on Form 10-Q filed on August 14, 2020).
10.16#	License Agreement, by and between the Company and Indiana University Research and Technology Corporation, effective as of November 30, 2016 (incorporated by reference to Exhibit 10.9 of the Company's Quarterly Report on Form 10-Q filed on August 14, 2020).
10.17#	First Amendment to License Agreement, by and between the Company, the Trustee of Indiana University and Indiana University Research and Technology Corporation, effective as of August 16, 2019 (incorporated by reference to Exhibit 10.10 of the Company's Quarterly Report on Form 10-Q filed on August 14, 2020).
10.18	Second Amendment to License Agreement, by and between the Company and The Trustees of Indiana University, effective as of May 28, 2020 (incorporated by reference to Exhibit 10.1 to the Company's Quarterly Report on Form 10-Q filed on August 11, 2022).
10.19	Third Amendment to License Agreement, by and between the Company and The Trustees of Indiana University, effective as of June 9, 2022 (incorporated by reference to Exhibit 10.2 to the Company's Quarterly Report on Form 10-Q filed on August 11, 2022).
10.20	Notice of Substitute Option Grant between the Company and a certain Optionee (incorporated by reference to Exhibit 4.5 of the Company's Registration Statement on Form S-8 filed on June 26, 2020).
10.21	Lease, dated as of August 8, 2019, by and between Larimar Therapeutics, Inc. and Bala Plaza Property, Inc. (incorporated by reference to Exhibit 10.20 to the Company's Form 10-K filed on March 14, 2024).
10.22	First Amendment to Lease, dated as of March 9, 2023, by and between the Company and Bala Plaza Property, Inc. (incorporated by reference to Exhibit 10.21 to the Company's Form 10-K filed on March 14, 2024).
10.23+	Commercial Lease by and between the Company and Shigo Center Plaza Owner, LLC dated as of February 12, 2019 (incorporated herein by reference to Exhibit 10.4 of the Registrant's Form 10-Q filed on May 9, 2019).
10.24+	Sublease, dated October 27, 2020 by and between the Company and Massachusetts Municipal Association, Inc. (incorporated by reference to Exhibit 10.1 to the Company's Current Report on Form 8-K filed on October 30, 2020).
10.25+	Master Services Agreement, dated as of September 20, 2017, by and between the Company and KBI Biopharma, Inc. (incorporated by reference to Exhibit 10.1 to the Company's Quarterly Report on Form 10-Q filed on May 10, 2021).
10.26+	First Amendment to Master Services Agreement, dated as of November 9, 2018, by and between the Company and KBI Biopharma, Inc. (incorporated by reference to Exhibit 10.2 to the Company's Quarterly Report on Form 10-Q filed on May 10, 2021).
10.27+	Second Amendment to Master Services Agreement, dated as of September 20, 2022, by and between the Company and KBI Biopharma, Inc. (incorporated by reference to Exhibit 10.23 to the Company's Form 10-K filed on March 14, 2023)

10.28	Sales Agreement, dated as of May 9, 2024, by and between the Company and Guggenheim Securities, LLC (incorporated by reference to Exhibit 10.1 to the Company's Quarterly Report on Form 10-Q filed on May 9, 2024).
19.1*^	Insider Trading Policy.
21.1*	Subsidiaries of Larimar Therapeutics, Inc.
23.1*	Consent of PricewaterhouseCoopers LLP, independent registered public accounting firm.
31.1*	Rule 13a-14(a)/15d-14(a) certification of Principal Executive Officer.
31.2*	Rule 13a-14(a)/15d-14(a) certification of Principal Financial Officer.
32.1**	Section 1350 certification of the Principal Executive Officer and Principal Financial Officer, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002 (furnished herewith).
97.1	Larimar Therapeutics. Inc.'s Compensation Recovery Policy (incorporated by reference to Exhibit 97.1 to the Company's Form 10-K filed on March 14, 2024.
101.INS	Inline XBRL Instance Document
101.SCH	Inline XBRL Taxonomy Extension Schema Document
101.CAL	Inline XBRL Taxonomy Extension Calculation Linkbase Document
101.DEF	Inline XBRL Taxonomy Extension Definition Linkbase Document
101.LAB	Inline XBRL Taxonomy Extension Label Linkbase Document
101.PRE	Inline XBRL Taxonomy Extension Presentation Linkbase Document
104	Cover Page Interactive Data File (formatted as inline XBRL and contained in Exhibit 101)

ITEM 16. Form 10-K Summary

None.

SIGNATURES

Pursuant to the requirements of Section 13 or 15(d) of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned, thereunto duly authorized.

LARIMAR THERAPEUTICS, INC.

By: /s/ Carole S. Ben-Maimon, M.D.
Name: Carole S. Ben-Maimon, M.D.
Title: President and Chief Executive Officer

Pursuant to the requirements of the Securities Exchange Act of 1934, this report has been signed by the following persons on behalf of the registrant and in the capacities and on the dates indicated.

Signature	Title	Date
/s/ Carole S. Ben-Maimon, M.D. Carole S. Ben-Maimon, M.D.	President, Chief Executive Officer and Director (principal executive officer)	March 24, 2025
/s/ Michael Celano Michael Celano	Chief Financial Officer (principal financial and accounting officer)	March 24, 2025
/s/ Joseph Truitt Joseph Truitt	Chairman, Board of Directors	March 24, 2025
/s/ Thomas E. Hamilton Thomas E. Hamilton	Director	March 24, 2025
/s/ Jonathan Leff Jonathan Leff	Director	March 24, 2025
/s/ Jeffrey W. Sherman, M.D., FACP Jeffrey W. Sherman, M.D., FACP	Director	March 24, 2025
/s/ Frank E. Thomas Frank E. Thomas	Director	March 24, 2025

INDEX

	Page
Report of Independent Registered Public Accounting Firm (PCAOB ID 238)	F-1
Consolidated Balance Sheets as of December 31, 2024 and 2023	F-3
Consolidated Statements of Operations and Comprehensive Loss for the years ended December 31, 2024 and 2023	F-4
Consolidated Statements of Changes in Stockholders Equity for the years ended December 31, 2024 and 2023	F-5
Consolidated Statements of Cash Flows for the years ended December 31, 2024 and 2023	F-6
Notes to Consolidated Financial Statements	F-7

Report of Independent Registered Public Accounting Firm

To the Board of Directors and Stockholders of Larimar Therapeutics, Inc.

Opinion on the Financial Statements

We have audited the accompanying consolidated balance sheets of Larimar Therapeutics, Inc. and its subsidiary (the "Company") as of December 31, 2024 and 2023, and the related consolidated statements of operations and comprehensive loss, of changes in stockholders' equity and of cash flows for the years then ended, including the related notes (collectively referred to as the "consolidated financial statements"). In our opinion, the consolidated financial statements present fairly, in all material respects, the financial position of the Company as of December 31, 2024 and 2023, and the results of its operations and its cash flows for the years then ended in conformity with accounting principles generally accepted in the United States of America.

Basis for Opinion

These consolidated financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on the Company's consolidated financial statements based on our audits. We are a public accounting firm registered with the Public Company Accounting Oversight Board (United States) (PCAOB) and are required to be independent with respect to the Company in accordance with the U.S. federal securities laws and the applicable rules and regulations of the Securities and Exchange Commission and the PCAOB.

We conducted our audits of these consolidated financial statements in accordance with the standards of the PCAOB. Those standards require that we plan and perform the audits to obtain reasonable assurance about whether the consolidated financial statements are free of material misstatement, whether due to error or fraud. The Company is not required to have, nor were we engaged to perform, an audit of its internal control over financial reporting. As part of our audits we are required to obtain an understanding of internal control over financial reporting but not for the purpose of expressing an opinion on the effectiveness of the Company's internal control over financial reporting. Accordingly, we express no such opinion.

Our audits included performing procedures to assess the risks of material misstatement of the consolidated financial statements, whether due to error or fraud, and performing procedures that respond to those risks. Such procedures included examining, on a test basis, evidence regarding the amounts and disclosures in the consolidated financial statements. Our audits also included evaluating the accounting principles used and significant estimates made by management, as well as evaluating the overall presentation of the consolidated financial statements. We believe that our audits provide a reasonable basis for our opinion.

Emphasis of Matter

As discussed in Note 1 to the consolidated financial statements, the Company will require additional financing to fund future operations. Management's evaluation of the events and conditions and management's plans to mitigate this matter are also described in Note 1.

Critical Audit Matters

The critical audit matter communicated below is a matter arising from the current period audit of the consolidated financial statements that was communicated or required to be communicated to the audit committee and that (i) relates to accounts or disclosures that are material to the consolidated financial statements and (ii) involved our especially challenging, subjective, or complex judgments. The communication of critical audit matters does not alter in any way our opinion on the consolidated financial statements, taken as a whole, and we are not, by communicating the critical audit matter below, providing a separate opinion on the critical audit matter or on the accounts or disclosures to which it relates.

Accrued Research and Development Expenses

As described in Notes 2 and 6 to the consolidated financial statements, the Company recognizes external research and development costs based on an evaluation of the progress to completion of specific tasks using information provided to the Company by its key service providers. Within accrued expenses, total accrued research and development expenses amounted to $17.1 million as of December 31, 2024. Management's process involves reviewing open contracts and purchase orders, communicating with personnel and outside vendors to identify

services that have been performed, and estimating the level of service performed and the associated costs incurred for the services when invoices or other notification of actual costs have not been received.

The principal considerations for our determination that performing procedures relating to accrued research and development expenses is a critical audit matter are (i) the significant judgment by management in developing the estimates of accrued research and development expenses and (ii) a high degree of auditor judgment, subjectivity and effort in performing procedures and evaluating audit evidence for these accrued research and development expenses related to management's estimates of the level of service performed and the associated costs incurred for the services when invoices or other notification of actual costs have not yet been received.

Addressing the matter involved performing procedures and evaluating audit evidence in connection with forming our overall opinion on the financial statements. These procedures also included, among others; (i) testing management's process for estimating accrued research and development expenses; (ii) evaluating the appropriateness of the method used by management to develop the estimate; (iii) testing the completeness and accuracy of the data used by management to develop the estimates related to the level of service performed and the associated costs incurred for the services when invoices or other notification of actual costs have not yet been received; (iv) evaluating the reasonableness of the estimated costs incurred for the services which have not been invoiced or other notification of actual costs have not yet been received, on a sample basis, by tracing to underlying supporting documentation, such as underlying contracts, invoices and information received from certain third party service vendors, where applicable, and; (v) testing a selection of invoices received by the Company subsequent to December 31, 2024.

/s/ PricewaterhouseCoopers LLP
Philadelphia, Pennsylvania
March 24, 2025

We have served as the Company's auditor since 2020.

LARIMAR THERAPEUTICS, INC.

CONSOLIDATED BALANCE SHEETS
(In thousands, except share and per share data)

	December 31, 2024	December 31, 2023
Assets		
Current assets:		
Cash and cash equivalents	$ 33,218	$ 26,749
Marketable securities	150,236	60,041
Prepaid expenses and other current assets	11,850	3,385
Total current assets	195,304	90,175
Property and equipment, net	881	684
Operating lease right-of-use assets	2,838	3,078
Restricted cash	606	1,339
Other assets	596	659
Total assets	$ 200,225	$ 95,935
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 2,424	$ 1,283
Accrued expenses	20,872	7,386
Operating lease liabilities, current	1,060	837
Total current liabilities	24,356	9,506
Operating lease liabilities	4,057	4,709
Total liabilities	28,413	14,215
Commitments and contingencies (See Note 8)		
Stockholders' equity:		
Preferred stock; $0.001 par value per share; 5,000,000 shares authorized as of December 31, 2024 and December 31, 2023; no shares issued and outstanding as of December 31, 2024 and December 31, 2023	—	—
Common stock, $0.001 par value per share; 115,000,000 shares authorized as of December 31, 2024 and December 31, 2023; 63,815,065 and 43,909,069 shares issued and outstanding as of December 31, 2024 and December 31, 2023, respectively	64	43
Additional paid-in capital	440,758	270,150
Accumulated deficit	(269,158)	(188,554)
Accumulated other comprehensive gain	148	81
Total stockholders' equity	171,812	81,720
Total liabilities and stockholders' equity	$ 200,225	$ 95,935

The accompanying notes are an integral part of these consolidated financial statements.

LARIMAR THERAPEUTICS, INC.

CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS
(In thousands, except share and per share data)

	Year Ended December 31,	
	2024	**2023**
Operating expenses:		
Research and development	$ 73,278	$ 27,670
General and administrative	17,612	14,088
Total operating expenses	90,890	41,758
Loss from operations	(90,890)	(41,758)
Other income, net	10,286	4,809
Net loss	$ (80,604)	$ (36,949)
Net loss per share, basic and diluted	$ (1.32)	$ (0.84)
Weighted average common shares outstanding, basic and diluted	61,256,084	43,901,241
Comprehensive loss:		
Net loss	$ (80,604)	$ (36,949)
Other comprehensive loss:		
Unrealized gain on marketable securities	67	112
Total other comprehensive gain	67	112
Total comprehensive loss	$ (80,537)	$ (36,837)

The accompanying notes are an integral part of these consolidated financial statements.

LARIMAR THERAPEUTICS, INC.

CONSOLIDATED STATEMENTS OF CHANGES IN STOCKHOLDERS' EQUITY
(In thousands, except share data)

	Common Stock Shares	Common Stock Par Value	Additional Paid-in Capital	Accumulated Deficit	Accumulated Other Comprehensive Gain	Total Stockholders' Equity
Balances as of December 31, 2023	43,909,069	$ 43	$ 270,150	$ (188,554)	$ 81	$ 81,720
Issuance of common stock, net	19,736,842	20	161,736	—	—	161,756
Vesting of restricted stock units	153,750	1	(1)	—	—	—
Exercise of stock options	15,404	—	77	—	—	77
Stock-based compensation expense	—	—	8,796	—	—	8,796
Unrealized gain on marketable debt securities	—	—	—	—	67	67
Net Loss	—	—	—	(80,604)	—	(80,604)
Balances as of December 31, 2024	63,815,065	$ 64	$ 440,758	$ (269,158)	$ 148	$ 171,812

	Common Stock Shares	Common Stock Par Value	Additional Paid-in Capital	Accumulated Deficit	Accumulated Other Comprehensive Gain (Loss)	Total Stockholders' Equity
Balances as of December 31, 2022	43,269,200	$ 43	$ 262,496	$ (151,605)	$ (31)	$ 110,903
Stock-based compensation expense	—	—	7,615	—	—	7,615
Exercise of stock options	11,466	—	33	—	—	33
Exercise of warrants	628,403	—	6	—	—	6
Unrealized gain on marketable securities	—	—	—	—	112	112
Net loss	—	—	—	(36,949)	—	(36,949)
Balances as of December 31, 2023	43,909,069	$ 43	$ 270,150	$ (188,554)	$ 81	$ 81,720

The accompanying notes are an integral part of these consolidated financial statements.

LARIMAR THERAPEUTICS, INC.

CONSOLIDATED STATEMENTS OF CASH FLOWS
(In thousands)

	Year Ended December 31, 2024	2023
Cash flows from operating activities:		
Net loss	$ (80,604)	$ (36,949)
Adjustments to reconcile net loss to net cash used in operating activities:		
Stock-based compensation expense	8,796	7,615
Lease expense	(189)	(82)
Depreciation expense	318	311
Net accretion of discount on marketable securities	(5,256)	(1,843)
Changes in operating assets and liabilities:		
Prepaid expenses and other current assets	(8,465)	(1,065)
Accounts payable	1,141	(403)
Accrued expenses	13,436	(1,022)
Other assets	63	(21)
Net cash used in operating activities:	(70,760)	(33,459)
Cash flows from investing activities:		
Purchase of property and equipment	(515)	(164)
Purchase of marketable securities	(227,872)	(101,233)
Maturities and sales of marketable securities	143,000	134,750
Net cash provided by (used in) investing activities	(85,387)	33,353
Cash flows from financing activities:		
Proceeds from sale of common stock, net of issuance costs	161,806	—
Proceeds from exercise of stock options and warrants	77	30
Net cash provided by financing activities	161,883	30
Net increase (decrease) in cash, cash equivalents and restricted cash	5,736	(76)
Cash, cash equivalents and restricted cash at beginning of period	28,088	28,164
Cash, cash equivalents and restricted cash at end of period	$ 33,824	$ 28,088
Supplemental disclosure of non-cash investing and financing activities:		
Leased assets obtained in exchange for new operating lease liabilities	$ 465	$ 790
Offering costs included in accounts payable and accrued expense	50	—
Proceeds from exercise of stock options included in other current assets	—	9

The accompanying notes are an integral part of these consolidated financial statements.

1.Description of Business and Basis of Presentation

Larimar Therapeutics, Inc., together with its subsidiary (the "Company" or "Larimar"), is a clinical-stage biotechnology company focused on developing treatments for patients suffering from complex rare diseases using its novel cell penetrating peptide technology platform. Larimar's lead product candidate, nomlabofusp (nomlabofusp is the International Nonproprietary Name and the United States Adopted Name for CTI-1601), is a subcutaneously administered, recombinant fusion protein intended to deliver human frataxin ("FXN"), an essential protein, to the mitochondria of patients with Friedreich's ataxia ("FA"). FA is a rare, progressive and fatal disease in which patients are unable to produce sufficient FXN due to a genetic abnormality.

Larimar has completed two Phase 1 clinical trials and a Phase 2 dose exploration trial, and has an ongoing Phase 2 OLE trial in patients with FA and an ongoing run-in study in adolescent patients with FA.

In May 2021, the Company reported positive top-line data from its Phase 1 FA program after completing dosing of the single ascending dose ("SAD") trial in December 2020 and of the multiple ascending dose ("MAD") trial in March 2021. Data from these trials demonstrated proof-of-concept by showing that daily subcutaneous injections of nomlabofusp for up to 13 days resulted in dose-dependent increases in FXN levels from baseline compared to placebo in all evaluated tissues (buccal cells, skin, and platelets). There were no serious adverse events associated with either the MAD or SAD trials.

In May 2023, the Company reported preliminary unblinded top-line data from the 25 mg cohort of the Phase 2 four-week, placebo-controlled, dose exploration trial of nomlabofusp in FA patients. Data from the cohort indicated nomlabofusp was generally well tolerated and showed increases in FXN levels from baseline compared to placebo in all evaluated tissues (skin and buccal cells) at day 14.

In July 2023, the FDA cleared initiation of a second cohort (50 mg) of the four-week, placebo-controlled, Phase 2 dose exploration trial of nomlabofusp in patients with FA and the initiation of the OLE trial with daily dosing of 25 mg.

In February 2024, the Company reported positive top-line data and successful completion of the Phase 2 dose exploration study. Nomlabofusp was generally well tolerated throughout the four-week treatment periods, had a predictable pharmacokinetic profile and led to 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. Patients who completed treatment in the Phase 2 dose exploration study or who previously completed a prior clinical trial of nomlabofusp are eligible to screen and possibly participate in the OLE study.

In March 2024, Larimar dosed the first patient in the OLE trial, evaluating daily subcutaneous injections of 25 mg of nomlabofusp self-administered or administered by a caregiver. The OLE study will evaluate the safety and tolerability, pharmacokinetics, and frataxin levels in peripheral tissues as well as other exploratory pharmacodynamic markers (lipid profiles and gene expression data) following long-term subcutaneous administration of nomlabofusp. In addition, following the completion of enrollment, as well as at least one participant completing one year of dosing, clinical assessments collected during the study will be compared to data from a matched control arm derived from participants in the Friedreich's Ataxia Clinical Outcome Measures Study ("FACOMS") database.

On May 30, 2024, Larimar announced that the FDA's Center for Drug Evaluation and Research ("CDER") had selected nomlabofusp as one of a few programs for participation in the Support for Clinical Trials Advancing Rare Disease Therapeutics ("START") Pilot Program. The objective of the program is to accelerate the development of drugs for rare diseases that lead to significant disability or death by facilitating frequent advice and regular communication with the FDA staff to expedite the review process of biologics and drugs.

In September 2024, the Company received access to the Medicines and Healthcare Regulatory Agency ("MHRA") Innovative Licensing and Access Pathway ("ILAP") for the treatment of adults and children with FA. The ILAP is facilitating patient access to novel treatments by accelerating time to market through opportunities for enhanced engagements with UK regulatory authorities and other stakeholders. Along with the receipt of the ILAP designation, nomlabofusp has already been granted orphan drug designations in the U.S. and the European Union (the "EU"), and access to the PRIME scheme in the EU.

In December 2024, the Company reported positive initial data from the ongoing OLE study. This data included safety, FXN levels, clinical, pharmacokinetic data and dose escalation:

•Nomlabofusp was generally well tolerated for up to 260 days in subjects. The most common adverse events were injection site reactions, with most being mild, brief in duration, and self-limited. Two participants had serious adverse events (one seizure and one anaphylactic reaction) that resolved within 24 hours and withdrew from the study;

•Long-term 25 mg tissue FXN levels showed positive mean change from baseline of 1.32 pg/µg in buccal cells and 9.28 pg/µg in skin cells at Day 90 and that 25 mg of nomlabofusp increased and maintained tissue FXN levels over time, increasing from a mean level of 15% of HV at baseline to 30% in buccal cells and from 16% to 72% in skin cells at Day 90;

•Tissue FXN levels appear to reach steady-state levels by Day 30 in buccal cells;

•Early clinical data showed trends toward improvement across a number of clinical outcomes for long-term 25 mg daily nomlabofusp including: (1) decreased values indicating early trends towards improvement in the Modified Friedreich Ataxia Rating Scale (mFARS) and the FARS-Activities of Daily Living ("ADL"), Modified Fatigue Impact Scale and 9 hole peg test at 90 days relative to baseline;

•Key pharmacokinetic data showed rapid absorption after subcutaneous administration with exposure appearing to reach steady state in plasma by day 30 with no further accumulation, which is consistent with data from our Phase 1 and Phase 2 studies.

Also in December 2024, Larimar announced that it was increasing the dose in the OLE to 50 mg of nomlabofusp daily for then currently enrolled and all future OLE study participants. In March 2025, the Company announced that its Safety Monitoring Team has deemed anaphylaxis as an adverse drug reaction likely associated with nomlabofusp and therefore, Larimar expects to see additional reactions. To reduce the risk of allergic reactions, including anaphylaxis, Larimar amended the OLE protocol to administer premedication for the first month of dosing. The OLE study is ongoing with seven sites activated and participants continuing to enroll. All study participants are currently receiving the 50 mg dose of nomlabofusp. The Company plans to provide an update on OLE data on at least 30 to 40 study participants, some of whom have been receiving nomlabofusp for more than a year, in September 2025.

In January 2025, Larimar initiated dosing of adolescents (12-17 years old) in a PK run-in study for pediatric patients with FA. Study participants in the PK run-in study are randomized 2:1 to receive either nomlabofusp at a weight-base dose expected to match the PK of adults receiving the 50 mg dose, or placebo, daily for seven days. Following assessment of safety and PK data, participants will be eligible to screen for the OLE study. The data from this cohort is expected to be presented during the nomlabofusp program update in September 2025.

In March 2025, Larimar 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. FDA recommended focusing on assessments of skin FXN concentrations rather than buccal FXN concentrations due to more consistent sampling and less variability. FDA acknowledged that data the Company recently submitted appear to support a relationship between increased FXN concentrations in skin cells and relevant tissues such as the heart, dorsal root ganglion and skeletal muscle. FDA also acknowledged that the nonclinical studies Larimar submitted were performed at relevant human doses. FDA also suggested that Larimar consider exploring the relationship between increases in FXN in skin and changes in pharmacodynamic (PD) markers such as lipid profiles and/or clinical measures to provide additional support for the use of FXN as an RLSE. Larimar plans to continue discussions with FDA regarding the adequacy of the safety data set for a BLA submission seeking accelerated approval targeted for year-end 2025.

Additionally, the Company has obtained feedback from both FDA and EMA on the global Phase 3 study protocol and is on track to initiate the study by mid-2025 with potential sites in the U.S., E.U., U.K, Canada, and Australia.

The Company is subject to risks and uncertainties common to pre-commercial companies in the biotechnology industry, including, but not limited to, development and commercialization by competitors of new technological innovations, dependence on key personnel, protection of proprietary technology, compliance with governmental regulations, failure to secure regulatory approval for its drug candidates or any other product candidates and the ability to secure additional capital to fund its operations. Product candidates under development will require extensive non-clinical and clinical testing and regulatory approval prior to commercialization. These efforts require significant amounts of additional capital, adequate personnel, infrastructure and extensive compliance-reporting

capabilities. Even if the Company's drug development efforts are successful, it is uncertain when, if ever, it will realize significant revenue from product sales.

Basis of Presentation

The consolidated financial statements include the accounts of Larimar and its wholly owned subsidiary. All intercompany balances and transactions have been eliminated. The accompanying consolidated financial statements have been prepared in conformity with U. S. Generally Accepted Accounting Principles ("GAAP").

Liquidity and Capital Resources

The Company's consolidated financial statements have been presented on the basis that it will continue as a going concern, which contemplates the realization of assets and satisfaction of liabilities in the normal course of business.

Since its inception, the Company has incurred significant recurring operating losses and negative cash flows from operations. The Company has incurred net losses of $80.6 million and $36.9 million for the years ended December 31, 2024, and 2023, respectively, and cash flow used in operations of $70.8 million and $33.5 million for the years ended December 31, 2024 and 2023, respectively. In addition, as of December 31, 2024, the Company had an accumulated deficit of $269.2 million. The Company expects to continue to generate operating losses for the foreseeable future. As of December 31, 2024, the Company had approximately $183.5 million of cash, cash equivalents and marketable securities available for use to fund its operations and capital requirements.

The Company has funded its operations to date primarily with proceeds from sales of common stock and proceeds from the sale of prefunded warrants for the purchase of common stock, the acquisition in 2020 of cash, cash equivalents and marketable securities upon the merger with Zafgen, Inc. ("Zafgen") and, prior to the 2020 merger with Zafgen, capital contributions from Chondrial Holdings, LLC.

In February 2024, the Company sold 19,736,842 shares of its common stock in an underwritten public offering at a price of $8.74 per share and received net proceeds of approximately $161.8 million.

In accordance with Accounting Standards Codification ("ASC") 205, "Disclosure of Uncertainties about an Entity's Ability to Continue as a Going Concern", the Company has evaluated whether there are certain conditions and events, considered in the aggregate, that raise substantial doubt about the Company's ability to continue as a going concern within one year after the date that these consolidated financial statements are issued. As of the issuance date of these consolidated financial statements, the Company expects its cash, cash equivalents and marketable securities will be sufficient to fund its forecasted operating expenses and capital expenditure requirements into the second quarter of 2026.

The Company has not yet commercialized any products and does not expect to generate revenue from the commercial sale of any products for several years, if at all. The Company expects that its research and development and general and administrative expenses will continue to increase and, as a result, that it will need additional capital to fund its future operating and capital requirements. Unless and until the Company can generate substantial revenue, management continuously evaluates different strategies to obtain the required funding for future operations. These strategies include seeking additional funding through a combination of public or private equity offerings, debt or royalty financings, collaborations and licensing arrangements, strategic partnerships with pharmaceutical and/or larger biotechnology companies, or other sources. The incurrence of indebtedness would result in increased payment obligations and the Company may be required to agree to certain restrictive covenants, such as limitations on its ability to incur additional debt, limitations on its ability to acquire, sell or license intellectual property rights, minimum required cash balances and other operating restrictions that could adversely impact the Company's ability to conduct its business. Any additional fundraising efforts may divert the Company's management from their day-to-day activities, which may adversely affect its ability to develop and commercialize its product candidates.

There can be no assurance that the Company will be able to raise sufficient additional capital on acceptable terms, if at all. If such additional financing is not available on satisfactory terms, or is not available in sufficient amounts, or if the Company does not have sufficient authorized shares, the Company may be required to delay, limit, or eliminate the development of business opportunities and its ability to achieve its business objectives, its competitiveness, and its business, financial condition, and results of operations will be materially adversely affected. The Company could also be required to seek funds through arrangements with collaborative partners or otherwise at an earlier stage than otherwise would be desirable and it may be required to relinquish rights to some of its technologies or product candidates or otherwise agree to terms unfavorable to it, any of which may have a material

adverse effect on the Company's business, operating results and prospects. In addition, geopolitical tensions, volatility of capital markets, and other adverse macroeconomic events, including those due to inflationary pressures, rising interest rates, bank instability and the ability of the U.S. government to manage federal debt limits as well as the potential impact of other health crises on the global financial markets may reduce the Company's ability to access capital, which could negatively affect its liquidity and ability to continue as a going concern.

If the Company is unable to obtain sufficient funding when needed and/or on acceptable terms, the Company may be required to significantly curtail, delay or discontinue one or more of its research and development programs, the manufacture of clinical and commercial supplies, product portfolio expansion, pre commercialization efforts and/or commercial operations, which could adversely affect its business prospects, or the Company may be unable to continue operations. If the timing of the Company's clinical assumptions are delayed or if there are other forecasted assumption changes that negatively impact its operating plan, the Company would reduce expenditures in order to further extend cash resources.

2.Summary of Significant Accounting Policies

Use of Estimates

The preparation of consolidated financial statements in conformity with GAAP requires management to make estimates and assumptions that affect the reported amounts of assets and liabilities and disclosure of contingent assets and liabilities at the date of the financial statements and the reported amounts of expenses during the reporting period. This process involves reviewing open contracts and purchase orders, communicating with our personnel and outside vendors to identify services that have been performed on our behalf and estimating the level of service performed and the associated costs incurred for the services when we have not yet been invoiced or otherwise notified of the actual costs. Significant estimates and assumptions reflected in these consolidated financial statements include, but are not limited to, the accrual of research and development expense, the recording as prepaid expense of payments made in advance of the actual provision of goods or services, valuation of stock-based awards and valuation of leases. Due to inherent uncertainty involved in making estimates, actual results reported in future periods may be affected by changes in these estimates. On an ongoing basis, the Company evaluates its estimates and assumptions.

Concentrations of Credit Risk and Significant Suppliers

Financial instruments that potentially subject the Company to concentrations of credit risk consist principally of cash, cash equivalents and marketable securities. Cash balances may be held in financial institutions which may exceed federally insured limits. The Company has not experienced realized losses related to its cash, cash equivalents or marketable securities.

The Company is highly dependent on third-party manufacturers to supply products for research and development activities in its programs, to scale and optimize their manufacturing processes and, ultimately, to provide commercial supply. The Company relies and expects to continue to rely on a small number of manufacturers to supply it with its requirements for drug substance and formulated drugs related to these programs. The drug substance which is in frozen liquid form for nomlabofusp is currently manufactured for the Company by a third-party manufacturer, and the frozen liquid form of drug product is made at another manufacturer. The Company is undertaking a program with a third manufacturer to begin to produce a lyophilized version of the drug product from the same drug substance, that, once available, the Company intends to use in certain of its future planned clinical trials. The Company’s 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.

Cash and Cash Equivalents

The Company considers all highly liquid investments with maturities of three months or less at the date of purchase to be cash equivalents. Cash equivalents consisted of money market funds as of December 31, 2024 and 2023.

Marketable securities

Marketable securities consist of debt investments with original maturities greater than ninety days. The Company classifies its marketable securities as available-for-sale. Accordingly, these investments are recorded at fair value, which is based on quoted market prices. When the fair value is below the amortized cost the amount of the expected credit loss is estimated. The credit-related impairment amount, if any, would be recognized in net

income; the remaining impairment amount and unrealized gains are reported as a component of accumulated other comprehensive income in stockholders' equity. Credit losses, if any, are recognized through the use of an allowance for credit losses account and subsequent improvements in expected credit losses are recognized as a reversal of the allowance account. If the Company has the intent to sell the security or if it is more likely than not that the Company would be required to sell the security prior to recovery of its amortized cost basis, the allowance for credit loss would be written off and the excess of the amortized cost basis of the asset over its fair value would be recorded in net income.

Property and Equipment

Property and equipment are stated at cost less accumulated depreciation. Depreciation expense is recognized using the straight-line method over a five or seven-year estimated useful life for equipment, furniture and fixtures and office equipment. Leasehold improvements are amortized over the shorter of the asset life or the term of the lease agreement. Expenditures for repairs and maintenance of assets are charged to expense as incurred. Upon retirement or sale, the cost and related accumulated depreciation of assets disposed of are removed from the accounts and any resulting gain or loss is included in loss from operations.

Impairment of Long-Lived Assets

Long-lived assets consist of property and equipment, net, and right-of-use assets. Long-lived assets to be held and used are tested for recoverability whenever events or changes in business circumstances indicate that the carrying amount of the assets may not be fully recoverable. Factors that the Company considers in deciding when to perform an impairment review include significant underperformance of the business in relation to expectations, significant negative industry or economic trends, and significant changes or planned changes in the use of the assets. If an impairment review is performed to evaluate a long-lived asset for recoverability, the Company compares forecasts of undiscounted cash flows expected to result from the use and eventual disposition of the long-lived asset to its carrying value. Any impairment loss, if indicated, is measured as the amount by which the carrying amount of the asset exceeds the estimated fair value of the asset.

Research and Development Costs

Costs associated with internal research and development and external research and development services, including drug manufacturing and scale- up, other drug development and testing costs, clinical studies and non-clinical studies, are expensed as incurred. Research and development expenses include costs for salaries, employee benefits, subcontractors, facility-related expenses, depreciation, stock-based compensation, third-party license fees, laboratory supplies, and external costs of outside vendors engaged to conduct discovery, non-clinical and clinical development activities and clinical trials as well as to manufacture clinical trial materials and to scale up the manufacturing process, and other costs. The Company recognizes external research and development costs based on an evaluation of the progress to completion of specific tasks using information provided to the Company by its key service providers.

Nonrefundable advance payments for goods or services to be received in the future for use in research and development activities are recorded as prepaid expenses. Such prepaid expenses are recognized as an expense when the goods have been delivered or the related services have been performed, or when it is no longer expected that the goods will be delivered, or the services rendered.

Upfront payments, milestone payments and annual maintenance fees under license agreements are currently expensed in the period in which they are incurred.

Patent Costs

All patent-related costs incurred in connection with filing and prosecuting patent applications are expensed as incurred due to the uncertainty about the recovery of the expenditure. Amounts incurred are classified as general and administrative expenses.

Stock-Based Compensation

The Company measures all stock-based awards granted to employees and directors based on the fair value on the date of grant using the Black-Scholes option-pricing model. Compensation expense of those awards is recognized over the requisite service period, which is the vesting period of the respective award. Typically, the Company issues awards with only service-based vesting conditions and records the expense for these awards using the straight-line method. The Company accounts for forfeitures as they occur.

The Company classifies stock-based compensation expense in its consolidated statements of operations and comprehensive loss in the same manner in which the award recipient's payroll costs are classified or in which the award recipient's service payments are classified.

Prior to May 28, 2020, the Company had been a private company and lacked company-specific historical and implied volatility information for its common stock. Prior to January 1, 2023, the Company estimated its expected common stock price volatility solely based on the historical volatility of publicly traded peer companies. Beginning on January 1, 2023, based on the availability of sufficient historical trading data of the Company's own common stock on the Nasdaq Global Market to calculate accurately its volatility, the Company began blending its volatility starting from June 2020 (following its merger with Zafgen in 2020) to the date of each stock-based award, and weighing the volatility of its peer group for the amount of time from May 31, 2020 backwards so that the blended volatility equals the expected term of the related stock-based award. The expected term of the Company's stock options has been determined utilizing the "simplified" method for awards that qualify as "plain-vanilla" options. The risk-free interest rate is determined by reference to the U.S. Treasury yield curve in effect at the time of grant of the award for time periods approximately equal to the expected term of the award. The expected dividend yield considers the fact that the Company has never paid cash dividends on common stock and does not expect to pay any cash dividends in the foreseeable future.

Segment Information

Operating segments are defined as components of an enterprise for which separate discrete information is available for evaluation by the chief operating decision maker, or decision making group, in deciding how to allocate resources in assessing performance. The Company is managed on a consolidated basis and has one operating and reportable segment related to the development of clinical and preclinical product candidates for the development of the Company's proprietary new therapies, primarily nomlabofusp, the Company's life science segment. The Company's chief operating decision maker ("CODM") is the Company's chief executive officer ("CEO").

The accounting policies of the life science segment are the same as those described in the summary of significant accounting policies. The CODM assesses performance for the Company and its reportable segment based on net loss, which is reported on the consolidated Statements of Operations. The measure of segment assets is reported on the balance sheet as total consolidated assets. All long-lived assets are located in the U.S.

To date, the Company has not generated any product revenue. The Company expects to continue to incur significant expenses and operating losses for the foreseeable future as it advances product candidates through all stages of development and clinical trials and regulatory approval.

The CODM uses consolidated financial information, including consolidated net loss, to evaluate performance, forecast future period financial results, allocate resources for the company by, among other things, comparing budgeted to actual results.

The table below summarizes the significant expense categories regularly reviewed by the CODM for the twelve months ended December 31, 2024 and 2023:

	2024	2023
Operating expenses:		
Technical operations	$ 44,805	$ 6,781
Development	24,361	15,733
Nomlabofusp support	4,112	5,156
General and administrative	15,944	14,088
Commercial	1,668	—
Total operating expenses	$ 90,890	$ 41,758
Other income, net	(10,286)	(4,809)
Net loss	$ (80,604)	$ (36,949)

Income Taxes

The Company accounts for income taxes using the asset and liability method, which requires the recognition of deferred tax assets and liabilities for the expected future tax consequences of events that have been recognized in the consolidated financial statements or in the Company's tax returns. Deferred tax assets and liabilities are determined based on the difference between the financial statement and tax basis of assets and liabilities using enacted tax rates in effect for the year in which the differences are expected to reverse. Changes in deferred tax assets and liabilities are recorded in the provision for income taxes. The Company assesses the likelihood that its deferred tax assets will be recovered from future taxable income and, to the extent it believes, based upon the weight of available evidence, that it is more likely than not that all or a portion of the deferred tax assets will not be realized, a valuation allowance is established through a charge to income tax expense. Potential for recovery of deferred tax assets is evaluated by estimating the future taxable profits expected and considering prudent and feasible tax planning strategies.

The Company accounts for uncertainty in income taxes recognized in the consolidated financial statements by applying a two-step process to determine the amount of tax benefit to be recognized. First, the tax position must be evaluated to determine the likelihood that it will be sustained upon external examination by the taxing authorities. If the tax position is deemed more-likely-than-not to be sustained, the tax position is then assessed to determine the amount of benefit to recognize in the consolidated financial statements. The amount of the benefit that may be recognized is the largest amount that has a greater than 50% likelihood of being realized upon ultimate settlement. The provision for income taxes includes the effects of any resulting tax reserves, or unrecognized tax benefits, that are considered appropriate as well as the related net interest and penalties.

Comprehensive Gain (Loss)

Comprehensive gain (loss) includes net loss as well as other changes in stockholders' equity that result from transactions and economic events other than those with stockholders. For the years ended December 31, 2024 and 2023, the Company's only element of other comprehensive gain (loss) was unrealized gain (loss) on marketable securities.

Net Loss Per Share

Basic net loss per share is computed by dividing the net loss attributable to common stockholders by the weighted average number of common shares outstanding for the period. Prior to August 11, 2023, basic shares outstanding includes the weighted average effect of the Company's prefunded warrants issued in June 2020, the exercise of which requires little or no consideration for the delivery of shares of common stock. These prefunded warrants were exercised on August 11, 2023 and the Company received cash proceeds of less than $0.1 million. Accordingly, the 628,403 shares were issued upon the exercise of these warrants and are included in issued and outstanding common stock.

Diluted net loss per share attributable to common stockholders is computed by dividing the diluted net loss attributable to common stockholders by the weighted average number of common shares, including potentially dilutive common stock equivalents assuming the dilutive effect of outstanding stock options, outstanding restricted stock units, and unvested restricted common shares, as determined using the treasury stock method. For periods in

which the Company has reported net losses (all periods since inception), diluted net loss per common share attributable to common stockholders is the same as basic net loss per common share attributable to common stockholders, since dilutive common stock equivalents are not assumed to have been issued if their effect is antidilutive.

The Company excluded 7,135,390 and 4,888,502 options to purchase common stock, outstanding as of December 31, 2024 and 2023 respectively, from the computation of diluted net loss per share for the twelve months ended December 31, 2024 and 2023, respectively, because they had an anti-dilutive impact due to the net loss incurred for the periods.

Recently Issued and Adopted Accounting Pronouncements

From time to time, new accounting guidance is issued by the FASB or other standard setting bodies that is adopted by us as of the effective date or, in some cases where early adoption is permitted, in advance of the effective date. We have assessed the recently issued guidance that is not yet effective and believe the new guidance will not have a material impact on the consolidated results of operations, cash flows or financial position.

In November 2023, the Financial Accounting Standards Board ("FASB") issued ASU 2023-07, Segment Reporting (Topic 280): Improvements to Reportable Segment Disclosures ("ASU 2023-07"). ASU 2023-07 requires disclosure of incremental segment information on an annual and interim basis. The amendments also require companies with a single reportable segment to provide all disclosures required by this amendment and all existing segment disclosures in ASC 280, Segment Reporting. The amendments are effective for fiscal years beginning after December 15, 2023, and interim periods beginning after December 15, 2024. The Company adopted ASU 2023-07, effective December 31, 2024, in these consolidated financial statements. ASU 2023-07 only impacted the disclosures and did not impact the consolidated financial statements. See Note 2, Segment Information, for disclosures related to the adoption of ASU 2023-07.

Recently Issued Accounting Pronouncements Not Yet Adopted

In December 2023, the FASB issued ASU No. 2023-09, "Improvements to Income Tax Disclosures (Topic 740)," which requires entities to disclose in their rate reconciliation table additional categories of information about federal, state and foreign income taxes as well as additional information about reconciling items if certain quantitative thresholds are met. This ASU will require all entities to disclose income taxes paid, net of refunds, disaggregated by federal (national), state and foreign taxes for annual periods and to disaggregate the information by jurisdiction based on a quantitative threshold. All entities are required to apply the guidance prospectively, with the option to apply it retrospectively. The ASU is effective for fiscal years beginning after December 15, 2024, with early adoption permitted. The Company has determined it will adopt this ASU on January 1, 2025, the adoption of which is not expected to have a material impact on the Company's consolidated results of operations or cash flows.

In November 2024, the FASB issued ASU 2024-03, "Income Statement - Reporting Comprehensive Income - Expense Disaggregation Disclosures (Subtopic 220-40): Disaggregation of Income Statement Expenses", which requires disaggregated disclosures in the notes of the financial statements of certain categories of expenses that are included in expense line items on the face of the income statement. This ASU is effective for annual periods beginning after December 15, 2026, and interim periods within annual reporting periods beginning after December 15, 2027. Early adoption is permitted. The Company will evaluate the impact adopting ASU 2024-03 will have on the Company's consolidated financial statements and disclosures.

3.Fair Value Measurements and Cash Equivalents/Marketable Securities

Fair Value Measurements

The Company's assets and liabilities that are measured at fair value on a recurring basis as of December 31, 2024 and December 31, 2023 are measured in accordance with the standards of ASC 820, *Fair Value Measurements and Disclosures*, which establishes a three-level valuation hierarchy for measuring fair value and expands financial statement disclosures about fair value measurements. The valuation hierarchy is based on upon the transparency of inputs to the valuation of an asset or liability as of the measurement date. The three levels are defined as follows:

Level – 1	Inputs to the valuation methodology are quoted prices (unadjusted) for identical assets or liabilities in active markets.
Level – 2	Inputs to the valuation methodology include quoted prices for similar assets and liabilities in active markets, and inputs that are observable for the asset or liability, either directly or indirectly, for substantially the full term of the financial instrument.
Level – 3	Inputs to the valuation methodology are unobservable and significant to the fair value measurement.

The Company's financial instruments consist primarily of cash and cash equivalents, marketable securities, accounts payable and accrued liabilities. For accounts payable and accrued liabilities, the carrying amounts of these financial instruments as of December 31, 2024 and 2023 were considered representative of their fair values due to their short term to maturity.

The following tables summarize the Company's cash equivalents and marketable securities as of December 31, 2024 and 2023:

	Total	Quoted Prices in Active Markets (Level 1)	Significant Other Observable Inputs (Level 2)	Significant Unobservable Inputs (Level 3)
		(in thousands)		
December 31, 2024				
Cash equivalents:				
Money market funds invested in government securities	$ 26,702	$ 26,702	$ —	$ —
Total cash equivalents	26,702	26,702	—	—
Marketable securities:				
U.S. Treasury Bills	2,947	2,947	—	—
U.S. Government securities	147,289	—	147,289	—
Total marketable securities	150,236	2,947	147,289	—
Total cash equivalents and marketable securities	$ 176,938	$ 29,649	$ 147,289	$ —
December 31, 2023				
Cash equivalents:				
Money market funds invested in government securities	$ 24,701	$ 24,701	$ —	$ —
Total cash equivalents	24,701	24,701	—	—
Marketable securities:				
U.S. Treasury Bills	17,334	17,334	—	—
U.S. Government securities	35,719	—	35,719	—
Corporate bonds	6,988	—	6,988	—
Total marketable securities	60,041	17,334	42,707	—
Total cash equivalents and marketable securities	$ 84,742	$ 42,035	$ 42,707	$ —

The accrued interest receivable related to the Company's investments was $1.1 million and $0.3 million as of December 31, 2024 and 2023, respectively, and is included in prepaid expenses and other current assets on the consolidated balance sheet.

The Company classifies its money market funds and U.S. treasury bills, which are valued based on quoted market prices in active markets with no valuation adjustment, as Level 1 assets within the fair value hierarchy.

The Company classifies its investments in U.S. government and agency securities, corporate commercial paper, and corporate bonds, if any, as Level 2 assets within the fair value hierarchy. The fair values of these investments are estimated by taking into consideration valuations obtained from third-party pricing services. The pricing services utilize industry standard valuation models, including both income- and market-based approaches, for which all significant inputs are observable, either directly or indirectly, to estimate fair value. These inputs include reported trades of and broker/dealer quotes on the same or similar securities, issuer credit spreads, benchmark securities, prepayment/default projections based on historical data and other observable inputs.

As of December 31, 2024, the unrealized loss for available-for-sale investments were non-credit related, and the Company does not intend to sell the investments that were in an unrealized loss position, nor will it be required to sell those investments before recovery of their amortized cost basis, which may be maturity. As of December 31, 2024 and 2023, no allowances for credit losses for the Company's investments were recorded. During the twelve months ended December 31, 2024 and 2023, the Company did not recognize any impairment losses related to investments.

As of December 31, 2024 and 2023, the Company's cash equivalents and marketable securities consisted of a U.S. government money market fund, U.S. Treasury Bills and U.S. government and agency securities, all held in our name in a separate custody account with U.S. Bank. The U.S. government money market fund has same-day liquidity access and the U.S. government and agency securities all have maturities of 360 days or less.

Marketable Securities

The following tables summarize the Company's marketable securities as of December 31, 2024 and 2023:

	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Value
		(in thousands)		
December 31, 2024				
Assets:				
U.S. Treasury Bills	2,945	2	—	2,947
U.S. Government securities	147,143	166	(20)	147,289
Total marketable securities	$ 150,088	$ 168	$ (20)	$ 150,236
December 31, 2023				
Assets:				
U.S. Treasury Bills	17,330	4	—	17,334
U.S. Government securities	35,653	66	—	35,719
Corporate bonds	6,977	11	—	6,988
	$ 59,960	$ 81	$ —	$ 60,041

4.Prepaid Expenses and Other Current Assets

Prepaid expenses and other current assets consisted of the following:

	December 31, 2024	December 31, 2023
	(in thousands)	
Prepaid research and development expenses	$ 9,780	$ 1,994
Interest receivable	1,054	332
Prepaid insurance	499	682
Other prepaid expenses and other assets	517	377
Total prepaid expenses and other assets	$ 11,850	$ 3,385

5.Fixed Assets

Fixed assets, net consisted of the following:

	Useful Life	December 31, 2024	December 31, 2023
		(in thousands)	
Computer equipment	5 years	$ 117	$ 117
Lab equipment	5 years	1,707	1,192
Furniture and fixtures	7 years	555	555
Leasehold improvements	lease term	45	45
Total property, plant and equipment		2,424	1,909
Less: Accumulated depreciation		(1,543)	(1,225)
Property, plant and equipment, net		$ 881	$ 684

Depreciation expense was $0.3 million and $0.2 million for the years ended December 31, 2024 and 2023, respectively. In addition, for the years ended December 31, 2024 and 2023, there was $0.1 million of depreciation related to sublet assets recorded as other expense.

6.Accrued Expenses

Accrued expenses consisted of the following:

	December 31, 2024	December 31, 2023
	(in thousands)	
Accrued research and development expenses	$ 17,057	$ 4,594
Accrued payroll and related expenses	3,254	2,365
Accrued other	561	427
Total accrued expenses and other current liabilities	$ 20,872	$ 7,386

7.Stockholders' Equity and Stock Options

Common Stock and Prefunded warrants

On May 28, 2020, the Company entered into a securities purchase agreement with certain accredited investors (the "Purchasers") for the sale by the Company in a private placement of 6,105,359 shares of the Company's common stock and prefunded warrants to purchase an aggregate of 628,403 shares of the Company's common stock, for a price of $11.88 per share of the common stock and $11.87 per prefunded warrant. The prefunded warrants were exercisable at an exercise price of $0.01 and were exercisable indefinitely. In August 2023, the 628,403 shares of prefunded warrants were exercised and the Company received cash proceeds of six thousand two hundred and eighty-four dollars. The private placement closed on June 1, 2020. The aggregate gross proceeds for the issuance and sale of the common stock and prefunded warrants were $80.0 million, transaction costs totaled $4.6 million and resulted in net proceeds of $75.4 million. The Company's Registration Statement on Form S-3, filed with the SEC on June 26, 2020, registered the resale of 6,105,359 shares of common stock sold and the 628,403 shares of common stock underlying the prefunded warrants. MTS Health Partners served as placement agent to the Company in connection with the private placement. As partial compensation for these services, the Company issued MTS Health Partners 35,260 shares of common stock.

As of December 31, 2024, the Company's Amended and Restated Certificate of Incorporation, authorized the Company to issue up to 115,000,000 shares of $0.001 par value common stock, of which 63,815,065 shares were issued and outstanding, and up to 5,000,000 shares of $0.001 par value undesignated preferred stock, of which no shares were issued or outstanding. The voting, dividend and liquidation rights of the holders of the Company's common stock are subject to and qualified by the rights, powers and preferences of the holders of the preferred stock. Each share of common stock entitles the holder to one vote on all matters submitted to a vote of the Company's stockholders. Common stockholders are entitled to receive dividends, as may be declared by the board of directors of the Company (the "Board"), if any. No cash dividends have been declared or paid to date.

In February 2024, the Company sold 19,736,842 shares of its common stock in an underwritten public offering price of $8.74 per share and received net proceeds of approximately $161.8 million.

2022 ATM Agreement

In November 2022, the Company entered into a sales agreement (the "2022 ATM Agreement") with a Guggenheim Securities, LLC in connection with the establishment of an "at-the-market" offering program under which the Company could sell up to an aggregate of $50.0 million of shares of common stock (the "ATM Shares") from time to time.

In February 2024, in connection with the underwritten public offering described above, the Company terminated the 2022 ATM Agreement. No ATM Shares were ever sold pursuant to the 2022 ATM Agreement.

In May 2024, the Company entered into a sales agreement (the "ATM Agreement") with Guggenheim Securities, LLC in connection with the establishment of an "at-the-market" offering program under which the Company could sell up to an aggregate of $100 million of shares of common stock (the "ATM Shares") from time to time. To date, no sales of common stock have been made under this ATM Agreement.

2020 Equity Incentive Plan

The Board adopted the 2020 Equity Incentive Plan (the "2020 Plan") on July 16, 2020 and the stockholders of the Company approved the 2020 Plan on September 29, 2020. The 2020 Plan replaced the predecessor plans (the "Prior Plans") that the Company assumed following its merger with Zafgen in May 2020. Options outstanding under the Prior Plans will remain outstanding, unchanged, and subject to the terms of the Prior Plans and the respective award agreements, and no further awards will be made under the Prior Plans. However, if any award previously granted under the Prior Plans, expires, terminates, is canceled, or is forfeited for any reason after the approval of the 2020 Plan, the shares subject to that award will be added to the 2020 Plan share pool so that they can be utilized for new grants under the 2020 Plan.

The 2020 Plan provides for the grant of incentive stock options ("ISOs"), nonstatutory stock options ("NSOs"), stock appreciation rights, restricted stock awards, restricted stock unit awards, performance-based restricted stock units, and cash or other stock-based awards. ISOs may be granted only to the Company's employees, including the Company's officers, and the employees of the Company's affiliates. All other awards may be granted to the Company's employees, including the Company's officers, the Company's non-employee directors and consultants, and the employees and consultants of the Company's affiliates.

The maximum number of shares that may be issued in respect of any awards under the 2020 Plan is the sum of: (i) 1,700,000 shares plus (ii) an annual increase on January 1, 2021 and each anniversary of such date thereafter through January 1, 2030, equal to the lesser of (A) 4% of the shares issued and outstanding on the last day of the immediately preceding fiscal year, or (B) such smaller number of shares as determined by the Board (collectively, the "Plan Limit"). The maximum aggregate number of shares that may be issued under the 2020 Plan is 8,000,000 over the ten-year term of the 2020 Plan.

As permitted by the 2020 Plan, the Company added 2,552,603 and 1,756,363 shares available for grant to the 2020 Plan on January 1, 2025 and January 1, 2024, respectively, increasing the maximum number of shares of the Company's common stock that may be issued under the 2020 Plan as of January 1, 2025 to 3,179,529 shares.

During the twelve months ended December 31, 2024 and 2023, respectively, options to purchase 1,242 and 224,437 shares issued under the Prior Plans were canceled and became available for grant under the 2020 Plan. In addition, as of December 31, 2024, 626,926 shares of common stock were available for grant under the 2020 Plan.

Stock Valuation

The following table presents, on a weighted average basis, the assumptions used in the Black-Scholes option-pricing model to determine the grant-date fair value of stock options granted to employees:

	2024	2023
Risk-free interest rate	4.12%	3.72%
Expected term (in years)	6.22	6.23
Expected volatility	95%	94%
Dividend yield	0.00%	0.00%

Stock Options

The following table summarizes the Company's stock option activity for the twelve months ended December 31, 2024 (amounts in millions, except for share and per share data):

	Number of Shares	Weighted Average Exercise Price	Weighted Average Remaining Contractual Term (in years)	Aggregate Intrinsic Value (a) (in millions)
Outstanding as of December 31, 2023	4,273,502	$ 9.06	7.8	
Granted	2,235,377	5.35		
Exercised	(15,404)	5.00		
Forfeited/Expired	(57,088)	10.08		
Outstanding as of December 31, 2024	6,436,387	$ 7.78	7.6	$ 0.4
Exercisable as of December 31, 2024	3,094,992	$ 10.45	6.3	$ 0.2
Vested and expected to vest as of December 31, 2024	6,436,387	$ 7.78	7.6	$ 0.4

(a)The aggregate intrinsic value is calculated as the difference between the exercise price of the underlying options and the fair value of the common stock for the options that were in the money at December 31, 2024.

2024 Option Grants

During the twelve months ended December 31, 2024, the Company granted options to purchase 2,235,377 shares of common stock to employees and directors under the 2020 Plan. The options have an exercise price equal to the closing stock price as of the grant date. Of the 2,235,377 options granted, 2,140,377 were granted to employees and vest over four years, with 25% vesting on the first anniversary of the grant and the remainder vesting in equal monthly installments thereafter. The remaining 95,000 options were annual grants to the Company's directors and vest one year from the grant date. The weighted-average grant date fair value of options granted under the 2020 Plan during the twelve months ended December 31, 2024 was $4.25.

As of December 31, 2024, total unrecognized compensation expense related to unvested stock options granted under the 2020 Plan was $10.7 million, which is expected to be recognized over a weighted average period of 2.63 years.

Inducement Stock Option Grant

There were no inducement awards granted in the twelve months ended December 31, 2024.

As of December 31, 2024, total unrecognized compensation expense related to unvested inducement options granted under the 2020 Plan was $0.7 million, which is expected to be recognized over a weighted average period of 2.28 years.

Restricted Stock Units

RSUs were granted under the 2020 Plan to the Company's employees in order to maintain retention of key employees. The value of an RSU award is based on the Company's stock price on the date of the grant. The shares underlying the RSUs are not issued until the RSUs vest.

Activity with respect to the Company's RSUs during the twelve months ended December 31, 2024 was as follows (in millions, except share, contractual term, and per share data):

	Number of Shares	Weighted Average Grant Date Fair Value	Weighted Average Remaining Contractual Term (in years)	Aggregate Intrinsic Value (a) (in millions)
Outstanding as of December 31, 2023	615,000	$ 4.94	1.6	
Restricted stock units granted	245,372	4.21		
Restricted stock units vested	(153,750)	4.94		
Restricted stock units forfeited	(7,619)	4.64		
Outstanding as of December 31, 2024	699,003	$ 4.69	1.2	$ 2.7
Unvested and expected to vest as of December 31, 2024	699,003	$ 4.69	1.2	$ 2.7

Restricted Stock Unit Grants

During the twelve months ended December 31, 2024, the Company granted 245,372 shares of RSUs to employees under the 2020 Plan. The RSUs vest annually over four years and have a weighted-average grant date fair value during the twelve months ended December 31, 2024 of $4.21.

As of December 31, 2024, total unrecognized compensation expense for RSUs was $2.3 million, which is expected to be recognized over a weighted-average period of 2.4 years.

January 2025 Option, Restricted Stock Unit Grants and Performance-Based Restricted Stock Units

On January 22, 2025, the Company granted options to purchase 2,084,162 shares of common stock, 347,360 shares of restricted stock units and 200,000 Performance-based restricted stock units to employees under the 2020 Plan. The options have an exercise price equal to the closing stock price as of the grant date, and vest over four years, with 25% vesting on the first anniversary of the grant and the remainder vesting in equal monthly installments thereafter. The shares of restricted stock will vest annually over four years. The performance-based restricted stock units were granted to the Company's named executive officers. The grants included two performance milestones. The fist milestone, which relates to 20% of each grant, will vest if the FDA accepts the filing of a biologic license application for accelerated approval of nomlabofusp by December 31, 2026. The second milestone, representing 80% of each grant will vest if the FDA approves the Company nomlabofusp BLA within 12 months of the BLA acceptance (no later than December 31, 2027.) If the first milestone is not achieved by December 31, 2026, the full grant will be forfeited.

Stock-Based Compensation

Stock-based compensation expense was classified in the consolidated statements of operations as follows:

	Year Ended December 31,	
	2024	2023
	(in thousands)	
Research and development	$ 4,053	$ 3,077
General and administrative	4,743	4,538
	$ 8,796	$ 7,615

8.Commitments and Contingencies

Intellectual Property Licenses

The Company is party to an exclusive License Agreement (the "WFUHS License"), dated November 30, 2016, as amended, with Wake Forest University Health Sciences ("WFUHS") and an exclusive License Agreement (the "IU License"), dated November 30, 2016, as amended, with Indiana University ("IU"). Such agreements provide for a transferable, worldwide license to certain patent rights regarding technology used by the Company with respect to the development of nomlabofusp. Both agreements continue from their effective date through the last to date of expiration of the licensed patents, unless earlier terminated by either party in accordance with their terms.

In partial consideration for the right and license granted under these agreements, the Company will pay each of WFUHS and IU a royalty of a low single digit percentage of net sales of licensed products depending on whether there is a valid patent covering such products. As additional consideration for these agreements, the Company is obligated to pay each of WFUHS and IU certain milestone payments of up to $2.6 million in the aggregate upon the achievement of certain developmental milestones, which commenced with the enrollment of the first patient in a Phase 1 clinical trial. The Company enrolled the first patient in its SAD trial on December 11, 2019 and paid WFUHS and IU less than $0.1 million. The Company will also pay each of WFUHS and IU sublicensing fees ranging from a high-single digit to a low double-digit percentage of sublicense consideration depending on the Company's achievement of certain regulatory milestones as of the time of receipt of the sublicense consideration. The Company is also obligated to reimburse WFUHS and IU for patent-related expenses. In the event that the Company disputes the validity of any of the licensed patents, the royalty rate would be tripled during such dispute. The Company is also obligated to pay to IU a minimum annual royalty of less than $0.1 million per annum.

In the event that the Company is required to pay IU consideration, then the Company may deduct 20% of such IU consideration on a dollar-for-dollar basis from the consideration due to WFUHS. In the event that the Company is required to pay WFUHS consideration, then the Company may deduct 60% of such WFUHS consideration on a dollar-for-dollar basis from the consideration due to IU.

In October 2022, the Company initiated dosing of a Phase 2 study. Pursuant to the terms of both the WFUHS License and the IU License, the company recognized milestone expense of $0.3 million within research and development expenses.

Both agreements continue from their effective date through the last date of expiration of the licensed patents, unless earlier terminated by either party in accordance with their terms.

Leases

Bala Cynwyd Office Space

On August 8, 2019, the Company entered into an operating lease for 4,642 square feet of office space in Bala Cynwyd, Pennsylvania, effective as of December 15, 2019, for a period of three years and six months with an option to extend the lease for three additional years. Due to required tenant improvements to be completed by the landlord, the Company did not take immediate possession of the leased property and the lease term commenced on February 15, 2020.

On March 9, 2023, the Company executed a lease extension agreement on its original 4,642 square footage of office space in Bala Cynwyd, Pennsylvania (which was set to expire in August 2023) and agreed to lease an additional 3,462 square feet of office space from the same landlord.

The lease extension on the original 4,642 square footage commenced on September 1, 2023 and the Company recorded a right of use asset and lease liability of $0.5 million as of that date.

The new lease on 3,462 additional square footage commenced on October 1, 2023 and the Company recorded a right of use asset and lease liability of $0.3 million as of that date.

The right of use assets and lease liabilities with these leases are reflected in the financial statements for the year ended December 31, 2024 as are the right of use asset and lease liability of the Company's Boston office space and lab space discussed below.

Boston Office Lease

In connection with the Company's 2020 merger with Zafgen described in footnote 1, on May 28, 2020, the Company acquired a non-cancellable operating lease for approximately 17,705 square feet of office space (the "Premises"). The lease expires on October 30, 2029. As part of the agreement, the Company was initially required to maintain a letter of credit, of $1.3 million. In October of 2024, upon the agreement with the landlord that we had achieved certain clinical development milestones required in the lease, this letter of credit was reduced to $0.6 million. During both periods presented, this cash deposit and is classified as restricted cash within the consolidated financial statements. In addition to the base rent, the Company is also responsible for its share of operating expenses, electricity and real estate taxes, which costs are not included in the determination of the leases' right-of-use assets or lease liabilities. The right-of-use asset is being amortized to other income/(expense) over the remaining lease term as a result of the sublease described below.

On October 27, 2020, the Company entered into a sublease agreement (the "Sublease") with Massachusetts Municipal Association, Inc. (the "Subtenant"), whereby the Company sublet the entire Premises to the Subtenant. The initial term of the Sublease commenced on December 4, 2020 and continues until October 30, 2029. In connection with the Sublease, the Company evaluated the need for impairment under ASC 360 "Impairment Testing: Long-Lived Assets Classified as Held and Used," and determined there was no impairment.

The Sublease provided for an initial annual base rent of $0.8 million, which increases annually up to a maximum annual base rent of $1.0 million. The Subtenant also is responsible for paying to the Company future increases in operating costs (commencing on January 1, 2022), future increases in annual tax costs (commencing July 1, 2021) and all utility costs (commencing March 1, 2021) attributable to the Premises during the term of the Sublease. As part of the Sublease, the subtenant deposited a letter of credit in the amount of $0.8 million to assure their performance under the sublease. If there are no uncured events of default under the sublease, the amount of this security deposit decreases over time to $0.4 million on the sixth anniversary of the Sublease. The Company records sublease income on this sublease on a straight-line basis as a component of other income/(expense).

Lab Space

On November 5, 2018, the Company entered into an operating lease for office and lab space in Philadelphia, Pennsylvania, effective as of January 1, 2019, and expiring on December 31, 2020 with an option to extend the lease for two additional years. On August 4, 2020, the Company executed the first option to extend the lease for an additional year, expiring on December 31, 2021. On August 9, 2021, the Company executed the remaining option to extend the lease for an additional year, expiring on December 31, 2022. In September 2023, the Company extended this lease for an additional year with the option to terminate with four months' notice. On March 28, 2024, the Company gave the requisite notice and vacated the property in May 2024.

On October 16, 2023, the Company entered into an operating lease for alternate lab space in King of Prussia, Pennsylvania for a period of four years. Due to required tenant improvements to be completed by the landlord, the Company did not take immediate possession of the leased property. The actual lease term commenced on May 10, 2024. Upon commencement of the lease term, the Company recorded a right of use asset and lease liability of $0.5 million which are reflected in these consolidated financial statements.

Lease Expense

Expense arising from operating leases was $0.5 million and $0.4 million during the twelve months ended December 31, 2024 and 2023, respectively. For operating leases, the weighted-average remaining lease term for leases at December 31, 2024 and 2023 was 4.5 and 5.5 years, respectively. For operating leases, the weighted average discount rate for leases at December 31, 2024 and 2023 was 11.0%. The Company has not entered into any financing leases.

Maturities of lease liabilities due under these lease agreements as of December 31, 2024 are as follows:

Year Ending December 31, (in thousands)	Operating Leases
2025	$ 1,543
2026	1,473
2027	1,267
2028	1,189
2029	959
Thereafter	—
Total lease payments	6,431
Less: imputed interest	(1,314)
Present value of lease liabilities	$ 5,117

Legal Proceedings

The Company is not currently a party to any litigation, nor is management aware of any pending or threatened litigation against the Company, that it believes would materially affect the Company's business, operating results, financial condition or cash flows.

9.Income Taxes

During the years ended December 31, 2024, and 2023, the Company recorded no income tax benefits for the net operating losses incurred in each year due to its uncertainty of realizing a benefit from those items.

The domestic and foreign components of loss before income taxes are as follows.

	Years ended December 31, 2024	2023
Domestic	$ (80,604)	$ (36,948)
Foreign	—	(1)
	$ (80,604)	$ (36,949)

A reconciliation of the U.S. federal statutory income tax rate to the Company's effective income tax rate is as follows:

	Year Ended December 31, 2024	2023
Federal statutory income tax rate	21.0%	21.0%
State taxes, net of federal benefit	5.3	4.9
Change in state tax rate	(1.3)	(1.7)
Federal and state research and development tax credit	14.5	8.2
Nondeductible permanent differences	(0.8)	(1.0)
Stock compensation forfeitures - DTA Write Off	—	(1.8)
Change in deferred tax asset valuation allowance	(38.7)	(29.6)
Effective income tax rate	0.0%	0.0%

Net deferred tax assets as of December 31, 2024 and 2023 consisted of the following:

	2024	2023
Deferred tax assets:		
Capitalized R&D - acquired in merger with Zafgen	$ 66,678	$ 66,678
Capitalized R&D - Section 174 Costs post 2021	22,616	9,411
Stock based compensation	4,879	3,490
Net operating loss carryforwards	50,177	45,195
Tax credit carryforwards	28,022	16,319
Other temporary differences	30	21
Accruals & reserves	19	19
Fixed assets & intangibles	119	107
Operating lease liability	1,196	1,298
Total deferred tax assets	$ 173,736	$ 142,538
Deferred tax liabilities:		
Operating right of use asset	(708)	(768)
Total deferred tax liabilities	$ (708)	$ (768)
Less: Valuation allowance	$ (173,028)	$ (141,770)
Net deferred tax assets / (liabilities)	$ —	$ —

Changes in the valuation allowance for deferred tax assets during the year ended December 31, 2024 and 2023 related primarily to the increase in net operating loss carryforwards and tax credit carryforwards and a decrease other deferred tax assets associated with a reduction in the Company's effective state rate. Changes to the valuation allowance were as follows:

	Years ended December 31,	
	2024	2023
Valuation allowance as of the beginning of the year	$ 141,770	$ 130,831
Increases recorded to income tax provision	31,258	10,939
Valuation allowance at end of year	$ 173,028	$ 141,770

As of December 31, 2024, the Company had net operating loss carryforwards that expire for federal, foreign and state income tax purposes of $198.2 million, $1.2 million and $164.5 million, respectively. The federal and state operating losses begin to expire in 2026 and 2030, while the foreign net loss carryforward can be carried forward indefinitely. As of December 31, 2024, the Company had federal net operating loss carryforwards that were generated after December 31, 2017 of $159.5 million that do not expire, however these carryforwards are limited to 80% of the taxable income in any one tax period. As of December 31, 2024, the Company also had available tax credit carryforwards for federal and state income tax purposes of $28.0 million which begin to expire in 2039. Utilization of the pre-Merger net operating loss carryforwards attributable to Zafgen, of approximately $33.5 million, are subject to a substantial annual limitation under Section 382 of the Internal Revenue Code of 1986 due to ownership changes occurred during the tax year associated with the Merger. In addition to the limitation of the pre-Merger NOL's of Zafgen, the net capitalized R&D deferred tax assets in the amount of $66.9 million is subject to the built-in loss rules under Section 382 and may not be realized if the underlying asset associated with the R&D is disposed within five years of the Merger, or May 28, 2025. In general, an ownership change, as defined by Section 382, results from transactions increasing the ownership of certain stockholders or public groups in the stock of a corporation by more than 50% over a three-year period. The ownership changes will limit the amount of pre-merger Zafgen carryforwards that can be utilized annually to offset future taxable income with an annual limitation of approximately $35 thousand per year. The Company has reduced their NOL and R&D tax credit deferred tax assets associated with the pre-Merger Zafgen operations as a result of the 382 analysis.

The Company believes that as a result of its merger with Zafgen, its 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, the Company's July 2021, September 2022 and February 2024 equity transactions could also limit its ability to utilize NOLs in the future. The Company has not conducted a study to assess whether a change of control has occurred or whether there have been multiple changes of control since inception. If the Company experienced a change of control, as defined by Section 382, at any time since inception, utilization of its net operating loss carryforwards or tax credit carryforwards would be subject to an annual limitation under Section 382, which is determined by first

multiplying the value of the Company's stock at the time of the ownership change by the applicable long-term tax-exempt rate, and then could be subject to additional adjustments, as required. Additionally, our July 2021, September 2022 and February 2024 equity transactions could also limit our ability to utilize NOLs in the future. Any limitation may result in expiration of a portion of the net operating loss carryforwards or tax credit carryforwards before utilization. Further, until a study is completed, and any limitation is known, no amounts are being presented as an uncertain tax position.

The Tax Cuts and Jobs Act ("TCJA") requires taxpayers to capitalize and amortize research and experimental expenditures under IRC Section 174 for tax years beginning after December 31, 2021. This rule became effective for the Company during the year ended December 31, 2022 and resulted in the capitalization of research and development costs of $69.2 million, $24.5 million and $22.2 million in 2024, 2023 and 2022, respectively. The Company is amortizing these costs for tax purposes over five years if the research and development was performed in the U.S. and over 15 years if the research and development was performed outside the U.S.

As of December 31, 2024 and 2023, the Company's net deferred tax asset balance before the valuation allowance was $173.0 million and $141.8 million, respectively, and was comprised principally of net operating loss carryforwards, capitalized research and development expenses and tax credit carryforwards. During the years ended December 31, 2024 and 2023, gross deferred tax assets increased due to deferred tax assets acquired as a result of additional net operating loss carryforwards and research and development tax credits generated.

The Company has evaluated the positive and negative evidence bearing upon its ability to realize the deferred tax assets. Management has considered the Company's history of cumulative net losses incurred since inception and its lack of commercialization of any products or generation of any revenue from product sales since inception and has concluded that it is more likely than not that the Company will not realize the benefits of the deferred tax assets. Accordingly, a full valuation allowance has been established against the deferred tax assets as of December 31, 2024 and 2023. Management reevaluates the positive and negative evidence at each reporting period.

The Company has not recorded any amounts for unrecognized tax benefits as of December 31, 2024 and 2023. The Company files tax returns as prescribed by the tax laws of the jurisdictions in which it operates. In the normal course of business, the Company is subject to examination by federal and state jurisdictions, where applicable. There are currently no pending income tax examinations. The Company's tax years are still open under statute from 2020 to the present. Earlier years may be examined to the extent that tax credit or net operating loss carryforwards are used in future periods. The Company's policy is to record interest and penalties related to income taxes as part of its income tax provision.

10. Related Party

In May 2024, the Company entered into an agreement with the Friedreich's Ataxia Research Alliance (FARA) to join the TRACK-FA Neuroimaging Consortium that includes pharmaceutical, biotechnology, academic and clinical partners. The consortium will conduct a natural history study designed to establish disease-specific neuroimaging biomarkers to track disease progression in the brain and spinal cord and provide a basis for utilizing these biomarkers in clinical trials. As an industry partner, the Company will help fund the study and contribute to the study design, research activities, and analysis. The Company will have access to all study data for use in its regulatory filings, as appropriate. During the twelve months ended December 31, 2024, the Company incurred $0.9 million of costs related to the Track-FA program and will fund future costs going forward. One of the Company's Directors is also a director of FARA.

Additionally, during the twelve months ended December 31, 2024, the Company sponsored patient and caregiver awareness events held by FARA for a cumulative $0.1 million.