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, 2023

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 001-38890

Quince Therapeutics, Inc.

(Exact name of registrant as specified in its Charter)

Delaware	**90-1024039**
(State or other jurisdiction of incorporation or organization)	**(I.R.S. Employer Identification No.)**
611 Gateway Boulevard, Suite 273 South San Francisco, California	**94080**
(Address of principal executive offices)	**(Zip Code)**

Registrant's telephone number, including area code: (415) 910-5717

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	QNCX	The Nasdaq Stock Market LLC
Series A Junior Participating Preferred Purchase Rights	N/A	The Nasdaq Stock Market LLC

Securities registered pursuant to Section 12(g) of the Act: **None**

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 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, 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 ☒

The aggregate market value of the common stock held by non-affiliates of the registrant as of June 30, 2023 (the last business day of the registrant's most recently completed second fiscal quarter) was approximately $50 million, based on the closing price of the registrant's common stock, as reported by the Nasdaq Global Select Market on June 30, 2023 of $1.505 per share.

The number of shares of the registrant's common stock outstanding as of March 25, 2024 was 43,215,233.

DOCUMENTS INCORPORATED BY REFERENCE

Part III incorporates by reference certain information from the registrant's definitive proxy statement (the "Proxy Statement") relating to its 2024 Annual Meeting of Stockholders. The Proxy Statement will be filed with the United States Securities and Exchange Commission within 120 days after the end of the fiscal year to which this report relates.

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Special Note Regarding Forward-Looking Statements

This Annual Report on Form 10-K contains forward-looking statements. All statements other than statements of historical facts contained in this report, including statements regarding our future results of operations and financial position, business strategy, drug candidates, planned preclinical studies and clinical trials, research and development costs, regulatory approvals, timing and likelihood of success, as well as plans and objectives of management for future operations, are forward-looking statements. In some cases, forward-looking statements may be identified by words such as "believe," "may," "will," "estimate," "continue," "anticipate," "intend," "could," "would," "expect," "objective," "plan," "potential," "seek," "grow," "target," "if," and similar expressions intended to identify forward-looking statements.

We have based these forward-looking statements largely on our current expectations and projections about future events and trends that we believe may affect our financial condition, results of operations, business strategy, short-term and long-term business operations and objectives and financial needs. These forward-looking statements are subject to known and unknown risks, uncertainties and assumptions, including risks described in the section titled “Risk Factors” set forth in Part I, Item 1A of this Annual Report on Form 10-K and in our other filings with the Securities and Exchange Commission (the "SEC"). It is not possible for our management to predict all risks, nor can we assess the impact of all factors on our business or the extent to which any factor, or combination of factors, may cause actual results to differ materially from those contained in any forward-looking statements we may make. In light of these risks, uncertainties and assumptions, the future events and trends discussed in this Annual Report on Form 10-K may not occur, and actual results may differ materially and adversely from those anticipated or implied in the forward-looking statements. Forward-looking statements contained in this Annual Report on Form 10-K include, but are not limited to, statements about:

- our ability to successfully execute on our current strategic direction;
- our ability to successfully integrate EryDel;
- future research and development activities, including the scope, success, cost and timing of any future development activities, preclinical studies and clinical trials, including clinical trials of EryDex or other pipeline compounds we advance through the drug development process;
- the timing and focus of any potential future clinical trials, and the reporting of data from those trials;
- our ability and timing of seeking and obtaining FDA and any other regulatory approvals for our drug candidates;
- the willingness of the FDA or other regulatory authorities to accept any future completed or planned clinical and preclinical studies and other work, as the basis for review and approval of our drug candidates for their respective indications;
- whether regulatory authorities determine that additional trials or data are necessary in order to accept a new drug application for review and/or approval;
- the ability of any future clinical trials to demonstrate safety and efficacy of our EryDex and other drug candidates, and other positive results;
- our financial performance;
- the sufficiency of our existing cash and cash equivalents to fund our future operating expenses and capital expenditure requirements;
- the accuracy of our estimates regarding expenses, future revenue, capital requirements, and needs for additional financing;
- our expectations related to the use of our available cash;
- our ability to obtain funding for our operations, including funding necessary to develop and commercialize our drug candidates;
- our expectations regarding the potential market size and the size of the patient populations for our drug candidates, if approved for commercial use, and the potential market opportunities for commercializing our drug candidates;
- our plans relating to commercializing our drug candidates, if approved;
- our plans and ability to establish sales, marketing and distribution infrastructure to commercialize any drug candidates for which we obtain approval;
- our ability to attract and retain key scientific and clinical personnel, in light of recent management changes and reduction in force;

- our ability to contract with third-party suppliers and manufacturers and their ability to perform adequately;
- our reliance on third parties to conduct clinical trials of our drug candidates, and for the manufacture of our drug candidates for preclinical studies and clinical trials;
- dependence upon the integrity of our supply chain, including multiple single-source suppliers;
- our reliance on third-party suppliers for certain of our raw materials and components;
- our ability to expand our drug candidates into additional indications and patient populations;
- the success of competing therapies that are or may become available;
- the beneficial characteristics, safety and efficacy of our drug candidates;
- governmental or regulatory delays, information requests, clinical holds, and regulatory developments in the United States and other jurisdictions;
- our ability to obtain and maintain regulatory approval of our drug candidates, and any related restrictions, limitations and/or warnings in the label of any approved drug candidate;
- our ability to obtain and maintain CE Certificates of conformity for the medical device components of our EryDex System in accordance with applicable legislation governing medical devices;
- our ability to transition CE Certifications under the previous Medical Device Directive, to a regulatory framework under MDR;
- our plans and ability to obtain or protect intellectual property rights, including extensions of existing patent terms where available;
- the scope of protection we are able to establish and maintain for intellectual property rights covering our drug candidates and technology;
- potential claims relating to our intellectual property; and
- our ability to grow our organization and increase the size of our facilities to meet our anticipated growth.

We caution you that the foregoing list may not contain all of the forward-looking statements made in this Annual Report on Form 10-K.

You should not rely upon forward-looking statements as predictions of future events. The events and circumstances reflected in the forward-looking statements may not be achieved or occur. Although we believe that the expectations reflected in the forward-looking statements are reasonable, we cannot guarantee future results, levels of activity, performance or achievements. Except as required by law, we do not intend to update any of these forward-looking statements after the date of this Annual Report on Form 10-K or to conform these statements to actual results or revised expectations.

You should read this Annual Report on Form 10-K with the understanding that our actual future results, levels of activity, performance and events and circumstances may be materially different from what we expect.

This Annual Report on Form 10-K contains estimates, projections and other information concerning our industry, our business and the markets for our drug candidates. We obtained the industry, market and similar data set forth in this report from our own internal estimates and research and from academic and industry research, publications, surveys and studies conducted by third parties, including governmental agencies. Information that is based on estimates, forecasts, projections, market research or similar methodologies is inherently subject to uncertainties and actual events or circumstances may differ materially from events and circumstances that are assumed in this information. While we believe that the data we use from third parties are reliable, we have not separately verified these data. Further, while we believe our internal research is reliable, such research has not been verified by any third party. You are cautioned not to give undue weight to any such information, projections and estimates.

Summary of Risk Factors

We may be unable for many reasons, including those that are beyond our control, to implement our business strategy successfully. The occurrence of any single risk or any combination of risks could materially and adversely affect our business, financial condition, results of operations, cash flows and the trading price of our common stock. Some of these risks are:

- We may experience difficulties integrating Quince and EryDel's operations and realizing the expected benefits of the EryDel Acquisition.
- We are substantially dependent on the success of our lead drug candidate, EryDex. The Phase 3 NEAT clinical trial of EryDex for A-T will be conducted under a protocol negotiated with FDA by EryDel and our execution of the trial may be delayed, may not be successful, and may not result in NDA approval, with adverse results for our business and share price.
- We have no drug candidates approved for commercial sale, we have never generated any revenue from sales, and we may never be profitable.
- We will require additional capital to fund development of EryDex.
- We may be required to make milestone payments to EryDel shareholders or pursuant to the EIB Facility in connection with our development and commercialization of EryDex, which could adversely affect the overall profitability of EryDex, if approved.
- Clinical drug development is a lengthy, expensive and uncertain process. The results of preclinical studies and early clinical trials are not always predictive of future results. Any drug candidate that we may advance into clinical trials may not achieve favorable results in later clinical trials, if any, or receive marketing approval.
- Results in earlier clinical trials may not be indicative of the results that may be obtained in further registrational clinical trials, which may delay or prevent obtaining regulatory approval.
- We will incur additional costs and may experience delays in completing, or ultimately be unable to complete, the development and commercialization of our drug candidates.
- Our drug candidates may cause or have attributed to them undesirable side effects or have other properties that delay or prevent their regulatory approval or limit their commercial potential.
- Clinical trials of our drug candidates may not uncover all possible AEs that patients may experience.
- If we are not able to successfully demonstrate a favorable differentiation between EryDex and currently available corticosteroids, our business would be harmed and our ability to generate revenue from that class of drugs would be severely impaired.
- Because the potential rare disease target patient populations of EryDex are small, and the addressable patient population even smaller, we may not be able to effectively complete clinical trials or commercialize the drug candidate.
- We are a clinical stage biotechnology company with a limited operating history, which may make it difficult to evaluate the prospects for our future viability.
- We will require substantial additional funding to finance our operations and discover and develop drug candidates. If we are unable to raise this funding when needed or on acceptable terms, we may be forced to delay, reduce or eliminate our drug development programs or other operations.
- We cannot be certain that the FDA or foreign regulatory authorities will permit us to proceed with any current or future proposed clinical trial designs. Our drug candidates may not receive regulatory approval, and without regulatory approval we will not be able to market our drug candidates.
- Clinical failure can occur at any stage of clinical development and we have never submitted an NDA or comparable foreign applications before.
- Clinical trials of our drug candidates have in the past been put on clinical holds by, and failed to demonstrate safety and efficacy to the satisfaction of, the FDA, and if any future clinical trials of our drug candidates are put on clinical holds by, or fail to demonstrate safety and efficacy to the satisfaction of, the FDA, the EMA, or similar regulatory authorities

outside the United States, or do not otherwise produce positive results, we may incur additional costs or experience delays in completing, or ultimately be unable to complete, the development and commercialization of our drug candidates.

- We currently rely and expect to continue to rely on third parties to conduct some of our preclinical studies and clinical trials and some aspects of our research and preclinical testing and on third-party contract manufacturing organizations to manufacture and supply our preclinical, clinical and commercial materials, and those third parties may not perform satisfactorily, including failing to meet deadlines for the completion of such trials, research, manufacturing or testing.
- If we or any of our third-party manufacturers or suppliers encounter difficulties in production of our future drug candidates, or fail to meet rigorously enforced regulatory standards, our ability to provide supply of our future drug candidates for clinical trials or for patients, if approved, could be delayed or stopped, or we may be unable to maintain a commercially viable cost structure.
- If, in the future, we are unable to establish sales and marketing capabilities or enter into agreements with third parties to sell and market any drug candidates we may develop, we may not be successful in commercializing those drug candidates if and when they are approved.
- If we are unable to obtain and maintain sufficient intellectual property protection for our current drug candidates, any future drug candidates, and other proprietary technology we develop, or if the scope of the intellectual property protection is not sufficiently broad, our competitors could develop and commercialize drug candidates similar or identical to ours, and our ability to successfully commercialize our current drug candidate, if approved, any future drug candidates, and other proprietary technologies if approved, may be adversely affected.
- Our stockholders may realize little or no value from the divesture of our legacy assets, and as a result our stock price may decline, we could be subject to litigation, and our business may be adversely affected.

DEFINED TERMS

Unless the context requires otherwise, references to "Quince," "the Company," "we," "us," or "our" in this Annual Report on Form 10-K refer to Quince Therapeutics, Inc. and its consolidated subsidiaries. We also have used several other terms in this Annual Report on Form 10-K, most of which are explained or defined below.

Abbreviated Term	Defined Term
2017 Tax Act	Tax Cuts and Jobs Act of 2017
3PLs	Third-party Logistics Providers
AE	Adverse Event
AIA	Leahy-Smith America Invents Act
AIDE	Autologous Intracellular Drug Encapsulation
ANDA	Abbreviated New Drug Application
ARB	Angiotensin Receptor Blockers
ASC	Accounting Standards Codification
ASU	Accounting Standards Update
A-T	Ataxia-Telangiectasia
ATTeST	Ataxia Telangiectasia Trial with the EryDex SysTem
CARES Act	Coronavirus Aid, Relief, and Economic Security Act
C-GIC	Clinical Global Impression of Change
GMP	Current Good Manufacturing Practice
Cmax	The highest (peak) concentration of a drug in the bloodstream or other part of the body after drug administration
CMC	Chemistry Manufacturing Controls
CMO	Contract Manufacturing Organization
CMS	Center for Medicare and Medicaid Services
the Code	Internal Revenue Code of 1986, as amended
COSO framework	Control-Integrated Framework issued by the Committee of Sponsoring Organizations of the Treadway Commission
COVID-19	Coronavirus disease
CPA	Certified Public Accountant
Credits	Tax credits
CRO	Contract Research Organization
CTA	Clinical Trial Application
DOJ	United States Department of Justice
DSMB	Data Safety Monitoring Board
DSP	Dexamethasone Sodium Phosphate
EC	European Commission
EMA	European Medicines Agency
EryDel	EryDel S.p.A.
EryDex	Red blood cell encapsulated dexamethasone sodium phosphate
EU	European Union
EryKit	Consumable treatment kit that provides EryDex
Exchange Act	Securities Exchange Act of 1934
FASB	Financial Accounting Standards Board
FCPA	Foreign Assets Controls, the United States Foreign Corrupt Practices Act of 1977
FDA	United States Food and Drug Administration
FFDCA	Federal Food, Drug, and Cosmetic Act
GAAP	accounting principles generally accepted in the United States
GCP	Good Clinical Practice
GDPR	General Data Protection Regulation
GLP	Good Laboratory Practice
GMP	Good Manufacturing Practice
HHS	United States Department of Health and Human Services

HIPAA	Health Insurance Portability and Accountability Act of 1996
HITECH	Health Information Technology for Economic and Clinical Health Act of 2009
HPA	Hypothalamic-Pituitary-Adrenal (HPA) Axis
HTA	Health Technology Assessment
ICARS	International Cooperative Ataxia Rating Scale
IND	Investigational New Drug
IPO	Initial Public Offering
IPR&D	*In-process Research and Development*
IRA	Inflation Reduction Act of 2022
IRB	Institutional Review Board
ITT	Intent To Treat
Jefferies	Jefferies LLC
Lighthouse	Lighthouse Pharmaceuticals, Inc.
LSM	Least Square Mean
MAA	Marketing Authorization Application
MAD	Multiple Ascending Dose
MDD	Medical Devices Directive
MDR	Medical Devices Regulation 2017/745
mICARS	Modified International Cooperative Ataxia Rating Scale
MHRA	United Kingdom Medicines and Healthcare Products Regulatory Agency
MPEEM	Multi-Period Excess Earnings Method
Nasdaq	The Nasdaq Stock Market LLC
NCE	New Chemical Entity
NDA	New Drug Application
NEAT	EryDex Phase 3 Trial (Neurologic Effects of EryDex on Subjects with A-T)
NIH	National Institute of Health
NOL	Net Operating Loss
Novosteo	Novosteo, Inc.
PCAOB	Public Company Accounting Oversight Board
PCT	Patent Cooperation Treaty
PD	Pharmacodynamic
PDMA	Prescription Drug Marketing Act
Process solutions	(Hypotonic Solutions 1& 2 and Hypertonic Solution PIGPA) sterile solutions to allow drug encapsulation and restoring the physiological osmolarity during EryDex process
PDUFA	Prescription Drug User Fee Act
PP	Per Protocol Population is all patients who enrolled into the study and fulfilled all inclusion/exclusion criteria, did not have any major protocol violations, and completed the initial treatment period of the study as planned.
PK	Pharmacokinetic
PMDA	Pharmaceutical and Medical Devices Agency of Japan
PPACA	Patient Protection and Affordable Care Act as amended by the Health Care and Education Reconciliation Act of 2010
PRF	Purdue Research Foundation
PTE	Patent Term Extension
R&D	Research and Development
RCL	Red Cell Loader, the machine that encapsulates drug into red blood cells
Registrational or pivotal trial	An adequate and well-controlled trial designed to be sufficient to apply for regulatory approval of a drug candidate, although notwithstanding the Company's design a regulatory agency may determine that further clinical studies or data are required
RmICARS	Rescored modified International Cooperative Ataxia Rating Scale
RBC	Red Blood Cell
RSA	Restricted Stock Awards
RSU	Restricted Stock Units
SAD	Single Ascending Dose
SAE	Serious Adverse Event
Sarbanes-Oxley Act	The Sarbanes-Oxley Act of 2002
SEC	United States Securities and Exchange Commission

Securities Act	Securities Act of 1933
SPA	Special Protocol Assessment
Syringe Kit	Device for anticoagulated blood collection and for the sterile connection to the EryKit
TEAE	Treatment-Emergent Adverse Effect
UPC	Unified Patent Court
USPTO	The United States Patent and Trademark Office
VA	Veterans Affairs

PART I

Item 1. Business.

Overview

Effective August 1, 2022, the Company, previously named Cortexyme, Inc. ("Cortexyme"), changed its name to Quince Therapeutics, Inc. (“Quince”, the “Company”, “we”, “us” or “our”). The Company was incorporated in the State of Delaware in June 2012 and is headquartered in South San Francisco, California. From inception, we have been focused on novel therapeutic approaches to improve the lives of patients with major, unmet medical needs. The Company was initially founded on the seminal discovery of the presence of Porphyromonas gingivalis, and its secreted toxic virulence factor proteases, called gingipains, in the relevant brain areas of both Alzheimer's and Parkinson's disease patients.

In May 2022, we completed the acquisition of Novosteo, a Delaware corporation, a privately held biotechnology company focused on targeted therapeutics to treat rare skeletal diseases, bone fractures and injury. The acquisition of Novosteo (the “Novosteo Acquisition”) and the addition of new executive management precipitated a strategic shift in our focus. In August 2022, we also announced our intent to actively seek compelling clinical-stage assets available for in-licensing and acquisition to expand our development pipeline with a focus on acquiring, developing, and commercializing innovative therapeutics for patients suffering from debilitating and rare diseases.

On January 30, 2023, we announced that we intended to prioritize capital resources toward the expansion of our development pipeline through opportunistic in-licensing and acquisition of clinical-stage assets targeting debilitating and rare diseases.

On October 20, 2023, we completed our acquisition of EryDel, a privately held, late-stage biotechnology company (the “EryDel Acquisition”) with a proprietary AIDE technology platform and Phase 3 lead asset, EryDex, that targets the potential treatment of a rare neurodegenerative disease, A-T, for which there are currently no approved treatments in any global market. EryDel is a variable interest entity and the Company is the primary beneficiary and sole shareholder. In addition, there are no restrictions on the use of the assets of EryDel.

We are a late-stage biotechnology company dedicated to harnessing the power of a patient’s own biology to deliver innovative therapies to those living with rare diseases.

Our proprietary AIDE technology platform is an innovative drug/device combination that uses an automated process designed to encapsulate a drug into the patient’s own red blood cells. Red blood cells have several characteristics that make them a potentially ideal vehicle for drug delivery, including potentially better tolerability, enhanced tissue distribution, reduced immunogenicity, and prolongation of circulating half-life. Our AIDE technology is designed to harness these benefits to allow for new and improved therapeutic options for patients living with high unmet medical needs. The AIDE technology platform is believed to confer several benefits over conventional therapies and can be applied to a broad range of small or large molecule drugs and biologics. Our Phase 3 lead asset, EryDex, leverages our AIDE technology to encapsulate DSP into a patient’s own red blood cells, and is targeted to treat a rare pediatric neurodegenerative disease, A-T. We expect to begin enrollment in a Phase 3 NEAT clinical trial of EryDex in A-T in the second quarter of 2024.

By pioneering the delivery of a drug encapsulated in a patient’s own red blood cells, we seek to advance proprietary therapeutics that hold the potential to redefine the standard of care and meaningfully improve the quality of life for patients with rare disease.

Strategy

We believe we are well-capitalized into 2026 and intend to focus our development expertise and financial resources toward the advancement of our proprietary AIDE technology platform and Phase 3 lead asset, EryDex. As of December 31, 2023, we had $75.1 million in cash, cash equivalents, and investments.

EryDex is the first drug in development that leverages our AIDE technology and is composed of DSP encapsulated in autologous red blood cells for the treatment of a rare pediatric neurodegenerative disease called A-T. DSP is a corticosteroid well described for its anti-inflammatory properties, but coupled with significant adverse effects, including potential long-term adverse effects due to adrenal suppression. EryDex is designed to maintain the known efficacy of corticosteroids but reduce or eliminate the significant adverse effects that accompany chronic use of corticosteroid treatment. Currently, there are no approved treatments for A-T and the global market, based on our internal estimates and assumptions, represents a $1+ billion peak commercial opportunity. We believe this makes EryDex an ideal lead asset to demonstrate the clinical and commercial potential of our AIDE technology.

Our strategic priority is to complete the Phase 3 clinical trial of EryDex, called NEAT (**N**eurologic **E**ffects of EryDex on Subjects with **A**-**T**; NCT06193200 / IEDAT-04-2022) to evaluate its safety and efficacy for the potential treatment of A-T. We also will investigate additional indications for EryDex and new pipeline programs using our AIDE technology platform. These priorities include the following activities and corporate milestones:

- Complete start up activities for our Phase 3 NEAT clinical trial by the end of second quarter of 2024.
- Enroll the first patient in our Phase 3 NEAT clinical trial in the second quarter of 2024.
- Report our Phase 3 NEAT clinical trial top-line results in the second half of 2025.
- Prepare for a U.S. NDA submission in 2026, provided we obtain positive NEAT study results.
- Pursue potential strategic partnerships to out-license ex U.S. regional territories to extend operational runway to support potential NDA approval of EryDex, as well as further advance other potential indications and programs discovered using the AIDE platform.

We benefit from a strong senior leadership team who possess a wide range of biotech expertise that encompasses all stages of drug development, regulatory submission and approval, and commercialization. Our team has previously been involved in drug programs that resulted in numerous FDA approvals, founded and sold companies, and participated in various public and private financings that resulted in hundreds of millions of dollars of company investment, in addition to a number of successful exits that generated billions of dollars in shareholder value. We believe this breadth of experience will meaningfully benefit the Company as we work to successfully execute our strategic priorities.

Fiscal Year 2023 Key Events

- On January 27, 2023, we sold our legacy small molecule protease inhibitor portfolio, including COR588, COR388, COR852, and COR803, pursuant to an asset purchase agreement with Lighthouse.
- On January 30, 2023, we announced our intention to prioritize capital resources toward the expansion of our development pipeline through opportunistic in-licensing and acquisition of clinical-stage assets targeting debilitating and rare diseases. Our plans also called for the out-licensing of our bone-targeting drug platform and precision bone growth molecule NOV004 designed for accelerated fracture repair in patients with bone fractures and osteogenesis imperfecta. In conjunction with this action, we approved a cost reduction program to align operations with changes in our corporate strategy. This resulted in an approximate 47% reduction in our workforce that was completed by April 2023 and incurred expenses of approximately $0.4 million. We approved the related mutual termination of License Agreement for our bone-targeting drug platform and precision bone growth molecule NOV004 with PRF effective as of October 31, 2023.
- On October 20, 2023, we completed the acquisition of EryDel, a privately held, late-stage biotechnology company with a proprietary AIDE technology platform and Phase 3 lead asset, EryDex, that targets the potential treatment of a rare pediatric neurodegenerative disease, A-T.

Proprietary AIDE Technology Platform

Our proprietary AIDE technology platform is a novel drug/device combination that uses an automated process designed to encapsulate a drug into the patient's own red blood cells. Red blood cells have several characteristics that make them a potentially ideal vehicle for drug delivery, including potentially better tolerability, enhanced tissue distribution, reduced immunogenicity, and prolongation of circulating half-life. Our AIDE technology is designed to harness these benefits to allow for the chronic administration of drugs that have limitations due to toxicity, poor biodistribution, suboptimal pharmacokinetics, or immune response. In this way, the flexibility of our AIDE technology is believed to confer several benefits over conventional therapies and can be applied to a broad range of small or large molecule drugs and biologics. Additionally, the AIDE technology's use of autologous red blood cells in the encapsulation process is different from standard cell therapies, such as synthetic or engineered cells, as well as distinct from typical blood transfusions that utilize donor red blood cells for drug administration to the patient. The use of autologous blood may minimize safety risks associated with the use of donor blood and may reduce the potential immunogenic risks associated donor cells and synthetic cell therapies.

The AIDE technology drug/device combination consists of a specialized automated equipment called the RCL, a sterile single-use consumable treatment kit called EryKit, Syringe Kit, drug, and process solutions.

The RCL is a proprietary CE marked non-invasive device that allows blood processing at the point of care. With a user-friendly touch screen interface, the RCL automates the AIDE process technology by handling the blood, drug, and processing solutions with the use of the proprietary EryKit. Our EryKit is a CE marked medical device that provides the essential single-use components for

loading the patient's red blood cells with a variety of drugs by using the RCL. The RCL, EryKit, and Syringe Kit have been CE marked in accordance with the MDR. Process solutions (Hypotonic Solution 1&2 and Hypertonic Solution PIGPA) are proprietary sterile solutions, CE marked according to the MDD, aimed to create suitable osmotic condition during the process to allow the drug diffusion into red blood cells and the subsequent restoring of physiological condition.

The automated AIDE process and treatment is designed to be completed at the point-of-care and includes a series of steps which take approximately two hours from start to finish. This process includes:

- Collection of 50mL of a patient's blood using our proprietary Syringe Kit.
- Processing the patient's collected blood in the RCL using our proprietary EryKit.
- Autologous red blood cells in the RCL are swollen and their pores are "opened" in two steps using two sequential hypotonic process solutions.
- Drug is added to the RCL and enters into the opened red blood cells.
- Physiological osmotic conditions are then restored by adding a hypertonic solution that "reseals" the red blood cells.
- Drug that is not encapsulated during the process is removed by extensive washing with an injectable saline solution.
- Upon completion of the AIDE process, the drug encapsulated red blood cells are infused into the patient.

Potential Benefits of Red Blood Cell Encapsulated Drug Delivery

Many efficacious drugs have limited therapeutic potential because of dose limiting toxicity, while other drugs may have efficacy ceilings due to inferior biodistribution, pharmacokinetics and pharmacodynamics. Our proprietary AIDE technology uses an automated process designed to encapsulate a drug into the patient's own red blood cells to deliver a therapy in a potentially more effective and safer way. Autologous red blood cells have several characteristics that make them an ideal vehicle for drug delivery:

- Potential for improved biodistribution as encapsulated drug in autologous red blood cells is designed to enable the slow release of the drug from the red blood cells traversing through the body, various tissue beds, and many capillaries for desired therapeutic effect.
- Potential for altered pharmacokinetics and pharmacodynamics, including long circulating half-life, and altered or improved tissue distribution. The altered pharmacokinetics and pharmacodynamics of the encapsulated drug delivery enabled by autologous red blood cells may significantly increase the desired therapeutic effect and/or improve the safety profile of the therapy.
- Potential for improved biocompatibility through the use of autologous red blood cells to, thereby avoiding issues with donor compatibility.
- Potential for the encapsulation of small or large molecules, peptides, and proteins inside of autologous red blood cells to limit biodegradability, thereby altering the encapsulated drug's metabolism and degradation.
- Potential for significantly decreasing adverse effects of treatments.

New Indications and Program Expansion Potential

The flexibility of the AIDE platform is believed to confer several potential benefits over conventional therapies and can be applied to a broad range of drugs ranging from small to large molecules, as well as biologics. Potential benefits include better tolerability, enhanced tissue distribution, reduced immunogenicity, and prolongation of circulating half-life which we believe could be an advantage for treating patients.

We intend to investigate other potential indications for EryDex where chronic corticosteroid treatment is – or has the potential to become – a standard of care if there were not corticosteroid-related safety concerns. This evaluation process spans across ataxia, neuromuscular, hematology, cancer, and autoimmune disease indications with a focus on rare diseases. To support this drug development pipeline expansion in a capital efficient structure, we would prioritize an investigator initiated clinical trial approach to evaluate EryDex for other potential rare disease indications. In addition, we intend to evaluate additional potential applications of the AIDE technology platform using drugs and biologics targeted at rare and debilitating diseases to further expand our drug development pipeline.

Our AIDE technology platform reflects more than 20 years of innovation and approximately $100 million of investment, which has resulted in innovation that creates high barriers to competitive entry. The RCL, EryKit, Syringe Kit, and process solutions are proprietary products and CE marked in the EU, in accordance with the MDR and MDD.

Phase 3 Lead Asset – EryDex for the Potential Treatment of A-T

EryDex is the first drug in development that leverages our AIDE technology and is composed of DSP encapsulated in autologous red blood cells targeted to treat a rare pediatric neurodegenerative disease, A-T. We call this drug/device combination the EryDex System.

A-T Background and Prevalence

A-T is an inherited autosomal recessive neurodegenerative and immunodeficiency disorder caused by mutations in ATM gene, which is responsible for cell homeostatic and cell division functions including but not limited to double-stranded DNA repair.

Typically, A-T is first diagnosed before the age of five as children begin to develop an altered gait and fall with greater frequency. Neurological symptoms worsen and patients with A-T frequently become wheelchair-bound by adolescence. Teenage years for patients with A-T are typically marked by repeated infections, pulmonary impairment, and malignancies. The median lifespan is approximately 25 to 30 years old with mortality due to infections and malignancy.

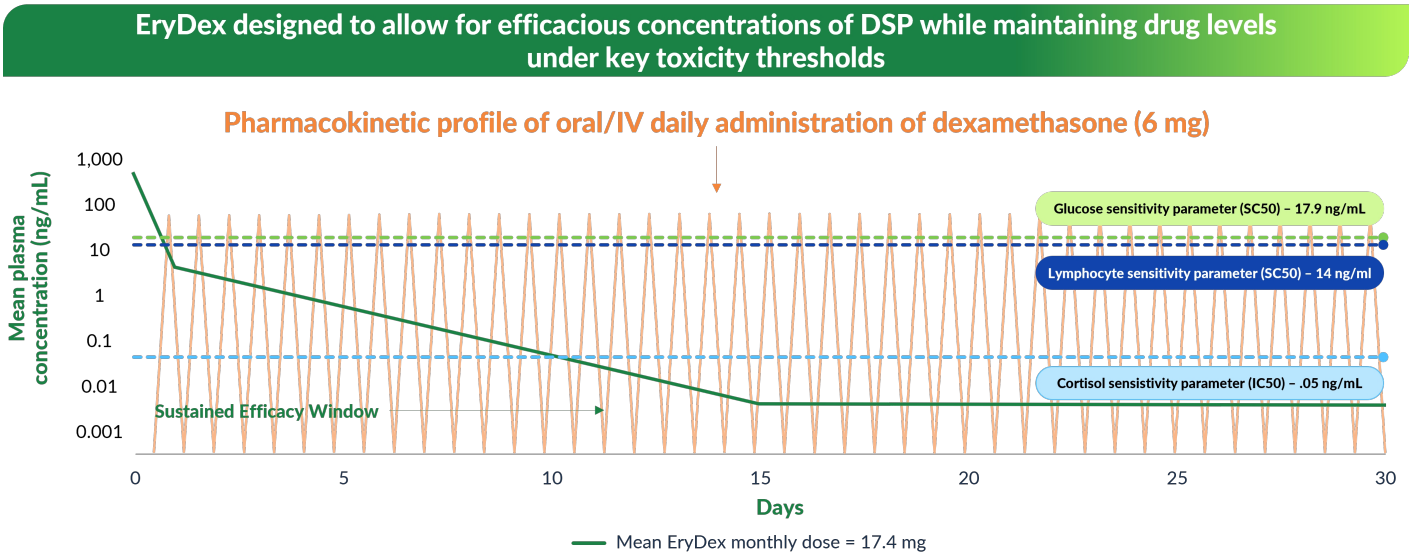
We estimate that there are approximately 5,000 patients with A-T in the U.S. and approximately 5,000 patients with A-T in the UK and EU4 countries. There are currently no approved therapeutic treatments in any global market for A-T.

Limitations of Chronic Corticosteroid Administration

DSP is a corticosteroid well known for its anti-inflammatory properties as well as its dose-limiting toxicity due to adrenal suppression. Our AIDE technology is designed to encapsulate DSP in a patient's own red blood cells and to alter the biodistribution, pharmacokinetics, and pharmacodynamics of the DSP allowing for both effective and safe treatment.

The optimal efficacy of corticosteroids is the result of two pharmacokinetic characteristics: 1) an initial bolus to achieve a high Cmax that results in high levels of corticosteroid receptor occupation; and 2) sufficient sustained tissue concentrations that allow for continued receptor site occupancy over time.

In order for a conventional corticosteroid to achieve these characteristics, the drug must be dosed frequently, typically daily. Long-term daily dosing regimens sufficient to ensure efficacy lead to significant and debilitating long-term adverse effects associated with HPA axis suppression. Based on many years of patients being dosed monthly with EryDex, the EryDex therapy may avoid these long-term safety concerns that are a major impediment to the chronic administration of corticosteroids. Corticosteroid therapy without significant long-term safety liabilities would represent a major advancement in the potential treatment of many chronic diseases where corticosteroids are already known to be beneficial.



Note: Information represented does not reflect a completed comparative study of EryDex versus oral/IV administration of DSP, but rather provides a comparison of published corticosteroid pharmacokinetic information relative to company data regarding EryDex. IC50 and SC50 refer to pharmacodynamic parameters of which IC50 reflects drug concentration eliciting 50% of the maximum inhibition and SC50 reflects drug concentration eliciting 50% of the maximum stimulation. References: Company prior Phase 3 ATTeST clinical trial data (ClinicalTrials.gov ID: NCT02770807); Montanha et al, Frontiers in Pharmacology (2022) 13: 814134; Krzyzanski et al, Journal of Pharmacokinetics and Pharmacodynamics (2021) 48: 411-438; Aljebab et al, PLOS ONE (2017) 10: 1371.

The standard delivery of corticosteroids by either intravenous, intramuscular, subcutaneous, or oral routes result in multiple peaks and troughs. Although corticosteroids can readily achieve Cmax levels required to establish efficacy, frequent dosing repeatedly exceeds toxicity thresholds associated with AEs, leading to the chronic adverse effects such as hyperglycemia, immunosuppression, and suppression of the HPA.

In contrast, EryDex delivers approximately 40% of the total dose in the first 24 hours of infusion to achieve an initial Cmax required for efficacy. Over the following approximately 30 days, DSP is dephosphorylated by red blood cell intracellular phosphatases, resulting in the slow diffusion of DSP from the red blood cell.

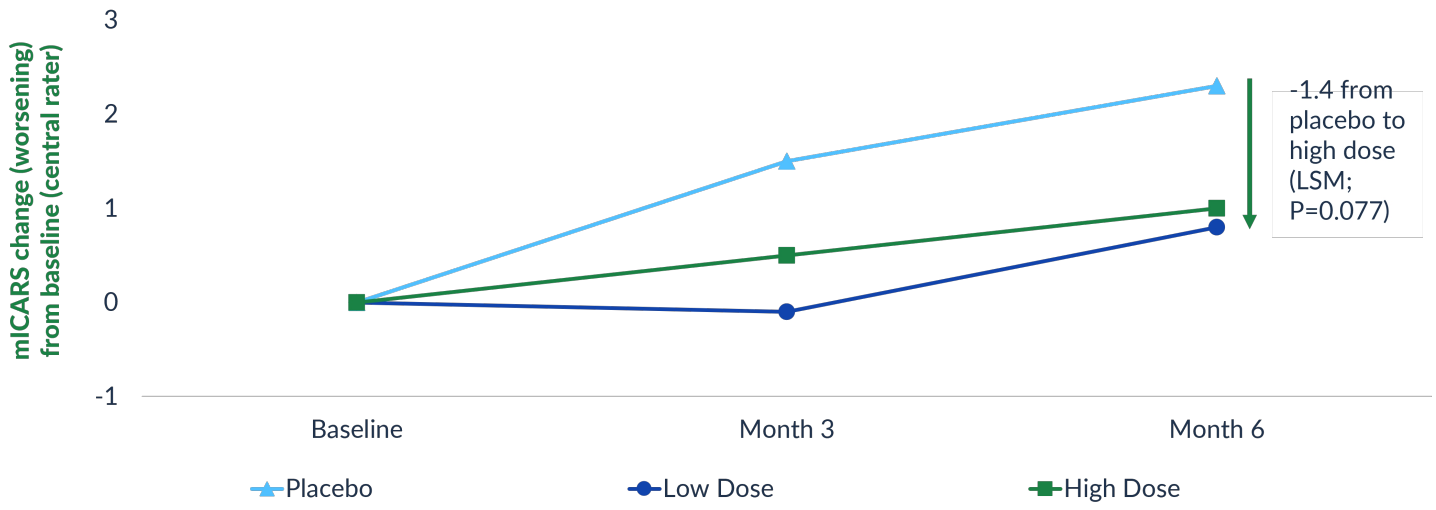
Prior ATTeST Phase 3 Clinical Trial Results in Patients with A-T

EryDel completed the largest global clinical trial of patients with A-T in a Phase 3 clinical trial called ATTeST (**A**taxia **T**elangiectasia**T**rial with the **E**ryDex **S**ys**T**em; NCT02770807 / IEDAT-02-2015). ATTeST was an international, multicenter, randomized, double-blind, placebo-controlled, Phase 3 clinical trial of patients ages six years and older. The objective of ATTeST was to evaluate the effect of two doses (Low Dose and High Dose DSP/infusion) of EryDex, compared to placebo, on central nervous system symptoms in subjects with A-T. The initial treatment period was six months. All participants who completed efficacy assessments over the initial six months were eligible to continue in an additional six-month, double blind, placebo-controlled treatment designed to collect longer-term safety and efficacy data. There were 176 patients randomized (1:1:1) between Low Dose (mean of 8.2 mg ± standard deviation of 3.3 mg), High Dose (mean of 17.4 mg ± standard deviation of 5.4 mg), and placebo. Participants received monthly doses for 12 months. At time points of month six and nine, one-third of participants on placebo switched to active drug, respectively. All participants who completed treatment in the ATTeST study were subsequently eligible to enroll in an open-label extension study (NCT03563053 / IEDAT-03-2018; OLE-IEDAT) with 104 participants receiving High Dose EryDex.

The primary efficacy endpoint of ATTeST was change in neurological symptoms from baseline to month six measured by mICARS and compared between EryDex active arms and the placebo control arm using a mixed model repeated measures analysis. The ICARS was developed to quantify the level of impairment as a result of ataxia as related to hereditary ataxias. The modified ICARS, or mICARS, was the result of discussions with the FDA. The key secondary efficacy endpoint of ATTeST was the change in participant's global clinical status from baseline until month six. This was measured by using the CGI-C. The safety objective was to evaluate the safety and tolerability of two dose levels of EryDex compared to placebo in participants with A-T, based on the occurrence of AEs.

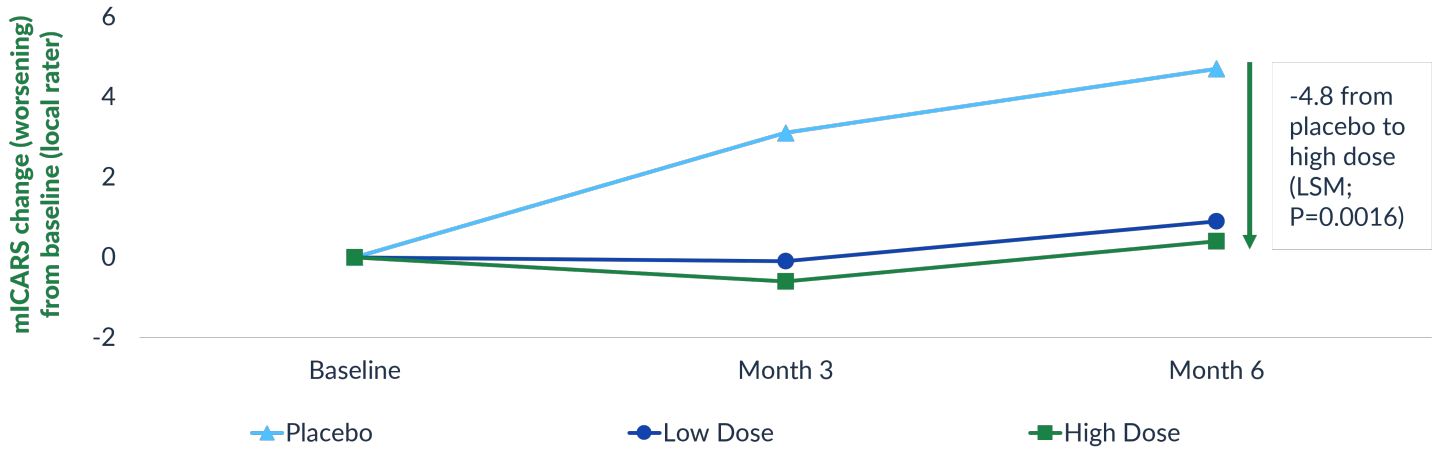
The point estimate of ATTeST's treatment effect LSM and a p-value are presented below. High Dose EryDex missed the pre-specified primary efficacy endpoint with a p-value of 0.077. However, High Dose EryDex demonstrated statistical significance in the per protocol (PP) population with a p-value of 0.019. Most of the clinical signs of neurodegeneration are observed in patients with A-T before the age of 10. By the age of 12, the vast majority of patients with A-T have become non-ambulatory and the neurological signs of disease progression slow significantly since most of the lower limb neuronal damage has already occurred. mICARS is focused on lower limb movement and published natural history data suggest two distinctive regression patterns between patients ages six to nine and 10 years or older with rapid neurological deterioration in the younger age group (six to nine years) compared to a much slower deterioration in older patients (10 years or older), many of whom are non-ambulatory. These data underpin the treatment effect in the patient population where neurodegeneration is most pronounced. Subgroup analyses were performed in the six to nine year old subgroup in the mITT population and there was a statistically significant difference for mICARS in the High Dose group as compared to placebo. Of note, the majority of patients eliminated from the PP population were due to COVID-19. COVID-19 uniquely impacted this trial because of two critical factors: 1) participants received their infusion at hospitals where essential personnel were diverted to care for COVID patients, and 2) participants with A-T are immunocompromised and there was hesitation in taking them into a high risk setting for acquiring the infection.

ATTeST Primary Endpoint (All Ages) in Intent to Treat Population (ITT)



Note: mICARS = Modified International Cooperative Ataxia Rating Scale by Central Rater • LSM = Least Square Means

ATTeST 6-9 Year Subgroup, mICARS



Note: mICARS = Modified International Cooperative Ataxia Rating Scale by Central Rater • LSM = Least Square Means

In the ATTeST clinical trial, EryDex treatment was well tolerated with only mild to moderate transient AEs that rapidly resolved without medical intervention in all dose groups. No patterns of clinically relevant AEs were observed. Additionally, there were no clear, clinically significant differences in potential corticosteroid-induced side effects observed between the treated and placebo groups, including adrenal insufficiency, cushingoid appearance, osteoporosis, growth and development through puberty, and serious systemic infections.

ATTeST Safety Summary

	ATTeST: Initial Treatment Period			ATTeST: Through Month 12		
	EDS-EP Low Dose (N=59)	EDS-EP High Dose (N=57)	Placebo (N=59)	EDS-EP Low Dose (N=59)	EDS-EP High Dose (N=57)	Non-switch Placebo (N=19)
Patients With Any TEAE (%)	73%	82%	73%	76%	88%	79%
Patients With Any Treatment-Related TEAE (%)	25%	37%	25%	32%	44%	26%
Patients With Any Serious TEAE (%)	10%	12%	12%	14%	16%	21%
Patients With Any Serious Treatment-Related TEAE (%)	0	2%	0	2%	2%	5%
Patients With Any TEAE Leading to Discontinuation (%)	0	4%	0	2%	4%	0
Patients With Any TEAE Leading to Death (%)	0	0	0	0	0	0

Note: TEAE = Treatment Emergent Adverse Event • EDS-EP = EryDex System End Product

Pivotal Phase 3 NEAT Clinical Trial of EryDex in Patients with A-T

We gained valuable learnings from the ATTeST clinical trial. Treating patients early is an important factor in A-T because the rate of neurological deterioration is most pronounced between the ages of six to nine years old.

Beginning in the second quarter of 2024, we expect to begin enrollment of the global Phase 3 clinical trial. NEAT is an international, multicenter, randomized, double-blind, placebo-controlled study to evaluate the neurological effects of EryDex in patients with A-T. We plan to enroll approximately 86 patients with A-T ages six to nine years old randomized (1:1) between EryDex or placebo, and approximately 20 patients with A-T ages 10 years or older. Participants who complete the full treatment period, complete the study assessments, and provide informed consent will be eligible to transition to an open label extension program after trial completion. The primary efficacy endpoint for the NEAT study will be measured by the change from baseline to last visit completion in RmICARS.

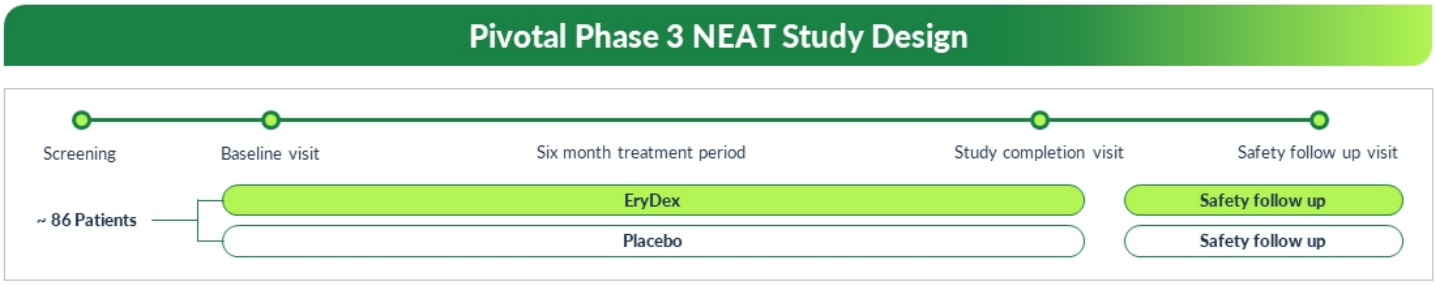


Figure: Phase 3 NEAT clinical study design.

Regulatory Interactions

The pivotal Phase 3 NEAT clinical trial will be conducted under an SPA agreement with the FDA, which should allow for the submission of an NDA following completion of this study, provided we obtain positive results. Our plan would be to submit the NDA in 2026 with expectations to submit a MAA with the EMA shortly thereafter. EryDex is considered to be a drug/device combination product . We anticipate submitting a 505(b)(2) NDA to the FDA, which allows us to rely upon the agency's prior findings of safety and effectiveness for the active pharmaceutical ingredient DSP.

EryDex has received orphan drug designation for the treatment of A-T from the FDA and EC. The EryDex System's RCL is a CE marked non-invasive device that allows blood processing at the point of care, and the EryKit is a CE marked medical device in accordance with the MDR. Process Solutions are CE marked according to the MDD.

Manufacturing

We currently operate one manufacturing facility in Medolla, Italy, which is authorized for the design and development, production, distribution, and servicing of our RCL machines, EryKit, and all proprietary medical devices. This production facility complies with EU ISO13485 and U.S. quality standards for medical device manufacturers. We also use several third-party manufacturers to produce key components and for final assemblies of the RCL and EryKit. We believe our current leased space is sufficient to meet our current needs to ensure adequate supply in our ongoing and future clinical trials, as well as anticipated early commercial needs, if EryDex is approved for marketing.

We also rely on third-party providers to manufacture sterile process solutions and drug product. Under unilateral development of our drug candidates, we are responsible for our internal manufacturing efforts, as well as for those of our third-party contract manufacturers, and we expect to continue to rely on internal manufacturing and multiple external manufacturers. We believe there are multiple sources for all of the materials required for the manufacture of our drug candidates. We intend to identify and qualify additional manufacturers to provide both process solutions and bulk drug product manufacturing services prior to submission of an NDA to the FDA as necessary to provide adequate commercial quantities of each of the sterile solutions. As our drug candidates advance through development, we expect to enter into long-term commercial supply agreements with key suppliers and manufacturers to fulfill and secure the ongoing and planned preclinical, clinical, and, if our drug candidates are approved for marketing, our commercial supply needs for ourselves and our collaborators.

Commercialization Plan

We plan to establish a commercial infrastructure in the U.S. to support the launch of EryDex, provided we obtain positive results from the Phase 3 NEAT clinical trial and subsequent regulatory approval from the FDA. We hired commercial leadership and are developing plans to ensure the necessary teams, infrastructure, systems, and processes are in place for a successful launch.

If we receive FDA approval for EryDex for the treatment of A-T, we plan to utilize a specialty distribution model to support drug availability to patients. We will also utilize a patient-centered hub to support education, insurance coverage, and compliance.

Commercial infrastructure for rare disease drugs typically consists of a targeted specialty sales force responsible for a focused group of stakeholders, including physicians, specialty distributors, and patient groups. Each sales territory collaborates closely with a cross functional team, including sales management, medical liaisons, internal sales support, marketing, and distribution. One challenge unique to commercializing therapies for rare diseases is the difficulty in identifying eligible patients due to the very small and sometimes heterogeneous disease populations.

Additional capabilities important to the rare disease marketplace include the management of key accounts such as centers of excellence, managed care organizations, group-purchasing organizations, specialty distributors and pharmacies, and government accounts. To develop the required commercial infrastructure, we will have to invest significant financial and management resources. Some of these resources will be committed prior to any regulatory confirmation that any of our drug candidates will be approved.

Outside of the United States, where appropriate and depending on the terms of contractual arrangements, we plan to commercialize EryDex through strategic partners. In certain instances, we may consider building our own commercial infrastructure.

Competition

We face competition from a number of different sources, including large and specialty pharmaceutical and biotechnology companies, academic research institutions, governmental agencies and public and private research institutions. We believe that the key competitive factors affecting the success of any drug candidates will include efficacy, safety profile, method of administration, cost, level of promotional activity, and intellectual property protection.

There are currently no therapies approved for A-T on the global market. If EryDex is approved for the treatment of A-T, it has the potential to be the first treatment on the market for this indication, but it currently faces pipeline competition. Pipeline competition for this rare disease results in competition for patient recruitment, as well as investigators' time and resources. There are drugs currently in development for A-T in the U.S., and other countries, including corticosteroids. GTX-102, an oral spray formulation of betamethasone, in development by Acasti; IB1001, N-Acetyl-L-Leucine, in development by IntraBio; MBM-01, an EPAS1/HIF1 inhibitor, in development by Matrix Biomed; splice-switching antisense oligonucleotide in development by the Boston Children's Hospital; triheptanoin, a medium-chain triglyceride marketed by Ultragenyx under the name Dojolvi, in development by The University of Queensland; and nicotinamide riboside, in development by Oslo University Hospital. In addition to drugs in development, there are many available corticosteroids, including prednisone, prednisolone, betamethasone, deflazacort, vamorolone, and many in development that could allow for longer half-lives and less AEs than approved corticosteroids.

If approved and launched commercially, EryDex may face competition from these drugs and drug candidates. Some of these drug candidates may enter the market prior to EryDex, and some of these drug candidates could limit the market or level of reimbursement available for EryDex, if it is commercialized.

Intellectual Property

The divesture of the numerous patents and patent applications relating to the compound NOV004 was completed on October 31, 2023. Under the Termination Agreement, we agreed to reimburse PRF for certain fees and costs incurred in connection with the prosecution of the licensed patents prior to termination. We also agreed to assign to PRF certain documents and materials developed by us in connection with the development of the licensed product under the License Agreement, subject to our retained right to use such documents and materials for internal research purpose. If during a specified period following the termination of the License Agreement, PRF assigns or grants any license, option or other rights under the licensed patents to certain third parties that we had identified in its prior efforts to pursue out-licensing opportunity, PRF would be required to pay us 35% of related payments received by PRF.

EryDel, our wholly owned subsidiary, owns numerous patents and patent applications covering EryDex and AIDE technology in the United States and in jurisdictions outside of the United States. Issued patents covering EryDex and AIDE technology have been obtained in the United States, Europe, Japan, and a number of other jurisdictions outside of the United States. Our patent portfolio consists of six published patent families. Two patent families are directed to the EryDex system and the process for loading a drug into an erythrocyte.

The first patent family consists of U.S. Patent No. 9,089,640 and select foreign counterparts. The ‘640 patent issued on July 28, 2015. The ‘640 patent is the U.S. national phase entry of International PCT Patent Appl. No. PCT/IB2011/000891, filed on April 26, 2011, which claims priority to U.S. Provisional Patent Appl. No. 61/373,018, filed on August 12, 2010. The patent has 154 days of PTA and will expire in 2031 (excluding PTE). The ‘640 patent was recorded as assigned to EryDel on January 4, 2013. The ‘640 patent discloses a portable and automated apparatus and kit for introducing compounds within erythrocytes. The apparatus has a reusable part provided with mechanical elements such as pumps and valves and electronic units such as a control unit, which introduces compounds into erythrocytes in an automated manner. The apparatus also has a disposable part which comes into contact with the sample containing the erythrocytes. The apparatus also provides for further concentration of the erythrocytes after they have been treated. There are foreign counterparts in the same family, including in Italy, Australia, Brazil, Canada, China, Israel, Japan, Mexico, Russia, Singapore, South Korea, and the EPO. The corresponding EPO patent is EP 2563343 B1. The claims of this patent cover the RCL and EryKit.

The second patent family consists of U.S. Patent No. 10,849,858 and select foreign counterparts. The ‘858 patent issued on December 1, 2020. The ‘858 patent was is the U.S. national phase entry of International PCT Patent Appl. No. PCT/IB2014/061338, filed on May 9, 2014, which claims priority to Italian Application numbers RM2013A0280 and RM2013A0610, filed May 10, 2013 and November 5, 2013, respectively. The ‘858 application was recorded as assigned to EryDel on December 11, 2015. The patent has 477 days of PTA and will expire in 2035 (excluding PTE). U.S. Pat. Appl. No. 17/083,771, which is a continuation application of the ‘858 patent, is currently pending in the U.S. The ‘858 patent discloses a second swelling step that differentiates it from the method of the prior art which only has one swelling step. The second swelling step of the ‘858 patent leads to significant improvement in the viability and tunability of the erythrocytes before and after drug loading. There are foreign counterparts in the same family, including in Italy, Australia, Brazil, Canada, China, Israel, Japan, Mexico, Philippines, Russia, Singapore, South Korea, and the EPO. The corresponding EPO patent is EP 2994117 B1. This patent covers the planned method of operation of the EryDex system.

Two provisional patent applications 63/625,213, filed January 25, 2024, and 63/626,398, filed January 29, 2024 have also been filed in the U.S. and are directed to the therapeutic use of drug-loaded erythrocytes in treating disease.

We actively protect our commercially important proprietary technology by, among other methods, obtaining, maintaining, and defending our patent rights. Issued patents can provide protection for varying periods of time, depending upon the date of filing of the patent application, the date of patent issuance and the legal term of patents in the countries in which they are obtained. In general, patents issued for applications filed in the United States can provide exclusionary rights for 20 years from the earliest effective non-provisional filing date. In addition, in certain instances, the term of an issued U.S. patent that covers or claims an FDA approved product can be extended to recapture a portion of the term effectively lost as a result of the FDA regulatory review period, which is called patent term extension. The period of patent term extension in the United States cannot be longer than five years and the total patent term, including the extension period, must not exceed 14 years following FDA approval. The term of patents outside of the United States varies in accordance with the laws of the foreign jurisdiction, but typically is also 20 years from the earliest effective non-provisional filing date. However, the actual protection afforded by a patent varies on a product-by-product basis, from country-to-country, and depends upon many factors, including the type of patent, the scope of its coverage, the availability of regulatory-related extensions, the availability of legal remedies in a particular country and the validity and enforceability of the patent. Some countries

also provide mechanisms to recapture a portion of the patent term lost during regulatory review, similar to patent term extension in the United States. The amount of patent term that can be recaptured depends on the laws of the relevant jurisdictions.

As with other biotechnology and pharmaceutical companies, our ability to maintain and solidify our proprietary and intellectual property position for our drug candidates and technologies will depend on our success in obtaining effective patent claims and enforcing those claims if granted. However, our pending patent applications, and any patent applications that we may in the future file or license from third parties may not result in the issuance of patents. We cannot guarantee that our owned pending patent application, or any patent applications that we may in the future file or license from third parties, will result in the issuance of patents. We also cannot predict the scope of claims that may be allowed or enforced in our patents. In addition, the coverage claimed in a patent application can be significantly reduced before the patent is issued, and its scope can be reinterpreted after issuance. Consequently, we may not obtain or maintain adequate patent protection for any of our programs and drug candidates. Any issued patents that we may receive in the future may be challenged, invalidated or circumvented. For example, we cannot be certain of the priority rights of inventions covered by pending third-party patent applications. If third parties prepare and file patent applications in the United States or other jurisdictions that also claim technology or therapeutics to which we have rights, we may have to participate in interference proceedings, post-grant review, reissue, or reexamination in the USPTO and equivalent foreign courts, which could result in substantial costs to us even if the eventual outcome, which is highly unpredictable, is favorable to us. In addition, because of the extensive time required for clinical development and regulatory review of a drug candidate we may develop, it is possible that, before any of our drug candidates can be commercialized, any related patent may expire or remain in force for only a short period following commercialization, thereby limiting any protection such patent would afford the respective product and any competitive advantage such patent may provide. For more information regarding the risks related to our intellectual property, see "Risk Factors—Risks Relating to Our Intellectual Property."

The term of individual patents depends upon the legal term of the patents in the countries in which they are obtained. In most countries in which we file, the patent term is 20 years from the earliest date of filing a non-provisional patent application in the United States. In the United States, the patent term of a patent that covers an FDA-approved drug may also be eligible for patent term extension, which permits patent term restoration as compensation for the patent term lost during the FDA regulatory review process. The Hatch-Waxman Act permits a patent term extension of up to five years beyond the expiration of the patent. The length of the patent term extension is related to the length of time the drug is under regulatory review. Patent term extension cannot extend the remaining term of a patent beyond a total of 14 years from the date of product approval, only one patent applicable to an approved drug may be extended and only those claims covering the approved drug, a method for using it, or a method for manufacturing it may be extended. Similar provisions are available in Europe and other foreign jurisdictions to extend the term of a patent that covers an approved drug. In the future, if and when our drug candidates receive FDA approval, we expect to apply for patent term extensions on patents covering those drug candidates. We plan to seek patent term extensions to any of our issued patents in any jurisdiction where these are available, however there is no guarantee that the applicable authorities, including the FDA in the United States, will agree with our assessment of whether such extensions should be granted, and if granted, the length of such extensions. For more information regarding the risks related to our intellectual property, see "Risk Factors—Risks Relating to Our Intellectual Property."

In addition to patent protection, we also rely on trademark registration, trade secrets, know-how, other proprietary information and continuing technological innovation to develop and maintain our competitive position. We seek to protect and maintain the confidentiality of proprietary information to protect aspects of our business that are not amenable to, or that we do not consider appropriate for, patent protection. Although we take steps to protect our proprietary information and trade secrets, including through contractual means with our employees and consultants, third parties may independently develop substantially equivalent proprietary information and techniques or otherwise gain access to our trade secrets or disclose our technology. Thus, we may not be able to meaningfully protect our trade secrets and we cannot guarantee, however, that these agreements will afford us adequate protection of our intellectual property and proprietary information rights. It is our policy to require our employees, consultants, outside scientific collaborators, sponsored researchers and other advisors to execute confidentiality agreements upon the commencement of employment or consulting relationships with us. These agreements provide that all confidential information concerning our business or financial affairs developed or made known to the individual during the course of the individual's relationship with us is to be kept confidential and not disclosed to third parties except in specific circumstances. Our agreements with employees also provide that all inventions conceived by the employee in the course of employment with us or from the employee's use of our confidential information are our exclusive property. However, such confidentiality agreements and invention assignment agreements can be breached, and we may not have adequate remedies for any such breach. Additionally, some of our trade secrets and know-how for which we decide to not pursue additional patent protection may, over time, be disseminated within the industry through independent development and public presentations describing the methodology. For more information regarding the risks related to our intellectual property, see "Risk Factors—Risks Relating to Our Intellectual Property."

The patent positions of biotechnology companies like ours are generally uncertain and involve complex legal, scientific and factual questions. Our commercial success will also depend in part on not infringing upon the proprietary rights of third parties. It is uncertain whether the issuance of any third-party patent would require us to alter our development or commercial strategies, or our drugs or processes, obtain licenses or cease certain activities. Our breach of any license agreements or our failure to obtain a license to

proprietary rights required to develop or commercialize our future drug candidates may have a material adverse impact on us. If third parties prepare and file patent applications in the United States that also claim technology to which we have rights, we may have to participate in interference or derivation proceedings in the USPTO to determine priority of invention. For more information, see "Risk Factors—Risks Relating to Our Intellectual Property."

Regulatory Matters

Government authorities in the United States at the federal, state and local level, and in other countries and jurisdictions, extensively regulate, among other things, the research, development, testing, manufacture, quality control, approval, labeling, packaging, storage, record-keeping, promotion, advertising, distribution, post-approval monitoring and reporting, marketing, sampling and export and import of pharmaceutical products. Generally, before a new drug can be marketed, considerable data demonstrating its quality, safety and efficacy must be obtained, organized into a format specific for each regulatory authority, submitted for review and approved by the regulatory authority.

Products composed of components that would normally be regulated by different centers at the FDA are known as combination products. Typically, the FDA's Office of Combination Products assigns a combination product to a specific agency center as the lead reviewer. The FDA determines which center will lead a product's review based upon the product's primary mode of action. Depending on the type of combination product, its approval, clearance or licensure may usually be obtained through the submission of a single marketing application. We anticipate that EryDex will be regulated as a drug, and that the FDA will permit a single regulatory submission seeking approval of EryDex. Even when a single marketing application is required for a combination product, such as an NDA for a combination pharmaceutical and device product, both the FDA's Center for Drug Evaluation and Research and the FDA's Center for Devices and Radiological Health may participate in the review. An applicant will also need to discuss with the agency how to apply certain premarket requirements and post-marketing regulatory requirements, including conduct of clinical trials, AE reporting and GMP, to their combination product.

U.S. Drug Development

In the United States, the FDA regulates drugs under the Federal Food, Drug, and Cosmetic Act, or FDCA, and its implementing regulations. The process of obtaining regulatory approvals and the subsequent compliance with appropriate federal, state, local and foreign statutes and regulations requires the expenditure of substantial time and financial resources. Failure to comply with the applicable U.S. requirements at any time during the product development process, approval process or post-market may subject an applicant to administrative or judicial sanctions. These sanctions could include, among other actions, the FDA's refusal to approve pending applications, withdrawal of an approval, a clinical hold, untitled or warning letters, product recalls or market withdrawals, product seizures, total or partial suspension of production or distribution, injunctions, fines, refusals of government contracts, restitution, disgorgement and civil or criminal penalties.

An applicant seeking approval to market and distribute a new drug product in the United States must typically undertake the following:

- completion of preclinical laboratory tests, animal studies and formulation studies in compliance with the FDA's GLP, regulations;
- submission to the FDA of an IND, which must take effect before human clinical trials may begin;
- approval by an independent IRB, representing each clinical site before each clinical trial may be initiated;
- performance of adequate and well-controlled human clinical trials in accordance with GCPs, to establish the safety and efficacy of the proposed drug product for each proposed indication;
- preparation and submission to the FDA of an NDA, requesting marketing for one or more proposed indications;
- review by an FDA advisory committee, where appropriate or if applicable;
- satisfactory completion of one or more FDA inspections of the manufacturing facility or facilities at which the product, or components thereof, are produced to assess compliance with current Good Manufacturing Practices, or cGMP, requirements and to assure that the facilities, methods and controls are adequate to preserve the product's identity, strength, quality and purity;
- satisfactory completion of FDA audits of clinical trial sites to assure compliance with GCPs and the integrity of the clinical data;
- payment of user fees and securing FDA approval of the NDA; and
- compliance with any post-approval requirements, including the potential requirement to implement a Risk Evaluation and Mitigation Strategy, or REMS, and the potential requirement to conduct post-approval studies.

Preclinical Studies

Before an applicant begins testing a compound with potential therapeutic value in humans, the drug candidate enters the preclinical testing stage. Preclinical studies include laboratory evaluation of product chemistry, toxicity and formulation, as well as *in vitro* and animal studies to assess the potential safety and activity of the drug for initial testing in humans and to establish a rationale for therapeutic use. The conduct of preclinical studies is subject to federal regulations and requirements, including GLP regulations. The results of the preclinical tests, together with manufacturing information, analytical data, any available clinical data or literature and plans for clinical trials, among other things, are submitted to the FDA as part of an IND. Some long-term preclinical testing, such as animal tests of reproductive AEs and carcinogenicity, may continue after the IND is submitted.

The IND and IRB Processes

The authorization for an IND must be secured prior to interstate shipment and administration of any new drug that is not the subject of an approved NDA. In support of a request for an IND, applicants must submit a protocol for each clinical trial and any subsequent protocol amendments must be submitted to the FDA as part of the IND. In addition, the results of the preclinical tests, together with manufacturing information, analytical data, any available clinical data or literature and plans for clinical trials, among other things, are submitted to the FDA as part of an IND. The FDA requires a 30-day waiting period after the filing of each IND before clinical trials may begin. This waiting period is designed to allow the FDA to review the IND to determine whether human research subjects will be exposed to unreasonable health risks. At any time during this 30-day period, the FDA may raise concerns or questions about the conduct of the trials as outlined in the IND and impose a clinical hold. In this case, the IND sponsor and the FDA must resolve any outstanding concerns before clinical trials can begin.

Following commencement of a clinical trial under an IND, the FDA may also place a clinical hold or partial clinical hold on that trial. A clinical hold is an order issued by the FDA to the sponsor to delay a proposed clinical investigation or to suspend an ongoing investigation. A partial clinical hold is a delay or suspension of only part of the clinical work requested under the IND. For example, a specific protocol or part of a protocol is not allowed to proceed, while other protocols may do so. Following issuance of a clinical hold or partial clinical hold, an investigation may only resume after the FDA has notified the sponsor that the investigation may proceed. The FDA will base that determination on information provided by the sponsor correcting the deficiencies previously cited or otherwise satisfying the FDA that the investigation can proceed.

A sponsor may choose, but is not required, to conduct a foreign clinical study under an IND. When a foreign clinical study is conducted under an IND, all IND requirements must be met unless waived. When the foreign clinical study is not conducted under an IND, the sponsor must ensure that the study complies with certain FDA regulatory requirements in order to use the study as support for an IND or application for marketing approval. Specifically, the FDA has promulgated regulations governing the acceptance of foreign clinical studies not conducted under an IND, establishing that such studies will be accepted as support for an IND or application for marketing approval if the study was conducted in accordance with GCP including review and approval by an independent ethics committee, or IEC, and informed consent from subjects, and the FDA is able to validate the data from the study through an on-site inspection if the FDA deems such inspection necessary. The GCP requirements encompass both ethical and data integrity standards for clinical studies. The FDA's regulations are intended to help ensure the protection of human subjects enrolled in non-IND foreign clinical studies, as well as the quality and integrity of the resulting data. They further help ensure that non-IND foreign studies are conducted in a manner comparable to that required for IND studies. If a marketing application is based solely on foreign clinical data, the FDA requires that the foreign data be applicable to the U.S. population and U.S. medical practice; the studies must have been performed by clinical investigators of recognized competence; and the FDA must be able to validate the data through an on-site inspection or other appropriate means, if the FDA deems such an inspection to be necessary.

In addition to the foregoing IND requirements, an IRB representing each institution participating in the clinical trial must review and approve the plan for any clinical trial before it commences at that institution, and the IRB must conduct continuing review and reapprove the study at least annually. The IRB must review and approve, among other things, the study protocol and informed consent information to be provided to study subjects. An IRB must operate in compliance with FDA regulations. An IRB can suspend or terminate approval of a clinical trial at its institution, or an institution it represents, if the clinical trial is not being conducted in accordance with the IRB's requirements or if the drug candidate has been associated with unexpected serious harm to patients.

Additionally, some trials are overseen by an independent group of qualified experts organized by the trial sponsor, known as a data safety monitoring board or committee. This group provides authorization for whether or not a trial may move forward at designated check points based on access that only the group maintains to available data from the study. Suspension or termination of development during any phase of clinical trials can occur if it is determined that the participants or patients are being exposed to an unacceptable health risk. Other reasons for suspension or termination may be made by us based on evolving business objectives and/or competitive climate.

Information about certain clinical trials must be submitted within specific timeframes to the NIH, for public dissemination on its ClinicalTrials.gov website.

Human Clinical Studies in Support of an NDA

Clinical trials involve the administration of the investigational product to human subjects under the supervision of qualified investigators in accordance with GCP requirements, which include, among other things, the requirement that all research subjects provide their informed consent in writing before their participation in any clinical trial. Clinical trials are conducted under written study protocols detailing, among other things, the inclusion and exclusion criteria, the objectives of the study, the parameters to be used in monitoring safety and the effectiveness criteria to be evaluated.

Human clinical trials are typically conducted in the following sequential phases, which may overlap or be combined:

- *Phase 1:* The drug is initially introduced into healthy human subjects or, in certain indications such as cancer, patients with the target disease or condition and tested for safety, dosage tolerance, absorption, metabolism, distribution, excretion and, if possible, to gain an early indication of its effectiveness and to determine optimal dosage.
- *Phase 2:* The drug is administered to 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 and optimal dosage.
- *Phase 3:* The drug is administered to an expanded patient population, generally at geographically dispersed clinical trial sites, in well-controlled clinical trials to generate enough data to statistically evaluate the efficacy and safety of the product for approval, to establish the overall risk-benefit profile of the product, and to provide adequate information for the labeling of the product.
- *Phase 4:* Post-approval studies, which are conducted following initial approval, are typically conducted to gain additional experience and data from treatment of patients in the intended therapeutic indication.

The clinical drug development phases described above are general guidelines. The phases are not clearly delineated from each other in every regard, and it is common practice to separate (e.g., Phase 1a and 1b trials) or combine (e.g., a Phase 2/3 trial) phases, which is accepted by the FDA and other global regulatory agencies.

Progress reports detailing the results of the clinical trials must be submitted at least annually to the FDA and more frequently if SAEs occur. In addition, IND safety reports must be submitted to the FDA for any of the following: serious and unexpected suspected adverse reactions; findings from other studies or animal or *in vitro* testing that suggest a significant risk in humans exposed to the drug; and any clinically important increase in the case of a serious suspected adverse reaction over that listed in the protocol or investigator brochure. Phase 1, Phase 2 and Phase 3 clinical trials may not be completed successfully within any specified period, or at all. Furthermore, the FDA or the sponsor may suspend or terminate a clinical trial at any time on various grounds, including a finding that the research subjects are being exposed to an unacceptable health risk. Similarly, an IRB can suspend or terminate approval of a clinical trial at its institution, or an institution it represents, if the clinical trial is not being conducted in accordance with the IRB's requirements or if the drug has been associated with unexpected serious harm to patients. The FDA will typically inspect one or more clinical sites to assure compliance with GCP and the integrity of the clinical data submitted.

Concurrent with clinical trials, companies often complete additional animal studies and must also develop additional information about the chemistry and physical characteristics of the drug as well as finalize a process for manufacturing the product in commercial quantities in accordance with cGMP requirements. The manufacturing process must be capable of consistently producing quality batches of the drug candidate and, among other things, must develop methods for testing the identity, strength, quality, and purity of the final drug. Additionally, appropriate packaging must be selected and tested and stability studies must be conducted to demonstrate that the drug candidate does not undergo unacceptable deterioration over its shelf life.

Special Protocol Assessment

A sponsor may request an SPA, the purpose of which is to reach agreement with the FDA on the Phase 3 clinical trial protocol design and analysis that will form the primary basis of an efficacy claim. An SPA request must be made before the proposed trial begins, and all open issues must be resolved before the trial begins for an SPA to be approved. If a written agreement is reached, it will be documented in an SPA letter or the minutes of a meeting between the sponsor and the FDA and made part of the administrative record.

Even if the FDA agrees to the design, execution and analyses proposed in protocols reviewed under the SPA process, the FDA may revoke or alter its agreement under the following circumstances:

- public health concerns emerge that were unrecognized at the time of the protocol assessment, or the director of the review division determines that a substantial scientific issue essential to determining safety or efficacy has been identified after testing has begun;
- a sponsor fails to follow a protocol that was agreed upon with the FDA; or
- the relevant data, assumptions, or information provided by the sponsor in a request for SPA change, are found to be false statements or misstatements, or are found to omit relevant facts.

A documented SPA may be modified, and such modification will be deemed binding by the FDA review division, except under the circumstances described above, if the FDA and the sponsor agree in writing to modify the protocol and such modification is intended to improve the study. However, an SPA does not guarantee that a trial will be successful.

Submission of an NDA to the FDA

Assuming successful completion of required clinical testing and other requirements, the results of the preclinical studies and clinical trials, together with detailed information relating to the product's chemistry, manufacture, controls and proposed labeling, among other things, are submitted to the FDA as part of an NDA requesting approval to market the drug product for one or more indications. Under federal law, the submission of most NDAs is additionally subject to an application user fee, and the sponsor of an approved NDA is also subject to annual program fees.

The FDA conducts a preliminary review of an NDA within 60 days of its receipt and informs the sponsor by the 74th day after the FDA's receipt of the submission to determine whether the application is sufficiently complete to permit substantive review. The FDA may request additional information rather than accept an NDA for filing. In this event, the application must be resubmitted with the additional information. The resubmitted application is also subject to review before the FDA accepts it for filing. Once the submission is accepted for filing, the FDA begins an in-depth substantive review. The FDA has agreed to specified performance goals in the review process of NDAs. Most such applications are meant to be reviewed within ten months from the filing date, and most applications for "priority review" products are meant to be reviewed within six months of the filing date. The review process and the Prescription Drug User Fee Act goal date may be extended by the FDA for three additional months to consider new information or clarification provided by the applicant to address an outstanding deficiency identified by the FDA following the original submission.

Before approving an NDA, the FDA typically will inspect the facility or facilities where the product is or will be manufactured. These pre-approval inspections may cover all facilities associated with an NDA submission, including drug component manufacturing (such as active pharmaceutical ingredients), finished drug product manufacturing, and control testing laboratories. The FDA will not approve an application 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, the FDA will typically inspect one or more clinical sites to assure compliance with GCP.

In addition, as a condition of approval, the FDA may require an applicant to develop a REMS. A REMS uses risk minimization strategies beyond the professional labeling to ensure that the benefits of the product outweigh the potential risks. To determine whether a REMS is needed, the FDA will consider the size of the population likely to use the product, seriousness of the disease, expected benefit of the product, expected duration of treatment, seriousness of known or potential AEs, and whether the product is a new molecular entity. A REMS can include medication guides, physician communication plans for healthcare professionals, and elements to assure safe use, or ETASU. ETASU may include, but are not limited to, special training or certification for prescribing or dispensing, dispensing only under certain circumstances, special monitoring, and the use of patient registries. The FDA may require a REMS before approval or post-approval if it becomes aware of a serious risk associated with use of the product. The requirement for a REMS can materially affect the potential market and profitability of a product.

The FDA is required to refer an application for a novel drug to an advisory committee or explain why such referral was not made. Typically, an advisory committee is a panel of independent experts, including clinicians and other scientific experts, that reviews, evaluates and provides 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.

The FDA's Decision on an NDA

On the basis of the FDA's evaluation of the NDA and accompanying information, including the results of the inspection of the manufacturing facilities, the FDA may issue an approval letter or a complete response letter. An approval letter authorizes commercial marketing of the product with specific prescribing information for specific indications. A complete response letter generally outlines the deficiencies in the submission and may require substantial additional testing or information in order for the FDA

to reconsider the application. If and when those deficiencies have been addressed to the FDA's satisfaction in a resubmission of the NDA, the FDA will issue an approval letter. The FDA has committed to reviewing such resubmissions in two or six months depending on the type of information included. Even with submission of this additional information, the FDA ultimately may decide that the application does not satisfy the regulatory criteria for approval.

If the FDA approves a product, it may limit the approved indications for use for the product, require that contraindications, warnings or precautions be included in the product labeling, require that post-approval studies, including Phase 4 clinical trials, be conducted to further assess the drug's safety after approval, require testing and surveillance programs to monitor the product after commercialization, or impose other conditions, including distribution restrictions or other risk management mechanisms, including REMS, which can materially affect the potential market and profitability of the product. The FDA may prevent or limit further marketing of a product based on the results of post-market studies or surveillance programs. After approval, many types of changes to the approved product, such as adding new indications, manufacturing changes and additional labeling claims, are subject to further testing requirements and FDA review and approval.

Post-Approval Requirements

Drugs manufactured or distributed pursuant to FDA approvals are subject to pervasive and continuing regulation by the FDA, including, among other things, requirements relating to recordkeeping, periodic reporting, product sampling and distribution, advertising and promotion and reporting of adverse experiences with the product. After approval, most changes to the approved product, such as adding new indications or other labeling claims, are subject to prior FDA review and approval. There also are continuing annual user fee requirements for any marketed products and the establishments at which such products are manufactured, as well as new application fees for supplemental applications with clinical data.

In addition, drug manufacturers and other entities involved in the manufacture and distribution of approved drugs are required to register their establishments with the FDA and state agencies and are subject to periodic unannounced inspections by the FDA and these state agencies for compliance with cGMP requirements. Changes to the manufacturing process are strictly regulated and often require prior FDA approval before being implemented. FDA regulations also require investigation and correction of any deviations from cGMP and impose reporting and documentation requirements upon the sponsor and any third-party manufacturers that the sponsor may decide to use. Accordingly, manufacturers must continue to expend time, money, and effort in the area of production and quality control to maintain cGMP compliance.

Once an approval is granted, the FDA may withdraw the approval if compliance with regulatory requirements and standards is not maintained or if problems occur after the product reaches the market. Later discovery of previously unknown problems with a product, including AEs of unanticipated severity or frequency, or with manufacturing processes, or failure to comply with regulatory requirements, may result in revisions to the approved labeling to add new safety information; imposition of post-market studies or clinical trials to assess new safety risks; or imposition of distribution or other restrictions under a REMS program. Other potential consequences include, among other things:

- restrictions on the marketing or manufacturing of the product, complete withdrawal of the product from the market or product recalls;
- safety alerts, Dear Healthcare Provider letters, press releases or other communications containing warning or other safety information about the product;
- fines, warning letters or holds on post-approval clinical trials;
- refusal of the FDA to approve pending NDAs or supplements to approved NDAs, or suspension or revocation of product license approvals;
- product seizure or detention, or refusal to permit the import or export of products; or
- injunctions or the imposition of civil or criminal penalties.

The FDA strictly regulates marketing, labeling, advertising and promotion of products that are placed on the market. Drugs may be promoted only for the approved indications and in accordance with the provisions of the approved label. The FDA and other agencies actively enforce the laws and regulations prohibiting the promotion of off-label uses, and a company that is found to have improperly promoted off-label uses may be subject to significant liability.

Medical Device Regulation

The medical device component of EryDex will be subject to additional FDA regulations, including:

- the FDA's Quality System Regulation, which requires manufacturers, including their suppliers, to follow stringent design, testing, control, documentation and other quality assurance procedures during all aspects of the manufacturing process;
- a requirement for Human Factors studies during development to support approval;
- medical device reporting regulations, which require that manufacturers report to the FDA if their device may have caused or contributed to a death or serious injury or malfunctioned in a way that would likely cause or contribute to a death or serious injury if the malfunction were to recur;
- medical device recalls, which require that manufacturers report to the FDA any recall of a medical device, provided the recall was initiated to either reduce a risk to health posed by the device, or to remedy a violation of the FDCA caused by the device that may present a risk to health; and
- post-market surveillance regulations, which apply when necessary to protect the public health or to provide additional safety and effectiveness data for the device.

Disclosure of Clinical Trial Information

Sponsors of clinical trials of FDA regulated products are required to register and disclose certain clinical trial information. Information related to the product, patient population, phase of investigation, trial sites and investigators, and other aspects of the clinical trial is then made public as part of the registration. Sponsors are also obligated to discuss the results of their clinical trials after completion. Disclosure of the results of these trials can be delayed in certain circumstances for up to two years after the date of completion of the trial. Competitors may use this publicly available information to gain knowledge regarding the progress of development programs.

The Orphan Drug Act

Under the Orphan Drug Act, the FDA may grant orphan drug designation to drugs intended to treat a rare disease or condition—generally a disease or condition that affects fewer than 200,000 individuals in the United States. Orphan drug designation must be requested before submitting an NDA. After the FDA grants orphan drug designation, the generic identity of the drug and its potential orphan use are disclosed publicly by the FDA. Orphan drug designation does not convey any advantage in, or shorten the duration of, the regulatory review and approval process. The first NDA applicant to receive FDA approval for a particular active ingredient to treat a particular disease with FDA orphan drug designation is entitled to a seven-year exclusive marketing period in the United States for that product, for that indication. During the seven-year exclusivity period, the FDA may not approve any other applications to market the same drug for the same disease, except in limited circumstances, such as a showing of clinical superiority to the product with orphan drug exclusivity. Orphan drug exclusivity does not prevent the FDA from approving a different drug for the same disease or condition, or the same drug for a different disease or condition. Among the other benefits of orphan drug designation are Credits for certain research and a waiver of the NDA application user fee.

Pediatric Exclusivity and Pediatric Use

Under the Best Pharmaceuticals for Children Act, certain drugs may obtain an additional six months of exclusivity in an indication, if the sponsor submits information requested in writing by the FDA in what is known as a Written Request, relating to the use of the active moiety of the drug in children. The FDA may not issue a Written Request for studies on unapproved or approved indications where it determines that information relating to the use of a drug in a pediatric population, or part of the pediatric population, may not produce health benefits in that population. The six-month period of pediatric exclusivity attaches to the end of all existing marketing exclusivity and patent periods listed in FDA's Approved Drug Products with Therapeutic Equivalence Evaluations ("Orange Book") at the time of granting.

To receive the six-month pediatric market exclusivity, a sponsor would have to receive a Written Request from the FDA and conduct the requested studies in accordance with a written agreement with the FDA. If there is no written agreement, studies would be conducted in accordance with commonly accepted scientific principles, and reports submitted of those studies. A Written Request may include studies for indications that are not currently in the labeling if the FDA determines that such information will benefit the public health. The FDA will accept the reports upon its determination that the studies were conducted in accordance with and are responsive to the original Written Request, agreement, or commonly accepted scientific principles, as appropriate, and that the reports comply with the FDA's filing requirements.

In addition, the Pediatric Research Equity Act (PREA) requires a sponsor to conduct pediatric studies for most drugs, for a new active ingredient, new indication, new dosage form, new dosing regimen, or new route of administration. Under PREA, original

NDAs and supplements thereto must contain a pediatric assessment unless the sponsor has received a deferral or waiver. The required assessment must include the evaluation of the safety and effectiveness of the product for the claimed indications in all relevant pediatric subpopulations and support dosing and administration for each pediatric subpopulation for which the product is safe and effective. The FDA, on its own initiative or at the request of the sponsor, may defer pediatric trial requirements for some or all of the pediatric subpopulations. A deferral may be granted by the FDA if it believes that additional safety or effectiveness data in the adult population need to be collected before the pediatric studies begin. The FDA must send a non-compliance letter to any sponsor that fails to submit the required assessment, keep a deferral current, or fails to submit a request for approval of a pediatric formulation. Unless otherwise required by regulation, PREA generally does not apply to a drug for an indication for which orphan designation has been granted with the exception of orphan-designated drugs if the drug is a molecularly targeted cancer product intended for the treatment of an adult cancer and is directed at a molecular target that the FDA has determined is substantially relevant to the growth or progression of a pediatric cancer.

Fast Track Designation, Accelerated Approval, and Priority Review

A sponsor may seek approval of its drug candidate under programs designed to accelerate the FDA's review and approval of NDAs. For example, Fast Track Designation may be granted to a drug intended for treatment of a serious or life-threatening disease or condition that has potential to address unmet medical needs for the disease or condition. The key benefits of Fast Track Designation are more frequent interactions with the FDA and rolling review (submission of portions of an application before the complete marketing application is submitted).

Under the accelerated approval programs, the FDA may approve an NDA on the basis of either 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. Post-marketing studies or completion of ongoing studies after marketing approval are required to verify the drug's clinical benefit in relationship to the surrogate endpoint or ultimate outcome in relationship to the clinical benefit. FDA is authorized to require a post-approval study to be underway prior to approval or within a specified time period following approval 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.

Based on results of the Phase 3 clinical trials submitted in an NDA, upon the request of an applicant, the FDA may grant the NDA a Priority Review designation, which sets the target date for FDA action on the application at eight months after the NDA submission. Priority Review is granted where there is evidence that the proposed product would be a significant improvement in the safety or effectiveness of the treatment, diagnosis, or prevention of a serious condition. If criteria are not met for Priority Review, the application is subject to the standard FDA review period of twelve months after NDA submission. Priority Review designation does not change the scientific/medical standard for approval or the quality of evidence necessary to support approval.

EU Drug Development

Clinical Trials in the EU

Similarly to the United States, the various phases of non-clinical and clinical research in the EU are subject to significant regulatory controls.

In the EU, clinical trials are governed by the Clinical Trials Regulation (EU) No 536/2014, or CTR, which entered into application on January 31, 2022 repealing and replacing the former Clinical Trials Directive 2001/20, or CTD. The CTR is intended to harmonize and streamline clinical trial authorizations, simplify adverse-event reporting procedures, improve the supervision of clinical trials and increase transparency. Specifically, the Regulation, which is directly applicable in all EU Member States, introduces a streamlined application procedure through a single-entry point, the "EU portal", the Clinical Trials Information System, or CTIS; a single set of documents to be prepared and submitted for the application; as well as simplified reporting procedures for clinical trial sponsors. A harmonized procedure for the assessment of applications for clinical trials has been introduced and is divided into two parts. Part I assessment is led by the competent authorities of a reference Member State selected by the trial sponsor and relates to clinical trial aspects that are considered to be scientifically harmonized across EU Member States. This assessment is then submitted to the competent authorities of all concerned Member States in which the trial is to be conducted for their review. Part II is assessed separately by the competent authorities and Ethics Committees in each concerned EU Member State. Individual EU Member States retain the power to authorize the conduct of clinical trials on their territory.

The extent to which on-going clinical trials will be governed by the CTR will depend on the duration of the individual clinical trial. For clinical trials in relation to which an application for approval was made on the basis of the CTD before January 31, 2023, the CTD will continue to apply on a transitional basis until January 31, 2025. By that date, all ongoing trials will become subject to the provisions of the CTR. The CTR will apply to clinical trials from an earlier date if the related clinical trial application was made on the basis of the CTR or if the clinical trial has already transitioned to the CTR framework before January 31, 2025.

In all cases, clinical trials must be conducted in accordance with GCP and the applicable regulatory requirements. Medicines used in clinical trials, including ATMPs, must be manufactured in accordance with the guidelines on cGMP and in a GMP licensed facility, which can be subject to GMP inspections.

EU Review and approval process

In the EU, medicinal products can only be commercialized after a related marketing authorization, or MA, has been granted. To obtain an MA for a product in the EU, an applicant must submit a Marketing Authorization Application, or MAA, either under a centralized procedure administered by the EMA, or one of the procedures administered by the competent authorities of EU Member States (decentralized procedure, national procedure or mutual recognition procedure). An MA may be granted only to an applicant established in the EU.

The centralized procedure provides for the grant of a single MA by the European Commission that is valid throughout the EEA (which is comprised of the 27 EU Member States plus Norway, Iceland and Liechtenstein). Pursuant to Regulation (EC) No 726/2004, the centralized procedure is compulsory for specific products, including for (i) medicinal products derived from biotechnological processes, (ii) products designated as orphan medicinal products, (iii) advanced therapy medicinal products, or ATMPs, and (iv) products with a new active substance indicated for the treatment of HIV/AIDS, cancer, neurodegenerative diseases, diabetes, auto-immune and other immune dysfunctions and viral diseases. For products with a new active substance indicated for the treatment of other diseases and products that are highly innovative or for which a centralized process is in the interest of patients, authorization through the centralized procedure is optional on related approval.

Under the centralized procedure, the EMA's Committee for Medicinal Products for Human Use, or CHMP, conducts the initial assessment of a product. The CHMP is also responsible for several post-authorization and maintenance activities, such as the assessment of modifications or extensions to an existing MA. The maximum timeframe for the evaluation of an MAA under the centralized procedure is 210 days, excluding clock stops when additional information or written or oral explanation is to be provided by the applicant in response to questions of the CHMP. Accelerated assessment may be granted by the CHMP in exceptional cases, when a medicinal product targeting an unmet medical need is expected to be of major interest from the point of view of public health and, in particular, from the viewpoint of therapeutic innovation. If the CHMP accepts a request for accelerated assessment, the time limit of 210 days will be reduced to 150 days (excluding clock stops). The CHMP can, however, revert to the standard time limit for the centralized procedure if it considers that it is no longer appropriate to conduct an accelerated assessment.

Unlike the centralized authorization procedure, the decentralized MA procedure requires a separate application to, and leads to separate approval by, the competent authorities of each EU Member State in which the product is to be marketed. This application is identical to the application that would be submitted to the EMA for authorization through the centralized procedure. The reference EU Member State prepares a draft assessment and drafts of the related materials within 120 days after receipt of a valid application. The resulting assessment report is submitted to the concerned EU Member States who, within 90 days of receipt, must decide whether to approve the assessment report and related materials. If a concerned EU Member State cannot approve the assessment report and related materials due to concerns relating to a potential serious risk to public health, disputed elements may be referred to the Heads of Medicines Agencies' Coordination Group for Mutual Recognition and Decentralized Procedures – Human, or CMDh, for review. The subsequent decision of the European Commission is binding on all EU Member States.

The mutual recognition procedure allows companies that have a medicinal product already authorized in one EU Member State to apply for this authorization to be recognized by the competent authorities in other EU Member States. Like the decentralized procedure, the mutual recognition procedure is based on the acceptance by the competent authorities of the EU Member States of the MA of a medicinal product by the competent authorities of other EU Member States. The holder of a national MA may submit an application to the competent authority of an EU Member State requesting that this authority recognize the MA delivered by the competent authority of another EU Member State.

An MA has, in principle, an initial validity of five years. The MA may be renewed after five years on the basis of a re-evaluation of the risk-benefit balance by the EMA or by the competent authority of the EU Member State in which the original MA was granted. To support the application, the MA holder must provide the EMA or the competent authority with a consolidated version of the Common Technical Document providing up-to-date data concerning the quality, safety and efficacy of the product, including all variations introduced since the MA was granted, at least nine months before the MA ceases to be valid. The European Commission or the competent authorities of the EU Member States may decide on justified grounds relating to pharmacovigilance, to proceed with one further five-year renewal period for the MA. Once subsequently definitively renewed, the MA shall be valid for an unlimited period. Any authorization which is not followed by the actual placing of the medicinal product on the EU market (for a centralized MA) or on the market of the authorizing EU Member State within three years after authorization ceases to be valid (the so-called sunset clause).

Innovative products that target an unmet medical need and are expected to be of major public health interest may be eligible for a number of expedited development and review programs, such as the Priority Medicines, or PRIME, scheme, which provides incentives similar to the breakthrough therapy designation in the U.S. PRIME is a voluntary scheme aimed at enhancing the EMA's

support for the development of medicinal products that target unmet medical needs. 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 EU or, if there is, the new medicinal product will bring a major therapeutic advantage) and they must demonstrate the potential to address the unmet medical need by introducing new methods of therapy or improving existing ones. Benefits accrue to sponsors of product candidates with PRIME designation, including but not limited to, early and proactive regulatory dialogue with the EMA, frequent discussions on clinical trial designs and other development program elements, and potentially accelerated MAA assessment once a dossier has been submitted.

In the EU, a "conditional" MA may be granted in cases where all the required safety and efficacy data are not yet available. The European Commission may grant a conditional MA for a medicinal product if it is demonstrated that all of the following criteria are met: (i) the benefit-risk balance of the medicinal product is positive; (ii) it is likely that the applicant will be able to provide comprehensive data post-authorization; (iii) the medicinal product fulfils an unmet medical need; and (iv) the benefit of the immediate availability to patients of the medicinal product is greater than the risk inherent in the fact that additional data are still required. The conditional MA is subject to conditions to be fulfilled for generating the missing data or ensuring increased safety measures. It is valid for one year and must be renewed annually until all related conditions have been fulfilled. Once any pending studies are provided, the conditional MA can be converted into a traditional MA. However, if the conditions are not fulfilled within the timeframe set by the EMA and approved by the European Commission, the MA will cease to be renewed.

An MA may also be granted "under exceptional circumstances" where the applicant can show that it is unable to provide comprehensive data on efficacy and safety under normal conditions of use even after the product has been authorized and subject to specific procedures being introduced. These circumstances may arise when the intended indications are very rare and, in the state of scientific knowledge at that time, it is not possible to provide comprehensive information, or when generating data may be contrary to generally accepted ethical principles. Like a conditional MA, an MA granted in exceptional circumstances is reserved to medicinal products intended to be authorized for treatment of rare diseases or unmet medical needs for which the applicant does not hold a complete data set that is required for the grant of a standard MA. However, unlike the conditional MA, an applicant for authorization in exceptional circumstances is not subsequently required to provide the missing data. Although the MA "under exceptional circumstances" is granted definitively, the risk-benefit balance of the medicinal product is reviewed annually, and the MA will be withdrawn if the risk-benefit ratio is no longer favorable.

Pediatric Development in the EU

In the EU, Regulation (EC) No 1901/2006 provides that all MAAs for new medicinal products have to include the results of trials conducted in the pediatric population, in compliance with a pediatric investigation plan, or PIP, agreed with the EMA's Pediatric Committee, or PDCO. The PIP sets out the timing and measures proposed to generate data to support a pediatric indication of the medicinal product for which MA is being sought. The PDCO can grant a deferral of the obligation to implement some or all of the measures provided in the PIP until there are sufficient data to demonstrate the efficacy and safety of the product in adults. Further, the obligation to provide pediatric clinical trial data can be waived by the PDCO when these data are not needed or appropriate because the product is likely to be ineffective or unsafe in children, the disease or condition for which the product is intended occurs only in adult populations, or when the product does not represent a significant therapeutic benefit over existing treatments for pediatric patients. Once the MA is obtained in all EU Member States and study results are included in the product information, even when negative, the product is eligible for a six-month extension to the Supplementary Protection Certificate, or SPC, if any is in effect at the time of authorization or, in the case of orphan medicinal products, a two-year extension of orphan market exclusivity.

Manufacturing Regulation in the EU

In addition to an MA, various other requirements apply to the manufacturing and placing on the EU market of medicinal products. The manufacturing of medicinal products in the EU requires a manufacturing authorization and import of medicinal products into the EU requires a manufacturing authorization allowing for import. The manufacturing authorization holder must comply with various requirements set out in the applicable EU laws, regulations and guidance, including EU cGMP standards. Similarly, the distribution of medicinal products within the EU is subject to compliance with the applicable EU laws, regulations and guidelines, including the requirement to hold appropriate authorizations for distribution granted by the competent authorities of EU Member States. Marketing authorization holders and/or manufacturing and import authorization, or MA holders and/or distribution authorization holders may be subject to civil, criminal or administrative sanctions, including suspension of manufacturing authorization, in case of non-compliance with the EU or EU Member States' requirements applicable to the manufacturing of medicinal products.

Data and Market Exclusivity in the EU

The EU provides opportunities for data and market exclusivity related to MAs. Upon receiving an MA, innovative medicinal products are generally entitled to receive eight years of data exclusivity and 10 years of market exclusivity. Data exclusivity, if granted, prevents regulatory authorities in the EU from referencing the innovator's data to assess a generic application

or bio similar application for eight years from the date of authorization of the innovative product, after which a generic or bio similar MAA can be submitted, and the innovator's data may be referenced. The market exclusivity period prevents a successful generic or bio similar applicant from commercializing its product in the EU until 10 years have elapsed from the initial MA of the reference product in the EU. The overall ten-year period may, occasionally, be extended for a further year to a maximum of 11 years if, during the first eight years of those ten years, the MA holder obtains an authorization for one or more new therapeutic indications which, during the scientific evaluation prior to their authorization, are held to bring a significant clinical benefit in comparison with existing therapies. However, there is no guarantee that a product will be considered by the EU's regulatory authorities to be a new chemical/biological entity, and products may not qualify for data exclusivity.

Orphan Designation in the EU

In the EU, Regulation (EC) No. 141/2000, as implemented by Regulation (EC) No. 847/2000 provides that a medicinal product can be designated as an orphan medicinal product by the European Commission if its sponsor can establish that: (i) the product is intended for the diagnosis, prevention or treatment of life-threatening or chronically debilitating conditions; (ii) either (a) such conditions affect not more than 5 in 10,000 persons in the EU when the application is made, or (b) the product without the benefits derived from orphan status, would not generate sufficient return in the EU to justify the necessary investment in developing the medicinal product; and (iii) there exists no satisfactory authorized method of diagnosis, prevention, or treatment of the condition that has been authorized in the EU, or even if such method exists, the product will be of significant benefit to those affected by that condition.

Regulation (EC) No 847/2000 sets out further provisions for implementation of the criteria for designation of a medicinal product as an orphan medicinal product. An application for the designation of a medicinal product as an orphan medicinal product must be submitted at any stage of development of the medicinal product but before filing of an MAA. An MA for an orphan medicinal product may only include indications designated as orphan. For non-orphan indications treated with the same active pharmaceutical ingredient, a separate marketing authorization must be sought.

Orphan medicinal product designation entitles an applicant to incentives such fee reductions or fee waivers, protocol assistance, and access to the centralized marketing authorization procedure. Upon grant of a marketing authorization, orphan medicinal products are entitled to a ten-year period of market exclusivity for the approved therapeutic indication, which means that the EMA cannot accept another marketing authorization application or accept an application to extend for a similar product and the European Commission cannot grant a marketing authorization for the same indication for a period of ten years. The period of market exclusivity is extended by two years for orphan medicinal products that have also complied with an agreed PIP. No extension to any supplementary protection certificate can be granted on the basis of pediatric studies for orphan indications. Orphan medicinal product designation does not convey any advantage in, or shorten the duration of, the regulatory review and approval process.

The period of market exclusivity may, however, be reduced to six years if, at the end of the fifth year, it is established that the product no longer meets the criteria on the basis of which it received orphan medicinal product destination, including where it can be demonstrated on the basis of available evidence that the original orphan medicinal product is sufficiently profitable not to justify maintenance of market exclusivity or where the prevalence of the condition has increased above the threshold. Additionally, an MA may be granted to a similar medicinal product with the same orphan indication during the 10 year period if: (i) if the applicant consents to a second original orphan medicinal product application, (ii) if the manufacturer of the original orphan medicinal product is unable to supply sufficient quantities; or (iii) if the second applicant can establish that its product, although similar, is safer, more effective or otherwise clinically superior to the original orphan medicinal product. A company may voluntarily remove a product from the register of orphan products.

Post-authorization Requirements

Where an MA is granted in relation to a medicinal product in the EU, the holder of the MA is required to comply with a range of regulatory requirements applicable to the manufacturing, marketing, promotion and sale of medicinal products. Similar to the United States, both MA holders and manufacturers of medicinal products are subject to comprehensive regulatory oversight by the EMA, the European Commission and/or the competent regulatory authorities of the individual EU Member States. The holder of an MA must establish and maintain a pharmacovigilance system and appoint an individual qualified person for pharmacovigilance who is responsible for oversight of that system. Key obligations include expedited reporting of suspected serious adverse reactions and submission of periodic safety update reports, or PSURs.

All new MAAs must include a risk management plan, or RMP, describing the risk management system that the company will put in place and documenting measures to prevent or minimize the risks associated with the product. The regulatory authorities may also impose specific obligations as a condition of the MA. Such risk-minimization measures or post-authorization obligations may include additional safety monitoring, more frequent submission of PSURs, or the conduct of additional clinical trials or post-authorization safety studies.

In the EU, the advertising and promotion of medicinal products are subject to both EU and EU Member States' laws governing promotion of medicinal products, interactions with physicians and other healthcare professionals, misleading and comparative advertising and unfair commercial practices. General requirements for advertising and promotion of medicinal products, such as direct-to-consumer advertising of prescription medicinal products are established in EU law. However, the details are governed by regulations in individual EU Member States and can differ from one country to another. For example, applicable laws require that promotional materials and advertising in relation to medicinal products comply with the product's Summary of Product Characteristics, or SmPC, which may require approval by the competent national authorities in connection with an MA. The SmPC is the document that provides information to physicians concerning the safe and effective use of the product. Promotional activity that does not comply with the SmPC is considered off-label and is prohibited in the EU.

Clinical Trial Data Disclosure

Many jurisdictions have mandatory clinical trial information obligations incumbent on sponsors. In the EU, transparency requirements relating to clinical trial information are established in the CTR. The CTR establishes a general principle according to which information contained in CTIS shall be made publicly accessible unless confidentiality is justified on grounds of protecting personal data, or commercially confidential information, necessary to protect confidential communications between EU Member States in relation to the preparation of an assessment report, or necessary to ensure effective supervision of the conduct of a clinical trial by EU Member States. This confidentiality exception may be overruled if there is an overriding public interest in disclosure. The publication of data and documents in relation to the conduct of a clinical trial will take place in accordance with specific timelines. The timelines are established by the EMA and are determined based on the documents and the categorization of the clinical trial.

In addition, Regulation No. 1049/2001 on access to documents, or the ATD Regulation, and the related EMA policy 0043 on access to documents, provide for a wide right for EU-based interested parties to submit an access to documents request to the EMA to access certain information held by the EMA. Only very limited information is exempted from disclosure (i.e., commercially confidential information, which is construed increasingly narrowly and protected personal data). It is possible for competitors to access and use this data in their own research and development programs anywhere in the world, once these data are in the public domain.

Combination Products

The EU regulates medical devices and medicinal products separately, and through different legislative instruments. Products that are a combination of a medicinal product and a medical device may be regulated as either a medicinal product, a medical device or, subject to certain requirements, on the basis of both sets of rules. The applicable requirements governing placing a drug-device combination on the EU market will vary depending on the type of drug-device combination product and on which of the components of the combination has the primary mode of action.

Drug-device combination products that form a single integral product that is not reusable and for which the action of the medicinal product is principal to that of the medical device are governed by the regulatory framework applicable to medicinal products. However, the General Safety and Performance Requirements, or GSPRs, of Annex I to Regulation (EU) 2017/745 on Medical Devices, or MDR, will be applicable to the safety and performance of the medical device part of the product in the context of its use with the medicinal product. In these circumstances, an MAA must be submitted to the competent authorities responsible for evaluating the safety and effectiveness of medicinal products. As part of the MAA, the applicant must also submit, where available, the results of the assessment of the conformity of the medical device part of the product with the MDR contained in the manufacturer's EU Declaration of Conformity of the device or the relevant Certificate of Conformity issued by a Notified Body. If the MAA does not include the results of the conformity assessment, and where the conformity assessment of the device, if used separately, requires the involvement of a Notified Body, the competent authorities must require the applicant to provide a Notified Body Opinion on the conformity of the device with the relevant GSPRs. Based on this approach, the competent authorities responsible for medicinal products will review the specific aspects of the medical devices part of the product which are relevant to the safety and efficacy of the medicinal product and the Notified Body, where applicable, will evaluate the relevant GSPRs of the device.

Drug-device combination products that form a single integral product that is not reusable and for which the action of the medicinal products is ancillary to that of the medical device are governed by the regulatory framework applicable to medical devices in accordance with the MDR. However, the quality, safety and usefulness of the medicinal product must also be verified as part of the device and a scientific opinion from a national competent authority of an EU Member State or from the EMA, depending on its nature and therapeutic intention, must be sought regarding the quality and safety of the medicinal product, including the benefit or risk of its incorporation into the medical device. Where a medical device incorporates a medicinal product as an integral part as a single use drug delivery system, which is intended exclusively for use in the given combination and which is not reusable, it is regulated as a medicinal product. In this case, the relevant General Safety and Performance Requirements, or GSPRs of the MDR will apply to the safety and performance of the device element.

By contrast, drug-device combination products which do not form a single integral product will be regulated separately. This may include, for example a drug-device combination product where a medical device and a medicinal product are co-packaged

and the medical device is intended solely to be used for the administration of the co-packaged medicinal product. In these circumstances, the medicinal product will be governed by the regulatory framework applicable to medicinal products and the medical device will be governed by the MDR. However, the characteristics of a medical device used for the administration of a medicinal product may impact the quality, safety and efficacy profile of the medicinal product. As a result, as part of the MAA submitted to the competent authorities for the medicinal product, the applicant may need to provide additional information regarding the characteristics of the co-packaged medical device that may impact on the quality, safety and/or efficacy of the medicinal product. Similar requirements may apply where the products are not co-packaged but the medicinal product information makes an explicit reference to a specific medical device.

Pharmaceutical Coverage, Pricing and Reimbursement

In the United States and markets in other countries, patients who are prescribed treatments for their conditions and providers performing the prescribed services generally rely on third-party payors to reimburse all or part of the associated healthcare costs. Patients are unlikely to use our products unless coverage is provided, and reimbursement is adequate to cover a significant portion of the cost of our products. Significant uncertainty exists as to the coverage and reimbursement status of products approved by the FDA and other government authorities. Even if our drug candidates are approved, sales of our products will depend, in part, on the extent to which third-party payors, including government health programs in the United States such as Medicare and Medicaid, commercial health insurers and managed care organizations, provide coverage, and establish adequate reimbursement levels for, such products. The process for determining whether a payor will provide coverage for a product is separate from the process for setting the price or reimbursement rate that the payor will pay for the product if coverage is approved. Third-party payors are increasingly challenging the prices charged, examining the medical necessity, and reviewing the cost-effectiveness of medical products and services and imposing controls to manage costs. Third-party payors may limit coverage to specific products on an approved list, also known as a formulary, which might not include all of the approved products for a particular indication.

In order to secure coverage and reimbursement for any product that might be approved for sale, a company may need to conduct expensive pharmacoeconomic studies in order to demonstrate the medical necessity and cost-effectiveness of the product, in addition to the costs required to obtain FDA or other comparable marketing approvals. Nonetheless, drug candidates may not be considered medically necessary or cost effective. A decision by a third-party payor not to cover our drug candidates could reduce physician utilization of our products once approved and have a material adverse effect on our sales, results of operations and financial condition. Additionally, a payor's decision to provide coverage for a product does not imply that an adequate reimbursement rate will be approved. Further, one payor's determination to provide coverage for a drug product does not assure that other payors will also provide coverage and reimbursement for the product, and the level of coverage and reimbursement can differ significantly from payor to payor. Third-party reimbursement and coverage may not be available to enable us to maintain price levels sufficient to realize an appropriate return on our investment in product development.

Within the United States, if we obtain appropriate approval in the future to market any of our drug product candidates, those products could potentially be covered by various government health benefit programs as well as purchased by government agencies. The participation in such programs or the sale of products to such agencies is subject to regulation.

The containment of healthcare costs also has become a priority of federal, state and foreign governments and the prices of drugs have been a focus in this effort. Governments have shown significant interest in implementing cost-containment programs, including price controls, restrictions on reimbursement and requirements for substitution of generic products. Adoption of price controls and cost-containment measures, and adoption of more restrictive policies in jurisdictions with existing controls and measures, could further limit a company's revenue generated from the sale of any approved products. Coverage policies and third-party reimbursement rates may change at any time. Even if favorable coverage and reimbursement status is attained for one or more products for which a company or its collaborators receive marketing approval, less favorable coverage policies and reimbursement rates may be implemented in the future. Any reduction in reimbursement from Medicare and other government programs may result in a similar reduction in payments from private payors. The implementation of cost containment measures or other healthcare reforms may prevent the generation of revenue, attainment of profitability, or commercialization of products. In addition, it is possible that there will be further legislation or regulation that could harm the business, financial condition and results of operations.

Outside the United States, ensuring adequate coverage and payment for our drug candidates will face challenges. Pricing of prescription pharmaceuticals is subject to governmental control in many countries. Pricing negotiations with governmental authorities can extend well beyond the receipt of marketing approval for a product and may require us to conduct a clinical study that compares the cost effectiveness of our drug candidates or products to other available therapies. The conduct of such a clinical study could be expensive and result in delays in our commercialization efforts.

In the EU, 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 drug candidate to currently available therapies (so called HTA) in order to obtain reimbursement or approval. This HTA process is the procedure according to which the assessment of the public health impact,

therapeutic impact and the economic and societal impact of use of a given medicinal product in the national healthcare systems of the individual country is conducted. The outcome of HTA regarding specific medicinal products will often influence the pricing and reimbursement status granted to these medicinal products by the competent authorities of individual EU Member States. For example, the EU provides options for its member states to restrict the range of products for which their national health insurance systems provide reimbursement and to control the prices of medicinal products for human use. EU member states may approve a specific price for a product or it may instead adopt a system of direct or indirect controls on the profitability of the company placing the product on the market. Other member states allow companies to fix their own prices for products but monitor and control prescription volumes and issue guidance to physicians to limit prescriptions. Recently, many countries in the EU have increased the amount of discounts required on pharmaceuticals and these efforts could continue as countries attempt to manage healthcare expenditures, especially in light of the severe fiscal and debt crises experienced by many countries in the EU. The downward pressure on healthcare costs in general, particularly prescription drugs, has become intense. As a result, increasingly high barriers are being erected to the entry of new products. Political, economic and regulatory developments may further complicate pricing negotiations, and pricing negotiations may continue after reimbursement has been obtained. Reference pricing used by various EU member states, and parallel trade (arbitrage between low-priced and high-priced member states), can further reduce prices. There can be no assurance that any country that has price controls or reimbursement limitations for pharmaceutical products will allow favorable reimbursement and pricing arrangements for any of our products, if approved in those countries.

Healthcare Law and Regulation

Healthcare providers and third-party payors play a primary role in the recommendation and prescription of drug products that are granted marketing approval. Arrangements with providers, consultants, third-party payors and customers are subject to broadly applicable fraud and abuse, anti-kickback, false claims laws, reporting of payments to physicians, certain other healthcare providers and teaching hospitals and patient privacy laws and regulations and other healthcare laws and regulations that may constrain our business and/or financial arrangements. Restrictions under applicable federal and state healthcare laws and regulations, include the following:

- the U.S. federal Anti-Kickback Statute, which prohibits, among other things, persons and entities from knowingly and willfully soliciting, offering, paying, receiving or providing remuneration, directly or indirectly, in cash or in kind, to induce or reward either the referral of an individual for, or the purchase, order or recommendation of, any good or service, for which payment may be made, in whole or in part, under a federal healthcare program such as Medicare and Medicaid;
- the federal civil and criminal false claims laws, including the civil False Claims Act, and civil monetary penalties laws, which prohibit individuals or entities from, among other things, knowingly presenting, or causing to be presented, to the federal government, claims for payment that are false, fictitious or fraudulent or knowingly making, using or causing to made or used a false record or statement to avoid, decrease or conceal an obligation to pay money to the federal government;
- the federal HIPAA, which created additional federal criminal laws that prohibit, among other things, knowingly and willfully executing, or attempting to execute, a scheme to defraud any healthcare benefit program or making false statements relating to healthcare matters;
- HIPAA, as amended by the HITECH, which impose obligations, including mandatory contractual terms, with respect to safeguarding the privacy, security and transmission of individually identifiable health information;
- the federal transparency requirements known as the federal Physician Payments Sunshine Act, under the PPACA, which requires certain manufacturers of drugs, devices, biologics and medical supplies to report annually to the CMS, within the HHS, information related to payments and other transfers of value made by that entity to physicians (defined to include to include doctors, dentists, optometrists, podiatrists and chiropractors), certain other healthcare providers (such as physician assistants and nurse practitioners), and teaching hospitals, as well as ownership and investment interests held by physicians and their immediate family members;
- analogous state and foreign laws and regulations, such as state and foreign anti-kickback and false claims laws, which may apply to healthcare items or services that are reimbursed by non-governmental third-party payors, including private insurers; and
- outside the United States, interactions between pharmaceutical companies and health care professionals are also governed by strict laws, such as national anti-bribery laws of European countries, national sunshine rules, regulations, industry self-regulation codes of conduct and physicians' codes of professional conduct. Failure to comply with these requirements could result in reputational risk, public reprimands, administrative penalties, fines or imprisonment.

Some state and foreign laws require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the government in addition to requiring drug manufacturers to report information related to payments to physicians and other healthcare providers or marketing expenditures and pricing information. State and foreign laws also govern the privacy and security of health information in some circumstances, many of which differ from each other in significant ways and often are not preempted by HIPAA, thus complicating compliance efforts.

Failure to comply with the aforementioned laws can result in the imposition of significant civil, criminal and administrative sanctions, damages, disgorgement, monetary fines, possible exclusion from participation in Medicare, Medicaid and other federal healthcare programs, imprisonment, and integrity oversight and reporting obligations.

Healthcare Reform

A primary trend in the United States healthcare industry and elsewhere is cost containment. There have been a number of federal and state proposals during the last few years regarding the pricing of pharmaceutical and biopharmaceutical products, limiting coverage and reimbursement for drugs and other medical products, government control and other changes to the healthcare system in the United States.

In March 2010, the United States Congress enacted the PPACA, which, among other things, includes changes to the coverage and payment for drug products under government healthcare programs.

Since its enactment, there have been executive, legal and political challenges to certain aspects of the PPACA. On June 17, 2021, the U.S. Supreme Court dismissed a challenge on procedural grounds that argued the PPACA is unconstitutional in its entirety because the "individual mandate" was repealed by Congress. In addition, on August 16, 2022, President Biden signed the IRA into law, which among other things, extends enhanced subsidies for individuals purchasing health insurance coverage in PPACA marketplaces through plan year 2025. The IRA also eliminates the "donut hole" under the Medicare Part D program beginning in 2025 by significantly lowering the beneficiary maximum out-of-pocket cost and through a newly established manufacturer discount program. It is possible that the PPACA will be subject to judicial or Congressional challenges in the future. It is unclear how any such challenges and additional healthcare reform measures will impact the PPACA and our business. Other healthcare reform measures that may be adopted in the future could have a material adverse effect on our industry generally and on our ability to maintain or increase sales of our existing products that we successfully commercialize or to successfully commercialize our drug candidates, if approved.

Other legislative changes have been proposed and adopted in the United States since the PPACA was enacted. In August 2011, the Budget Control Act of 2011, among other things, created measures for spending reductions by Congress. A Joint Select Committee on Deficit Reduction, tasked with recommending a targeted deficit reduction of at least $1.2 trillion for the years 2013 through 2021, was unable to reach required goals, thereby triggering the legislation's automatic reduction to several government programs. This includes aggregate reductions of Medicare payments to providers up to 2% per fiscal year, which went into effect in April 2013 and will remain in effect through 2031, unless additional Congressional action is taken. Additionally, on March 11, 2021, President Biden signed the American Rescue Plan Act of 2021 into law, which eliminates the statutory Medicaid drug rebate cap, currently set at 100% of a drug's average manufacturer price, for single-source and innovator multiple source drugs, effective January 1, 2024. In addition, Congress is considering additional health reform measures.

In addition to the PPACA, there will continue to be proposals by legislators at both the federal and state levels, regulators and third-party payors to keep healthcare costs down while expanding individual healthcare benefits. For example, there has been increasing legislative and enforcement interest in the United States with respect to drug pricing practices. Specifically, there have been several recent presidential executive orders, Congressional inquiries, and proposed federal legislation designed to, among other things, bring more transparency to drug pricing, reduce the cost of prescription drugs under Medicare, review the relationship between pricing and manufacturer patient programs, and reform government program reimbursement methodologies for drugs. For example, in July 2021, the Biden administration released an executive order, "Promoting Competition in the American Economy," with multiple provisions aimed at prescription drugs. In response to Biden's executive order, on September 9, 2021, HHS released a Comprehensive Plan for Addressing High Drug Prices that outlines principles for drug pricing reform and sets out a variety of potential legislative policies that Congress could pursue as well as potential administrative actions HHS can take to advance these principles. Further, the IRA will, among other things (i) allow HHS to negotiate the price of certain high-expenditure, single-source drugs and biologics covered under Medicare, and subject drug manufacturers to civil monetary penalties and a potential excise tax by offering a price that is not equal to or less than the "negotiated fair price" under the law and (ii) impose rebates with respect to certain drugs and biologics covered under Medicare Part B or Medicare Part D to penalize price increases that outpace inflation. On August 29, 2023, HHS announced the list of the first ten drugs that will be subject to price negotiations, although the Medicare drug price negotiation program is currently subject to legal challenges. It is unclear how the IRA will be effectuated but is likely to have a significant impact on the pharmaceutical industry. In response to the Biden administration's October 2022 executive order, on February 14, 2023, HHS released a report outlining three new models for testing by the CMS Innovation Center which will be evaluated on their ability to lower the cost of drugs, promote accessibility, and improve quality of care. It is unclear whether the models will be utilized in any

health reform measures in the future. Further, on December 7, 2023, the Biden administration announced an initiative to control the price of prescription drugs through the use of march-in rights under the Bayh-Dole Act. On December 8, 2023, the National Institute of Standards and Technology published for comment a Draft Interagency Guidance Framework for Considering the Exercise of March-In Rights which for the first time includes the price of a product as one factor an agency can use when deciding to exercise march-in rights. While march-in rights have not previously been exercised, it is uncertain if that will continue under the new framework. 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. Any reduction in reimbursement from Medicare and other government programs may result in a similar reduction in payments from private payors. The implementation of cost containment measures or other healthcare reforms may prevent the generation of revenue, attainment of profitability, or commercialization of products. In addition, it is possible that there will be further legislation or regulation that could harm the business, financial condition and results of operations.

In the EU, in December 2021, Regulation No 2021/2282 on HTA, or HTA Regulation, was adopted. The HTA Regulation is intended to boost cooperation among EU Member States in assessing health technologies, including new medicinal products, and providing the basis for cooperation at EU level for joint clinical assessments in these areas. When it enters into application in 2025, the HTA Regulation will be intended to harmonize the clinical benefit assessment of HTA Regulation across the EU.

Employees and Human Capital

Our human capital objectives include identifying, recruiting, retaining, incentivizing, and integrating our employees. Through equity incentive plans, we aim to attract, retain, and motivate selected staff. Our success hinges on retaining highly skilled employees, and we offer competitive salaries, bonuses, equity opportunities, development programs, and a comprehensive well-being package.

As of December 31, 2023, we had 32 total employees, of which 18 are in research and development and 14 are in general and administrative. Our employees are primarily located in South San Francisco, California, Medolla, Italy and Bresso Italy, and others work remotely from their residences located across the United States. None of our employees are represented by a labor union or are a party to a collective bargaining agreement and we believe that we have good relations with our employees.

On January 30, 2023, our Board approved a plan to reduce our workforce by approximately 47% as of such date, in order to preserve cash and to align operations with the change in our corporate strategy.

On October 20, 2023, upon the completion of the EryDel Acquisition, our headcount increased by 20 after the integration of the non-US employees, of which 16 are in research and development, while 4 are in general and administrative functions. Our newly acquired human capital is directly contributing to the advancement of our Phase 3 lead asset, EryDex, as an integral part of our proprietary drug-device combination technology platform.

Corporate Information

We were incorporated in Delaware on June 20, 2012. Our principal executive offices are located at 611 Gateway Blvd Suite 273, South San Francisco, CA 94080. Our telephone number at that location is (415) 910-5717. Our corporate website address is www.quincetx.com. Information contained on, or that may be accessed through, our website is not incorporated by reference into this Annual Report on Form 10-K and should not be considered a part of this Annual Report on Form 10-K.

Quince is a registered trademark of Quince Therapeutics, Inc. All other brand names or trademarks appearing in this Annual Report on Form 10-K are the property of their respective holders. Solely for convenience, the trademarks and trade names in this Annual Report on Form 10-K are referred to without the ® and ™ symbols, but such references should not be construed as any indicator that their respective owners will not assert, to the fullest extent under applicable law, their rights thereto.

Item 1A. Risk Factors.

Our operations and financial results are subject to various risks and uncertainties, including those described below that could adversely affect our business, financial condition, results of operations, cash flows and the trading price of our common stock. You should carefully consider the following risks, together with all of the other information in this Annual Report on Form 10-K, including our consolidated financial statements and the related notes included elsewhere in this Annual Report on Form 10-K.

Risks Relating to Our Business

We may experience difficulties integrating Quince and EryDel's operations and realizing the expected benefits of the EryDel Acquisition.

The success of the EryDel Acquisition will depend in part on our ability to realize the expected operational efficiencies and associated cost synergies and anticipated business opportunities and growth prospects from the EryDel Acquisition in an efficient and effective manner. We may not be able to fully realize the operational efficiencies and associated cost synergies or leverage the potential business opportunities and growth prospects to the extent anticipated or at all.

Challenges associated with the integration may include those related to retaining and motivating executives and other key employees, blending corporate cultures, eliminating duplicative operations, and making necessary modifications to internal control over financial reporting and other policies and procedures in accordance with applicable laws. Some of these factors are outside our control, and any of them could delay or increase the cost of our integration efforts. The integration process could take longer than anticipated and could result in the loss of key employees, the disruption of each company's ongoing businesses, increased tax costs, inefficiencies, and inconsistencies in standards, controls, information technology systems, policies and procedures, any of which could adversely affect our ability to maintain relationships with employees or third parties, or our ability to achieve the anticipated benefits of the transaction, and could harm our financial performance. If we are unable to successfully integrate certain aspects of the operations of EryDel, including relevant human resource functions, or experience delays, we may incur unanticipated liabilities and be unable to fully realize the potential benefit of future revenue and other anticipated benefits resulting from the arrangement, and our business, results of operations and financial condition could be adversely affected.

We are substantially dependent on the success of our lead drug candidate, EryDex.

Our business and future success depends on our ability to successfully develop, obtain regulatory approval for and successfully commercialize our lead drug candidate, EryDex, which is under clinical development for A-T. EryDex is our only drug candidate in late-stage clinical development, and our business currently depends heavily on its successful development. In the previous Phase 3 ATTeST trial, the trial did not meet the primary efficacy endpoint. The trial saw statistically significant results in the age group of six to nine years old and we expect to initiate the NEAT study in this population in the second quarter 2024. We expect to announce the results of the NEAT study in the second half of 2025, but cannot guarantee that the results of this study will be positive or that they will allow further development in this therapeutic indication.

EryDex will require additional clinical and non-clinical development, regulatory review and approval in multiple jurisdictions, substantial investment, access to sufficient commercial manufacturing capacity and significant marketing efforts before we can generate any revenue from product sales. We cannot be certain EryDex will receive regulatory approval or be successfully commercialized even if we receive regulatory approval. In addition, because EryDex is our most advanced drug candidate, and because our other drug candidates are based on the same AIDE platform technology, if EryDex encounters safety or efficacy problems, developmental delays or regulatory issues or other problems, our development plans and business would be significantly harmed.

We have no drug candidates approved for commercial sale, we have never generated any revenue from sales, and we may never be profitable.

We have no drug candidates approved for sale, have never generated any revenue from sales, have never been profitable and do not expect to be profitable in the foreseeable future. We have incurred net losses in each year since our inception. For the years ended December 31, 2023 and 2022, our net losses were $31.4 million and $51.7 million, respectively. We had an accumulated deficit of $319.6 million as of December 31, 2023.

Before we are able to generate any revenue, we will need to commit substantial funds to the anticipated clinical and development activities related to EryDex, and we may not be able to obtain sufficient funds on acceptable terms, if at all. Any additional debt financing or additional equity that we raise may contain terms that are not favorable to us and/or result in dilution to our stockholders.

We expect that it could take several years, if ever, before we may have a drug candidate ready for commercialization. We expect to continue to incur losses for the foreseeable future, and we expect these losses to increase as we pursue our current strategic direction, and seek regulatory approvals for any drug candidates, prepare for and begin the commercialization of any approved drug candidates, and add infrastructure and personnel to support our drug development efforts and operations as a public company. We anticipate that any such losses could be significant for the next several years. These net losses and negative cash flows have had, and will continue to have, an adverse effect on our stockholders' equity and working capital. Further, these net losses have fluctuated significantly in the past and are expected to continue to significantly fluctuate from quarter-to-quarter or year-to-year. To become and remain profitable, we must develop and eventually commercialize a drug with significant revenue.

We may never succeed in developing a commercial drug. On January 25, 2022, the FDA placed a full clinical hold on the IND for atuzaginstat (COR388), one of our assets that has since been out-licensed. On March 8, 2023 The FDA placed a partial clinical hold on the IND for EryDex related to extractables and leachables of new components used in the EryKit. The FDA subsequently lifted the partial clinical hold on September 23, 2023. Additionally, the Phase 3 ATTeST study conducted by EryDel failed to meet the primary endpoint. The FDA may place additional clinical holds on our current or currently contemplated clinical programs or otherwise limit our ability to proceed with other clinical programs in our pipeline, which will harm our business, financial condition, results of operations and may force us to cease our operations.

We expect to explore partnership and licensing opportunities to support the future development of EryDex and other drug candidates. We may also encounter other unforeseen expenses, difficulties, complications, delays and other known and unknown challenges as we pursue our current strategic direction.

There are numerous risks and uncertainties, we are unable to accurately predict the timing or amount of increased expenses or when, or if, we will be able to generate revenues or achieve profitability. If we do achieve profitability, we may not be able to sustain or increase profitability on a quarterly or annual basis and we will continue to incur substantial research and development and other expenditures to develop and market additional drug candidates.

We may be required to make milestone payments to the EryDel Shareholders or pursuant to the EIB Facility in connection with our development and commercialization of EryDex, which could adversely affect the overall profitability of EryDex, if approved.

In connection with the EryDel Acquisition, we may be required to make additional payments to EryDel Shareholders of up to an aggregate of $485.0 million in potential cash payments, comprised of up to $5.0 million upon the achievement of a specified development milestone, $25.0 million at NDA acceptance, up to $60.0 million upon the achievement of specified approval milestones, and up to $395.0 million upon the achievement of specified on market and sales milestones, with no royalties paid to EryDel. These milestone obligations could impose substantial additional costs on us, divert resources from other aspects of our business, and adversely affect the overall profitability of EryDex, if approved. We may need to obtain additional financing to satisfy these milestone payments, and cannot be sure that any additional funding, if needed, will be available on terms favorable to us, or at all.

Additionally, in connection with the EIB Facility, we are also required to make additional payments to the EIB consisting of (i) interest payments on the outstanding loans thereunder, (ii) payments based on a percentage of the revenue derived from the acquisition of EryDel USA, Inc. on July 21, 2023, which will be payable annually with respect to the immediately preceding fiscal year commencing on June 30, 2027, and (iii) repayments of the principal amount of the loans under the EIB Facility upon the occurrence of certain events. The occurrence of certain events of default under the EIB Facility, including failure to make payments as they become due (subject to a grace period of three (3) business days) to the EIB, would result in the EIB having the right to accelerate and demand immediate payment of all outstanding obligations, together with accrued interest, if any, and any prepayment fees, under the EIB Facility. We may need additional funding in order to make such payments.

Our future results could suffer if we do not effectively manage our operations.

In connection with our new strategic pursuits, we may expand our size and operations through the EryDel Acquisition. Our future success depends, in part, upon our ability to manage such expanded business, which may pose substantial challenges for management, including challenges related to the management and monitoring of new operations and associated increased costs and complexity. There can be no assurances that we will be successful or that we will realize the expected synergies and other benefits anticipated from any future acquisitions or strategic transactions that we may undertake in the future.

Our financial results have been in the past and may in the future be adversely affected by impairment charges from the recording of goodwill and intangible assets.

Our financial results have been in the past and may in the future be adversely affected by impairment charges from the recording of goodwill and intangible assets incurred in connection with acquisitions. For example, we incurred a $0.8 million goodwill impairment charge in the quarter ended September 30, 2022 and a $5.9 million IPR&D Intangible Asset impairment charge for the quarter ended March 31, 2023 in connection with the Novosteo Acquisition. Further, our failure to identify or accurately assess the magnitude of necessary technology investments we assumed as a result of the EryDel Acquisition could result in unexpected litigation or regulatory exposure, unfavorable accounting charges, a loss of anticipated tax benefits or other adverse effects on our business, operating results or financial condition.

Risks Related to the Development of Our Drug Candidates

The Phase 3 NEAT clinical trial of EryDex for A-T will be conducted under a protocol negotiated with FDA by EryDel and our execution of the trial may be delayed, may not be successful, and may not result in NDA approval, with adverse results for our business and share price.

With the acquisition of our Phase 3 lead asset, EryDex, we intend to initiate the Phase 3 NEAT clinical trial in the first half of 2024. The NEAT protocol is the subject of an SPA, agreement with FDA. The FDA may revoke or alter its SPA agreement under the following circumstances:

- public health concerns emerge that were unrecognized at the time of the protocol assessment, or the director of the review division determines that a substantial scientific issue essential to determining safety or efficacy has been identified after testing has begun;
- a sponsor fails to follow a protocol that was agreed upon with the FDA; or
- the relevant data, assumptions, or information provided by the sponsor in a request for SPA change, are found to be false statements or misstatements, or are found to omit relevant facts.

A documented SPA may be modified, and such modification will be deemed binding on the FDA review division, except under the circumstances described above, if the FDA and the sponsor agree in writing to modify the protocol and such modification is intended to improve the study. An SPA, however, does not guarantee that a trial will be successful, and our execution of the Phase 3 NEAT clinical trial may be delayed and even if successful may not result in approval by the FDA.

We, or any future development partner with whom we enter into a related agreement, are required to conduct clinical and nonclinical trials in accordance with the study plan and protocols and applicable regulatory requirements. The FDA or comparable regulatory authorities outside the U.S., including in the EU, may disagree with the design or implementation of our or any of our future development partners' clinical trials. We or any of our future development partners may be unable to demonstrate to the satisfaction of the FDA or other regulatory authorities outside the U.S., including in the EU, that a product candidate is safe and effective for any indication.

In addition, we are responsible for ensuring that each of our nonclinical studies and clinical trials are conducted in accordance with the study plan and protocols and applicable regulatory requirements and that drug candidates are manufactured and tested in accordance with applicable GMP requirements and other applicable regulatory requirements. If we or any future development partner are unable to demonstrate that our candidate drugs were manufactured and clinical trials were conducted in accordance with applicable regulations we may be unable to submit appropriate evidence to support applications for drug approval and the authorities may reject or related applications.

Clinical drug development is a lengthy, expensive and uncertain process. The results of preclinical studies and early clinical trials are not always predictive of future results. Any drug candidate that we may advance into clinical trials may not achieve favorable results in later clinical trials, if any, or receive marketing approval.

The research and development of drugs is extremely risky. Only a small percentage of drug candidates that enter the development process ever receive marketing approval. Before obtaining marketing approval from regulatory authorities for the sale of any drug candidate, we must complete preclinical development and then conduct extensive clinical trials to demonstrate the safety and efficacy of our drug candidates in humans. Clinical testing is expensive and can take many years to complete, and its outcome is inherently uncertain.

The results of preclinical studies and completed clinical trials are not necessarily predictive of future results, and our current drug candidate may not be further developed or have favorable results in later studies or trials. Clinical trial failure may result from a multitude of factors including, but not limited to, flaws in study design, dose selection, placebo effect, patient enrollment criteria and failure to demonstrate favorable safety or efficacy traits. As such, failure in clinical trials can occur at any stage of testing. The Phase 3 ATTeST clinical trial conducted by EryDel failed to meet the primary endpoint and was potentially negatively affected by missing date during the COVID-19 pandemic. Several companies in the pharmaceutical industry have suffered setbacks in the advancement of their drug candidates into later-stage clinical trials due to lack of efficacy or adverse safety profiles, notwithstanding results in earlier preclinical studies or clinical trials. In addition, data obtained from preclinical trials and clinical trials are susceptible to varying interpretations, and regulatory authorities may not interpret our data as favorably as we do, which may further delay, limit or prevent development efforts, clinical trials or marketing approval. Furthermore, as more competing drug candidates within a particular class of drugs proceed through clinical development to regulatory review and approval, the amount and type of clinical data that may be required by regulatory authorities may increase or change.

If we are unable to complete preclinical studies or clinical trials of any future drug candidates, due to safety or efficacy concerns, or if the results of these trials are not sufficient to convince regulatory authorities of their safety or efficacy, we will not be able to obtain marketing approval for commercialization on a timely basis or at all. Even if we are able to obtain marketing approval for our current and any future drug candidates, those approvals may be for indications or dose levels that deviate from our desired approach or may contain other limitations that would adversely affect our ability to generate revenue from sales of those drug candidates.

Results in earlier clinical trials may not be indicative of the results that may be obtained in registrational clinical trials, which may delay or prevent obtaining regulatory approval.

Clinical development is expensive and can take many years to complete, and its outcome is inherently uncertain. Failure can occur at any time during the clinical trial process. Success in preclinical studies and early clinical trials may not be predictive of results in larger clinical trials, and previous results from early or small clinical trials may not be replicated or show as favorable an outcome in further clinical trials, even if successful. For example, EryDel had previously endeavored to develop EryDex for the potential treatment of A-T. While we have not seen evidence of significant safety concerns throughout EryDex Phase 3 clinical development for A-T, it failed to meet the primary endpoint, but showed statistically effective results in a certain population, six to nine years old. We plan to conduct a Phase 3 NEAT clinical trial in the population (*i.e.* six to nine years old) that was found to be statistically effective. However, further studies in this population may not replicate previous results. Accordingly, the previous clinical trials that EryDel conducted may not have uncovered safety issues, even if they exist. The biochemical pathways that we believe are affected by EryDex are implicated in a variety of biological processes and disease conditions, and it is possible that the use of our drug candidates to treat larger numbers of patients will demonstrate unanticipated AEs, which may negatively affect their safety profile.

Many companies in the pharmaceutical and biotechnology industries have suffered significant setbacks in late-stage clinical trials after achieving positive results in early-stage development, or after achieving positive results in pivotal trials, and we have had, and may face, similar setbacks. In addition, the patient populations under investigation with EryDex have many co-morbidities that may cause severe illness or death, which may be attributed to EryDex in a manner that negatively affects the safety profile of our drug candidate. If the results of our ongoing or future clinical trials for EryDex are inconclusive with respect to efficacy, if we do not meet our clinical endpoints with statistical significance, or if there are unanticipated safety concerns or AEs that emerge during clinical trials, we may be prevented from or delayed in obtaining marketing approval, and even if we obtain marketing approval, any sales may suffer.

We will incur additional costs and may experience delays in completing, or ultimately be unable to complete, the development and commercialization of our drug candidates.

The risk of failure is high for any drug candidates we may acquire that are in clinical and preclinical development. The clinical trials and manufacturing of our drug candidates are, and the manufacturing and marketing of our drug candidates, if approved, will be, subject to extensive and rigorous review and regulation by numerous government authorities in the United States and in other countries where we intend to test and market our drug candidates. Before obtaining regulatory approvals for the commercial sale of any of our drug candidates, we must demonstrate thorough lengthy, complex and expensive preclinical testing and clinical trials that our drug candidates are both safe and effective for use in each target indication. We may not be able to develop a trial design that the FDA and other foreign regulatory authorities can accept. Each drug candidate must demonstrate an adequate risk versus benefit profile in its intended patient population and for its intended use.

Clinical trials are expensive and can take many years to complete, and their outcomes are inherently uncertain. We cannot guarantee that any future clinical trials will be conducted as planned or completed on schedule, if at all. Failure can occur at any time during the clinical trial process. For example, on January 25, 2022, the FDA placed a full clinical hold on the IND for atuzaginstat (COR388). Additionally, the Phase 2/3 study with COR388 in Alzheimer's disease failed to meet the primary endpoint. COR388 is one of our assets that has since been out-licensed. On March 8, 2023 The FDA placed a partial clinical hold on the IND for EryDex related to extractables and leachables of new components used in the EryKit. The FDA subsequently lifted the partial clinical hold on September 23, 2023. Additionally, the Phase 3 ATTeST study conducted by EryDel failed to meet the primary endpoint. Even if any future clinical trials are completed as planned, we cannot be certain that their results will support the safety and effectiveness of our potential drug candidates for their targeted indications or support continued clinical development of such drug candidates. Our ongoing and any future clinical trial results may not be successful.

In addition, even if such trials are successfully completed, we cannot guarantee that the FDA or foreign regulatory authorities will interpret the results as we do, and more trials could be required before we submit our drug candidates for approval. Moreover, results acceptable to support approval in one jurisdiction may be deemed inadequate by another regulatory authority to support regulatory approval in that other jurisdiction. To the extent that the results of the trials are not satisfactory to the FDA or

foreign regulatory authorities for support of a marketing application, we may be required to expend significant resources, which may not be available to us, to conduct additional trials in support of potential approval of our drug candidates.

If we are required to conduct preclinical studies, clinical trials or other testing of our drug candidates beyond those that we currently contemplate, if we are unable to successfully complete preclinical studies, clinical trials of our drug candidates or other testing, if the results of these trials or tests are not positive or are only modestly positive or if there are safety of efficacy concerns, we may:

- be delayed in obtaining marketing approval for our drug candidates;
- not obtain marketing approval at all or regulatory authorities may suspend, vary or withdraw marketing approvals for approved products;
- obtain approval for indications, dosages or patient populations that are not as broad as intended or desired;
- obtain approval with labeling that includes significant use or distribution restrictions or safety warnings, including boxed warnings;
- be subject to additional post-marketing testing requirements; or
- have the medicine removed from the market after obtaining marketing approval.

Drug development costs will also increase if we experience delays in testing or in obtaining marketing approvals. We do not know whether any clinical trials will begin as planned, will need to be amended or will be completed on schedule, or at all. Significant preclinical studies and clinical trial delays also could shorten any periods during which we may have the exclusive right to commercialize our drug candidates, could allow our competitors to bring drug candidates to market before we do, and could impair our ability to successfully commercialize our drug candidates, if approved, any of which may harm our business and results of operations. In addition, many of the factors that cause, or lead to a delay in the commencement or completion of, clinical trials may also ultimately lead to termination or suspension of a clinical trial. Any of these occurrences may harm our business, financial condition and prospects significantly. Any termination of any clinical trial of our drug candidates will harm our commercial prospects and our ability to generate revenues.

Our drug candidates may cause or have attributed to them undesirable side effects or have other properties that delay or prevent their regulatory approval or limit their commercial potential.

Undesirable side effects caused by our drug candidates or that may be identified as related to our drug candidates by investigators conducting our clinical trials or even related to competing products in development that utilize a similar mechanism of action or act through a similar biological disease pathway could cause us or regulatory authorities to interrupt, delay, or halt clinical trials and could result in the delay or denial of regulatory approval by the FDA or other regulatory authorities and potential product liability claims. AEs and SAEs that emerge during treatment with our drug candidates or other compounds acting through similar biological pathways may be deemed to be related to our drug candidate. This may require longer and more extensive Phase 3 clinical development, or regulatory authorities may increase the amount of data and information required to approve, market, or maintain our drug candidates and could result in negative labeling or a restrictive REMS or comparable foreign strategy. This may also result in an inability to obtain approval of our drug candidates.

The occurrence of any or all of these events may cause the development of our drug candidates to be delayed or terminated, which could materially and adversely affect our business and prospects. Our drug candidates have in the past and may in the future be deemed to cause AEs and SAEs.

Clinical trials of our drug candidates may not uncover all possible AEs that patients may experience.

Clinical trials are conducted in representative samples of the potential patient population, which may have significant variability. By design, clinical trials are based on a limited number of subjects and are of limited duration of exposure to the product, to determine whether the drug candidate demonstrates the substantial evidence of efficacy and safety necessary to obtain regulatory approval. As with the results of any statistical sampling, we cannot be sure that all side effects of our drug candidates may be uncovered. It may be the case that only with a significantly larger number of patients exposed to the drug candidate for a longer duration may there be a more complete safety profile be identified. Further, even larger clinical trials may not identify rare SAEs, and the duration of such studies may not be sufficient to identify when those events may occur. Other products have been approved by the regulatory authorities for which safety concerns have been uncovered following approval. Such safety concerns have led to labeling changes, restrictions on distribution through use of a REMS, or comparable foreign strategy, or withdrawal of products from the market, and any of our drug candidates may be subject to similar risks.

Although to date we have not seen evidence of significant safety concerns with our drug candidates in the patient populations currently undergoing clinical trials with EryDex, if approved, may experience previously unreported adverse reactions, and it is possible that the FDA or other regulatory authorities may ask for additional safety data as a condition of, or in connection with, our efforts to obtain approval of our drug candidates. If safety problems occur or are identified after our products, if any, reach the market, we may make the decision or be required by regulatory authorities to amend the labeling of our products, recall our products, or even withdraw approval for our products.

If we are not able to successfully demonstrate a favorable differentiation between EryDex and currently available corticosteroids, our business would be harmed and our ability to generate revenue from that class of drugs would be severely impaired.

Our business model is to pursue the development of off-patent drugs for which we would directly pursue the development of a red blood cell encapsulated formulation through the FDA's 505(b)(2) regulatory pathway. In order to receive sufficient reimbursement and utilization, our drug candidates, will require showing differentiation against currently available generic products. If we are not able to differentiate EryDex from currently available corticosteroids by showing a safety or efficacy benefit that is reflected in the approved label, our business would be harmed and our ability to generate revenue from that class of drugs would be severely impaired.

Because the potential rare disease target patient populations of EryDex are small, and the addressable patient population even smaller, we may not be able to effectively complete clinical trials or commercialize the drug candidate.

EryDex is in development for rare disease. Our projections of both the number of people who have these diseases, as well as the subset of people with these diseases who have the potential to benefit from treatment with EryDex are based on our beliefs and estimates. These estimates have been derived from a variety of sources, including the scientific literature, or patient foundations, and may prove to be incorrect or contain errors. New studies have in the past and may continue to change the estimated incidence or prevalence of these diseases. We cannot accurately predict the number of patients for whom treatment might be possible. Additionally, since the potentially addressable patient population for this drug candidate is limited, we may fail to enroll a sufficient number of patients in our clinical trials in a timely manner. Furthermore, even if we successfully develop this asset, and obtain commercial approval, we may not be able to achieve significant market share for EryDex. Because the potential target populations are very small, we may not effectively complete clinical trials on a timely basis or at all and may not realize any significant return from the development or potential commercialization of this asset.

Risks Relating to Our Financial Position

We are a clinical stage biotechnology company and have a limited history operating a newly acquired business, which may make it difficult to evaluate the prospects for our future viability.

From our inception, we have been focused on novel therapeutic approaches to improve the lives of patients diagnosed with Alzheimer's and other degenerative diseases. After the Novosteo Acquisition in 2022, we shifted our operational focus on the development of our bone-targeting drug platform and lead compound NOV004 for development for rare skeletal diseases, bone fractures, and injury. In January 2023, we made a strategic decision to out-license our bone-targeting drug platform and prioritize capital resources toward the expansion of our development pipeline through the completion the acquisition of EryDel in October 2023. We have a limited history operating our newly acquired business, which may make it difficult to evaluate the success of our business to date and assess our future viability. Drug development is a highly uncertain undertaking and involves a substantial degree of risk. To date, we have only initiated two late-stage clinical trials, one of which was initiated by EryDel, and we have not obtained marketing approval for any drug candidate, manufactured a commercial scale drug candidate, arranged for a third party to do so on our behalf, or conducted sales and marketing activities necessary for successful drug candidate commercialization. Our short operating history as a company makes any assessment of our future success and viability subject to significant uncertainty. We will encounter risks and difficulties frequently experienced by clinical stage biotechnology companies in rapidly evolving fields, and we have not yet demonstrated an ability to overcome such risks and difficulties successfully. If we do not address these risks and difficulties successfully, our business will suffer.

We will require substantial additional funding to finance our operations and evaluate future drug candidates. If we are unable to raise this funding when needed or on acceptable terms, we may be forced to delay, reduce or eliminate our drug development programs or other operations.

Since our inception, we have used substantial amounts of cash to fund our operations, and we expect our expenses to increase substantially in the foreseeable future in connection with our ongoing activities, particularly as we evaluate and develop drug candidates. In addition, if we obtain marketing approval for any future drug candidates, we expect to incur significant commercialization expenses related to sales, marketing, manufacturing and distribution.

Further development of EryDex will require us to incur significant additional expenses. Moreover, we expect to require substantial additional funding to finance such payments and to advance the development and optimize the commercialization of EryDex, and there can be no assurance that such additional funding will be available on terms that are acceptable to us, or at all. If adequate funds are not available on a timely basis, we may not be able to effectively implement our strategic plan.

Accordingly, we will need to obtain substantial additional funding in order to fully execute on our corporate strategy. As of December 31, 2023, we had $75.1 million in cash, cash equivalents and investments. Our balance sheet includes publicly-traded corporate debt securities. We may be required to recognize impairments in the value of these investments if the relevant companies are materially adversely affected, become unable to repay debt securities when due, or experience credit rating downgrades, or if the public trading price of these securities decreases.

We believe that our existing capital resources will be sufficient to fund our projected operations, which would include anticipated clinical and development activities related to EryDel's lead asset through the Phase 3 NEAT clinical trial, into 2026. However, changing circumstances may cause us to increase our spending significantly faster than we currently anticipate, and we may need to spend more money than currently expected because of circumstances beyond our control. We may need to raise additional funds sooner than we anticipate if we choose to expand more rapidly than we presently anticipate. The amount and timing of our future funding requirements will depend on many factors, some of which are outside of our control, including but not limited to:

- the rate of progress in the development of and the conduct of clinical trials with respect to our product candidates;
- our ability to successfully identify partnership and licensing opportunities to support the future development of EryDex;
- the outcome, costs and timing of seeking and obtaining FDA and any other regulatory approvals;
- the number and characteristics of drug candidates that we acquired or pursue;
- our ability to manufacture sufficient quantities of our drug candidates and devices;
- our need to expand our research and development activities;
- the costs associated with securing and establishing commercialization and manufacturing capabilities;
- the costs of acquiring, licensing or investing in businesses, drug candidates and technologies;
- our ability to maintain, expand and defend the scope of our intellectual property portfolio, including the amount and timing of any payments we may be required to make, or that we may receive, in connection with the licensing, filing, prosecution, defense and enforcement of any patents or other intellectual property rights;
- our need and ability to retain management and hire scientific and clinical personnel;
- the effect of competing drugs and drug candidates and other market developments;
- our need to implement additional internal systems and infrastructure, including financial and reporting systems;
- the costs to grow our organization and increase the size of our facilities to meet our anticipated growth;
- the economic and other terms, timing of and success of any collaboration, licensing or other arrangements into which we may enter in the future; and
- our ability and timing of future milestones payments to EryDel shareholders and repayment of obligations in respect of the EIB Facility.

Additional funding may not be available to us on acceptable terms or at all. Any such funding may result in dilution to stockholders, imposition of debt covenants and repayment obligations, or other restrictions that may affect our business. We also could be required to seek funds through arrangements with collaborative partners or otherwise that may require us to relinquish rights to some of our technologies or drug candidates or otherwise agree to terms unfavorable to us.

The terms of the EIB Facility place restrictions on our operating and financial flexibility.

In connection with the EIB Facility, we are subject to operating restrictions and covenants that restrict our ability to finance our operations, engage in business activities or expand or fully pursue its business strategies. For example, unless we get approval from EIB, the EIB Facility limits our ability to, among other things:

- incur additional debt or provide guarantees in respect of debt;

- incur liens;
- make investments, acquisitions, loans or advances;
- sell assets;
- make distributions to equity holders, including dividends and distributions on, and redemptions, repurchases or retirement of, our capital stock;
- enter into certain hedging transactions;
- enter into fundamental changes, including mergers and consolidations;
- enter into transactions with affiliates;
- change the nature of our business; and
- change our management.

In addition, the EIB Facility requires that we meet certain reporting and operating covenants, including an obligation to maintain a certain minimum unrestricted balance of cash or cash equivalents. Our ability to comply with these covenants may be affected by events beyond our control, and we may not be able to meet those covenants.

The EIB Facility includes customary events of default, including failure to pay principal, interest or certain other amounts when due; material inaccuracy of representations and warranties; breach of covenants; cross-default to other indebtedness (resulting in a right of the other lender to accelerate such indebtedness after giving effect to any grace periods); certain bankruptcy and insolvency events; certain undischarged judgments; and material adverse change. A breach of any of these covenants could result in an event of default under the EIB Facility. If an event of default occurs and is ongoing under the terms of the Finance, EIB may accelerate all of the obligations of EryDel thereunder and demand payment from us pursuant to the guarantees. Any declaration by the lender of an event of default could significantly harm our business and prospects and could cause the price of our common stock to decline.

Unstable market and global economic conditions, including adverse developments affecting the financial services industry, such as actual events or concerns involving liquidity, defaults or non-performance by financial institutions, may have adverse consequences on our business, financial condition and stock price.

The global credit and financial markets have experienced volatility, including as a result of the COVID-19 pandemic, changes in interest rates, and economic inflation, which has included diminished liquidity and credit availability, declines in consumer confidence, declines in economic growth, high inflation, uncertainty about economic stability and changes in unemployment rates. The financial markets and the global economy may also be adversely affected by the current or anticipated impact of military conflict, acts of terrorism or other geopolitical events. Sanctions imposed by the United States and other countries in response to such conflicts, including the one in Ukraine, may also continue to adversely impact the financial markets and the global economy, and any economic countermeasures by the affected countries or others could heighten market and economic instability. There can be no assurance that further deterioration in credit and financial markets and confidence in economic conditions will not occur. Our business strategy may be adversely affected by any such economic downturn, volatile business environment or continued unpredictable and unstable market conditions. Failure to secure any necessary financing in a timely manner could have a material adverse effect on our growth strategy, financial performance and stock price.

We regularly maintain cash balances at third-party financial institutions in excess of the FDIC insurance limit. Although we assess our banking relationships as we believe necessary or appropriate, our access to funding sources 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 institutions with which we have arrangements directly, or the financial services industry or economy in general. These factors could involve financial institutions or financial services industry companies with which we have financial or business relationships, but could also include factors involving financial markets or the financial services industry generally.

Our failure to maintain certain tax benefits applicable to Italian biotechnology companies may adversely affect our results of operations, our cash flows and our financial condition.

As a Company with an Italian biotechnology subsidiary, we have benefited from certain tax advantages, including, for example, the R&D tax credit, which an Italian tax credit aimed at stimulating research and development. The R&D tax credit can be offset payments of certain taxes and contributions (e.g., social contributions, VAT payables, registration fees, income and withholding taxes and all other tax-related items that companies usually pay monthly). For eligible research and development activities, the tax credits were equal to 20% of the costs incurred in fiscal years 2022 and 2021, with a maximum annual amount of $4.4 million (Euro 4

million). In 2023 the tax credit rate was decreased to 10% of the eligible expenses for certain activities, and the annual ceiling of the credit increased to $5.5 million (EUR 5 million). Expenses incurred by the Company for years ended December 31, 2021, 2022, and 2023 generated a total tax credit amounting to $2.0 million (Euro 1.8 million), $1.1 million (Euro 1 million), and $877,000 (Euro 800k), respectively. The Italian tax authorities may audit each research and development program in respect of which a R&D tax credit has been claimed and assess whether such program qualifies in its view for the R&D tax credit. The Italian tax authorities may challenge our eligibility for, or our calculation of, certain tax reductions or deductions in respect of our research and development activities. Should the Italian tax authorities be successful, the R&D tax credit, may be reduced, which would have a negative impact on our results of operations and future cash flows. We believe, due to the nature of our business operations, that we will continue to be eligible to receive the R&D tax credit. However, if the Italian government decides to eliminate, or to reduce the scope or the rate of, the R&D tax credit, either of which it could decide to do at any time, our results of operations could be adversely affected.

Risks Relating to Regulatory Review and Approval of Our Drug Candidates and Other Legal Compliance Matters

We cannot be certain that the FDA or foreign regulatory authorities will permit us to proceed with any current or future proposed clinical trial designs. Our drug candidates may not receive regulatory approval, and without regulatory approval we will not be able to market our drug candidates.

We currently have no drug candidates approved for sale and we cannot guarantee that we will ever have marketable drug candidates. Our ability to generate revenue related to sales, if ever, will depend on the successful development and regulatory approval of our drug candidates.

The development of a drug candidate and issues relating to its approval and marketing are subject to extensive regulation by the FDA in the United States and regulatory authorities in other countries, with regulations differing from country to country. We are not permitted to market any drug candidates in the United States until we receive approval of an NDA from the FDA. Similar requirements apply in foreign countries. We have not submitted any marketing applications for a drug candidate.

Because EryDex utilizes DSP, we believe it will qualify for FDA approval through the FDA's 505(b)(2) regulatory pathway and through corresponding regulatory paths in other foreign jurisdictions. The clinical requirements for a 505(b)(2) drug candidate can vary widely from product to product depending primarily on whether the drug candidate claims a new indication, provides for a different route of administration, or claims improved safety compared to the existing approved product, and may include bioequivalence trials, limited safety and efficacy trials, or full Phase 1 through 3 trials.

NDAs must include extensive preclinical and clinical data and supporting information to establish the drug candidate's safety and effectiveness for each desired indication. NDAs must also include significant information regarding the chemistry, manufacturing and controls for the drug. Obtaining approval of an NDA is a lengthy, expensive and uncertain process, and we may not be successful in obtaining approval. The FDA review processes can take years to complete and approval is never guaranteed. If we submit an NDA to the FDA, the FDA must decide whether to accept or reject the submission for filing. We cannot be certain that any submissions will be accepted for filing and review by the FDA. Regulators of other jurisdictions may impose similar requirements and have their own procedures for approval of drug candidates. Even if a drug is approved, the FDA or a comparable foreign regulatory authority may limit the indications for which the drug may be marketed, require extensive warnings on the drug labeling or require expensive and time-consuming clinical trials or reporting as conditions of approval. Regulatory authorities in countries outside of the United States also have requirements for approval of drug candidates with which we must comply prior to marketing in those countries. Obtaining regulatory approval for marketing of a drug candidate in one country does not ensure that we will be able to obtain regulatory approval in any other country. In addition, delays in approvals or rejections of marketing applications in the United States or other countries may be based upon many factors, including regulatory requests for additional analyses, reports, data, preclinical studies and clinical trials, regulatory questions regarding different interpretations of data and results, changes in regulatory policy during the period of drug development and the emergence of new information regarding our drug candidates or other drug candidates. Also, regulatory approval for any of our drug candidates may be withdrawn.

Clinical failure can occur at any stage of clinical development and we have never submitted an NDA or comparable foreign application before.

The FDA or other foreign regulatory authorities may limit our ability to proceed with potential clinical programs, which could have a materially adverse impact on us. The submission of a successful NDA or comparable foreign applications is a complicated process. As an organization, we have never conducted a registrational clinical trial and have limited experience in preparing, submitting and prosecuting regulatory filings, and have not submitted an NDA or comparable foreign applications. Failure to commence or complete, or delays in, our planned clinical trials would prevent us from or delay us in seeking approval for, and if approved, commercializing our drug candidates, and failure to successfully complete any of these activities in a timely manner for any

of our drug candidates could have a material adverse impact on our business and financial performance. The commencement, enrollment and completion of clinical trials can be delayed or suspended for a variety of reasons, including:

- inability to obtain sufficient funds required for a clinical trial;
- inability to reach agreements on acceptable terms with prospective CROs and trial sites, the terms of which can be subject to extensive negotiation and may vary significantly among different CROs and trial sites;
- clinical holds, other regulatory objections to commencing or continuing a clinical trial or the inability to obtain regulatory approval to commence a clinical trial in countries that require such approvals;
- discussions with the FDA or non-U.S. regulators regarding the scope or design of our clinical trials;
- inability to identify and maintain a sufficient number of trial sites, many of which may already be engaged in other clinical trial programs, including some that may be for the same indications targeted by our drug candidates;
- inability to obtain approval from IRBs or positive ethics committee opinions to conduct a clinical trial at their respective sites;
- severe or unexpected drug-related adverse effects experienced by patients, which have resulted and may result in a full or partial clinical hold by the FDA or non-U.S. regulators;
- inability to timely manufacture sufficient quantities of the drug candidate or devices required for a clinical trial;
- difficulty recruiting and enrolling patients to participate in clinical trials for a variety of reasons, including ability to find patients with a rare disease, meeting the enrollment criteria for our study and competition from other clinical trial programs for the same indications as our drug candidates;
- inability to retain enrolled patients after a clinical trial is underway; and
- enrollment may be delayed or interrupted or patients may drop out of clinical trials due to or the fear of natural disasters, such as earthquakes, tsunamis, power shortages or outages, floods, or monsoons, public health crises, such as pandemics and epidemics, political crisis, such as terrorism, war, political instability or other conflict, cyberattacks, or other events outside of our control occurring at or around our clinical trials sites in the United States or Europe.

In addition, the design of a clinical trial can determine whether its results will support approval of a drug and flaws in the design of a clinical trial may not become apparent until the clinical trial is well-advanced. Changes in regulatory requirements and guidance may also occur and we may need to amend clinical trial protocols to reflect these changes with appropriate regulatory authorities. Amendments may require us to resubmit clinical trial protocols to IRBs or ethics committees for re-examination, which may impact the costs, timing or successful completion of a clinical trial.

In addition, if we are required to conduct additional clinical trials or other preclinical studies of our drug candidates beyond those contemplated, our ability to obtain regulatory approval of these drug candidates and generate revenue from their sales would be similarly harmed.

Clinical trials of our drug candidates have in the past been put on clinical holds by, and failed to demonstrate safety and efficacy to the satisfaction of, the FDA, and if any future clinical trials of our drug candidates are put on clinical holds by, or fail to demonstrate safety and efficacy to the satisfaction of, the FDA, the EMA, or similar regulatory authorities outside the United States, or do not otherwise produce positive results, we may incur additional costs or experience delays in completing, or ultimately be unable to complete, the development and commercialization of our drug candidates.

Before obtaining regulatory approvals for the commercial sale of any of our drug candidates, we must demonstrate through lengthy, complex and expensive preclinical studies and clinical trials that our drug candidates are both safe and effective for use in each target indication. Each drug candidate must demonstrate an adequate risk versus benefit profile in its intended patient population and for its intended use.

Clinical testing is expensive and can take many years to complete, and its outcome is inherently uncertain. Failure can occur at any time during the clinical trial process. Clinical trials of our drug candidates have in the past been put on clinical holds imposed by, and failed to demonstrate safety and efficacy to the satisfaction of, the FDA, the EMA, or similar regulatory authorities outside of the United States. For example, on January 25, 2022, the FDA placed a full clinical hold on the IND for atuzaginstat (COR388), one of our assets that has since been out-licensed. On March 8, 2023, the FDA placed a partial clinical hold on the IND for EryDex related to extractables and leachables of new components used in the EryKit. The FDA subsequently lifted the partial clinical hold on September 23, 2023. The FDA may place additional clinical holds on our current or currently contemplated clinical programs or

otherwise limit our ability to proceed with other clinical programs in our pipeline. Additionally, the results of preclinical studies of our drug candidates may not be predictive of the results of early-stage or later-stage clinical trials, and results of early clinical trials of our drug candidates may not be predictive of the results of later-stage clinical trials. For example, the Phase 3 ATTeST study conducted by EryDel failed to meet its primary endpoint. The results of clinical trials in one set of patients or disease indications may not be predictive of those obtained in another. In some instances, there can be significant variability in safety or efficacy results between different clinical trials of the same drug candidate due to numerous factors, including changes in trial procedures set forth in protocols, differences in the size and type of the patient populations, changes in and adherence to the dosing regimen and other clinical trial protocols and the rate of dropout among clinical trial participants. Drug candidates in later stages of clinical trials may fail to show the desired safety and efficacy profile despite having progressed through preclinical studies and initial clinical trials. A number of companies in the pharmaceutical industry have suffered significant setbacks in advanced clinical trials due to lack of efficacy or unacceptable safety issues, notwithstanding promising results in earlier trials. This is particularly true in degenerative diseases, where failure rates historically have been higher than in many other disease areas. Most drug candidates that begin clinical trials are never approved by regulatory authorities for commercialization.

In addition, even if such clinical trials are successfully completed, we cannot guarantee that the FDA, the EMA, or other foreign regulatory authorities will interpret the results as we do, and more trials could be required before we submit our drug candidates for approval. Moreover, principal investigators for our clinical trials may serve as scientific advisors or consultants to us from time to time and receive compensation in connection with such services. Under certain circumstances, we may be required to report some of these relationships to the FDA, the EMA, or other regulatory authorities. The FDA, the EMA, or other regulatory authorities may conclude that a financial relationship between us and a principal investigator has created a conflict of interest or otherwise affected the integrity of the study. The FDA, the EMA, or other regulatory authorities may therefore question the integrity of the data generated at the applicable clinical trial site and the utility of the clinical trial itself may be jeopardized. This could result in a delay in approval, or rejection, of our marketing applications by the FDA, the EMA, or other regulatory authorities, as the case may be, and may ultimately lead to the denial of marketing approval of any of our drug candidates. To the extent that the results of the trials are not satisfactory to the FDA, the EMA, or foreign regulatory authorities for support of a marketing application, we may be required to expend significant resources, which may not be available to us, to conduct additional trials in support of potential approval of our drug candidates. Even if regulatory approval is secured for any of our drug candidates, the terms of such approval may limit the scope and use of our drug candidate, which may also limit its commercial potential.

We currently rely and expect to continue to rely on third parties to conduct some of our preclinical studies and clinical trials and some aspects of our research and preclinical testing and on third-party contract manufacturing organizations to manufacture and supply our preclinical, clinical and commercial materials, and those third parties may not perform satisfactorily, including failing to meet deadlines for the completion of such trials, research, manufacturing or testing.

We rely and expect to continue to rely on third parties, such as CROs, clinical data management organizations, medical institutions, and clinical investigators, to conduct some aspects of our research and preclinical testing and our clinical trials. We also currently rely on and expect to continue to rely on, third-party CMOs to manufacture and supply our preclinical, clinical and commercial materials. Any of these third parties may terminate their engagements with us or be unable to fulfill their contractual obligations. If we need to enter into alternative arrangements, it would delay our future drug development activities.

Our reliance on these third parties for research and development activities reduces our control over these activities but does not relieve us of our responsibilities. For example, we remain responsible for ensuring that each of our clinical trials is conducted in accordance with the general investigational plan and protocols for the trial. Moreover, the FDA requires us to comply with current GCP regulations for conducting, recording, and reporting the results of clinical trials to assure that data and reported results are credible, reproducible and accurate and that the rights, integrity, and confidentiality of trial participants are protected. We also are required to register any future clinical trials and post the results of completed clinical trials on a government-sponsored database within certain timeframes. Failure to do so can result in fines, adverse publicity, and civil and criminal sanctions. Similar requirements and consequences may apply in countries outside the United States.

Reliance on third-party manufacturers entails additional risks, such as the possible breach of the manufacturing agreement by the third party, the possible termination or nonrenewal of the agreement by the third party at a time that is costly or inconvenient for us and reliance on the third party for regulatory compliance, quality assurance, safety and related reporting. Third-party manufacturers may not be able to comply with cGMP regulations or similar regulatory requirements outside the United States.

If these third parties do not successfully carry out their contractual duties, meet expected deadlines, or conduct our clinical trials in accordance with regulatory requirements or our stated protocols, we will not be able to obtain, or may be delayed in obtaining, marketing approvals for any drug candidates we may develop and will not be able to, or may be delayed in our efforts to, successfully commercialize our drug candidates.

We also expect to rely on other third parties to store and distribute drug supplies for our future clinical trials. Any performance failure on the part of our distributors could delay clinical development or marketing approval of any drug candidates we may develop or commercialization of our medicinal products, producing additional losses and depriving us of drug revenue.

Risks Related to the Production and Manufacturing of our Drug Candidates and Future Products

Our production capacity could prove insufficient for our needs.

Our production capacity may prove insufficient in the future to meet the growth of our business, including producing sufficient quantities of drug candidates for clinical trials and, ultimately, our customers and distributors. There is no guarantee that we will or have properly estimated our required manufacturing capacities or that the third parties we rely on to provide required machinery and materials for the manufacturing process will be able to perform on our proposed timelines or meet our manufacturing demands, if at all. Also, if we must increase production capacity for any reason, we may need to make considerable investments that could lead to significant financing needs or require us to enter into subcontracting agreements in order to outsource part of the production.

We may not have access to the raw materials and other components, necessary for the manufacturing of our drug candidates.

We are dependent on third parties for the supply of various materials that are necessary to produce our drug candidates for clinical trials.

If our agreements with one or more of these suppliers were to be terminated or if one or more of these suppliers are unable to meet our demands, we could experience delays in our research or planned clinical trials or commercialization. We could be unable to find alternative suppliers of acceptable quality, in the appropriate volumes and at an acceptable cost.

In addition, these materials are subject to stringent manufacturing processes and rigorous testing. Delays in the completion and validation of facilities and manufacturing processes of these materials could adversely affect our ability to complete trials and commercialize our products in a cost-effective and timely manner. If we encounter difficulties in the supply of these materials, chemicals or biological products, or if we were not able to maintain our supply agreements, or establish new supply agreements in the future, our product development and our business prospects could be significantly compromised.

Our manufacturing facilities are subject to significant government regulations and approvals. If we or our third-party manufacturers fail to comply with these regulations or maintain these approvals, our business will be materially harmed.

We currently partially manufacture our Red Cell Loader machines and EryKit in our facility in Medolla, Italy. We and our third-party manufacturers are subject to ongoing regulation and periodic inspection by the FDA competent authorities of EU Member States and other regulatory bodies to ensure compliance with cGMP, as part of our clinical trials. Any failure to follow and document our or their adherence to such cGMP regulations or other regulatory requirements may lead to significant delays in the availability of products for commercial sale or clinical trials, may result in the termination of or a hold on a clinical trial, or may delay or prevent filing or approval of marketing applications for our products.

Failure to comply with applicable regulations could also result in the European Commission, FDA, the national authorities in the individual EU Member States, or other applicable regulatory authorities taking various actions, including:

- levying fines and other civil penalties;
- imposing consent decrees or injunctions;
- requiring us to suspend or put on hold one or more of our clinical trials;
- suspending, varying or withdrawing regulatory approvals;
- delaying or refusing to approve pending applications or supplements to approved applications;
- requiring us to suspend manufacturing activities or product sales, imports or exports;
- requiring us to communicate with physicians and other customers about concerns related to actual or potential safety, efficacy, and other issues involving our products;
- mandating product recalls or seizing products;
- imposing operating restrictions; and

- seeking criminal prosecutions.

Any of the foregoing actions could be detrimental to our reputation, business, financial condition or operating results. Furthermore, our key suppliers may not continue to be in compliance with all applicable regulatory requirements, which could result in our failure to produce our products on a timely basis and in the required quantities, if at all. In addition, before any additional products would be considered for marketing approval in the United States, the EU or elsewhere, our suppliers will have to pass an audit by the applicable regulatory authorities. We are dependent on our suppliers' cooperation and ability to pass such audits, and the audits and any audit remediation may be costly. Failure to pass such audits by us or any of our suppliers would affect our ability to commercialize our drug candidates in the United States, the EU or elsewhere.

Our production costs may be higher than we currently estimate.

We manufacture our drug candidates according to manufacturing best practices applicable to drugs for clinical trials and to specifications approved by the applicable regulatory authorities. If any of our drug candidates are found to be non-compliant, we would be required to manufacture the drug candidates again, which would entail additional costs and may prevent delivery of the drug candidates to patients on time.

Other risks inherent in the production process may have the same effect, such as:

- contamination of the controlled atmosphere area;
- unusable premises and equipment;
- new regulatory requirements requiring a partial and/or extended stop to the production unit to meet the requirements;
- unavailable qualified personnel;
- power failure of extended duration;
- logistical error; and
- rupture in the cold chain, which is a system for storing and transporting blood and blood products within the correct temperature range and conditions.

In addition, a rise in direct or indirect energy rates may increase product manufacturing and logistical costs. Any of these risks, should they occur, could disrupt our activities and compromise our financial position, results, reputation or growth.

The manufacture of our products requires strict adherence to regulatory requirements governing medical devices and if we or our suppliers encounter problems our business could suffer.

The manufacture of our products must comply with strict regulatory requirements governing Class II medical devices in the U.S. and other regulatory requirements in foreign locations. Problems may arise during manufacturing, quality control, storage, or distribution of our products for a variety of reasons, including equipment malfunction, failure to follow specific protocols and procedures, manufacturing quality concerns, or problems with raw materials, electromechanical, software and other components, supplier issues, and natural disasters. If problems arise during production, the affected products may have to be discarded. In the EU, our RCL and EryKit medical devices, Syringe Kit, and process solutions, are subject to periodic inspections by our Notified Body to maintain CE Certificates of Conformity permitting us to affix the CE mark to our medical devices. We may also be subject to unannounced audits by national competent authorities to ensure compliance with applicable regulatory requirements.

As a result of the transitional provisions in the MDR, some CE Certificates of Conformity issued by Notified Bodies in accordance with the MDD from May 2017, and which remained valid on May 26, 2021 will remain valid until December 31, 2027 for Class III and Class IIb implantable medical devices and until December 31, 2028 for other Class IIb, Class IIa and Class I devices with a measuring function or which are sterile. Class I medical devices, for which the conformity assessment procedure in accordance with the MDD did not require the involvement of a Notified Body but will require the involvement of a Notified Body in accordance with the MDR and for which an EU Declaration of Conformity was issued in accordance with the MDD prior to May 26, 2021, can continue to be placed on the EEA market until December 31, 2028. Manufacturers of medical devices may only benefit from the above extended transitional provisions deadlines if the following conditions are fulfilled: (i) the devices continue to comply with the requirements of the MDD, (ii) there are no significant changes in the design and intended purpose, (iii) the devices do not present an unacceptable risk to the health or safety of patients, users or other persons, or to other aspects of the protection of public health, (iv) the manufacturer implements a quality management system by May 26, 2024 which complies with the requirements of the MDR, (v) by May 26, 2024 an application is lodged with a Notified Body for conduct of the conformity assessment of the devices covered by

the CE Certificate of Conformity, or the devices intended to substitute for such devices, in accordance with the MDR and a related written agreement is signed with the Notified Body by September 26, 2024, and (vi) from May 26, 2021, compliance with the MDR relating to post-market surveillance, market surveillance, vigilance, registration of economic operators and of devices is ensured in place of the corresponding requirements in the MDD.

In addition, these CE Certificates of Conformity will remain valid in accordance with the extended transitional deadlines above only if either (i) the manufacturer signed a written agreement with a Notified Body for the conformity assessment of the device covered by the expired CE Certificate of Conformity, or the device intended to substitute that device, in accordance with the MDR before the date of expiry of the CE Certificate of Conformity, or (ii) a competent authority of an EU Member State has granted a derogation from the application conformity assessment procedure in accordance with Article 59(1) or Article 97(1) of the MDR.

Any failure to comply with any of these obligations may impact our activities in the EEA, the renewal of our existing CE Certificates of Conformity and future conformity assessment activities.

Manufacturing problems or delays could also lead to increased costs, lost sales, damage to customer relations, failure to supply penalties, time and expense spent investigating the cause and depending on the cause, similar losses with respect to other batches of products. If problems are not discovered before the product is released to the market, voluntary recalls, corrective actions, or product liability related costs may also be incurred. If unanticipated problems with our products arise, or if we or our suppliers fail to comply with regulatory requirements following CE marking, we may also become subject to enforcement actions such as restrictions on manufacturing processes, warning letters, suspension, variation or withdrawal of CE Certificates of Conformity, civil or criminal penalties. Should we encounter difficulties in the manufacture of our products or be subject to a product recall, our business could suffer materially.

If we or any of our third-party manufacturers or suppliers encounter difficulties in production of our future drug candidates, or fail to meet rigorously enforced regulatory standards, our ability to provide supply of our future drug candidates for clinical trials or for patients, if approved, could be delayed or stopped, or we may be unable to maintain a commercially viable cost structure.

The processes involved in manufacturing our drug candidates are highly regulated and subject to multiple risks. As drug candidates are developed through preclinical studies to late-stage clinical trials towards 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 processes and results. Such changes carry the risk that they will not achieve these intended objectives, and any of these changes could cause our drug candidates to perform differently and affect the results of planned clinical trials or other future clinical trials.

In order to conduct clinical trials of our drug candidates, or supply future commercial drug candidates or devices, if approved, we will need to manufacture them in small and large quantities. Our manufacturing partners may be unable to successfully modify or scale-up the manufacturing capacity for any of our drug candidates or devices in a timely or cost-effective manner, or at all. In addition, quality issues may arise during scale-up activities. If our manufacturing partners are unable to successfully scale-up the manufacture of our drug candidates or devices in sufficient quality and quantity, the development, testing and clinical trials of that drug candidate may be delayed or become infeasible, and regulatory approval or commercial launch of any resulting drug may be delayed or not obtained, which could significantly harm our business. The supply of any of these materials used in EryKits or RCLs may be limited or any of the supply manufacturers may not meet relevant regulatory requirements, and if we are unable to obtain any of these materials in sufficient amounts, in a timely manner and at reasonable prices, or if we encounter delays or difficulties in our relationships with manufacturers or suppliers, the production of EryKits and RCLs may be delayed. If any of our suppliers is unwilling or unable to meet its supply obligations and we are unable to secure an alternative supply source in a timely manner and on favorable terms, our business, financial condition, and results of operations may be harmed and the market price of our common stock and other securities may decline. The same risks apply to our internal manufacturing facilities.

In addition, the manufacturing process for any drug candidates is subject to FDA and foreign regulatory requirements, and continuous oversight, and we will need to contract with manufacturers who can meet all applicable FDA and foreign regulatory authority requirements, including complying with cGMPs, on an ongoing basis. If we or our third-party manufacturers are unable to reliably produce drug candidates in accordance with the requirements of the FDA or other regulatory authorities, we may not obtain or maintain the approvals we need to commercialize such future drug candidates. Even if we obtain regulatory approval for any of our drug candidates, there is no assurance that either we or our third-party contract manufacturers will be able to manufacture the approved drug in accordance with the requirements of the FDA or other regulatory authorities, to produce it in sufficient quantities to meet the requirements for the potential launch of the drug, or to meet potential future demand. Moreover, we, or our contract manufacturers, any future collaborators and their contract manufacturers could be subject to periodic unannounced inspections by the FDA, competent authorities of EU Member States or other comparable foreign regulatory authorities, to monitor and ensure compliance with cGMP. Despite our efforts to audit and verify regulatory compliance, one or more of our third-party manufacturing vendors may be found on regulatory inspection by the FDA, competent authorities of EU Member States or other comparable foreign regulatory authorities to be

noncompliant with cGMP regulations. Our failure, or the failure of our third-party manufacturers, to comply with applicable regulations could result in sanctions being imposed on us, including shutdown of the third-party vendor or invalidation of drug product lots or processes, fines, injunctions, civil penalties, delays, suspension, variation or withdrawal of approvals, license revocation, seizures or recalls of drug candidates or drugs, operating restrictions and criminal prosecutions, any of which could significantly and adversely affect supplies of our products, if approved, and significantly harm our business, financial condition, results of operations and prospects. Any of these challenges could delay completion of clinical trials, require bridging clinical trials or the repetition of one or more clinical trials, increase clinical trial costs, delay approval of our drug candidate, impair commercialization efforts, increase our cost of goods, and have an adverse effect on our business, financial condition, results of operations and growth prospects.

If the market opportunities for our drug candidates are smaller than we believe they are, our revenue may be adversely affected, and our business may suffer. Because the target patient populations of our drug candidates are small, we must be able to successfully identify patients and acquire a significant market share to achieve profitability and growth.

We focus our research and product development on treatments for rare and ultra-rare diseases. Given the small number of patients who have the diseases that we are targeting, our profitability and growth depend on successfully identifying patients with these rare and ultra-rare diseases. Our projections of both the number of people who have these diseases, as well as the subset of people with these diseases who have the potential to benefit from treatment with our drug candidates, are based on our beliefs and internal estimates. These estimates have been derived from a variety of sources, including scientific literature, surveys of clinics, patient foundations, and market research, and may prove to be incorrect. Further, new studies may change the estimated incidence or prevalence of these diseases, and, as a result, the number of patients with these diseases may turn out to be lower than expected. Our effort to identify patients with diseases we seek to treat is in early stages, and we cannot accurately predict the number of patients for whom treatment might be possible. Additionally, the potentially addressable patient population for each of our drug candidates may be limited or may not be amenable to treatment with our drug candidates, and new patients may become increasingly difficult to identify or gain access to, which would adversely affect our results of operations and our business. If patients who currently receive assistance from us in paying for the cost of our drugs continue to receive assistance, if approved, or who receive free drugs in the future, will negatively impact our profitability. If EryDex is only approved for patients with A-T who are between six and nine years old, it will be limiting an already small patient population. Finally, even if we obtain significant market share for our drug candidates, because the potential target populations are very small, we may never achieve profitability despite obtaining such significant market share.

If, in the future, we are unable to establish sales and marketing capabilities or enter into agreements with third parties to sell and market any drug candidates we may develop and for which we obtain approval, we may not be successful in commercializing those drug candidates if and when they are approved.

We do not have a sales or marketing infrastructure and have no experience in the sale, marketing, or distribution of pharmaceutical drug candidates, if approved, or devices. To achieve commercial success for any approved drug candidate for which we retain sales and marketing responsibilities, we must either develop a sales and marketing organization or outsource these functions to third parties. In the future, we may choose to build a focused sales, marketing, and commercial support infrastructure to sell, or participate in sales activities with collaborators for, some of our drug candidates if and when they are approved.

There are risks involved with both establishing our own commercial capabilities and entering into arrangements with third parties to perform these services. For example, factors that may inhibit our efforts to commercialize any drug candidates, if and when approved, whether alone or in collaboration with others:

- our inability to recruit and retain adequate numbers of effective sales, marketing, coverage or reimbursement, customer service, medical affairs, and other support personnel;
- the inability of sales personnel to obtain access to physicians or our failure to educate adequate numbers of physicians on the benefits of any future approved drug candidates;
- the inability of reimbursement professionals to negotiate arrangements for formulary access, reimbursement, and other acceptance by payors;
- the inability to price our drug candidates, if approved, at a sufficient price point to ensure an adequate and attractive level of profitability;
- the pricing of our products, particularly as compared to alternative treatments;
- availability of alternative effective treatments for indications our candidates are intended to treat and the relative risks, benefits and costs of those treatments;

- restricted or closed distribution channels that make it difficult to distribute our drug candidates, if approved, to segments of the patient population;
- the lack of complementary drug candidates, if approved, to be offered by sales personnel, which may put us at a competitive disadvantage relative to companies with more extensive drug candidate lines; and
- unforeseen costs and expenses associated with creating an independent commercialization organization.

If the commercial launch of a future drug candidate, if approved, for which we recruit a sales force and establish marketing and other commercialization capabilities is delayed or does not occur for any reason, we would have prematurely or unnecessarily incurred these commercialization expenses. This may be costly, and our investment would be lost if we cannot retain or reposition our commercialization personnel.

If we enter into arrangements with third parties to perform sales, marketing, commercial support, and distribution services, our sales revenue or the profitability of sales revenue may be lower than if we were to market and sell any drug candidates, if approved, we may develop ourselves. In addition, we may not be successful in entering into arrangements with third parties to commercialize our drug candidates, if approved, or may be unable to do so on terms that are favorable to us. We may have little control over such third parties, and any of them may fail to devote the necessary resources and attention to sell and market our drug candidates, if approved, effectively. If we do not establish commercialization capabilities successfully, either on our own or in collaboration with third parties, we will not be successful in commercializing our drug candidates if approved in the future.

If product liability lawsuits are brought against us, we may incur substantial liabilities and may be required to limit commercialization of our drug candidates, if approved.

We face an inherent risk of product liability as a result of the clinical testing of our drug candidates and will face an even greater risk when and if we commercialize any drug candidates, if approved. For example, we may be sued if our drug candidates cause or are perceived to cause injury or are found to be otherwise unsuitable during clinical testing, early access program, manufacturing, marketing or sale. 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, negligence, strict liability or a breach of warranties. Product liability claims may be brought against us by participants enrolled in our clinical trials, patients, health care providers or others using, administering our drug candidates or selling our drug candidates, if approved. Claims could also be asserted under state consumer protection acts. If we cannot successfully defend ourselves against product liability claims, we may incur substantial liabilities or be required to limit testing and commercialization of our drug candidates. Even successful defense would require significant financial and management resources. Regardless of the merits or eventual outcome, liability claims may result in:

- decreased or interrupted demand for our drug candidates;
- withdrawal of clinical trial participants and inability to continue clinical trials;
- initiation of investigations by regulators;
- costs to defend the related litigation;
- a diversion of management's time and our resources;
- substantial monetary awards to trial participants or patients;
- drug recalls, withdrawals or labeling, marketing or promotional restrictions;
- termination of clinical trial sites or entire trial programs;
- injury to our reputation and significant negative media attention;
- loss of revenue;
- exhaustion of any available insurance and our capital resources;
- the inability to commercialize any drug candidate, if approved; and
- a decline in our share price.

Our inability to obtain sufficient product liability insurance at an acceptable cost to protect against potential product liability claims could prevent or inhibit the commercialization of drug candidates we develop, alone or with potential collaborators. Our insurance policies may have various exclusions, and we may be subject to a product liability claim for which we have no coverage.

We may have to pay any amounts awarded by a court or negotiated in a settlement that exceed our coverage limitations or that are not covered by our insurance, and we may not have, or be able to obtain, sufficient capital to pay such amounts. Even if our agreements with any future corporate collaborators entitle us to indemnification against losses, such indemnification may not be available or adequate should any claim arise.

We may be exposed to a variety of international risks that could materially adversely affect our business.

Our business is subject to risks associated with conducting business internationally. Some of our suppliers and clinical trial centers are located outside of the United States. We may enter into agreements with third parties for the development and commercialization of drug candidates in international markets. We also plan to seek regulatory approval of our drug candidates outside of the United States. International business relationships will subject us to additional risks that may materially adversely affect our ability to attain or sustain profitable operations, including:

- differing regulatory requirements for drug approvals internationally;
- rejection or qualification of foreign clinical trial data by the competent authorities of other countries;
- price controls on our drug products;
- complexities and difficulties in obtaining, maintaining, protecting and enforcing our intellectual property;
- potential third-party patent rights in countries outside of the United States;
- different United States and foreign drug import and export rules;
- different reimbursement systems and different competitive drugs indicated to treat the indications for which our drug candidates are being developed;
- the potential for so-called "parallel importing," which is what occurs when a local seller, faced with relatively high local prices, opts to import goods from another jurisdiction with relatively low prices, rather than buying them locally;
- the potential for so-called "parallel exporting," which is what occurs when a local seller buys goods meant for the locals and sells the goods for a higher price in another country, potentially causing or aggravating supply problems;
- unexpected changes in tariffs, trade barriers and regulatory requirements;
- economic weakness, including inflation, bank failures, or political instability, particularly in non-U.S. economies and markets, including several countries in Europe;
- compliance with tax, including withholding of payroll taxes, employment, immigration and labor laws for employees living or traveling abroad;
- regulatory and compliance risks that relate to anti-corruption compliance and record-keeping that may fall within the purview of the U.S. Foreign Corrupt Practices Act, its accounting provisions or its anti-bribery provisions or provisions of anti-corruption or anti-bribery laws in other countries;
- taxes in other countries;
- financial risks, such as longer payment cycles, difficulty collecting accounts receivable, the impact of local and regional financial crises on demand and payment for our products and exposure to foreign currency exchange rate fluctuations;
- foreign currency fluctuations, which could result in increased operating expenses and reduced revenue, and other obligations incident to doing business in another country;
- workforce uncertainty in countries where labor unrest is more common than in the United States;
- production shortages resulting from any events affecting raw material supply or manufacturing capabilities abroad;
- business interruptions resulting from geo-political actions, including war and terrorism, public health crises, such as pandemics and epidemics, or natural disasters, including earthquakes, volcanoes, typhoons, floods, hurricanes and fires; and
- compliance with evolving and expansive international data privacy laws, such as the EU GDPR.

Any of these factors could harm our ongoing international clinical operations and supply chain, as well as any future international expansion and operations and, consequently, our business, financial condition, prospects and results of operations.

For example, the UK has voluntarily departed from the EU, commonly referred to as "Brexit." We do not know to what extent Brexit will impact the business and regulatory environment in the UK, the EU, or other countries. Changes impacting our ability to conduct business in the UK, or other EU countries, or changes to the regulatory regime in those countries, may impact certain portions of our research and general business operations in the UK and the EU.

The United Kingdom's withdrawal from the EU may have a negative effect on global economic conditions, financial markets and our business, which could reduce the price of our common shares.

Following Brexit, the UK and the EU signed a EU-UK Trade and Cooperation Agreement, or TCA, which became provisionally applicable on January 1, 2021 and entered into force on May 1, 2021. This agreement provides details on how some aspects of the UK and EU's relationship will operate going forwards however there are still uncertainties. The TCA primarily focuses on ensuring free trade between the EU and the UK in relation to goods, including medicinal products. Among the changes that have occurred are that Great Britain (England, Scotland and Wales) is treated as a "third country," a country that is not a member of the EU and whose citizens do not enjoy the EU right to free movement. Northern Ireland continues to follow many aspects of the EU regulatory rules, particularly in relation to trade in goods. As part of the TCA, the EU and the UK recognize GMP inspections carried out by the other party and the acceptance of official GMP documents issued by the other party. The TCA also encourages, although it does not oblige, the parties to consult one another on proposals to introduce significant changes to technical regulations or inspection procedures. Among the areas of absence of mutual recognition are batch testing and batch release. The UK has unilaterally agreed to accept EU batch testing and batch release. However, the EU continues to apply EU laws that require batch testing and batch release to take place in the EU territory. This means that medicinal products that are tested and released in the UK must be retested and re-released when entering the EU market for commercial use.

As it relates to marketing authorizations, Great Britain has a separate regulatory submission process, approval process and a separate national marketing authorization. Northern Ireland continues, however, to be covered by the marketing authorizations granted by the European Commission. For example, the scope of a marketing authorization for a medicinal product granted by the European Commission or by the competent authorities of EU Member States no longer encompasses Great Britain (England, Scotland and Wales). In these circumstances, a separate marketing authorization granted by the UK competent authorities is required to place medicinal products on the market in Great Britain. Northern Ireland continues, however, to be covered by the marketing authorizations granted by the European Commission.

On February 27, 2023, the UK Government and the European Commission reached a political agreement on the so-called "Windsor Framework". The Framework is intended to revise the Northern Ireland Protocol to address some of the perceived shortcomings in its operation. The agreement was adopted at the Withdrawal Agreement Joint Committee on March 24, 2023. If the changes are adopted in the form proposed, medicinal products to be placed on the market in the UK will be authorized solely in accordance with UK laws. Northern Ireland would be reintegrated back into a UK-only regulatory environment under the authority of the MHRA with respect to all medicinal products. The implementation of the Windsor Framework would occur in stages, with new arrangements relating to the supply of medicinal products into Northern Ireland anticipated to take effect in 2025.

A significant proportion of the regulatory framework in the UK applicable to medicinal products is currently derived from EU Directives and Regulations. The potential for UK legislation to diverge from EU legislation following Brexit could materially impact the regulatory regime with respect to the development, manufacture, import, approval, and commercialization of our drug candidates in the UK or the EU. If we are slow or unable to adapt to changes in existing requirements or the adoption of new requirements or policies governing clinical trials, our development plans may be impacted.

All of these changes could increase our costs and otherwise adversely affect our business. Any delay in obtaining, or an inability to obtain, any regulatory approvals, as a result of Brexit or otherwise, would prevent us from commercializing our drug candidates in the UK or the EU and restrict our ability to generate revenue and achieve and sustain profitability. In addition, we may be required to pay taxes or duties or be subjected to other hurdles in connection with the importation of our drug candidates into the EU. If any of these outcomes occur, we may be forced to restrict or delay efforts to seek regulatory approval in the UK or the EU for our drug candidates, or incur significant additional expenses to operate our business, which could significantly and materially harm or delay our ability to generate revenues or achieve profitability of our business. Any further changes in international trade, tariff and import/export regulations as a result of Brexit or otherwise may impose unexpected duty costs or other non-tariff barriers on us. These developments, or the perception that any of them could occur, may significantly reduce global trade and, in particular, trade between

the impacted nations and the UK. It is also possible that Brexit may negatively affect our ability to attract and retain employees, particularly those from the EU.

We may not be able to manage our business effectively if we are unable to attract and retain key personnel and consultants, and the loss of such persons could negatively impact the operations of the company.

We may not be able to attract or retain qualified management, finance, scientific and clinical personnel and consultants due to the intense competition for qualified personnel and consultants among biotechnology, pharmaceutical and other businesses or any other circumstances that would cause them no longer to provide their professional services to us in the near future. If we are not able to attract and retain necessary personnel and consultants to accomplish our business objectives, we may experience constraints that will significantly impede the achievement of our development objectives, our ability to raise additional capital and our ability to implement our business strategy. In addition, we may need to adjust the size of our workforce as a result of changes to our expectations for our business, which can result in diversion of management attention, disruptions to our business, and related expenses.

In addition, we previously announced a reduction in force, impacting a number of employees. Any further reduction in force may yield unintended consequences and costs, such as the loss of institutional knowledge and expertise, attrition beyond the intended reduction in force, the distraction of employees, reduced employee morale and could adversely affect our reputation as an employer, which could make it more difficult for us to hire new employees in the future and increase the risk that we may not achieve the anticipated benefits from the cost reduction program.

Our industry has experienced a high rate of turnover of management personnel in recent years. Potential changes in management could be disruptive to our business and may also result in our loss of unique skills and loss of knowledge about our business. Such turnover may also result in the departure of other existing employees or partners.

Replacing executive officers, key employees and consultants may be difficult and may take an extended period because of the limited number of individuals in our industry with the breadth of skills and experience required to develop, gain regulatory approval of and commercialize drug candidates successfully. Competition to hire and retain employees and consultants from this limited pool is intense, and we may be unable to hire, train, retain or motivate these additional key personnel and consultants. Our failure to retain or replace key personnel or consultants could materially harm our business. Additionally, the members of our management team have limited experience managing a public company, interacting with public company investors, and complying with the increasingly complex laws, rules and regulations that specifically govern public companies, which could cause our management to have to expend time and resources helping them become familiar with such requirements. We may lose our ability to implement our business strategy successfully and could be seriously harmed. Any of our executive officers or key employees or consultants may terminate their employment at any time.

We have scientific and clinical advisors and consultants who assist us in formulating our research, development and clinical strategies. These 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. Non-compete agreements are not permissible or are limited by law in certain jurisdictions and, even where they are permitted, these individuals typically will not enter into non-compete agreements with us. If a conflict of interest arises between their work for us and their work for another entity, we may lose their services. In addition, our advisors may have arrangements with other companies to assist those companies in developing drug candidates or technologies that may compete with ours.

Our employees, independent contractors, consultants, commercial partners and vendors may engage in misconduct or other improper activities, including noncompliance with regulatory standards and requirements and insider trading, which could significantly harm our business.

We are exposed to the risk of fraud or other misconduct by our employees, independent contractors, consultants, commercial partners and vendors. Misconduct by these parties could include intentional failures to comply with the regulations of the FDA and non-U.S. regulators, provide accurate information to the FDA and non-U.S. regulators, comply with health care fraud and abuse laws and regulations in the United States and abroad, report financial information or data accurately or disclose unauthorized activities to us. In particular, sales, marketing and business arrangements in the health care industry are subject to extensive laws and regulations intended to prevent fraud, misconduct, kickbacks, self-dealing and other abusive practices. These laws and regulations may restrict or prohibit a wide range of pricing, discounting, marketing and promotion, sales commission, customer incentive programs and other business arrangements. Employee misconduct could also involve the improper use of information obtained in the course of clinical trials, which could result in regulatory sanctions and serious harm to our reputation. It is not always possible to identify and deter employee misconduct, and the precautions we take to detect and prevent this activity may not be effective in controlling unknown or unmanaged risks or losses or in protecting us from governmental investigations or other actions or lawsuits stemming from a failure to

comply with these laws or regulations. If any such actions are instituted against us, those actions could have a significant impact on our business, including the imposition of significant fines or other sanctions.

Our insurance policies are expensive and only protect us from some business risks, which will leave us exposed to significant uninsured liabilities.

We do not carry insurance for all categories of risk that our business may encounter. Some of the policies we currently maintain include general liability, products liability and directors' and officers' insurance. We do not know, however, if we will be able to maintain insurance with adequate levels of coverage. Any significant uninsured liability may require us to pay substantial amounts, which would adversely affect our financial position and results of operations.

Failure (or perceived failure) to comply with health and data protection laws and regulations could lead to government enforcement actions, which could include civil or criminal penalties, private litigation, and/or adverse publicity and could negatively affect our operating results and business.

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, sensitive third-party data, business plans, transactions, financial information and (collectively, "sensitive data"). As a result, we and our collaborators are or may become subject to various federal, state, and foreign data protection laws and regulations (i.e., laws and regulations that address privacy and data security).

In the United States, numerous federal and state laws and regulations including federal health information privacy laws, state comprehensive consumer privacy laws, state data breach notification laws, state health information privacy laws, and federal and state consumer protection laws that govern the collection, use, disclosure, and protection of health-related and other personal information apply or could apply to our operations or the operations of our collaborators. Similar laws are being considered in various other states, as well as at the federal and local levels, and we expect more states to pass similar laws in the future. These developments may further complicate compliance efforts and increase legal risk and compliance costs for us and the collaborators upon whom we rely. In addition, we may obtain health information from third parties (including research institutions from which we obtain clinical trial data) that are subject to privacy and security requirements under the federal HIPAA as amended by the HITECH. Depending on the facts and circumstances, we could be subject to civil, criminal, and administrative penalties if we violate (or are perceived to violate) HIPAA.

Many foreign jurisdictions, including the EU, its member states, the United Kingdom and Australia, among others, have also adopted legislation and regulations that increase or change the requirements governing the collection, use, disclosure and transfer of the personal information of individuals in these jurisdictions. These laws, and similar laws being considered in other countries, and regulations are complex and change frequently, at times due to changes in political climate, and existing laws and regulations are subject to different and conflicting interpretations, which adds to the complexity of processing personal data from these jurisdictions. These laws have the potential to increase costs of compliance, risks of noncompliance and penalties for noncompliance.

For example, the EU's GDPR imposes numerous requirements for the collection, use and disclosure of personal information, including more stringent requirements relating to consent and the information that must be shared with data subjects about how their personal information is used, the obligation to notify regulatory authorities and affected individuals of personal data breaches, extensive internal privacy governance obligations, and obligations to honor expanded rights of individuals in relation to their personal information (for example, the right to access, correct and delete their data). In addition, the GDPR generally maintains restrictions on cross-border data transfer, and as a result we may be unable to transfer personal data from Europe and other jurisdictions to the United States or other countries. The GDPR may increase our responsibility and liability in relation to personal data that we process, and may also increase our costs of compliance.

Compliance with U.S. and international data protection laws and regulations could require us to take on more onerous obligations in our contracts, restrict our ability to collect, use and disclose data, or in some cases, impact our ability to operate in certain jurisdictions. Failure (or perceived failure) to comply with these laws and regulations could result in government enforcement actions (which could include civil, criminal and administrative penalties), private litigation, and/or adverse publicity and could negatively affect our operating results and business. Actual or perceived failure to comply with privacy laws may also cause clinical trial subjects, employees and other individuals about whom we or our potential collaborators obtain personal information, as well as the providers who share this information with us, to limit our ability to collect, use and disclose personal information. Claims that we have violated individuals' privacy rights, failed to comply with data protection laws, or breached our contractual obligations, even if we are not found liable, could be expensive and time-consuming to defend and could result in adverse publicity that could harm our business.

Changes in healthcare law and implementing regulations, as well as changes in healthcare policy, may impact our business in ways that we cannot currently predict, and may have a significant adverse effect on our business and results of operations.

In the United States and some foreign jurisdictions, there have been, and continue to be, several legislative and regulatory changes and proposed changes regarding the healthcare system that could prevent or delay marketing approval of drug candidates, restrict or regulate post-approval activities, and affect our ability to profitably sell any drug candidates for which we obtain marketing approval. Among policy makers and payors in the United States and elsewhere, including in the EU, there is significant interest in promoting changes in healthcare systems with the stated goals of containing healthcare costs, improving quality and/or expanding access. In the United States, the pharmaceutical industry has been a particular focus of these efforts and has been significantly affected by major legislative initiatives.

The PPACA substantially changed the way healthcare is financed by both the government and private insurers, and significantly impacts the U.S. pharmaceutical industry.

Since its enactment, there have been executive, judicial and Congressional challenges to certain aspects of the PPACA. It is possible that the PPACA will be subject to judicial or Congressional challenges in the future. It is unclear how any such challenges and the healthcare reform measures will impact the PPACA and our business. Other healthcare reform measures that may be adopted in the future could have a material adverse effect on our industry generally and on our ability to maintain or increase sales of our existing products that we successfully commercialize or to successfully commercialize our drug candidates, if approved. In addition to the PPACA, there will continue to be proposals by legislators at both the federal and state levels, regulators and third-party payors to keep healthcare costs down while expanding individual healthcare benefits. For example, on August 16, 2022, President Biden signed the IRA into law, which among other things, extends enhanced subsidies for individuals purchasing health insurance coverage in PPACA marketplaces through plan year 2025. The IRA also eliminates the "donut hole" under the Medicare Part D program beginning in 2025 by significantly lowering the beneficiary maximum out-of-pocket cost and through a newly established manufacturer discount program. It is unclear how these or similar policy initiatives will impact the PPACA and our business.

Other legislative changes have been proposed and adopted since the PPACA was enacted. These changes include aggregate reductions to Medicare payments to providers of up to 2% per fiscal year pursuant to the Budget Control Act of 2011 and subsequent laws, which began in 2013 and, due to subsequent legislative amendments to the statute, will remain in effect until 2032, unless additional Congressional action is taken. New laws may result in additional reductions in Medicare and other healthcare funding, which may adversely affect customer demand and affordability for our drug candidates and, accordingly, the results of our financial operations.

Also, there has been heightened governmental scrutiny recently over the manner in which drug manufacturers set prices for their marketed drug candidates, which has resulted in several Congressional inquiries and proposed and enacted federal and state legislation designed to, among other things, bring more transparency to drug pricing, review the relationship between pricing and manufacturer patient programs, and reform government program reimbursement methodologies for drugs. For example, in July 2021, the Biden administration released an executive order, "Promoting Competition in the American Economy," with multiple provisions aimed at prescription drugs. In response to Biden's executive order, on September 9, 2021, the Department of Health and Human Services (HHS) released a Comprehensive Plan for Addressing High Drug Prices that outlines principles for drug pricing reform and sets out a variety of potential legislative policies that Congress could pursue as well as potential administrative actions HHS can take to advance these principles. Further, the IRA will, among other things (i) allow HHS to negotiate the price of certain high-expenditure, single-source drugs and biologics covered under Medicare, and subject drug manufacturers to civil monetary penalties and a potential excise tax by offering a price that is not equal to or less than the "negotiated fair price" under the law and (ii) impose rebates with respect to certain drugs and biologics covered under Medicare Part B or Medicare Part D to penalize price increases that outpace inflation. The IRA permits HHS to implement many of these provisions through guidance, as opposed to regulation, for the initial years. HHS has and will continue to issue and update guidance as these programs are implemented. It is currently unclear how the IRA will be effectuated but is likely to have a significant impact on the pharmaceutical industry. Further, in response to the Biden administration's October 2022 executive order, on February 14, 2023, HHS released a report outlining three new models for testing by the CMS Innovation Center which will be evaluated on their ability to lower the cost of drugs, promote accessibility, and improve quality of care. It is unclear whether the models will be utilized in any health reform measures in the future. 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.

We expect that these and other healthcare reform measures that may be adopted in the future may result in more rigorous coverage criteria and lower reimbursement, and in additional downward pressure on the price that we receive for any approved drug candidate. Any reduction in reimbursement from Medicare or other government-funded programs may result in a similar reduction in

payments from private payors. The implementation of cost containment measures or other healthcare reforms may prevent us from being able to generate revenue, attain profitability or commercialize our drug candidates, once marketing approval is obtained.

Our ability to successfully commercialize any drugs that we develop depends in part on the extent to which coverage and adequate reimbursement are available from government health administration authorities, private health insurers, and other organizations.

Our ability to successfully commercialize any drugs that we develop depends in part on the extent to which coverage and adequate reimbursement are available from government health administration authorities, private health insurers, and other organizations. Government authorities and third-party payors, such as private health insurers and health maintenance organizations, each individually decide which medications they will pay for and establish reimbursement levels. A primary trend in the U.S. healthcare industry and elsewhere is cost containment. Government authorities and third-party payors have attempted to control costs by limiting coverage and the amount of reimbursement for particular medications. Government authorities currently impose mandatory discounts for certain patient groups, such as Medicare, Medicaid and VA hospitals, and may seek to increase such discounts at any time. Future regulation may negatively impact the price of our drug candidates, if approved. 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. We cannot be sure that coverage or reimbursement will be available for any drug candidate that we commercialize and, if coverage or reimbursement is available, the level of reimbursement. Reimbursement may impact the demand for, or the price of, any drug candidate for which we obtain marketing approval. In order to get coverage and reimbursement, physicians may need to show that patients have superior treatment outcomes with our products compared to standard of care drugs, including lower-priced generic versions of standard of care drugs. It is possible that a third-party payor may consider our drug candidates, once approved, and other therapies as substitutable and only offer to reimburse patients for the less expensive product. Even if we show improved efficacy or improved convenience of administration with our drug candidates, once approved, compared to existing products, pricing of existing products may limit the amount we will be able to charge for our drug candidates. Third-party payors may deny or revoke the reimbursement status of a given drug product or establish prices for new or existing marketed products at levels that are too low to enable us to realize an appropriate return on our investment in product development. Because EryDex is still in development, we are unable at this time to determine the likely level or method of coverage and reimbursement from third-party payors. If reimbursement is not available or is available only to limited levels, we may not be able to successfully commercialize any drug candidate for which we obtain marketing approval. In the United States, no uniform policy of coverage and reimbursement for products exists among third-party payors, and coverage decisions and reimbursement levels for products can differ significantly from payor to payor. As a result, the coverage determination process is often a time consuming and costly process that may require us to provide scientific and clinical support for the use of our products to each payor separately, with no assurance that coverage and adequate reimbursement will be applied consistently or obtained.

There may be significant delays in obtaining coverage and reimbursement for newly approved drugs, and coverage may be more limited than the purposes for which the medicine is approved by the FDA or other comparable foreign regulatory authorities. Moreover, eligibility for reimbursement does not imply that any drug will be paid for in all cases or at a rate that covers our costs, including research, development, manufacture, sale, and distribution. Interim reimbursement levels for new drugs, if applicable, may also not be sufficient to cover our costs and may not be made permanent. Reimbursement rates may vary according to the use of the drug and the clinical setting in which it is used, may be based on reimbursement levels already set for lower cost drugs and may be incorporated into existing payments for other services. Net prices for drugs may be reduced by mandatory discounts or rebates required by government healthcare programs or private payors and by any future relaxation of laws that presently restrict imports of drugs from countries where they may be sold at lower prices than in the United States. Third-party payors often rely upon Medicare coverage policy and payment limitations in setting their own reimbursement policies, but make their determinations independently and may impose additional restrictions. Our inability to promptly obtain and maintain coverage and profitable payment rates from both government-funded and private payors for any approved products we may develop could have a material adverse effect on our operating results, our ability to raise capital needed to commercialize drug candidates, and our overall financial condition. Further, coverage policies and third-party payor reimbursement rates may change at any time. Therefore, even if favorable coverage and reimbursement status is attained, less favorable coverage policies and reimbursement rates may be implemented in the future.

In the EU, coverage and reimbursement status of any drug candidates for which we obtain regulatory approval are provided for by the national laws of EU member states. The requirements may differ across the EU member states. The EU provides options for EU Member States to restrict the range of medicinal products for which their national health insurance systems provide reimbursement and to control the prices of medicinal products for human use. An EU Member State may approve a specific price for the medicinal product, it may refuse to reimburse a product at the price set by the manufacturer or it may instead adopt a system of direct or indirect controls on the profitability of the company placing the medicinal product on the market. Many EU Member States also periodically review their reimbursement procedures for medicinal products, which could have an adverse impact on reimbursement status.

Moreover, in order to obtain reimbursement for our products in some European countries, including some EU Member States, we may be required to compile additional data comparing the cost-effectiveness of our products to other available therapies.

This HTA of medicinal products is becoming an increasingly common part of the pricing and reimbursement procedures in some EU Member States, including those representing the larger markets. The HTA process is the procedure to assess therapeutic, economic and societal impact of a given medicinal product in the national healthcare systems of the individual country. The outcome of an HTA will often influence the pricing and reimbursement status granted to these medicinal products by the competent authorities of individual EU Member States. The extent to which pricing and reimbursement decisions are influenced by the HTA of the specific medicinal product currently varies between EU Member States. In December 2021, Regulation No 2021/2282 on HTA amending Directive 2011/24/EU, was adopted in the EU. This Regulation, which entered into force in January 2022 and will apply as of January 2025, is intended to boost cooperation among EU Member States in assessing health technologies, including new medicinal products, and providing the basis for cooperation at EU level for joint clinical assessments in these areas. The Regulation foresees a three-year transitional period and will permit EU Member States to use common HTA tools, methodologies, and procedures across the EU, working together in four main areas, including joint clinical assessment of the innovative health technologies with the most potential impact for patients, joint scientific consultations whereby developers can seek advice from HTA authorities, identification of emerging health technologies to identify promising technologies early, and continuing voluntary cooperation in other areas. Individual EU Member States will continue to be responsible for assessing non-clinical (e.g., economic, social, ethical) aspects of health technologies, and making decisions on pricing and reimbursement. If we are unable to maintain favorable pricing and reimbursement status in EU Member States for drug candidates that we may successfully develop and for which we may obtain regulatory approval, any anticipated revenue from and growth prospects for those products in the EU could be negatively affected. In light of the fact that the United Kingdom has left the EU, Regulation No 2021/2282 on HTA will not apply in the United Kingdom. However, the UK MHRA is working with UK HTA bodies and other national organizations, such as the Scottish Medicines Consortium, the National Institute for Health and Care Excellence, and the All-Wales Medicines Strategy Group, to introduce new pathways supporting innovative approaches to the safe, timely and efficient development of medicinal products.

Interim, top-line and preliminary data from our future clinical trials that we announce or publish from time to time may change as more patient data become available and are subject to audit and verification procedures that could result in material changes in the final data.

From time to time, we may publicly disclose preliminary or top-line data from our future clinical studies, which are based on a preliminary analysis of then-available data, and the results and related findings and conclusions are subject to change following a more comprehensive review of the data related to the particular study or trial. We also make assumptions, estimations, calculations and conclusions as part of our analyses of data, and we may not have received or had the opportunity to fully and carefully evaluate all data. As a result, the top-line results that we report may differ from future results of the same studies, or different conclusions or considerations may qualify such results, once additional data have been received and fully evaluated. 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, top-line data should be viewed with caution until the final data are available. From time to time, we may also disclose interim data from our clinical studies.

In addition, we may report interim analyses of only certain endpoints rather than all endpoints. Interim data from future 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. Adverse differences between preliminary or interim data and final data could significantly harm our business prospects. Further, disclosure of interim data by us or by our competitors could result in volatility in the price of our common stock. Further, others, including regulatory agencies, may not accept or agree with our assumptions, estimates, calculations, conclusions or analyses or may interpret or weigh the importance of data differently, which could impact the value of the particular program, the approvability or commercialization of the particular drug candidate or product and our company in general. In addition, the information we choose to publicly disclose regarding a particular study or clinical trial is based on what is typically extensive information, and you or others may not agree with what we determine is the material or otherwise appropriate information to include in our disclosure, and any information we determine not to disclose may ultimately be deemed significant with respect to future decisions, conclusions, views, activities or otherwise regarding a particular drug, drug candidate or our business. If the top-line data that we report differ from actual results, or if others, including regulatory authorities, disagree with the conclusions reached, our ability to obtain approval for, and commercialize, our drug candidates may be harmed, which could harm our business, operating results, prospects or financial condition.

Even if we obtain regulatory approval for a drug candidate, it will remain subject to extensive ongoing regulatory review and requirements.

If any of our future drug candidates are approved, they will be subject to ongoing regulatory requirements for manufacturing, labeling, packaging, storage, advertising, promotion, sampling, record-keeping, conduct of post-marketing studies, and submission of safety, efficacy, and other post-market information, including both federal and state requirements in the United States and requirements of comparable foreign regulatory authorities.

Manufacturers and manufacturers' facilities are required to comply with extensive requirements imposed by the FDA and comparable foreign regulatory authorities, including ensuring that quality control and manufacturing procedures conform to cGMPs regulations. As such, we and our contract manufacturers will be subject to continual review and inspections to assess compliance with cGMP and adherence to commitments made in any NDA. Accordingly, we and others with whom we work must continue to expend time, money, and effort in all areas of regulatory compliance, including manufacturing, production and quality control. We also are required to register our establishments and list our products with the FDA and certain state agencies. We and any third- party manufacturers or suppliers must continually adhere to federal regulations setting forth cGMP (for drugs) and QSR (for medical devices), and their foreign equivalents, which are enforced by the FDA and other national regulatory bodies through their facilities inspection programs. In complying with cGMP and foreign regulatory requirements, we and any of our third-party manufacturers or suppliers will be obligated to expend time, money and effort in production, record-keeping and quality control to ensure that our products meet applicable specifications and other requirements. QSR requirements also impose extensive testing, control and documentation requirements. State regulatory authorities and the regulatory agencies of other countries have similar requirements. In addition, we will be required to comply with regulatory requirements of the FDA, state regulatory agencies and the regulatory agencies of other countries concerning the reporting of AEs and device malfunctions, corrections and removals (e.g., recalls), promotion and advertising and general prohibitions against the manufacture and distribution of adulterated and misbranded devices.

Failure to comply with these regulatory requirements could result in enforcement actions, including, but not limited to, significant civil fines, product seizures, injunctions and/or criminal prosecution of responsible individuals and us. Any such actions would have a material adverse effect on our business, financial condition and results of operations.

Manufacturers and manufacturers' facilities are required to comply with extensive requirements imposed by the FDA and comparable foreign regulatory authorities, including ensuring that quality control and manufacturing procedures conform to cGMPs regulations. As such, we and our contract manufacturers will be subject to continual review and inspections to assess compliance with cGMP and adherence to commitments made in any NDA or comparable foreign application. Accordingly, we and others with whom we work must continue to expend time, money, and effort in all areas of regulatory compliance, including manufacturing, production and quality control.

Any regulatory approvals that we receive for our potential drug candidates will be subject to limitations on the approved indicated uses for which the drug candidate may be marketed and promoted or to the conditions of approval (including the potential for a requirement to implement a Risk Evaluation and Mitigation Strategy) or contain requirements for potentially costly post-marketing testing. We will be required to report certain adverse reactions and production problems, if any, to the FDA and comparable foreign regulatory authorities. Any new legislation addressing drug safety issues could result in delays in drug development or commercialization, or increased costs to assure compliance. The FDA and other agencies, including the Department of Justice, as well as foreign regulatory authorities closely regulate and monitor the post-approval marketing and promotion of drug candidates to ensure that they are manufactured, marketed and distributed only for the approved indications and in accordance with the provisions of the approved labeling. We will have to comply with requirements concerning advertising and promotion for our potential drug candidates and any products for which we receive approval. Promotional communications with respect to prescription drugs are subject to a variety of legal and regulatory restrictions and must be consistent with the information in the drug candidate's approved label. As such, we may not promote our potential drug candidates for indications or uses for which they do not have approval. In the EU, the advertising and promotion of medicinal products are subject to both EU and EU Member States' laws governing promotion of medicinal products, interactions with physicians and other healthcare professionals, misleading and comparative advertising and unfair commercial practices. General requirements for advertising and promotion of medicinal products, such as direct-to-consumer advertising of prescription medicinal products are established in EU law. However, the details are governed by regulations in individual EU Member States and can differ from one country to another. For example, applicable laws require that promotional materials and advertising in relation to medicinal products comply with the product's Summary of Product Characteristics, or SmPC, which may require approval by the competent national authorities in connection with an MA. The SmPC is the document that provides information to physicians concerning the safe and effective use of the product. Promotional activity that does not comply with the SmPC is considered off-label and is prohibited in the EU.

The holder of an approved NDA or equivalent foreign application must submit new or supplemental applications and obtain approval for certain changes to the approved drug candidate labeling, or manufacturing process. We could also be asked to conduct post-marketing clinical trials to verify the safety and efficacy of our potential drug candidates in general or in specific patient subsets. If original marketing approval was obtained via the accelerated approval pathway, we could be required to conduct a successful post-marketing clinical trial to confirm clinical benefit for our drug candidates. An unsuccessful post-marketing study or failure to complete such a study could result in the withdrawal of marketing approval.

If a regulatory authority discovers previously unknown problems with a drug or device, such as AEs of unanticipated severity or frequency, or problems with the facility where the drug candidate is manufactured, or disagrees with the promotion, marketing or labeling of a drug candidate, including if approved, such regulatory authority may impose restrictions on that drug candidate, an

approved drug, or us, including requiring withdrawal of the approved drug from the market. If we fail to comply with applicable regulatory requirements, a regulatory authority or enforcement authority may, among other things:

- issue warning or untitled letters that would result in adverse publicity;
- impose civil or criminal penalties;
- suspend, vary or withdraw regulatory approvals;
- suspend any of our ongoing clinical trials;
- mandate modifications to promotional materials or require us to provide corrective information to healthcare practitioners;
- require us to enter into a consent decree or permanent injunction, which can include imposition of various fines, reimbursements for inspection costs, required due dates for specific actions and penalties for noncompliance;
- refuse to approve pending applications or supplements to approved applications submitted by us;
- impose restrictions on our operations, including closing our contract manufacturers' facilities;
- seize or detain drug candidates or approved drugs; or
- require a drug candidate or approved drugs recall.

Any government investigation of alleged violations of law could require us to expend significant time and resources in response and could generate negative publicity. Any failure to comply with ongoing regulatory requirements may significantly and adversely affect our ability to commercialize and generate revenue from our drug candidates. If regulatory sanctions are applied or if regulatory approval is suspended, varied or withdrawn, the value of our company and our operating results will be adversely affected.

Non-compliance by us or any future collaborator with regulatory requirements, including safety monitoring or pharmacovigilance, and with requirements related to the development of products for the pediatric population can also result in significant financial penalties.

We may be unable to obtain and retain orphan drug designations for some of our drug candidates or to maintain the benefits associated with orphan drug designation status, including market exclusivity, which may cause our revenue, if any, to be reduced.

Regulatory authorities in some jurisdictions, including the United States and the EU, may designate drugs for relatively small patient populations as orphan drugs. Under the Orphan Drug Act, the FDA may grant orphan drug designation to a drug intended to treat a rare disease or condition, defined as a disease or condition with a patient population of fewer than 200,000 in the United States, or a patient population greater than 200,000 in the United States when there is no reasonable expectation that the cost of developing and making available the drug in the United States will be recovered from sales in the United States for that drug. Orphan drug designation must be requested before submitting an NDA. In the United States, orphan drug designation entitles a party to financial incentives such as opportunities for grant funding towards clinical trial costs, tax advantages, and user-fee waivers. After the FDA grants orphan drug designation, the generic identity of the drug and its potential orphan use are disclosed publicly by the FDA. Orphan drug designation does not convey any advantage in, or shorten the duration of, the regulatory review and approval process. In the EU, the European Commission, following an opinion from the EMA's Committee for Orphan Medicinal Products may grant orphan drug designation to promote the development of products (i) that are intended for the diagnosis, prevention, or treatment of a life-threatening or chronically debilitating conditions; (ii) either such conditions affect not more than five in 10,000 persons in the EU community, or without incentives, it is unlikely that sales of the drug in the EU would be sufficient to justify the necessary investment in developing the drug or biological product; and (iii) there exists no satisfactory authorized method of diagnosis, prevention, or treatment of the condition that has been authorized in the EU, or even if such method exists, the product will be of significant benefit to those affected by that condition. In the EU, orphan drug designation provides a range of potential incentives for medicinal products that have been granted an orphan designation by the European Commission, including protocol assistance, access to the centralized authorization procedure and fee reductions.

If a product that has orphan drug designation subsequently receives the first FDA approval for a particular active ingredient for the disease 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, including an NDA, to market the same drug for the same indication for seven years, except in limited circumstances such as a showing of clinical superiority to the product with orphan product exclusivity or if the FDA finds that the holder of the orphan product exclusivity has not shown that it can assure the availability of sufficient quantities of the orphan drug to meet the needs of patients with the disease or condition for which the drug was designated. A product may obtain orphan drug

exclusivity for each indication that has been designated upon approval of the indication, subject to the qualifications above. Any orphan drug exclusivity granted for second or subsequent indications applies only to those subsequent indications and does not block approval of a product for the first indication once the initial period of exclusivity has expired. Moreover, even if one of our drug candidates receives orphan product exclusivity, the FDA can still approve other drugs that have a different active ingredient for use in treating the same indication or disease.

In the EU, upon grant of a marketing authorization, orphan medicinal products are entitled to a ten-year period of market exclusivity for the approved therapeutic indication, which means that the EMA cannot accept another marketing authorization application or accept an application to extend for a similar product and the European Commission cannot grant a marketing authorization for the same indication for a period of ten years. The period of market exclusivity is extended by two years for orphan medicinal products that have also complied with an agreed PIP. No extension to any supplementary protection certificate can be granted on the basis of pediatric studies for orphan indications. Orphan medicinal product designation does not convey any advantage in, or shorten the duration of, the regulatory review and approval process. The period of market exclusivity may, however, be reduced to six years if, at the end of the fifth year, it is established that the product no longer meets the criteria on the basis of which it received orphan medicinal product destination, including where it can be demonstrated on the basis of available evidence that the original orphan medicinal product is sufficiently profitable not to justify maintenance of market exclusivity or where the prevalence of the condition has increased above the threshold. Additionally, an MA may be granted to a similar medicinal product with the same orphan indication during the 10 year period if: (i) if the applicant consents to a second original orphan medicinal product application; (ii) if the manufacturer of the original orphan medicinal product is unable to supply sufficient quantities; or (iii) if the second applicant can establish that its product, although similar, is safer, more effective or otherwise clinically superior to the original orphan medicinal product. A company may voluntarily remove a product from the register of orphan products.

We have received orphan drug designation by the FDA and European Commission for EryDex for the treatment of A-T. We may seek orphan drug designation in the United States, the EU and other European countries for additional orphan indications in which there is a medically plausible basis, including other rare diseases. In the future, exclusive marketing rights in the United States, if granted, may be limited if we seek approval for an indication broader than the orphan drug designated indication and may be lost if the FDA later determines that the request for the orphan drug designation was materially defective or if the manufacturer is unable to assure sufficient quantities of the product to meet the needs of patients with the rare disease or condition. In addition, although we have sought or intend to seek orphan drug designation, we may never receive approval for such designations.

If we fail to comply with healthcare laws, we could face substantial penalties and our business, operations and financial condition could be adversely affected.

Our operations are subject to various federal and state fraud and abuse and other healthcare laws. The laws that may impact our operations include:

- federal Anti-Kickback Statute, which prohibits, among other things, persons and entities from knowingly and willfully soliciting, receiving, offering or paying any remuneration (including any kickback, bribe, or rebate), directly or indirectly, overtly or covertly, in cash or in kind, to induce, or in return for, either the referral of an individual, or the purchase, lease, order or recommendation of any good, facility, item or service for which payment may be made, in whole or in part, under a federal healthcare program, such as the Medicare and Medicaid programs. A person or entity does not need to have actual knowledge of the statute or specific intent to violate it in order to have committed a violation. In addition, the government may assert that a claim including items or services resulting from a violation of the federal Anti-Kickback Statute constitutes a false or fraudulent claim for purposes of the False Claims Act;
- federal civil and criminal false claims laws, including the False Claims Act, and civil monetary penalty laws, which impose criminal and civil penalties, including through civil “qui tam” or “whistleblower” actions, against individuals or entities from, among other things, knowingly presenting, or causing to be presented, claims for payment or approval from Medicare, Medicaid, or other third-party payors that are false or fraudulent or knowingly making a false statement to improperly avoid, decrease or conceal an obligation to pay money to the federal government. Similar to the federal Anti-Kickback Statute, a person or entity does not need to have actual knowledge of these statutes or specific intent to violate them in order to have committed a violation;
- the federal HIPAA, which created new federal criminal statutes that prohibit, among other things, knowingly and willfully executing, or attempting to execute, a scheme to defraud any healthcare benefit program or obtain, by means of false or fraudulent pretenses, representations, or promises, any of the money or property owned by, or under the custody or control of, any healthcare benefit program, regardless of the payor (e.g., public or private) and

knowingly and willfully falsifying, concealing or covering up by any trick or device a material fact or making any materially false statements in connection with the delivery of, or payment for, healthcare benefits, items or services relating to healthcare matters;

- HIPAA, as amended by HITECH, and their respective implementing regulations, which impose requirements on certain covered healthcare providers, health plans, and healthcare clearinghouses as well as their respective business associates and their subcontractors that perform services for them that involve the use, or disclosure of, individually identifiable health information, relating to the privacy, security and transmission of individually identifiable health information without appropriate authorization;
- the federal Physician Payment Sunshine Act, created under the PPACA, and its implementing regulations, which require certain manufacturers of drugs, devices, biologics and medical supplies for which payment is available under Medicare, Medicaid or the Children's Health Insurance Program to report annually to the U.S. Department of Health and Human Services under the Open Payments Program, information related to payments or other transfers of value made to physicians, (defined to include doctors, dentists, optometrists, podiatrists and chiropractors), certain other healthcare professionals (such as physician assistants and nurse practitioners), and teaching hospitals, as well as ownership and investment interests held by physicians and their immediate family members;
- federal consumer protection and unfair competition laws, which broadly regulate marketplace activities and activities that potentially harm consumers; and
- analogous state and foreign laws and regulations, such as state and foreign anti-kickback, false claims, consumer protection and unfair competition laws which may apply to pharmaceutical business practices, including but not limited to, research, distribution, sales and marketing arrangements as well as submitting claims involving healthcare items or services reimbursed by any third-party payor, including commercial insurers; state and foreign laws that require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the government that otherwise restricts payments that may be made to healthcare providers and other potential referral sources; state and foreign laws that require the registration of sales representatives; state and foreign laws that require drug manufacturers to file reports with states or foreign regulatory authorities regarding pricing and marketing information, such as the tracking and reporting of gifts, compensations and other remuneration and items of value provided to healthcare professionals and entities; and state and foreign laws governing the privacy and security of health information in certain circumstances, many of which differ from each other in significant ways and may not have the same effect, thus complicating compliance efforts.

Outside the United States, interactions between pharmaceutical companies and health care professionals are also governed by strict laws, such as national anti-bribery laws of European countries, national sunshine rules, regulations, industry self-regulation codes of conduct and physicians' codes of professional conduct. Failure to comply with these requirements could result in reputational risk, public reprimands, administrative penalties, fines or imprisonment.

Because of the breadth of these laws and the narrowness of the statutory exceptions and safe harbors available, it is possible that some of our business activities, including compensating physicians with stock or stock options, could, despite our efforts to comply, be subject to challenge under one or more of such laws. Efforts to ensure that our business arrangements will comply with applicable healthcare laws may involve substantial costs. It is possible that governmental and enforcement authorities will conclude that our business practices may not comply with current or future statutes, regulations or case law interpreting applicable fraud and abuse or other healthcare laws and regulations. If any such actions are instituted against us, those actions could have a significant impact on our business, including the imposition of civil, criminal and administrative penalties, damages, disgorgement, monetary fines, possible exclusion from participation in Medicare, Medicaid and other federal healthcare programs, imprisonment, contractual damages, reputational harm, diminished profits and future earnings, and curtailment of our operations, any of which could adversely affect our ability to operate our business and our results of operations. In addition, the approval and commercialization of any of our drug candidates, if approved, outside the United States will also likely subject us to foreign equivalents of the healthcare laws mentioned above and comparable risks, among other foreign laws.

We are subject to substantial government regulation that is subject to change and could force us to make modifications to how we develop, manufacture, market, and price our products in the future.

The medical device industry is regulated extensively by governmental authorities, principally the FDA in the U.S. and corresponding state and foreign regulatory authorities. The majority of our manufacturing processes are required to comply with quality systems regulations, including cGMP requirements that cover the methods and documentation of the design, testing, production, control, quality assurance, labeling, packaging and shipping of our products. Failure to comply with applicable medical device regulatory requirements could result in, among other things, warning letters, fines, injunctions, civil penalties, repairs, replacements, refunds, recalls or seizures of products, total or partial suspensions of production, refusal of the FDA or other regulatory authorities to grant pre-market clearances or approvals for our products, withdrawals, or suspensions of future or current clearances or approvals and criminal prosecution.

In addition, our products are subject to pre-clearance requirements by the FDA and similar foreign regulatory authorities that govern a wide variety of product activities from design and development to labeling, manufacturing, promotion, sales, and distribution. Compliance with these regulations may be time consuming, burdensome, and expensive for us. The failure to obtain, or the loss or suspension of any such pre-approval, would negatively affect our ability to sell our products and harm our anticipated revenues.

Foreign governmental authorities that regulate the manufacture and sale of medical devices have become increasingly stringent and, to the extent we sell our products in foreign countries, we may be subject to rigorous regulation in the future. Regulatory changes could result in restrictions on our ability to carry on or expand our operations, higher than anticipated costs or lower than anticipated revenue.

The policies of the FDA and of other regulatory authorities may change and additional government regulations may be enacted that could prevent, limit or delay regulatory approval of our potential drug candidates. We cannot predict the likelihood, nature or extent of government regulation that may arise from future legislation or administrative action, either in the United States or abroad. For instance, the regulatory landscape related to clinical trials in the EU recently evolved. The EU Clinical Trials Regulation, or CTR, which was adopted in April 2014 and repeals the EU Clinical Trials Directive, became applicable on January 31, 2022. The CTR allows sponsors to make a single submission to both the competent authority and an ethics committee in each EU Member State, leading to a single decision for each EU Member State. The assessment procedure for the authorization of clinical trials has been harmonized as well, including a joint assessment by all EU Member States concerned, and a separate assessment by each EU Member State with respect to specific requirements related to its own territory, including ethics rules. Each EU Member State's decision is communicated to the sponsor via the centralized EU portal. Once the clinical trial approved, clinical study development may proceed. The CTR foresees a three-year transition period. The extent to which ongoing and new clinical trials will be governed by the CTR varies. For clinical trials in relation to which application for approval was made on the basis of the Clinical Trials Directive before January 31, 2023, the Clinical Trials Directive will continue to apply on a transitional basis until January 31, 2025. By that date, all ongoing trials will become subject to the provisions of the CTR. The CTR will apply to clinical trials from an earlier date if the related clinical trial application was made on the basis of the CTR or if the clinical trial has already transitioned to the CTR framework before January 31, 2025. Compliance with the CTR requirements by us and our third-party service providers, such as CROs, may impact our developments plans. In light of the entry into application of the CTR on January 31, 2022, we may be required to transition clinical trials for which we have obtained regulatory approvals in accordance with the CTD to the regulatory framework of the CTR. Transition of clinical trials governed by the CTD to the CTR will be required for clinical trials which will have at least one site active in the EU on January 30, 2025. A transitioning application would need to be submitted to the competent authorities of EU Member States through the Clinical Trials Information Systems and related regulatory approval obtained to continue the clinical trial past January 30, 2025. This would require financial, technical and human resources. If we are unable to transition our clinical trials in time, the conduct of those clinical trials may be negatively impacted.

It is currently unclear to what extent the UK will seek to align its regulations with the EU in the future. The UK regulatory framework in relation to clinical trials is derived from existing EU legislation (as implemented into UK law, through secondary legislation). On January 17, 2022, the UK Medicines and Healthcare products Regulatory Agency, or MHRA, launched an eight-week consultation on reframing the UK legislation for clinical trials. The UK Government published its response to the consultation on March 21, 2023 confirming that it would bring forward changes to the legislation. These resulting legislative amendments will determine how closely the UK regulations will align with the CTR. Failure of the UK to closely align its regulations with the EU may have an effect on the cost of conducting clinical trials in the UK as opposed to other countries and/or make it harder to seek a marketing authorization for the Company's drug candidates on the basis of clinical trials conducted in the United Kingdom.

In addition, on April 26, 2023, the European Commission adopted a proposal for a new Directive and Regulation to revise the existing pharmaceutical legislation. If adopted in the form proposed, the recent European Commission proposals to revise the existing EU laws governing authorization of medicinal products may result in a decrease in data and market exclusivity opportunities for our drug candidates in the EU and make them open to generic or biosimilar competition earlier than is currently the case with a related reduction in reimbursement status.

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 future marketing approval that we may have obtained and we may not achieve or sustain profitability, which would adversely affect our business, prospects, financial condition and results of operations.

If we or any contract manufacturers and suppliers we engage fail to comply with environmental, health, and safety laws and regulations, we could become subject to fines or penalties or incur costs that could have a material adverse effect on the success of our business.

We and any contract manufacturers and suppliers we engage are subject to numerous federal, state, and local environmental, health, and safety laws, regulations, and permitting requirements, including those governing laboratory procedures; the generation, handling, use, storage, treatment, and disposal of hazardous and regulated materials and wastes; the emission and discharge of hazardous materials into the ground, air, and water; and employee health and safety. Our operations involve the use of hazardous and flammable materials, including chemicals and radioactive materials. Our operations also produce hazardous waste. We generally contract with third parties for the disposal of these materials and wastes. We cannot eliminate the risk of contamination or injury from these materials. In the event of contamination or injury resulting from our use of hazardous materials, we could be held liable for any resulting damages, and any liability could exceed our resources. Under certain environmental laws, we could be held responsible for costs relating to any contamination at our current or past facilities and at third-party facilities. We also could incur significant costs associated with civil or criminal fines and penalties.

Compliance with applicable environmental laws and regulations may be expensive, and current or future environmental laws and regulations may impair our research, drug development and manufacturing efforts. In addition, we cannot entirely eliminate the risk of accidental injury or contamination from these materials or wastes. Although we maintain workers' compensation insurance to cover us for costs and expenses we may incur due to injuries to our employees resulting from the use of hazardous materials, this insurance may not provide adequate coverage against potential liabilities. We do not carry specific hazardous waste insurance coverage, and our property, casualty, and general liability insurance policies specifically exclude coverage for damages and fines arising from hazardous waste exposure or contamination. Accordingly, in the event of contamination or injury, we could be held liable for damages or be penalized with fines in an amount exceeding our resources, and our clinical trials or regulatory approvals could be suspended, which could have a material adverse effect on our business, financial condition, results of operations, and prospects.

Our business activities may be subject to the FCPA and similar anti-bribery and anti-corruption laws.

Our business activities may be subject to the FCPA and similar anti-bribery or anti-corruption laws, regulations or rules of other countries in which we may operate, including the UK Bribery Act. The FCPA generally prohibits offering, promising, giving, or authorizing others to give anything of value, either directly or indirectly, to a non-U.S. government official in order to influence official action, or otherwise obtain or retain business. The FCPA also requires public companies to make and keep books and records that accurately and fairly reflect the transactions of the corporation and to devise and maintain an adequate system of internal accounting controls. Our business is heavily regulated and therefore involves significant interaction with public officials, including officials of non-U.S. governments. Additionally, in many other countries, the health care providers who prescribe pharmaceuticals are employed by their government, and the purchasers of pharmaceuticals are government entities; therefore, our dealings with these prescribers and purchasers are subject to regulation under the FCPA. Recently the SEC and Department of Justice have increased their FCPA enforcement activities with respect to biotechnology and pharmaceutical companies. There is no certainty that all of our employees, agents, contractors, or those of our affiliates, will comply with all applicable laws and regulations, particularly given the high level of complexity of these laws. Violations of these laws and regulations could result in fines, criminal sanctions against us, our officers, or our employees, the closing down of our facilities, requirements to obtain export licenses, cessation of business activities in sanctioned countries, implementation of compliance programs, and prohibitions on the conduct of our business. Any such violations could include prohibitions on our ability to offer our drug candidates in one or more countries and could materially damage our reputation, our brand, our international expansion efforts, our ability to attract and retain employees, and our business, prospects, operating results, and financial condition.

Any collaboration arrangements that we may enter into in the future may not be successful, which could adversely affect our ability to develop and commercialize potential future drug candidates.

We may consider collaboration arrangements with pharmaceutical or biotechnology companies for the development or commercialization of drug candidates depending on the merits of retaining or divesting some or all commercialization rights. We will face, to the extent that we decide to enter into collaboration agreements, significant competition in seeking appropriate collaborators. Moreover, collaboration arrangements are complex and time-consuming to negotiate, document, implement and maintain. We may not be successful in our efforts to establish and implement collaborations or other alternative arrangements should we so chose to enter into such arrangements. The terms of any collaborations or other arrangements that we may establish may not be favorable to us.

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. Collaborations are subject to numerous risks, which may include that:

- collaborators have significant discretion in determining the efforts and resources that they will apply to collaborations;
- collaborators may not pursue development and commercialization of our drug candidates or may elect not to continue or renew development or commercialization programs based on clinical trial results, changes in their strategic focus due to the acquisition of competitive drug candidates, availability of funding or other external factors, such as a business combination that diverts resources or creates competing priorities;
- collaborators may delay clinical trials, provide insufficient funding for a clinical trial program, stop a clinical trial, abandon a drug candidate, repeat or conduct new clinical trials or require a new formulation of a drug candidate for clinical testing;
- collaborators could independently develop, or develop with third parties, drug candidates that compete directly or indirectly with our drug candidates;
- a collaborator with marketing, manufacturing and distribution rights to one or more drug candidates may not commit sufficient resources to or otherwise not perform satisfactorily in carrying out these activities;
- we could grant exclusive rights to our collaborators that would prevent us from collaborating with others;
- collaborators may not properly maintain or defend our intellectual property rights or may use our intellectual property or proprietary information in a way that gives rise to actual or threatened litigation that could jeopardize or invalidate our intellectual property or proprietary information or expose us to potential liability;
- disputes may arise between us and a collaborator that causes the delay or termination of the research, development or commercialization of our current or future drug candidates or that results in costly litigation or arbitration that diverts management attention and resources;
- collaborations may be terminated, and, if terminated, may result in a need for additional capital to pursue further development or commercialization of the applicable current or future drug candidates;
- collaborators may own or co-own intellectual property covering our drug candidates that results from our collaborating with them, and in such cases, we would not have the exclusive right to develop or commercialize such intellectual property; and
- a collaborator's sales and marketing activities or other operations may not be in compliance with applicable laws resulting in civil or criminal proceedings.

Risks Relating to Our Intellectual Property

If we are unable to obtain and maintain sufficient intellectual property protection for our current drug candidates, any future drug candidates, and other proprietary technology we develop, or if the scope of the intellectual property protection is not sufficiently broad, our competitors could develop and commercialize drug candidates similar or identical to ours, and our ability to successfully commercialize our current drug candidate, if approved, any future drug candidates, and other proprietary technologies if approved, may be adversely affected.

Our commercial success will depend in part on obtaining and maintaining a combination of patent protection, trade secret protection and confidentiality agreements to protect the intellectual property related to our current and future drug candidates and the methods used to manufacture them, as well as successfully defending these patents against third-party challenges. Our ability to stop third parties from making, using, selling, offering to sell or importing our drug candidates is dependent upon the extent to which we have rights under valid and enforceable patents or trade secrets that cover these activities.

The patent positions of biotechnology and pharmaceutical companies can be highly uncertain and involve complex legal and factual questions for which important legal principles remain unresolved. No consistent policy regarding the breadth of claims allowed in pharmaceutical patents has emerged to date in the United States or in many jurisdictions outside of the United States. Changes in either the patent laws or interpretations of patent laws in the United States and other countries may diminish the value of our intellectual property. Accordingly, we cannot predict the breadth of claims that may be enforced in the issued patents that we currently own, or in patents that may issue from the applications we currently or may in the future own or license from third parties. Further, if

any patents we obtain or license are deemed invalid and unenforceable, our ability to commercialize or license our technology could be adversely affected.

Others may have filed, and in the future are likely to file, patent applications covering drug candidates that are similar, identical or competitive to ours or important to our business. We cannot be certain that any patent application owned by a third party will not have priority over patent applications filed or in-licensed by us, or that we or our licensors will not be involved in interference, opposition or invalidity proceedings before U.S. or non-U.S. patent offices.

The degree of future protection for our proprietary rights is uncertain because legal means afford only limited protection and may not adequately protect our rights or permit us to gain or keep our competitive advantage. Only limited protection may be available and may not adequately protect our rights or permit us to gain or keep any competitive advantage. If we do not adequately protect our intellectual property and proprietary technology, competitors may be able to use our current or future drug candidates and proprietary technologies and erode or negate any competitive advantage we may have, which could have a material adverse effect on our financial condition and results of operations. For example:

- others may be able to make compounds that are similar to our drug candidates but that are not covered by the claims of our patents;
- we might not have been the first to make the inventions covered by our pending patent applications;
- we might not have been the first to file patent applications for these inventions;
- others may independently develop similar or alternative technologies or duplicate any of our technologies;
- any patents that we obtain may not provide us with any competitive advantages;
- we may not develop additional proprietary technologies that are patentable; or
- the patents of others may have an adverse effect on our business.

Should any of these events occur, they would significantly harm our business, results of operations and prospects.

We have applied, and we intend to continue applying, for patents covering aspects of our current drug candidates and device, any future drug candidates, any future improvements on the device or other proprietary technologies and their uses that we deem appropriate. However, we may not be able to apply for patents on certain aspects of our current or future drug candidates, proprietary technologies and their uses in a timely fashion, at a reasonable cost, in all jurisdictions, or at all, and any potential patent coverage we obtain may not be sufficient to prevent substantial competition.

Without patent protection on our current or future drug candidates, our ability to assert our patents to stop others from using or selling our current or future drug candidates may be limited. Due to the patent laws of a country, or the decisions of a patent examiner in a country, or our own filing strategies, we may not obtain patent coverage for all of our current or future drug candidates or methods involving the use of these candidates in a particular patent application. We plan to pursue divisional patent applications or continuation patent applications in the United States and other countries, where applicable, to obtain claim coverage for inventions which were disclosed but not claimed in a particular parent patent application.

The patent application process is subject to numerous risks and uncertainties, and there can be no assurance that we or any of our actual or potential future collaborators will be successful in protecting our current drug candidates, any future drug candidate, and other proprietary technologies and their uses by obtaining, defending, and enforcing patents. These risks and uncertainties include the following:

- the U.S. Patent and Trademark Office, or USPTO, and various foreign governmental patent agencies require compliance with a number of procedural, documentary, fee payment and other provisions during the patent process, the noncompliance with which can result in abandonment or lapse of a patent or patent application, and partial or complete loss of patent rights in the relevant jurisdiction;
- patent applications may not result in any patents being issued;
- patents that may be issued or in-licensed may be challenged, invalidated, modified, revoked, circumvented, found to be unenforceable or otherwise may not provide any competitive advantage;

- our competitors, many of whom have substantially greater resources than we do and many of whom have made significant investments in competing technologies, may seek or may have already obtained patents that will limit, interfere with or eliminate our ability to make, use and sell our drug candidates;
- other parties may have designed around our claims or developed technologies that may be related or competitive to our platform, may have filed or may file patent applications and may have received or may receive patents that overlap or conflict with our patent applications, either by claiming the same compounds, compositions of matter, or methods, or formulations, or by claiming subject matter that could dominate our patent position;
- any successful opposition to any patents owned by or licensed to us could deprive us of rights necessary to prevent others from practicing our technologies or to successfully commercialize any drug candidates that we may develop;
- because patent applications in the United States and most other countries are confidential for a period of time after filing, we cannot be certain that we or our licensors were the first to file any patent application related to our current drug candidates, any future drug candidates, and other proprietary technologies and their uses;
- an interference proceeding can be provoked by a third party or instituted by the USPTO to determine who was the first to invent any of the subject matter covered by the patent claims of applications we may in-license which have an effective filing date before March 16, 2013;
- there may be significant pressure on the U.S. government and international governmental bodies to limit the scope of patent protection both inside and outside the United States for disease treatments that prove successful, as a matter of public policy regarding worldwide health concerns; and
- countries other than the United States may have patent laws less favorable to patentees than those upheld by U.S. courts, allowing foreign competitors a better opportunity to create, develop and market competing drug candidates in those countries.

The patent prosecution process is also expensive and time-consuming, and we may not be able to file and prosecute all necessary or desirable patent applications at a reasonable cost or in a timely manner. It is also possible that we will fail to identify patentable aspects of our research and development output before it is too late to obtain patent protection. Although we enter into non-disclosure and confidentiality agreements with parties who have access to patentable aspects of our research and development output, such as our employees, corporate collaborators, outside scientific collaborators, CROs, contract manufacturers, consultants, advisors and other third parties, any of these parties may breach such agreements and disclose such output before a patent application is filed, thereby jeopardizing our ability to seek patent protection for such output. In addition, our ability to obtain and maintain valid and enforceable patents depends on whether the differences between our inventions and the prior art allow our inventions to be patentable over the prior art. Furthermore, publications of discoveries in the scientific literature often lag behind the actual discoveries, and patent applications in the United States and other jurisdictions are typically not published until 18 months after filing, or in some cases not at all. Therefore, we cannot be certain that we or our licensors were the first to make the inventions claimed in any of our owned or licensed patents or pending patent applications, or that we or our licensors were the first to file for patent protection of such inventions. We may also rely on trade secrets to protect our technology, especially where we do not believe patent protection is appropriate or feasible. However, trade secrets are difficult to protect. Although we use reasonable efforts to protect our trade secrets, our employees, consultants, contractors, outside scientific collaborators and other advisors may unintentionally or willfully disclose our information to competitors. Enforcing a claim that a third party illegally obtained and is using any of our trade secrets is expensive and time consuming, and the outcome is unpredictable. In addition, courts outside the United States are sometimes less willing to protect trade secrets. Moreover, our competitors may independently develop equivalent knowledge, methods and know-how.

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. Further, our issued patents could be found invalid or unenforceable if challenged in court, and we may incur substantial costs as a result of litigation or other proceedings relating to patent and other intellectual property rights.

Third parties, including competitors, may infringe, misappropriate or otherwise violate our patents, patents that may issue to us in the future, or the patents of our licensors that are licensed to us. To counter infringement or unauthorized use, we may need to choose to file infringement claims, which can be expensive and time-consuming. We may not be able to prevent, alone or with our licensors, infringement, misappropriation, or other violation of our intellectual property, particularly in countries where the laws may not protect those rights as fully as in the United States. If we choose to go to court to stop another party from using the inventions claimed in any patents we obtain, that individual or company has the right to ask the court to rule that such patents are invalid or should not be enforced against that third party for any number of reasons. In patent litigation in the United States, defendant counterclaims alleging invalidity and/or unenforceability are commonplace. Grounds for a validity challenge include an alleged failure

to meet any of several statutory requirements for patentability, including lack of novelty, obviousness or non-enablement. Grounds for an unenforceability assertion could include an allegation that someone connected with prosecution of the patent withheld relevant information from the USPTO or made a misleading statement during prosecution. Third parties may also raise similar claims before the USPTO, even outside the context of litigation. Similar mechanisms for challenging the validity and enforceability of a patent exist in non-U.S. patent offices and may result in the revocation, cancellation, or amendment of any non-U.S. patents we hold in the future. The outcome following legal assertions of invalidity and unenforceability is unpredictable, and prior art could render our patents, or those of our licensor's, invalid. 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 one or more drug candidates. Such a loss of patent protection would have a material adverse impact on our business.

These lawsuits are expensive and would consume time and resources and divert the attention of managerial and scientific personnel even if we were successful in stopping the infringement of such patents. In addition, there is a risk that the court will decide that such patents are not valid and that we do not have the right to stop the other party from using the claimed inventions. There is also the risk that, even if the validity of such patents is upheld, the court will refuse to stop the other party on the ground that such other party's activities do not infringe our rights to such patents. In addition, the U.S. Supreme Court has recently modified some tests used by the USPTO in granting patents over the past 20 years, which may decrease the likelihood that we will be able to obtain patents and increase the likelihood of challenge of any patents we obtain or license.

Interference or derivation proceedings provoked by third parties or brought by us or declared by the USPTO may be necessary to determine the priority of inventions with respect to our patents or patent applications, or those of our licensor's. An unfavorable outcome could require us to cease using the related technology or to attempt to license rights to it from the prevailing party. Our business could be harmed if the prevailing party does not offer us a license on commercially reasonable terms or at all, or if a non-exclusive license is offered and our competitors gain access to the same technology. 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. In addition, the uncertainties associated with litigation could have a material adverse effect on our ability to raise the funds necessary to continue our clinical trials, continue our research programs, license necessary technology from third parties or enter into development or manufacturing partnerships that would help us bring our current and any future drug candidates to market.

Even if resolved in our favor, litigation or other legal proceedings relating to our intellectual property rights may cause us to incur significant expenses, and could distract our technical and management personnel from their normal responsibilities. Such litigation or proceedings could substantially increase our operating losses and reduce the resources available for development activities or any future sales, marketing or distribution activities. We may not have sufficient financial or other resources to conduct such litigation or proceedings adequately. Some of our competitors may be able to sustain the costs of such litigation or proceedings more effectively than we can because of their greater financial resources. Uncertainties resulting from the initiation and continuation of patent litigation or other proceedings could compromise our ability to compete in the marketplace.

Furthermore, because of the substantial amount of discovery required in connection with intellectual property litigation, there is a risk that some of our confidential information could be compromised by disclosure during this type of litigation. There could also be public announcements of the results of hearings, motions or other interim proceedings or developments. If securities analysts or investors perceive these results to be negative, it could have a material adverse effect on the price of our common stock.

Our ability to enforce our patent rights depends on our ability to detect infringement. It may be difficult to detect infringers who do not advertise the components or methods that are used in connection with their drug candidates. Moreover, it may be difficult or impossible to obtain evidence of infringement in a competitor's or potential competitor's drug candidate. 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, including those of our licensor's, 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 our drug candidates are invalidated or found unenforceable, or if a court found that valid, enforceable patents held by third parties covered one or more of our drug candidates, our competitive position could be harmed or we could be required to incur significant expenses to enforce or defend our rights. If we initiate lawsuits to protect or enforce our patents, or litigate against third party claims, such proceedings would be expensive and would divert the attention of our management and technical personnel.

We may infringe the intellectual property rights of others, which may prevent or delay our drug development efforts and stop us from commercializing or increase the costs of commercializing our drug candidates.

Our success will depend in part on our ability to operate without infringing the intellectual property rights of third parties. We cannot guarantee that our drug candidates, or manufacture or use of our drug candidates, will not infringe third-party patents.

Furthermore, a third party may claim that we or our manufacturing or commercialization collaborators are using inventions covered by the third party's patent rights and may go to court to stop us from engaging in our normal operations and activities, including making or selling our drug candidates. These lawsuits are costly and could affect our results of operations and divert the attention of managerial and scientific personnel. There is a risk that a court would decide that we or our commercialization collaborators are infringing the third party's patents and would order us or our collaborators to stop the activities covered by the patents. In that event, we or our commercialization collaborators may not have a viable way around the patent and may need to halt commercialization of the relevant drug candidate. In addition, there is a risk that a court will order us or our collaborators to pay the other party damages for having violated the other party's patents. If we collaborate with third parties in the development of technology in the future, our collaborators may not properly maintain or defend our intellectual property rights or may use our proprietary information in such a way as to invite litigation that could jeopardize or invalidate our intellectual property or proprietary information or expose us to litigation or potential liability. Further, our collaborators may infringe the intellectual property rights of third parties, which may expose us to litigation and potential liability. In the future, we may agree to indemnify our collaborators against certain intellectual property infringement claims brought by third parties. The pharmaceutical and biotechnology industries have produced a proliferation of patents, and it is not always clear to industry participants, including us, which patents cover various types of drug candidates or methods of use. The coverage of patents is subject to interpretation by the courts, and the interpretation is not always uniform.

Any claims of patent infringement asserted by third parties would be time consuming and could:

- result in costly litigation;
- divert the time and attention of our technical personnel and management;
- cause development delays;
- prevent us from out-licensing or commercializing EryDex, or our other drug candidates until the asserted patent expires or is finally held invalid, unenforceable, or not infringed in a court of law;
- require us to develop non-infringing technology, which may not be possible on a cost-effective basis;
- require us to pay damages to the party whose intellectual property rights we may be found to be infringing, which may include treble damages if we are found to have been willfully infringing such intellectual property;
- require us to pay the attorney's fees and costs of litigation to the party whose intellectual property rights we may be found to be infringing; and/or
- require us to enter into royalty or licensing agreements, which may not be available on commercially reasonable terms, or at all.

If we are sued for patent infringement, we would need to demonstrate that our drug candidates or methods either do not infringe the patent claims of the relevant patent or that the patent claims are invalid or unenforceable, and we may not be able to do this. Proving invalidity or unenforceability is difficult.

For example, in the United States, proving invalidity requires a showing of clear and convincing evidence to overcome the presumption of validity enjoyed by issued patents. Even if we are successful in these proceedings, we may incur substantial costs and divert management's 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, which may not be available, defend an infringement action or challenge the validity or enforceability of the patents in court. Patent litigation is costly and time consuming. We may not have sufficient resources to bring these actions to a successful conclusion. In addition, if we do not obtain a license, develop or obtain non-infringing technology, fail to defend an infringement action successfully or have infringed patents declared invalid or unenforceable, we may incur substantial monetary damages, encounter significant delays in bringing our drug candidates to market and be precluded from manufacturing or selling our drug candidates.

We do not routinely conduct independent reviews of pending patent applications of and patents issued to third parties. We cannot be certain that others have not filed patent applications for technology covered by our pending applications, or that we were the first to invent the technology, because:

- some patent applications in the United States may be maintained in secrecy until the patents are issued;
- patent applications in the United States and elsewhere can be pending for many years before issuance, or unintentionally abandoned patents or applications can be revived;

- pending patent applications that have been published can, subject to certain limitations, be later amended in a manner that could cover our technologies, our drug candidates or the use of our drug candidates;
- identification of third-party patent rights that may be relevant to our technology is difficult because patent searching is imperfect due to differences in terminology among patents, incomplete databases and the difficulty in assessing the meaning of patent claims;
- patent applications in the United States are typically not published until 18 months after the priority date; and
- publications in the scientific literature often lag behind actual discoveries.

Furthermore, the scope of a patent claim is determined by an interpretation of the law, the written disclosure in a patent and the patent's prosecution history and can involve other factors such as expert opinion. Our interpretation of the relevance or the scope of claims in a patent or a pending application may be incorrect, which may negatively impact our ability to market our drug candidates. Further, we may incorrectly determine that our technologies, or drug candidates are not covered by a third-party patent or may incorrectly predict whether a third party's pending patent application will issue with claims of relevant scope. Our determination of the expiration date of any patent in the United States or abroad that we consider relevant may be incorrect, which may negatively impact our ability to develop and market our drug candidates.

Our competitors may have filed, and may in the future file, patent applications covering technology similar to ours, and others may have or obtain patents or proprietary rights that could limit our ability to make, use, sell, offer for sale or import our drug candidates and future approved products or impair our competitive position. Numerous third-party U.S. and foreign issued patents and pending patent applications exist in the fields in which we are developing drug candidates. There may be third-party patents or patent applications with claims to compositions, formulations, methods of manufacture or methods for treatment related to the use or manufacture of our drug candidates. Any such patent application may have priority over our patent applications, which could further require us to obtain rights to issued patents covering such technologies. If another party has filed a U.S. patent application on inventions similar to ours, we may have to participate in an interference proceeding declared by the USPTO to determine priority of invention in the United States. The costs of these proceedings could be substantial, and it is possible that such efforts would be unsuccessful if, unbeknownst to us, the other party had independently arrived at the same or similar inventions prior to our own inventions, resulting in a loss of our U.S. patent position with respect to such inventions. Other countries have similar laws that permit secrecy of patent applications, and may be entitled to priority over our applications in such jurisdictions.

Some of our competitors may be able to sustain the costs of complex patent litigation more effectively than we can because they have substantially greater resources. In addition, any uncertainties resulting from the initiation and continuation of any litigation could have a material adverse effect on our ability to raise the funds necessary to continue our operations.

We may not identify relevant third-party patents or may incorrectly interpret the relevance, scope or expiration of a third-party patent, which might adversely affect our ability to develop and market our products.

As the biotechnology industry expands and more patents are issued, the risk increases that our drug candidates may be subject to claims of infringement of the patent rights of third parties. There can be no assurance that our operations do not, or will not in the future, infringe existing or future third-party patents. Identification of third-party patent rights that may be relevant to our operations is difficult because patent searching is imperfect due to differences in terminology among patents, incomplete databases and the difficulty in assessing the meaning of patent claims. We cannot guarantee that any of our patent searches or analyses, including the identification of relevant patents, the scope of patent claims or the expiration of relevant patents, are complete or thorough, nor can we be certain that we have identified each and every third-party patent and pending application in the United States and abroad that is relevant to or necessary for the commercialization of our drug candidates in any jurisdiction.

Numerous U.S. and foreign patents and pending patent applications exist in our market that are owned by third parties. Our competitors in both the United States and abroad, many of which have substantially greater resources and have made substantial investments in patent portfolios and competing technologies, may have applied for or obtained or may in the future apply for and obtain, patents that will prevent, limit or otherwise interfere with our ability to make, use and sell our products. We do not always conduct independent reviews of pending patent applications of and patents issued to third parties. Patent applications in the United States and elsewhere are typically published approximately 18 months after the earliest filing for which priority is claimed, with such earliest filing date being commonly referred to as the priority date. Certain U.S. applications that will not be filed outside the U.S. can remain confidential until patents issue. In addition, patent applications in the United States and elsewhere can be pending for many years before issuance, or unintentionally abandoned patents or applications can be revived. Furthermore, pending patent applications

that have been published can, subject to certain limitations, be later amended in a manner that could cover our technologies, our products or the use of our products. As such, there may be applications of others now pending or recently revived patents of which we are unaware. These patent applications may later result in issued patents, or the revival of previously abandoned patents, that will prevent, limit or otherwise interfere with our ability to make, use or sell our products.

The scope of a patent claim is determined by an interpretation of the law, the written disclosure in a patent and the patent's prosecution history. Our interpretation of the relevance or the scope of a patent or a pending application may be incorrect. For example, we may incorrectly determine that our products are not covered by a third-party patent or may incorrectly predict whether a third-party's pending application will issue with claims of relevant scope. Our determination of the expiration date of any patent in the United States or abroad that we consider relevant may be incorrect. Our failure to identify and correctly interpret relevant patents may negatively impact our ability to develop and market our products.

We cannot provide any assurances that third-party patents do not exist which might be enforced against our current technology, including our research programs, drug candidates, their respective methods of use, manufacture and formulations thereof, and could result in either an injunction prohibiting our manufacture or future sales, or, with respect to our future sales, an obligation on our part to pay royalties and/or other forms of compensation to third parties, which could be significant.

Obtaining and maintaining our patent protection depends on compliance with various procedural, document submission, fee payment and other requirements imposed by governmental patent agencies, and our patent protection could be reduced or eliminated for non-compliance with these requirements.

Periodic maintenance fees, renewal fees, annuity fees and various other governmental fees on any issued patents and/or pending applications are due to be paid to the USPTO and various governmental patent agencies outside of the United States in several stages over the lifetime of the patents and/or applications. We have systems in place to remind us to pay these fees, and we employ an outside firm to pay these fees due to the USPTO and non-U.S. patent agencies. The USPTO and various non-U.S. governmental patent agencies require compliance with a number of procedural, documentary, fee payment and other similar provisions during the patent application process. We employ reputable law firms 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. If we license intellectual property, we may have to rely upon our licensors to comply with these requirements and effect payment of these fees with respect to any patents and patent applications that we license. 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. In such an event, our competitors might be able to enter the market and this circumstance would have a material adverse effect on our business.

We may be subject to claims that we have wrongfully hired an employee from a competitor or that we or our employees have wrongfully used or disclosed alleged confidential information or trade secrets of their former employers.

We may be subject to claims that our employees have wrongfully used or disclosed alleged trade secrets of their former employers. If we are not able to adequately prevent disclosure of trade secrets and other proprietary information, the value of our technology and drug candidate could be significantly diminished.

As is common in the biotechnology and pharmaceutical industries, we employ individuals who were previously employed at other biotechnology or pharmaceutical companies, including our competitors or potential competitors. We may be subject to claims that these employees, or we, have inadvertently or otherwise used or disclosed trade secrets or other proprietary information of their former employers. 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. In addition, we may face claims by third parties that our agreements with employees, contractors or consultants obligating them to assign intellectual property to us are ineffective or in conflict with prior or competing contractual obligations of assignment, which could result in ownership disputes regarding intellectual property we have developed or will develop and interfere with our ability to capture the commercial value of such intellectual property. Litigation may be necessary to defend against these claims. If we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights, which could adversely affect our business. Even if we are successful in defending against these claims, litigation could result in substantial costs and be a distraction to management.

If we are unable to protect the confidentiality of our trade secrets, our business and competitive position would be harmed. Our reliance on third parties requires us to share our trade secrets, which increases the possibility that a competitor will discover them or that our trade secrets will be misappropriated or disclosed.

We rely on trade secrets to protect our proprietary technologies, especially where we do not believe patent protection is appropriate or obtainable. However, trade secrets are difficult to protect. We rely in part on confidentiality agreements with our

employees, consultants, outside scientific collaborators, sponsored researchers and other advisors, and invention assignment agreements with employees, consultants and advisors, to protect our trade secrets and other proprietary information. In addition to contractual measures, we try to protect the confidential nature of our proprietary information using commonly accepted physical and technological security measures. Despite these efforts, we cannot provide any assurances that all such agreements have been duly executed, and these agreements may not effectively prevent disclosure of confidential information and may not provide an adequate remedy in the event of unauthorized disclosure of confidential information. In addition, others may independently discover our trade secrets and proprietary information. For example, the FDA, as part of its Transparency Initiative, is currently considering whether to make additional information publicly available on a routine basis, including information that we may consider to be trade secrets or other proprietary information, and it is not clear at the present time how the FDA's disclosure policies may change in the future, if at all. Costly and time-consuming litigation could be necessary to enforce and determine the scope of our proprietary rights, and failure to obtain or maintain trade secret protection could adversely affect our competitive business position.

In addition, such security measures may not provide adequate protection for our proprietary information, for example, in the case of misappropriation of a trade secret by an employee, consultant, customer or third party with authorized access. Our security measures may not prevent an employee, consultant or customer from misappropriating our trade secrets and providing them to a competitor, and any recourse we take against such misconduct may not provide an adequate remedy to protect our interests fully. Monitoring unauthorized uses and disclosures is difficult, and we do not know whether the steps we have taken to protect our proprietary technologies will be effective. Unauthorized parties may also attempt to copy or reverse engineer certain aspects of our drug candidates that we consider proprietary. Enforcing a claim that a party illegally disclosed or misappropriated a trade secret can be difficult, expensive and time-consuming, and the outcome is unpredictable. Even though we use commonly accepted security measures, the criteria for protection of trade secrets can vary among different jurisdictions.

Enforcing a claim that a party illegally disclosed or misappropriated a trade secret is difficult, expensive and time-consuming, and the outcome is unpredictable. In addition, some courts inside and outside the United States are less willing or unwilling to protect trade secrets. Moreover, third parties may still obtain this information or may come upon this or similar information independently, and we would have no right to prevent them from using that technology or information to compete with us. Trade secrets could over time be disseminated within the industry through independent development, the publication of journal articles and the movement of personnel skilled in the art from company to company or academic to industry scientific positions.

Though our agreements with third parties typically restrict the ability of our advisors, employees, collaborators, licensors, suppliers, third-party contractors and consultants to publish data potentially relating to our trade secrets, our agreements may contain certain limited publication rights. If any of our trade secrets were to be lawfully obtained or independently developed by a competitor, we would have no right to prevent such competitor from using that technology or information to compete with us, which could harm our competitive position. Because from time to time we expect to rely on third parties in the development, manufacture, and distribution of our drug candidates and provision of our services, we must, at times, share trade secrets with them. Despite employing the contractual and other security precautions described above, the need to share trade secrets increases the risk that such trade secrets become known by our competitors, are inadvertently incorporated into the technology of others, or are disclosed or used in violation of these agreements. If any of these events occurs or if we otherwise lose protection for our trade secrets, the value of this information may be greatly reduced and our competitive position would be harmed.

In the future, we may need to obtain licenses of third-party technology that may not be available to us or are available only on commercially unreasonable terms, and which may cause us to operate our business in a more costly or otherwise adverse manner that was not anticipated.

From time to time we may be required to license technology from third parties to further develop or commercialize our drug candidates. Should we be required to obtain licenses to any third-party technology, including any such patents required to manufacture, use or sell our drug candidates, such licenses may not be available to us on commercially reasonable terms, or at all. The inability to obtain any third-party license required to develop or commercialize any of our drug candidates could cause us to abandon any related efforts, which could seriously harm our business and operations.

Where we obtain licenses from or collaborate with third parties, in some circumstances, we may not have the right to control the preparation, filing and prosecution of patent applications, or to maintain the patents, covering technology that we license from third parties, or such activities, if controlled by us, may require the input of such third parties. We may also require the cooperation of our licensors and collaborators to enforce any licensed patent rights, and such cooperation may not be provided. Therefore, these patents and applications may not be prosecuted and enforced in a manner consistent with the best interests of our business, in compliance with applicable laws and regulations, which may affect the validity and enforceability of such patents or any patents that may issue from such applications. Moreover, if we do obtain necessary licenses, we will likely have obligations under those licenses, including making royalty and milestone payments, and any failure to satisfy those obligations could give our licensor the right to terminate the license.

Termination of a necessary license, or expiration of licensed patents or patent applications, could have a material adverse impact on our business. Our business would suffer if any such licenses terminate, if the licensors fail to abide by the terms of the license, if the licensors fail to enforce licensed patents against infringing third parties, if the licensed patents or other rights are found to be invalid or unenforceable, or if we are unable to enter into necessary licenses on acceptable terms. Furthermore, if any exclusive licenses terminate, or if the underlying patents fail to provide the intended exclusivity, competitors or other third parties may gain the freedom to seek regulatory approval of, and to market, drug candidates identical to ours. Moreover, our licensors may own or control intellectual property that has not been licensed to us and, as a result, we may be subject to claims, regardless of their merit, that we are infringing or otherwise violating the licensor's rights. In addition, while we cannot currently determine the amount of the royalty obligations, we would be required to pay on sales of future drug candidates, if any, the amounts may be significant. The amount of our future royalty obligations will likely depend on the technology and intellectual property we use in drug candidates that we successfully develop and commercialize, if any. Therefore, even if we successfully develop and commercialize drug candidates, we may be unable to achieve or maintain profitability.

Intellectual property rights do not necessarily address all potential threats to our competitive advantage.

The degree of future protection afforded by our intellectual property rights is uncertain because intellectual property rights have limitations and may not adequately protect our business or permit us to maintain our competitive advantage. For example:

- others may be able to make drug candidates that are similar to ours but that are not covered by the claims of the patents that we own;
- we or future collaborators might not have been the first to make the inventions covered by the issued patent or pending patent application that we own or have exclusively licensed;
- we or future collaborators might not have been the first to file patent applications covering certain of our inventions;
- others may independently develop similar or alternative technologies or duplicate any of our technologies without infringing our intellectual property rights;
- it is possible that our pending patent applications will not lead to issued patents;
- issued patents that we own or have exclusively licensed may be held invalid or unenforceable, as a result of legal challenges by our competitors;
- our competitors might conduct research and development activities in countries where we do not have patent rights and then use the information learned from such activities to develop competitive drug candidates for sale in our major commercial markets;
- we cannot ensure that any of our patents, or any of our pending patent applications, if issued, or those of our licensors, will include claims having a scope sufficient to protect our drug candidates;
- we cannot ensure that any patents issued to us or our licensors will provide a basis for an exclusive market for our commercially viable drug candidates or will provide us with any competitive advantages;
- we cannot ensure that our commercial activities or drug candidates will not infringe upon the patents of others;
- we cannot ensure that we will be able to successfully commercialize our drug candidates on a substantial scale, if approved, before the relevant patents that we own or license expire;
- we may not develop additional proprietary technologies that are patentable; and
- the patents of others may have an adverse effect on our business, including if others obtain patents claiming subject matter similar to or improving that covered by our patents and patent applications;

Should any of these events occur, they would significantly harm our business, results of operations and prospects.

Because of the expense and uncertainty of litigation, we may not be in a position to enforce our intellectual property rights against third parties.

Because of the expense and uncertainty of litigation, we may conclude that even if a third party is infringing our issued patent, any patents that may be issued as a result of our pending or future patent applications or other intellectual property rights, the risk-adjusted cost of bringing and enforcing such a claim or action may be too high or not in the best interest of our company or our

stockholders. Our competitors or other third parties may be able to sustain the costs of complex patent litigation or proceedings more effectively than we can because of their greater financial resources and more mature and developed intellectual property portfolios. In such cases, we may decide that the more prudent course of action is to simply monitor the situation or initiate or seek some other non-litigious action or solution. In addition, the uncertainties associated with litigation could compromise our ability to raise the funds necessary to continue our clinical trials, continue our internal research programs, in-license needed technology or other drug candidates, or enter into development partnerships that would help us bring our drug candidates to market. In such cases, we may decide that the more prudent course of action is to simply monitor the situation or initiate or seek some other non-litigious action or solution.

We may not be able to protect our intellectual property rights throughout the world.

Patents are of national or regional effect, and filing, prosecuting and defending patents on all of our drug candidates throughout the world would be prohibitively expensive, and our intellectual property rights in some countries outside the United States can be less extensive than those in the United States. As such, 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 drug candidates made using our inventions in and into the United States or other jurisdictions. Further, 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 pharmaceuticals, which could make it difficult for us to stop the infringement of our patents or marketing of competing drug candidates in violation of our proprietary rights generally. In addition, certain developing countries, including China and India, have compulsory licensing laws under which a patent owner may be compelled to grant licenses to third parties. In addition, some countries limit the enforceability of patents against third parties, including government agencies or government contractors. In these countries, patents may provide limited or no benefit, and in those countries, we and our licensors and licensees may have limited remedies if patents are infringed or if we or our licensors or licensees are compelled to grant a license to a third party, which could diminish the value of those patents. This could limit our potential revenue opportunities. Further, competitors may use our technologies in jurisdictions where we have not obtained patent protection to develop their own drug candidates and, further, may export otherwise infringing drug candidates to territories where we have patent protection but where enforcement is not as strong as that in the United States. These drug candidates may compete with our drug candidates, and our patents or other intellectual property rights may not be effective or sufficient to prevent them from competing. Proceedings to enforce our patent rights in foreign jurisdictions could result in substantial costs and divert our efforts and attention from other aspects of our business, could put our patents at risk of being invalidated or interpreted narrowly and our patent applications at risk of not issuing and could provoke third parties to assert claims against us. We may not prevail in any lawsuits that we initiate, and the damages or other remedies awarded, if any, may not be commercially meaningful. Accordingly, our efforts to enforce our intellectual property rights around the world may be inadequate to obtain a significant commercial advantage from the intellectual property that we develop or license.

In Europe, beginning June 1, 2023, European applications and patent may be subjected to the jurisdiction of the Unified Patent Court (the "UPC"). Also, European applications will have the option, upon grant of a patent, of becoming a Unitary Patent which will be subject to the jurisdiction of the UPC. This will be a significant change in European patent practice. As the UPC is a new court system, there is no precedent for the court, increasing the uncertainty. As a single court system can invalidate a European patent, we, where applicable may opt out of the UPC and as such, each European patent would need to be challenged in each individual country.

Geo-political actions in the United States and in foreign countries could increase the uncertainties and costs surrounding the prosecution or maintenance of our patent applications or those of any current or future licensors and the maintenance, enforcement or defense of our issued patents or those of any current or future licensors.

Changes in patent law in the United States and other jurisdictions could diminish the value of patents in general, thereby impairing our ability to protect our drug candidates.

As is the case with other biotechnology companies, our success is heavily dependent on intellectual property, particularly patents. Our patent rights may be affected by developments or uncertainty in U.S. or non-U.S. patent statutes, patent case laws in USPTO rules and regulations or in the rules and regulations of non-U.S. patent offices.

Obtaining and enforcing patents in the pharmaceutical industry involves both technological and legal complexity and is therefore costly, time consuming and inherently uncertain. Changes in either the patent laws or interpretation of the patent laws in the United States could increase the uncertainties and costs. Recent patent reform legislation in the United States and other countries, including the AIA, signed into law on September 16, 2011, could increase those uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents.

The AIA includes a number of significant changes to U.S. patent law. These include provisions that affect the way patent applications are prosecuted, redefine prior art and provide more efficient and cost-effective avenues for competitors to challenge the validity of patents. These include allowing third-party submission of prior art to the USPTO during patent prosecution and additional procedures to attack the validity of a patent by USPTO administered post-grant proceedings, including post-grant review, inter parties review, and derivation proceedings. After March 2013, under the AIA, the United States transitioned to a first inventor to file system in which, assuming that the other statutory requirements are met, the first inventor to file a patent application will be entitled to the patent on an invention regardless of whether a third party was the first to invent the claimed invention. However, the AIA and its implementation could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents, all of which could have a material adverse effect on our business, financial condition, results of operations and prospects. In addition, Congress may pass patent reform legislation that is unfavorable to us.

The U.S. Supreme Court has ruled on several patent cases in recent years, narrowing the scope of patent protection available in certain circumstances and weakening the rights of patent owners in certain situations. Depending on future actions by the U.S. Congress, the U.S. courts, the USPTO 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 become subject to claims challenging the inventorship or ownership of our patents and other intellectual property.

We may be subject to claims that former employees, collaborators or other third parties have an interest in our patents, trade secrets or other intellectual property as an inventor or co-inventor. The failure to name the proper inventors on a patent application can result in the patents issuing thereon being unenforceable. Inventorship disputes may arise from conflicting views regarding the contributions of different individuals named as inventors, the effects of foreign laws where foreign nationals are involved in the development of the subject matter of the patent, conflicting obligations of third parties involved in developing our drug candidates or as a result of questions regarding co-ownership of potential joint inventions. Litigation may be necessary to resolve these and other claims challenging inventorship and/or ownership. Alternatively, or additionally, we may enter into agreements to clarify the scope of our rights in such intellectual property. If we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights, such as exclusive ownership of, or right to use, valuable intellectual property. Such an outcome could have a material adverse effect on our business. Even if we are successful in defending against such claims, litigation could result in substantial costs and be a distraction to management and other employees.

Our licensors may have relied on third-party consultants or collaborators or on funds from third parties, such as the U.S. government, such that our licensors are not the sole and exclusive owners of the patents we in-licensed. If other third parties have ownership rights or other rights to our in-licensed patents, they may be able to license such patents to our competitors, and our competitors could market competing products and technology. This could have a material adverse effect on our competitive position, business, financial conditions, results of operations, and prospects.

In addition, we may be unsuccessful in executing agreements assigning such intellectual property to us with each party who, in fact, conceives or develops intellectual property that we regard as our own. The assignment of intellectual property rights may not be self-executing, or the assignment agreements may be breached, and we may be forced to bring claims against third parties, or defend claims that they may bring against us, to determine the ownership of what we regard as our intellectual property. Such claims could have a material adverse effect on our business, financial condition, results of operations, and prospects.

Patent terms may be inadequate to protect our competitive position on our drug candidates for an adequate amount of time, and if we do not obtain patent term extension for our drug candidates, our business may be materially harmed.

Patent rights are of limited duration. In the United States, the natural expiration of a patent is generally 20 years after its first effective non-provisional filing date. In addition, although upon issuance a U.S. patent's life can be increased based on certain delays caused by the USPTO, this increase can be reduced or eliminated based on certain delays caused by the patent applicant during patent prosecution. Given the amount of time required for the development, testing and regulatory review of new drug candidates, patents protecting such candidates might expire before or shortly after such drug candidates are commercialized. Even if patents covering our drug candidates are obtained, once the patent life has expired for a drug candidate, we may be open to competition from generic products. A patent term extension of up to five years based on regulatory delay may be available in the United States under the Hatch-Waxman Act. However, only a single patent can be extended for each marketing approval, and any patent can be extended only once, for a single drug candidate. Moreover, the scope of protection during the period of the patent term extension does not extend to the full scope of the claim, but instead only to the scope of the drug candidate as approved. Further, a patent term extension cannot extend the remaining term of a patent beyond a total of 14 years from the date of drug candidate approval and only those claims covering such approved drug candidate, a method for using it or a method for manufacturing it may be extended. Laws governing analogous patent term extensions in foreign jurisdictions vary widely, as do laws governing the ability to obtain multiple patents from a single patent

family. Additionally, we may not receive an extension if we fail to apply within applicable deadlines, fail to apply prior to expiration of relevant patents or otherwise fail to satisfy applicable requirements. Moreover, the applicable time period or the scope of patent protection afforded could be less than we request. If we are unable to obtain patent term extension or restoration, or the term of any such extension is less than we request, the period during which we will have the right to exclusively market our drug candidate will be shortened and our competitors may obtain approval of competing drug candidates following our patent expiration, and our revenue could be reduced.

If our trademarks and trade names are not adequately protected, then we may not be able to build name recognition in our markets of interest and our business may be adversely affected.

Our current or future trademarks or trade names may be challenged, infringed, circumvented or declared generic or determined to be infringing on other marks. We may not be able to protect our rights to these trademarks and trade names, which we need to build name recognition among potential partners or customers in our markets of interest. At times, competitors may adopt trade names or trademarks similar to ours, thereby impeding our ability to build brand identity and possibly leading to market confusion. In addition, there could be potential trade name or trademark infringement claims brought by owners of other trademarks or trademarks that incorporate variations of our registered or unregistered trademarks or trade names. Over the long term, if we are unable to establish name recognition based on our trademarks and trade names, then we may not be able to compete effectively and our business may be adversely affected. We may license our trademarks and trade names to third parties, such as distributors. Though these license agreements may provide guidelines for how our trademarks and trade names may be used, a breach of these agreements or misuse of our trademarks and trade names by our licensees may jeopardize our rights in or diminish the goodwill associated with our trademarks and trade names. Our efforts to enforce or protect our proprietary rights related to trademarks, trade names, trade secrets, domain names, copyrights or other intellectual property may be ineffective and could result in substantial costs and diversion of resources and could adversely affect our financial condition or results of operations.

Moreover, any name we have proposed to use with our drug candidate in the United States must be approved by the FDA, regardless of whether we have registered it, or applied to register it, as a trademark. Similar requirements exist in Europe. The FDA typically conducts a review of proposed drug candidate names, including an evaluation of potential for confusion with other drug candidate names. If the FDA (or an equivalent administrative body in a foreign jurisdiction) objects to any of our proposed proprietary drug candidate names, we may be required to expend significant additional resources in an effort to identify a suitable substitute name that would qualify under applicable trademark laws, not infringe the existing rights of third parties and be acceptable to the FDA. Furthermore, in many countries, owning and maintaining a trademark registration may not provide an adequate defense against a subsequent infringement claim asserted by the owner of a senior trademark. At times, competitors or other third parties may adopt trade names or trademarks similar to ours, thereby impeding our ability to build brand identity and possibly leading to market confusion. In addition, there could be potential trade name or trademark infringement claims brought by owners of other registered trademarks or trademarks that incorporate variations of our registered or unregistered trademarks or trade names. If we assert trademark infringement claims, a court may determine that the marks we have asserted are invalid or unenforceable, or that the party against whom we have asserted trademark infringement has superior rights to the marks in question. In this case, we could ultimately be forced to cease use of such trademarks.

We cannot ensure that patent rights relating to inventions described and claimed in our pending patent applications will issue or that patents based on our patent applications will not be challenged and rendered invalid and/or unenforceable.

The patent application process is subject to numerous risks and uncertainties, and there can be no assurance that we or any of our potential future collaborators will be successful in protecting our drug candidates by obtaining and defending patents. We have pending U.S. and foreign patent applications in our portfolio; however, we cannot predict:

- if and when patents may issue based on our patent applications;
- the scope of protection of any patent issuing based on our patent applications;
- whether the claims of any patent issuing based on our patent applications will provide protection against competitors;
- whether or not third parties will find ways to invalidate or circumvent our patent rights;
- whether or not others will obtain patents claiming aspects similar to those covered by our patents and patent applications;
- whether we will need to initiate litigation or administrative proceedings to enforce and/or defend our patent rights which will be costly whether we win or lose;
- whether the patent applications that we own or in-license will result in issued patents with claims that cover our drug candidates or uses thereof in the United States or in other foreign countries; and/or

- whether we may experience patent office interruption or delays to our ability to timely secure patent coverage to our drug candidates.

We cannot be certain that the claims in our pending patent applications directed to our drug candidates and/or technologies will be considered patentable by the USPTO or by patent offices in foreign countries. There can be no assurance that any such patent applications will issue as granted patents. One aspect of the determination of patentability of our inventions depends on the scope and content of the "prior art," information that was or is deemed available to a person of skill in the relevant art prior to the priority date of the claimed invention. There may be prior art of which we are not aware that may affect the patentability of our patent claims or, if issued, affect the validity or enforceability of a patent claim. Even if the patents do issue based on our patent applications, third parties may challenge the validity, enforceability or scope thereof, which may result in such patents being narrowed, invalidated or held unenforceable. Furthermore, even if they are unchallenged, patents in our portfolio may not adequately exclude third parties from practicing relevant technology or prevent others from designing around our claims. If the breadth or strength of our intellectual property position with respect to our drug candidates is threatened, it could dissuade companies from collaborating with us to develop and threaten our ability to commercialize our drug candidates. In the event of litigation or administrative proceedings, we cannot be certain that the claims in any of our issued patents will be considered valid by courts in the United States or foreign countries.

We may not be successful in obtaining or maintaining necessary rights to our drug candidates through acquisitions and in-licenses.

Because our development programs may in the future require the use of proprietary rights held by third parties, the growth of our business may depend in part on our ability to acquire, in-license, or use these third-party proprietary rights. We may be unable to acquire or in-license any compositions, methods of use, processes or other third-party intellectual property rights from third parties that we identify as necessary for our drug candidates. The licensing and acquisition of third-party intellectual property rights is a competitive area, and a number of more established companies may pursue strategies to license or acquire third-party intellectual property rights that we may consider attractive or necessary. These established companies may have a competitive advantage over us due to their size, capital resources and greater clinical development and commercialization capabilities. In addition, companies that perceive us to be a competitor may be unwilling to assign or license rights to us. We also may be unable to license or acquire third-party intellectual property rights on terms that would allow us to make an appropriate return on our investment or at all. If we are unable to successfully obtain rights to required third-party intellectual property rights or maintain the existing intellectual property rights we have, we may have to abandon development of the relevant program or drug candidate, which could have a material adverse effect on our business, financial condition, results of operations, and prospects.

While we normally seek to obtain the right to control prosecution, maintenance and enforcement of the patents relating to our drug candidates, there may be times when the filing and prosecution activities for patents and patent applications relating to our drug candidates are controlled by our future licensors or collaboration partners. If any of our future licensors or collaboration partners fail to prosecute, maintain and enforce such patents and patent applications in a manner consistent with the best interests of our business, including by payment of all applicable fees for patents covering our drug candidates, we could lose our rights to the intellectual property or our exclusivity with respect to those rights, our ability to develop and commercialize those drug candidates may be adversely affected and we may not be able to prevent competitors from making, using and selling competing products. In addition, even where we have the right to control patent prosecution of patents and patent applications we have licensed to and from third parties, we may still be adversely affected or prejudiced by actions or inactions of our licensees, our future licensors and their counsel that took place prior to the date upon which we assumed control over patent prosecution.

We may enter into license agreements in the future with others to advance our existing or future research or allow commercialization of our existing or future drug candidates. These licenses may not provide exclusive rights to use such intellectual property and technology in all relevant fields of use and in all territories in which we may wish to develop or commercialize our technology and products in the future.

In addition, subject to the terms of any such license agreements, we may not have the right to control the preparation, filing, prosecution, maintenance, enforcement, and defense of patents and patent applications covering the technology that we license from third parties. In such an event, we cannot be certain that these patents and patent applications will be prepared, filed, prosecuted, maintained, enforced, and defended in a manner consistent with the best interests of our business. If our future licensors fail to prosecute, maintain, enforce, and defend such patents or patent applications, or lose rights to those patents or patent applications, the rights we have licensed may be reduced or eliminated, and our right to develop and commercialize any of our future drug candidates that are subject of such licensed rights could be adversely affected.

Our future licensors may rely on third-party consultants or collaborators or on funds from third parties such that our future licensors are not the sole and exclusive owners of the patents we in-license. If other third parties have ownership rights to our future in-licensed patents, they may be able to license such patents to our competitors, and our competitors could market competing products

and technology. This could have a material adverse effect on our competitive position, business, financial conditions, results of operations, and prospects.

It is possible that we may be unable to obtain licenses at a reasonable cost or on reasonable terms, if at all. Even if we are able to obtain a license, it may be non-exclusive, thereby giving our competitors access to the same technologies licensed to us. In that event, we may be required to expend significant time and resources to redesign our technology, drug candidates, or the methods for manufacturing them or to develop or license replacement technology, all of which may not be feasible on a technical or commercial basis. If we are unable to do so, we may be unable to develop or commercialize the affected drug candidates, which could harm our business, financial condition, results of operations, and prospects significantly. We cannot provide any assurances that third-party patents do not exist which might be enforced against our current technology, manufacturing methods, drug candidates, or future methods or products resulting in either an injunction prohibiting our manufacture or future sales, or, with respect to our future sales, an obligation on our part to pay royalties and/or other forms of compensation to third parties, which could be significant.

Disputes may arise between us and our future licensors regarding intellectual property subject to a license agreement, including:

- the scope of rights granted under the license agreement and other interpretation-related issues;
- whether and the extent to which our technology and processes infringe on intellectual property of the licensor that is not subject to the licensing agreement;
- our right to sublicense patents and other rights to third parties;
- our diligence obligations under the license agreement and what activities satisfy those diligence obligations;
- our right to transfer or assign the license;
- the inventorship and ownership of inventions and know-how resulting from the joint creation or use of intellectual property by our future licensors and us and our partners; and
- the priority of invention of patented technology.

In addition, the agreements under which we 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 license in the future prevent or impair our ability to maintain our licensing arrangements on commercially acceptable terms, we may be unable to successfully develop and commercialize the affected drug candidates, which could have a material adverse effect on our business, financial conditions, results of operations, and prospects.

In spite of our best efforts, our future licensors might conclude that we materially breached our license agreements and might therefore terminate the license agreements, thereby removing our ability to develop and commercialize products and technology covered by these license agreements. If these in-licenses are terminated, or if the underlying patents fail to provide the intended exclusivity, competitors would have the freedom to seek regulatory approval of, and to market, products identical to ours. This could have a material adverse effect on our competitive position, business, financial conditions, results of operations, and prospects.

From time to time, we may be required to license technologies relating to our therapeutic research programs from additional third parties to further develop or commercialize our drug candidates. Should we be required to obtain licenses to any third-party technology, including any such patents required to manufacture, use or sell our drug candidates, such licenses may not be available to us on commercially reasonable terms, or at all. The inability to obtain any third-party license required to develop or commercialize any of our drug candidates could cause us to abandon any related efforts, which could seriously harm our business and operations.

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. Collaborations are subject to numerous risks, which may include that:

- collaborators have significant discretion in determining the efforts and resources that they will apply to collaborations;
- collaborators may not pursue development and commercialization of our products or may elect not to continue or renew development or commercialization programs based on trial or test results, changes in their strategic focus due to the acquisition of competitive products, availability of funding or other external factors, such as a business combination that diverts resources or creates competing priorities;

- collaborators could independently develop, or develop with third parties, products that compete directly or indirectly with our products or drug candidates;
- a collaborator with marketing, manufacturing and distribution rights to one or more products may not commit sufficient resources to or otherwise not perform satisfactorily in carrying out these activities;
- we could grant exclusive rights to our collaborators that would prevent us from collaborating with others;
- collaborators may not properly maintain or defend our intellectual property rights or may use our intellectual property or proprietary information in a way that gives rise to actual or threatened litigation that could jeopardize or invalidate our intellectual property or proprietary information or expose us to potential liability;
- disputes may arise between us and a collaborator that causes the delay or termination of the research, development or commercialization of our future drug candidates or that results in costly litigation or arbitration that diverts management attention and resources;
- collaborations may be terminated, and, if terminated, may result in a need for additional capital to pursue further development or commercialization of the applicable current and future drug candidates;
- collaborators may own or co-own intellectual property covering our products that results from our collaborating with them, and in such cases, we would not have the exclusive right to develop or commercialize such intellectual property; and
- a collaborator's sales and marketing activities or other operations may not be in compliance with applicable laws resulting in civil or criminal proceedings.

We may encounter difficulties in managing our growth and expanding our operations successfully.

As we seek to advance our product candidates through clinical trials and, if approved, through commercialization, we will need to expand our development, regulatory, quality assurance, manufacturing, commercialization, compliance, and administration capabilities or contract with third parties to provide these capabilities for us. As our operations expand, we expect that we will need to increase the responsibilities on members of management and manage any future growth effectively. Our failure to effectively manage our growth in this regard could prevent us from successfully implementing our strategy and maintaining the confidence of investors in our company.

Our stockholders may realize little or no value from the divestiture of our legacy assets, and as a result our stock price may decline, we could be subject to litigation, and our business may be adversely affected.

We have sold our legacy small molecule protease inhibitor portfolio to Lighthouse, which is a newly organized, private development stage company in the start-up phase, and has only recently commenced its operations. There is currently no existing public market for the shares of Lighthouse's common stock, and there can be no assurance that an active public market will ever develop. The absence of an active public market for these securities would make it difficult for us to sell the shares of Lighthouse's common stock and realize any value from them. To date, Lighthouse's operations have been primarily limited to organizing and staffing its company and completing the acquisition of our legacy assets. Accordingly, it is difficult if not impossible to predict Lighthouse's future performance or to evaluate its business and prospects, or ability to develop our legacy assets. For these and other reasons, our stockholders may realize little or no value from the divestiture of our legacy assets.

The divestiture of our legacy assets or previously announced change in our corporate strategy, including the termination of the license for NOV004, could result in litigation against us, including litigation arising from or related to the value, received in the sale of our legacy assets to Lighthouse. For example, some of our investors purchased shares of our common stock because they were interested in the opportunities presented by our small molecule protease inhibitor portfolio, others because they were interested in our bone-targeting drug platform. Thus, certain stockholders may have attributed substantial financial value to our legacy assets or NOV004. If our stockholders believe that the financial value which is or may be received by us or them from the divestiture of our assets is inadequate, our stock price may decline and litigation may occur. As a result of these and other factors, we may be exposed to a number of risks, including declines or fluctuations in our stock price, additional legal fees, and distractions to our management caused by activities undertaken in connection with resolving any disputes related to these transactions. The occurrence of any one or more of the above could have an adverse impact on our business and financial condition.

Risks Relating to Owning Our Common Stock

The market price of our common stock is likely to be volatile and could fluctuate or decline, resulting in a substantial loss of your investment.

The market price of our common stock has been and may continue to be volatile and could be subject to wide fluctuations in response to many risk factors listed in this section, and others beyond our control, including:

- our integration efforts related to our acquisition of EryDel;
- changes in our business strategy;
- timing and results of clinical trials;
- our ability to identify partnership and licensing opportunities to support the future development of EryDex;
- market opportunity for A-T and future indications;
- any delays in manufacturing of drug supplies, results of preclinical studies and clinical trials for drug candidates;
- regulatory actions with respect to our drug candidates or our competitors' drug candidates;
- actual or anticipated fluctuations in our financial condition and operating results, including fluctuations in our quarterly and annual results;
- announcement of actual or anticipated reduction in force, including our recent reduction in force;
- announcements of technological innovations by us or our competitors;
- overall conditions in our industry and the markets in which we operate;
- addition or loss of significant customers, or other developments with respect to significant customers;
- changes in laws or regulations applicable to our drug candidates;
- actual or anticipated changes in our growth rate relative to our competitors;
- announcements by us or our competitors of significant acquisitions, strategic partnerships, joint ventures or capital commitments;
- additions or departures of key personnel;
- competition from existing drug candidates or new drug candidates that may emerge;
- issuance of new or updated research or reports by securities analysts;
- cash runway expectations;
- fluctuations in the valuation of companies perceived by investors to be comparable to us;
- disputes or other developments related to proprietary rights, including patents, litigation matters, and our ability to obtain intellectual property protection for our technologies;
- announcement or expectation of additional financing efforts;
- sales of our common stock by us or our stockholders;
- share price and volume fluctuations attributable to inconsistent trading volume levels of our shares;
- market conditions for pharmaceutical stocks in general;
- the expiration of contractual lock-up agreements with our executive officers, directors and stockholders;
- general economic and market conditions; and
- ineffectiveness of our disclosure controls or internal controls.

Furthermore, the stock markets have experienced price and volume fluctuations that have affected and continue to affect the market prices of equity securities of many companies. These fluctuations often have been unrelated or disproportionate to the operating performance of those companies. These broad market and industry fluctuations, as well as general economic, political and

market conditions such as recessions, interest rate changes or international currency fluctuations, may negatively impact the market price of our common stock. In the past, stockholders have instituted securities class action litigation following periods of market volatility. If we were to become involved in securities litigation, it could subject us to substantial costs and divert our management's attention from other business concerns, which could seriously harm our business.

We may be subject to securities class action and stockholder derivative actions. These, and potential similar or related litigation, could result in substantial damages and may divert management's time and attention from our business and adversely impact our business, results of operations and financial condition.

We may become the target of securities class actions or stockholder derivative claims. Securities-related class action litigation has often been brought against companies, including many biotechnology companies, which experience volatility in the market price of their securities. This risk is especially relevant for us because biotechnology companies often experience significant stock price volatility in connection with their product development programs. Any preclinical or clinical trial results that the investors may deem as unfavorable, volatility in our stock price and other matters affecting our business and operations may subject us to actual and threatened securities class actions or stockholder derivative claims. In addition, we may be exposed to increased litigation from stockholders, customers, suppliers, consumers and other third parties due to the combination of EryDel's and Novosteo's business and ours following the EryDel and Novosteo Acquisitions, out-licensing of our legacy assets and NOV004. These types of proceedings may result in substantial costs, divert management's attention from other business concerns and adversely impact our business, results of operations and financial condition.

Future sales of our common stock in the public market could cause our share price to fall.

Sales of a substantial number of shares of our common stock in the public market, or the perception that these sales might occur, could depress the market price of our common stock and could impair our ability to raise capital through the sale of additional equity securities. Certain holders of our common stock have rights, subject to conditions, to require us to file registration statements covering their shares or to include their shares in Securities Act registration statements that we may file for ourselves or other stockholders. Once we register these shares, they can be freely sold in the public market. Moreover, we have also registered under the Securities Act shares of common stock that we may issue under our equity compensation plans.

In addition, the issuance of shares under awards granted under existing or future employee equity benefit plans may cause immediate and substantial dilution to our existing stockholders. In the future, we may issue additional shares of common stock or other equity or debt securities convertible into common stock in connection with a financing, acquisition, litigation settlement, employee arrangements or otherwise. Any such issuance could result in substantial dilution to our existing stockholders and could cause our stock price to decline.

We have in the past and may in the future fail to continue to meet the listing standards of Nasdaq, and as a result our common stock may be delisted, which could have a material adverse effect on the liquidity of our common stock.

Our common stock currently trades on the Nasdaq. The Nasdaq has requirements that a company must meet in order to remain listed on Nasdaq. For example, Nasdaq rules require us to maintain a minimum closing bid price of $1.00 per share of our common stock.

On December 4, 2023, we received a letter from the Listing Qualifications Staff, or the "Nasdaq Staff" of Nasdaq notifying us that for the last 30 consecutive business days, the bid price of our common stock had closed below $1.00 per share, the minimum closing bid price required by the continued listing requirements of Nasdaq Listing Rule 5450(a)(1). The notification received had no immediate effect on the listing of our common stock on the Nasdaq. In accordance with Nasdaq Listing Rule 5810(c)(3)(A), we had 180 calendar days to regain compliance with the minimum bid price requirement by having shares of our common stock maintain a minimum closing bid price of at least $1.00 per share for a minimum of 10 consecutive trading days. On December 29, 2023, we received a letter from the Nasdaq Staff notifying us that the closing bid price of our common stock had been at $1.00 per share or greater for 10 consecutive business days, from December 11, 2023 to December 28, 2023, and accordingly, we had regained compliance with Nasdaq Listing Rule 5450(a)(1). There can be no assurance that we will continue to meet the minimum bid price requirement, or any other Nasdaq requirements, in the future.

In addition, we may be unable to meet other applicable Nasdaq listing requirements, including maintaining minimum levels of stockholders' equity or market values of our common stock, in which case our common stock could be delisted. If our common stock were to be delisted, the liquidity of our common stock would be adversely affected, and the market price of our common stock could decrease.

We have never paid dividends on our common stock and we do not intend to pay dividends for the foreseeable future. Consequently, any gains from an investment in our common stock will likely depend on whether the price of our common stock increases.

We have never declared or paid any dividends on our common stock and do not intend to pay any dividends in the foreseeable future. We anticipate that we will retain all of our future earnings for use in the operation of our business and for general corporate purposes. Any determination to pay dividends in the future will be at the discretion of our board of directors. Accordingly, investors must rely on sales of their common stock after price appreciation, which may never occur, as the only way to realize any future gains on their investments.

General Risk Factors

Our charter documents and Delaware law could prevent a takeover that stockholders consider favorable and could also reduce the market price of our stock.

Our amended and restated certificate of incorporation and our amended and restated bylaws contain provisions that could delay or prevent a change in control of our company. These provisions could also make it more difficult for stockholders to elect directors and take other corporate actions. These provisions include:

- providing for a classified board of directors with staggered, three-year terms;
- authorizing our board of directors to issue preferred stock with voting or other rights or preferences that could discourage a takeover attempt or delay changes in control;
- prohibiting cumulative voting in the election of directors;
- providing that vacancies on our board of directors may be filled only by a majority of directors then in office, even though less than a quorum;
- prohibiting the adoption, amendment or repeal of our amended and restated bylaws or the repeal of the provisions of our amended and restated certificate of incorporation regarding the election and removal of directors without the required approval of at least 66.67% of the shares entitled to vote at an election of directors;
- prohibiting stockholder action by written consent;
- limiting the persons who may call special meetings of stockholders; and
- requiring advance notification of stockholder nominations and proposals.

These provisions may frustrate or prevent any attempts by our stockholders to replace or remove our current management by making it more difficult for stockholders to replace members of our board of directors, which is responsible for appointing the members of our management. In addition, the provisions of Section 203 of the Delaware General Corporate Law, or the DGCL, govern us. These provisions may prohibit large stockholders, in particular those owning 15% or more of our outstanding voting stock, from merging or combining with us for a certain period of time without the consent of our board of directors.

In addition, in April 2023, we implemented the Rights Agreement, also called a "poison pill," that may have the effect of discouraging or preventing a change of control by, among other things, making it uneconomical for a third party to gain control of us through open market accumulation of shares without paying all stockholders an appropriate control premium or without the consent of our board of directors. The Rights will expire on April 5, 2024, unless the Rights are earlier redeemed or exchanged by the Company.

These and other provisions in our amended and restated certificate of incorporation and our amended and restated bylaws and under Delaware law could discourage potential takeover attempts, reduce the price investors might be willing to pay in the future for shares of our common stock and result in the market price of our common stock being lower than it would be without these provisions.

Our amended and restated certificate of incorporation provides that the Court of Chancery of the State of Delaware is the sole and exclusive forum for substantially all disputes between us and our stockholders, which could limit our stockholders' abilities to obtain a favorable judicial forum for disputes with us or our directors, officers or employees.

Our amended and restated certificate of incorporation provides that, unless we consent to the selection of an alternative forum, to the fullest extent permitted by law, the Court of Chancery of the State of Delaware shall be the sole and exclusive forum for:

- any derivative action or proceeding brought on our behalf;

- any action asserting a claim of breach of a fiduciary duty owed by, or other wrongdoing by, any of our directors, officers, employees or agents or our stockholders;
- any action asserting a claim against us arising under the DGCL, our amended and restated certificate of incorporation, or our amended and restated bylaws; and
- any action asserting a claim against us that is governed by the internal-affairs doctrine;

provided that, the exclusive forum provision will not apply to suits brought to enforce any liability or duty created by the Exchange Act or any other claim for which the federal courts have exclusive jurisdiction; and provided further that, if and only if the Court of Chancery of the State of Delaware dismisses any such action for lack of subject matter jurisdiction, such action may be brought in another state or federal court sitting in the State of Delaware. Our amended and restated certificate of incorporation also provides that the federal district courts of the United States of America will be the exclusive forum for the resolution of any complaint asserting a cause of action against us or any of our directors, officers, employees or agents and arising under the Securities Act.

We believe these provisions may benefit us by providing increased consistency in the application of Delaware law and federal securities laws by chancellors and judges, as applicable, particularly experienced in resolving corporate disputes, efficient administration of cases on a more expedited schedule relative to other forums and protection against the burdens of multi-forum litigation. However, these provisions may limit a stockholder's ability to bring a claim in a judicial forum that it finds favorable for disputes with us or our directors, officers, or other employees. While the Delaware Supreme Court recently determined that such choice of forum provisions are facially valid, a stockholder may nevertheless seek to bring such a claim arising under the Securities Act against us, our directors, officers, or other employees in a venue other than in the federal district courts of the United States of America. In such instance, we would expect to vigorously assert the validity and enforceability of the exclusive forum provisions of our amended and restated certificate of incorporation, and this may require significant additional costs associated with resolving such action in other jurisdictions.

Claims for indemnification by our directors and officers may reduce our available funds to satisfy successful third-party claims against us and may reduce the amount of money available to us.

Our amended and restated certificate of incorporation and amended and restated bylaws provide that we will indemnify our directors and officers, in each case to the fullest extent permitted by Delaware law.

In addition, as permitted by Section 145 of the DGCL, our amended and restated bylaws and our indemnification agreements that we have entered into with our directors and officers provide that:

- we will indemnify our directors and officers for serving us in those capacities or for serving other business enterprises at our request, to the fullest extent permitted by Delaware law. Delaware law provides that a corporation may indemnify such person if such person acted in good faith and in a manner such person reasonably believed to be in or not opposed to the best interests of the registrant and, with respect to any criminal proceeding, had no reasonable cause to believe such person's conduct was unlawful;
- we may, in our discretion, indemnify employees and agents in those circumstances where indemnification is permitted by applicable law;
- we are required to advance expenses, as incurred, to our directors and officers in connection with defending a proceeding, except that such directors or officers shall undertake to repay such advances if it is ultimately determined that such person is not entitled to indemnification;
- we will not be obligated pursuant to our amended and restated bylaws to indemnify a person with respect to proceedings initiated by that person against us or our other indemnitees, except with respect to proceedings authorized by our board of directors or brought to enforce a right to indemnification;
- the rights conferred in our amended and restated bylaws are not exclusive, and we are authorized to enter into indemnification agreements with our directors, officers, employees and agents and to obtain insurance to indemnify such persons; and
- we may not retroactively amend our amended and restated bylaw provisions to reduce our indemnification obligations to directors, officers, employees and agents.

Our internal computer systems, or those used by our third-party research institution collaborators, CROs or other contractors or consultants, may fail or suffer security breaches, which could result in adverse consequences including, but not limited to, regulatory investigations or actions, litigation, fines/penalties, disruptions of our business operations, reputational harm, and loss of revenue or profits.

In the ordinary course of our business, we and the third parties upon which we rely process sensitive data, and, as a result, we and the third parties upon which we rely face a variety of evolving threats that could cause security incidents. Such threats are prevalent and continue to rise, are increasingly difficult to detect, and come from a variety of sources, including traditional computer "hackers," "hacktivists," individual threat actors, organized criminal threat actors, personnel (such as through theft or misuse), sophisticated nation states, and nation-state-supported actors.

Despite the implementation of security measures designed to detect and mitigate vulnerabilities, our internal computer systems and those of our CROs and other contractors and consultants may be vulnerable to damage from sources including, but not limited to, malicious code (*e.g.*, computer viruses), malware, ransomware attacks, software or hardware failures, telecommunications failures, and unauthorized access (including as a result of personnel misconduct or error). In particular, severe ransomware attacks are becoming increasingly prevalent and can lead to significant interruptions in our operations, ability to provide our products or services, loss of sensitive data and income, reputational harm, and diversion of funds. Additionally, remote work has become more common and has increased risks to our information technology systems and data, as more of our employees utilize network connections, computers, and devices outside our premises or network.

Although to our knowledge we have not experienced any such material system failure or security breach to date, if such an event were to occur and cause interruptions in our operations, it could result in a material disruption of our development programs and our business operations, as well as adverse consequences including, but not limited to, investigations, fines/penalties, litigation, and reputational harm. For example, the loss of clinical trial data from completed, ongoing or future clinical trials could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data. Likewise, we rely on our third-party research institution collaborators for research and development of our drug candidates and other third parties for the manufacture of our drug candidates and to conduct clinical trials, and similar events relating to their computer systems could also have a material adverse effect on our business. Our reliance on third-party service providers could also introduce new cyber security risks and vulnerabilities, such as supply-chain attacks. To the extent that any disruption or security breach were to result in a loss of, or damage to, our data or systems, or inappropriate disclosure of confidential or proprietary information, we could incur liability and the further development and commercialization of our drug candidates could be delayed. We cannot be sure that our insurance coverage will be adequate or sufficient to protect us from or to mitigate liabilities arising out of our privacy and security practices, that such coverage will continue to be available on commercially reasonable terms or at all, or that such coverage will pay future claims.

Our ability to utilize our federal net operating loss and tax credit carryforwards may be limited.

Our net operating loss, or NOL, carryforwards 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 tax years ending on or prior to December 31, 2017 are only permitted to be carried forward for 20 taxable years under applicable U.S. federal tax law. Moreover, under the Tax Act as modified by the CARES Act, federal NOLs generated in tax years beginning after December 31, 2017 may be carried forward indefinitely, but the deductibility of such federal NOLs may be limited to 80% of taxable income for tax years beginning January 1, 2018.

Under Sections 382 and 383 of the Internal Revenue Code, limitations on a corporation's ability to use its NOLs and tax credit carryforwards apply if a corporation undergoes an "ownership change," which is generally defined as a greater than 50 percentage point change (by value) in its equity ownership by certain stockholders over a three-year period. If we have experienced an ownership change at any time since our incorporation, we may already be subject to limitations on our ability to utilize our existing NOL carryforwards and other tax attributes to offset taxable income or tax liability. In addition, future changes in our stock ownership, which may be outside of our control, may trigger an ownership change. Similar provisions of state tax law may also apply to limit our use of accumulated state tax attributes. As a result, even if we earn net taxable income in the future, our ability to use our pre-change NOL carryforwards and other tax attributes to offset such taxable income or tax liability may be subject to limitations, which could potentially result in increased future income tax liability to us.

Item 1B. Unresolved Staff Comments.

None

Item 1C. Cybersecurity.

Our board of directors addresses the Company's cybersecurity risk management as part of its general oversight function. The board of directors' Audit Committee is responsible for overseeing Company's our risk management processes, including oversight and mitigation of risks from cybersecurity threats. Management is responsible for the day-to-day administration of our risk management program and our cybersecurity policies, processes, and practices.

Cybersecurity Risk Management and Strategy

We have implemented and maintain various information security processes designed to identify, assess and manage material risks from cybersecurity threats 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 (collectively, "Information Systems and Data").

We have implemented a cross-functional approach to assessing, identifying and managing material cybersecurity threats and incidents. Our Information Systems Representative and Chief Operating Officer identify and assess risks from cybersecurity threats by monitoring and evaluating our threat environment. We use various methods designed to accomplish this task including, for example: manual and automated tools, subscriptions to reports and services that identify cybersecurity threats, analyzing reports of threats and threat actors, and evaluating threats reported to us.

Depending on the relevant information systems environment, we implement and maintain various technical, physical, and organizational measures, processes, standards and policies designed to manage and mitigate material risks from cybersecurity threats to our Information Systems and Data, including, for example: incident detection and response strategies, systems monitoring, personnel training, cybersecurity insurance, data encryption strategies, network security controls, access controls, physical security controls, and asset management (such as tracking and disposal of Company information systems).

Our assessment and management of material risks from cybersecurity threats are integrated into the Company's overall risk management processes. For example, our IT Department works with management in an effort 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 service providers to assist us from time to time in an effort to identify, assess, and manage material risks from cybersecurity threats, including, for example, cybersecurity software providers and professional services firms (including legal counsel). We also use service providers to perform a variety of functions throughout our business, such as application providers, data hosting providers, and CROs. We have a vendor management strategy designed to manage cybersecurity risks associated with our use of these providers. Depending on the nature of the services provided, the sensitivity of the Information Systems and Data at issue, and the identity of the provider, our vendor management strategies may involve different levels of assessment designed to help identify cybersecurity risks associated with a provider and impose contractual obligations related to cybersecurity on the provider, such as reviewing their information security documentation and imposing contractual obligations on them with respect to their information security controls.

For a description of the risks from cybersecurity threats that may materially affect the Company and how they may do so, see our risk factors under Part 1. Item 1A. Risk Factors in this Annual Report on Form 10-K, including *Our internal computer systems, or those used by our third-party research institution collaborators, CROs or other contractors or consultants, may fail or suffer security breaches, which could result in adverse consequences including, but not limited to, regulatory investigations or actions, litigation, fines/penalties, disruptions of our business operations, reputational harm, and loss of revenue or profits.*

Governance

Our board of directors addresses the Company's cybersecurity risk management as part of its general oversight function. The board of directors' Audit Committee is responsible for overseeing Company's cybersecurity risk management processes, including oversight and mitigation of risks from cybersecurity threats. Our Audit Committee receives regular presentations and reports on developments in the cybersecurity space, including risk management practices, recent developments, evolving standards, threats, risks and mitigation. Our Audit Committee also receives prompt and timely information regarding any cybersecurity risk that meets pre-established reporting thresholds, as well as ongoing updates regarding any such risk.

Our Information Systems Representative, in coordination with senior management including our Chief Operating Officer works collaboratively across our company to implement a program designed to protect our information systems from cybersecurity threats and to promptly respond to any material cybersecurity incidents in accordance with our incident response and recovery plans. To facilitate the success of our cybersecurity program, cross-functional teams throughout our company address cybersecurity threats

and respond to cybersecurity incidents. Through ongoing communications with these teams, the Information Systems Representative and senior management are informed about and monitor the prevention, detection, mitigation and remediation of cybersecurity threats and incidents in real time and report such threats and incidents to the Audit Committee when appropriate. The Information Systems Representative has served in various roles in information technology and information security for over 25 years, including serving as the Director of Information Technology of another public company. Our Chief Operating Officer has over 7 years of experience managing information technology, including cybersecurity and risk management.

Item 2. Properties.

Our corporate headquarters are currently located in South San Francisco, California, where we signed a lease agreement for a smaller office space pursuant to a lease agreement that expires in November 2024. We also have leases in Medolla, in the Province of Modena, Italy where we have our manufacturing facility pursuant to a lease agreement that expires in August 31, 2028 and in Bresso, in the Province of Milan, Italy, for office space pursuant to a lease agreement that expires in January 31, 2036. We believe that these facilities will be adequate for our near-term needs. If required, we believe that suitable additional or alternative space would be available in the future on commercially reasonable terms.

Item 3. Legal Proceedings.

From time to time, we may become involved in legal proceedings arising in the ordinary course of business. We are not currently a party to any litigation or legal proceedings that, in the opinion of our management, are likely to have a material adverse effect on our business. Regardless of outcome, litigation can have an adverse impact on us because of defense and settlement costs, diversion of management resources, negative publicity and reputational harm and other factors.

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 listed on the Nasdaq under the trading symbol "QNCX".

Our common stock has been traded under the ticker symbol "CRTX" on the Nasdaq since May 9, 2019, and since August 1, 2022 under the ticker symbol "QNCX".

Stockholders

As of March 25, 2024, there were 50 holders of record of our common stock. The actual number of stockholders is greater than this number of record holders and includes stockholders who are beneficial owners but whose shares are held in street name by brokers and other nominees.

Dividend Policy

We have never declared or paid any cash dividends on our capital stock. We currently intend to retain any future earnings and do not expect to pay any dividends in the foreseeable future. Additionally, the EIB Facility prohibits the payment of dividends. Any future determination to declare cash dividends will be made at the discretion of our board of directors, subject to applicable laws, and will depend on a number of factors, including our financial condition, results of operations, capital requirements, contractual restrictions, general business conditions, and other factors that our board of directors may deem relevant.

Sales of Unregistered Securities

None

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 our consolidated financial statements and related notes, and Item 1 thereto included elsewhere in this Annual Report on Form 10-K. This discussion contains forward-looking statements that involve risk and uncertainties, such as statements of our plans, objectives, expectations, and intentions, that are based on the beliefs of our management. Our actual results could differ materially from those discussed in these forward-looking statements. Factors that could cause or contribute to such differences include, but are not limited to, those discussed in the "Risk Factors" section of this Annual Report on Form 10-K.

Overview

Following our acquisition of EryDel in October 2023, we are a late-stage biotechnology company dedicated to unlocking the power of a patient's own biology to deliver innovative therapies to those living with rare diseases.

Our proprietary AIDE technology platform is an innovative drug/device combination platform that uses an automated process to encapsulate a drug into a patient's own red blood cells. Red blood cells have several characteristics that make them a potentially ideal vehicle for drug delivery, including potentially better tolerability, enhanced tissue distribution, reduced immunogenicity, and prolongation of circulating half-life. Our AIDE technology is designed to harness many of these benefits to allow for new and improved therapeutic options for patients living with high unmet medical needs. The AIDE technology platform is believed to confer several benefits over conventional therapies and can be applied to a broad range of small or large molecule drugs and biologics.

EryDex is the first drug in development that leverages our AIDE technology and is composed of DSP encapsulated in autologous red blood cells targeted to treat a rare pediatric neurodegenerative disease called A-T. DSP is a corticosteroid well known for its anti-inflammatory properties, as well as its dose-limiting toxicity due to adrenal suppression. EryDex is designed to alter the pharmacokinetics and biodistribution of the DSP allowing for effective and safe treatment.

Currently, there are no approved treatments for A-T and the global market, based on our internal estimates and assumptions, represents a more than $1 billion peak commercial opportunity. We believe this makes EryDex an ideal lead asset to demonstrate the clinical and commercial potential of our AIDE technology.

2023 Events:

- On January 27, 2023, we sold our legacy small molecule protease inhibitor portfolio, including COR588, COR388, COR852, and COR803, pursuant to an asset purchase agreement with Lighthouse.
- On January 30, 2023, we announced that we intended to prioritize capital resources toward the expansion of our development pipeline through opportunistic in-licensing and acquisition of clinical-stage assets targeting debilitating and rare diseases. Our plans also called for the out-licensing of our bone-targeting drug platform and precision bone growth molecule NOV004 designed for accelerated fracture repair in patients with bone fractures and osteogenesis imperfecta. In conjunction with this action, we approved a cost reduction program to align operations with changes in our corporate strategy. This resulted in an approximate 47% reduction in our workforce that was completed by April 2023 and incurred expenses of approximately $0.4 million. We approved the related mutual termination of License Agreement for our bone-targeting drug platform and precision bone growth molecule NOV004 with PRF effective as of October 31, 2023.
- On October 20, 2023, we completed our acquisition of EryDel, a privately held, late-stage biotechnology company (the "EryDel Acquisition") with a Phase 3 lead asset, EryDex, that targets the potential treatment of a rare neurodegenerative disease, A-T, for which there are currently no approved treatments in any global market.
- The EryDel Acquisition was completed pursuant to that certain Stock Purchase Agreement, dated as of July 21, 2023, (the "Purchase Agreement"), by and among the Company, EryDel, EryDel Italy, Inc., holders of EryDel capital stock and the managers of EryDel (the "EryDel Shareholders") and Shareholder Representative Services LLC, a Colorado limited liability company solely in its capacity as the representative, agent and attorney-in-fact of the EryDel Shareholders. Pursuant to the terms of the Purchase Agreement, we issued 6,525,315 shares of common stock of the Company to the EryDel Shareholders. Up to an additional 725,037 shares of the Company's common stock may be issued to the EryDel Shareholders upon the first anniversary of the closing of the EryDel Acquisition. The EryDel Shareholders have a contingent right to receive up to an aggregate of $485.0 million in potential cash payments, comprised of up to $5.0 million upon the achievement of a specified development milestone, $25.0 million at NDA acceptance by the FDA, up to $60.0 million upon the achievement of specified approval milestones, and up to $395.0 million upon the achievement of specified on market and sales milestones, with no royalties paid to EryDel. Refer to Note 15 Business Combination for additional details.

Financial Overview

Following our acquisition of EryDel in October 2023, we shifted our strategic focus to become a late-stage biotechnology company dedicated to unlocking the power of a patient's own biology to deliver innovative therapeutics to those living with rare diseases. We intend to focus our development expertise and financial resources toward advancing a Phase 3 NEAT clinical trial, which is an international multicenter, randomized, double-blind, placebo-controlled study to evaluate the neurological effects of EryDex on patients with A-T. Enrollment for the Phase 3 NEAT clinical trial is expected to begin in the second quarter of 2024. We plan to enroll approximately 86 patients with A-T aged six to nine years old and approximately 20 A-T patients aged 10 years or older. This pivotal clinical trial will be conducted under an SPA agreement with the FDA, which should allow for the submission of an NDA following completion of this study, provided we obtain positive results. With $75.1 million of cash, cash equivalents and short-term investments as of December 31, 2023, we believe we are well-capitalized into 2026 with the ability to fully fund our lead asset, EryDex, through Phase 3 NEAT topline results and prepare for a potential NDA submission, provided we obtain positive results.

Previously, we had devoted substantially all of our efforts and financial resources to building our research and development capabilities, establishing our corporate infrastructure, and most recently, our acquisition of EryDel and subsequent development of EryDex, and prior to that, since commencing material operations in 2014, executing our Phase1a, Phase 1b and Phase 2/3 clinical trials of our legacy small molecule protease inhibitor portfolio, including atuzaginstat (COR388), our Phase 1 SAD/MAD clinical trial of COR588 and readying our bone targeting drug NOV004 for Phase 1 clinical trials. We have sold our legacy small molecule protease inhibitor portfolio and terminated our license for NOV004.

To date, we have not generated any revenue and we have never been profitable. We have incurred net losses since the commencement of our operations. As of December 31, 2023, we had an accumulated deficit of $319.6 million. We incurred a net loss of $31.4 million in the year ended December 31, 2023. We do not expect to generate product revenue unless and until we obtain marketing approval for and commercialize a drug candidate, and we cannot assure you that we will ever generate significant revenue or profits.

To date, we have financed our operations primarily through the issuance and sale of convertible promissory notes and redeemable convertible preferred stock and common stock. From inception through December 31, 2023, we received net proceeds of approximately $303.8 million from the issuance of redeemable convertible preferred stock, convertible promissory notes and common stock.

On December 23, 2021, we entered into an Open Market Sales Agreement, with Jefferies (the "Sales Agreement"). During the year ended December 31, 2023 and 2022, we sold zero and 51,769 shares of common stock, respectively, under the Sales Agreement and received net proceeds of $0 and $0.6 million, respectively.The registration statement registering the shares subject to the Sales Agreement has expired as of June 1, 2023.

As of December 31, 2023 and 2022, we had cash, cash equivalents and short-term investments of $75.1 million and $90.2 million, respectively. The balances exclude long-term investments of $0 and $3.6 million as of those same periods. Our cash equivalents, short-term and long-term investments are held in money market funds, certificate of deposits, repurchase agreements, investments in corporate debt securities, municipal debt obligations and government agency obligations.

We believe that our existing cash, cash equivalents and short-term investments will be sufficient to fund our planned operations into 2026. This is expected to include the ability to fully fund activities related to our recently acquired lead asset, EryDex, through a Phase 3 NEAT clinical trial and open label extension, and prepare for a potential NDA submission to the FDA, provided we obtain positive results. We expect the cost of our Phase 3 NEAT study and direct trial costs for the open label extension to be approximately $20 million and approximately $15 million, respectively. We have based these estimates on assumptions that may prove to be wrong, and we could utilize our available capital resources sooner than we expect.

In response to the reprioritization of our pipeline on January 30, 2023, the Board approved a cost reduction program to align operations with the change in corporate strategy to prioritize capital resources toward the expansion of our development pipeline through opportunistic in-licensing and acquisition of clinical-stage assets targeting debilitating and rare diseases. Under the Plan, we reduced headcount by approximately 47% through a reduction in our workforce. The reduction in force began in February 2023 was completed in April 2023.

We will need substantial additional funding to support our continuing operations and pursue our development strategy. Until such time as we can generate significant revenue from sales of an approved drug, if ever, we expect to finance our operations through the sale of equity, debt financings or other capital sources. Adequate funding may not be available to us on acceptable terms,

or at all. If we fail to raise capital or enter into such agreements as, and when, needed, we may have to significantly delay, scale back or discontinue the development and commercialization of our drug candidates or delay our efforts to expand our product pipeline.

Critical Accounting Policies, Significant Judgments and Use of Estimates

For a description of our significant accounting policies, see Note 2 to our consolidated financial statements.

The preparation of our consolidated financial statements in conformity with U.S. GAAP requires management to make estimates and assumptions in certain circumstances that affect the amounts reported in the accompanying consolidated financial statements and related footnotes. Actual results may differ from these estimates. We base our judgments on our experience and on various assumptions that we believe to be reasonable under the circumstances.

Of our policies, the following are considered critical to an understanding of our consolidated financial statements as they require the application of subjective and complex judgment, involving critical accounting estimates and assumptions impacting our consolidated financial statements.

The critical accounting estimates relate to the following:

- Research and Development Expenses
- Stock-based Compensation Expenses
- Income Taxes
- Business Combination
- Goodwill
- Identifiable Intangible Assets
- Contingent Consideration
- Long-term Debt

Research and Development Expenses

Research and development costs are expensed as incurred. Research and development expenses consist primarily of clinical trial and contract manufacturing expenses related to development of our drug candidates. Also included are personnel costs for our research and product development employees, non-personnel costs such as professional fees payable to third parties for preclinical studies and research services, laboratory supplies and equipment maintenance, product licenses, and other consulting costs.

We estimate preclinical and clinical study and research expenses based on the services performed, pursuant to arrangements with CROs that conduct and manage preclinical and clinical studies and research services on our behalf. Research and development contracts vary significantly in length, and may be for a fixed amount, based on milestones or deliverables, a variable amount based on actual costs incurred, capped at a certain limit, or for a combination of these elements. The financial terms of these agreements vary from contract to contract and may result in uneven expenses and payment flows. We estimate these expenses based on regular reviews with internal management personnel and external service providers as to the progress or stage of completion of services and the contracted fees to be paid for such services. Based upon the combined inputs of internal and external resources, if the actual timing of the performance of services or the level of effort varies from the original estimates, we will adjust the accrual accordingly. Although we do not expect our estimates to be materially different from amounts actually incurred, our understanding of the status and timing of services performed relative to the actual status and timing of services performed may vary and could result in us reporting amounts that are too high or too low in any particular period. Our accrual is dependent, in part, upon the receipt of timely and accurate reporting from clinical research organizations and other third-party vendors. Payments associated with licensing agreements to acquire exclusive licenses to develop, use, manufacture and commercialize products that have not reached technological feasibility and do not have alternate commercial use are expensed as incurred. Payments made to third parties under these arrangements in advance of the performance of the related services by the third parties are recorded as prepaid expenses until the services are rendered.

To date, there have been no material differences from our accrued estimated expenses to the actual clinical trial expenses and our methodology and assumptions used in developing these estimates have not changed materially during the periods presented. If we do not identify costs that we have begun to incur or if we underestimate or overestimate the level of services performed or the costs of these services, our actual expenses could differ from our estimates, which could materially affect our results of operations. Adjustments to our accruals are recorded as changes in estimates become evident. Furthermore, based on amounts invoiced to us by

our service providers, we may also record payments made to those providers as prepaid expenses that will be recognized as expense in future periods as services are rendered. Due to the nature of estimates, we cannot assure you that we will not make changes to our estimates in the future as we become aware of additional information about the status or conduct of our research and development activities.

Stock-Based Compensation Expense

We measure and record compensation expense using the applicable accounting guidance for share-based payments related to stock options and performance-based awards granted to our directors and employees. The fair value of stock options is determined by using the Black-Scholes option-pricing model. The Black-Scholes valuation model incorporates assumptions as to stock price volatility, the expected life of options or awards, a risk-free interest rate and dividend yield. In valuing our stock options and market-based stock awards, significant judgment is required in determining the expected volatility of our common stock and the expected life that individuals will hold their stock options prior to exercising. Expected volatility for stock options is based on the historical volatility of our own stock and the stock of companies within our defined peer group. Further, our expected volatility may change in the future, which could substantially change the grant-date fair value of future awards and, ultimately, the expense we record.

We expense stock-based compensation for stock options and performance awards over the requisite service period. For awards with only a service condition, we expense stock-based compensation using the straight-line method over the requisite service period for the entire award. For awards with a market condition, we expense over the vesting period regardless of the value that the award recipients ultimately receive.

We estimate the fair value of stock-based compensation utilizing the Black-Scholes option-pricing model, which is impacted by the following variables:

Expected Term—We have opted to use the "simplified method" for estimating the expected term of options, whereby the expected term equals the arithmetic average of the vesting term and the original contractual term of the option (generally 10 years).

Expected Volatility—Due to our limited operating history and a lack of company specific historical and implied volatility data, we have based our estimate of expected volatility on the historical volatility of our own stock and the stock of companies within our defined peer group. The historical volatility data was computed using the daily closing prices for the selected companies' shares during the equivalent period of the calculated expected term of the stock-based awards.

Risk-Free Interest Rate—The risk-free rate assumption is based on the U.S. Treasury instruments with maturities similar to the expected term of our stock options.

Expected Dividend—We have not issued any dividends in our history and do not expect to issue dividends over the life of the options and therefore have estimated the dividend yield to be zero.

Income Taxes

We prepare and file income tax returns based on our interpretation of each jurisdiction's tax laws and regulations. In preparing our consolidated financial statements, we estimate our income tax liability in each of the jurisdictions in which we operate by estimating our actual current tax expense together with assessing temporary differences resulting from differing treatment of items for tax and financial reporting purposes. These differences result in deferred tax assets and liabilities, which are included in our consolidated balance sheets.

Significant management judgment is required in assessing the realizability of our deferred tax assets. In performing this assessment, we consider whether it is more likely than not that some portion or all of the deferred tax assets will not be realized. The ultimate realization of deferred tax assets is dependent upon the generation of future taxable income during the periods in which those temporary differences become deductible. In making this determination, under the applicable financial accounting standards, we are allowed to consider the scheduled reversal of deferred tax liabilities, projected future taxable income and the effects of tax planning strategies. In the event that actual results differ from our estimates, we adjust our estimates in future periods and we may need to increase or decrease our valuation allowance, when management determines it is more likely than not that some or all of the tax benefits will not be realized. This could materially impact our consolidated financial position and results of operations.

We account for uncertain tax positions using a "more likely than not" threshold for recognizing and resolving uncertain tax positions. We evaluate uncertain tax positions on a quarterly basis and consider various factors including, but not limited to, changes in tax law, the measurement of tax positions taken or expected to be taken in tax returns, the effective settlement of matters subject to

audit, information obtained during in process audit activities and changes in facts or circumstances related to a tax position. We adjust the level of the liability to reflect any subsequent changes in the relevant facts surrounding the uncertain positions. Our liabilities for uncertain tax positions can be relieved only if the contingency becomes legally extinguished, through either payment to the taxing authority or the expiration of the statute of limitations, the recognition of the benefits associated with the position meet the "more likely than not" threshold or the liability becomes effectively settled through the examination process. We consider matters to be effectively settled once the taxing authority has completed all of its required or expected examination procedures, including all appeals and administrative reviews, we have no plans to appeal or litigate any aspect of the tax position and we believe that it is highly unlikely that the taxing authority would examine or re-examine the related tax position. We also accrue for potential interest and penalties related to unrecognized tax benefits in income tax expense. Judgments concerning the recognition and measurement of a tax benefit might change as new information becomes available.

Business Combination

We make certain judgments to determine whether transactions should be accounted for as acquisitions of assets or as business combinations. If it is determined that substantially all of the fair value of gross assets acquired in a transaction is concentrated in a single asset (or a group of similar assets), the transaction is treated as an acquisition of assets. We evaluate the inputs, processes, and outputs associated with the acquired set of activities. If the assets in a transaction include an input and a substantive process that together significantly contribute to the ability to create outputs, the transaction is treated as an acquisition of a business. We account for business combinations using the acquisition method of accounting, which requires that assets acquired and liabilities assumed generally be recorded at their fair values as of the acquisition date.

The Company accounts for business combinations using the acquisition method pursuant to the FASB ASC Topic 805. This method requires, among other things, that results of operations of acquired companies are included in the Company's financial results beginning on the respective acquisition dates, and that identifiable assets acquired and liabilities assumed are recognized at fair value as of the acquisition date. Intangible assets acquired in a business combination are recorded at fair value using a discounted cash flow model. The discounted cash flow model requires assumptions about the timing and amount of future net cash flows, the cost of capital and terminal values from the perspective of a market participant. Any excess of the fair value of consideration transferred (the "Purchase Price") over the fair values of the net assets acquired is recognized as goodwill. The fair value of identifiable assets acquired and liabilities assumed in certain cases may be subject to revision based on the final determination of fair value during a period of time not to exceed 12 months from the acquisition date. Legal costs, due diligence costs, business valuation costs and all other acquisition-related costs are expensed when incurred.

Goodwill

When we acquire a business, the assets acquired and liabilities assumed are recorded at their respective fair values at the acquisition date. Goodwill represents the excess of the acquisition consideration over the fair value of assets acquired and liabilities assumed. We test goodwill for impairment annually and when events or changes in circumstances indicate that the carrying value may not be recoverable. We have determined that we operate in a single segment and have a single reporting unit associated with the development and commercialization of pharmaceutical products. In performing the annual impairment test, the fair value of the reporting unit is compared to its corresponding carrying value, including goodwill. If the carrying value exceeds the fair value of the reporting unit an impairment loss will be recognized for the amount by which the reporting unit's carrying amount exceeds its fair value, not to exceed the carrying amount of goodwill. The Company first assesses qualitative factors to determine whether it is more likely than not that the fair value of the Company is less than its carrying amount, including goodwill. If that is the case, the Company performs a quantitative impairment test, and, if the carrying amount of the Company exceeds its fair value, then the Company will recognize an impairment charge for the amount by which its carrying amount exceeds its fair value, not to exceed the carrying amount of the goodwill.

Identifiable Intangible Assets

We have acquired intangible assets through our recent business combinations with EryDel in the fourth quarter of 2023, and with Novosteo in 2022. When significant identifiable intangible assets are acquired, we engage an independent third party valuation firm to assist in determining the fair values of these assets as of the acquisition date. Discounted cash flow models are typically used in these valuations, which require the use of significant estimates and assumptions, including but not limited to:

- estimating the timing of and expected costs to complete the in-process projects;
- projecting regulatory approvals;
- estimating future cash flows including revenues and operating profits resulting from completed products and in-process projects; and

- developing appropriate discount rates and probability rates by project.

We believe the fair value that we assign to the intangible asset acquired are based upon reasonable estimates and assumptions given available facts and circumstances as of the acquisition dates. No assurance can be given, however, that the underlying assumptions used to estimate expected cash flows will transpire as estimated. In addition, we are required to estimate the period of time over which to amortize the intangible assets, which requires significant judgment.

Impairment of Intangible Assets

Finite-lived intangible asset consist primarily of the tradename and is amortized on a straight-line basis over their estimated useful lives. Indefinite lived intangible assets are not amortized, Intangible assets related to IPR&D acquired in a business combination or an acquisition that are used in IPR&D shall be considered indefinite lived until the completion or abandonment of the associated research and development efforts. IPR&D is not amortized but is tested for impairment annually or when events or circumstances indicate that the fair value may be below the carrying value of the asset. If the carrying value of the assets is not expected to be recovered, the assets are written down to their estimated fair values. Please refer to Note 16, Intangible Assets to the Notes to the consolidated financial statements of this Annual Report on Form 10-K, for further information about our intangible assets as of December 31, 2023.

Contingent Consideration

We determine the acquisition date fair value of contingent consideration using a discounted cash flow method, with significant inputs that are not observable in the market and thus represents a Level 3 fair value measurement as defined in ASC Topic 820, Fair Value Measurement. The significant inputs in the Level 3 measurement not supported by market activity included our probability assessments of expected future cash flows related to our acquisition of EryDel in October 2023, during the contingent consideration period, appropriately discounted considering the uncertainties associated with the earnout obligation, and calculated in accordance with the terms of the definitive agreement. The liabilities for the contingent consideration are established at the time of the acquisition and will be evaluated on a quarterly basis based on additional information as it becomes available. Any change in the fair value adjustment is recorded in the earnings of that period. During the year ended December 31, 2023, we recorded a $1.6 million adjustment to increase the fair value of our contingent consideration related to the acquisition of EryDel. The adjustment is reflected within operating loss on the consolidated statement of operations and comprehensive loss. Changes in the fair value of the contingent consideration obligations may result from changes in probability assumptions with respect to the likelihood of achieving the various contingent payment obligations. Significant increases or decreases in the inputs noted above in isolation would result in a significantly lower or higher fair value measurement.

Long-Term Debt

We determined that we are eligible for the fair value option election in connection with the EIB Loan (as defined below) as the instrument met the definition of a “recognized financial liability” which is an acceptable financial instrument eligible for the fair value option under ASC 825. At the date of inception of the EIB Loan through the EryDel Acquisition, the fair value for each instrument is derived from the instrument’s implied discount rate at inception.

Components of Operating Results

Operating Expenses

Research and Development Expenses

Our research and development expenses consist of expenses incurred in connection with the research and development of our research programs. These expenses include payroll and personnel expenses, including stock-based compensation, for our research and product development employees, laboratory supplies, product licenses, consulting costs, contract research, regulatory, quality assurance, preclinical and clinical expenses, allocated rent, facilities costs and depreciation. We expense both internal and external research and development costs as they are incurred. Non-refundable advance payments and deposits for services that will be used or rendered for future research and development activities are recorded as prepaid expenses and recognized as an expense as the related services are performed.

To date, our research and development expenses have supported the advancement of atuzaginstat (COR388) and COR588 and to a lesser extent the clinical and regulatory development of NOV004. With the acquisition of EryDel, we expect our research and

development expenses to increase significantly from current levels as we begin enrollment and proceed with the Phase 3 NEAT clinical trial.

In addition, the probability of success of any in-licensed drug candidate will depend on numerous factors, including safety, efficacy, competition, manufacturing capability and commercial viability. We will need to determine which programs to pursue and how much to fund each program in response to the scientific and clinical success of each drug candidate, as well as an assessment of each drug candidate's commercial potential.

Because our drug candidates have not been in-licensed the outcome of these efforts is uncertain, we cannot estimate the actual amounts necessary to successfully complete the development and commercialization of our drug candidate or whether, or when, we may achieve profitability.

General and Administrative Expenses

General and administrative expenses consist principally of personnel-related costs, including payroll and stock-based compensation, for personnel in executive, finance, human resources, business and corporate development, and other administrative functions, professional fees for legal, consulting, insurance and accounting services, allocated rent and other facilities costs, depreciation, and other general operating expenses not otherwise classified as research and development expenses.

We anticipate that our general and administrative expenses will increase from current levels due to the acquisition of EryDel and the increase in headcount as the size of our business and research and development operations grows to support additional research and development activities.

Interest Income

Interest income consists primarily of interest earned on our short-term and long-term investments portfolio.

Other Expense, net

Other expense, net consists primarily of the effects of foreign currency exchange rates.

Results of Operations

Comparison of the Years Ended December 31, 2023 and 2022

The following table summarizes our results of operations for the periods indicated (dollars in thousands):

	Year Ended December 31,		Change	
	2023	2022	$	%
Operating expenses:				
Research and development	$ 9,447	$ 25,178	$ (15,731)	(62.5) %
General and administrative	17,695	26,012	(8,317)	(32.0) %
Goodwill impairment charge	—	825	(825)	(100.0) %
Intangible asset impairment charge	5,900	—	5,900	100.0 %
Fair value adjustment for contingent consideration	1,578	—	1,578	100.0 %
Loss from operations	(34,620)	(52,015)	(17,395)	(33.4) %
Fair value adjustment for long-term debt	(338)	—	(338)	100.0 %
Interest income	3,478	1,068	(2,410)	(225.7) %
Other expense, net	(102)	(997)	(895)	(89.8) %
Net loss before income tax benefit	(31,582)	(51,944)	(20,362)	(39.2) %
Income tax benefit	197	284	87	30.6 %
Net loss	$ (31,385)	$ (51,660)	$ (20,275)	(39.2) %

Research and Development Expenses

The following table summarizes our research and development expenses: (dollars in thousands)

	Year Ended December 31,		Change	
	2023	2022	$	%
Direct research and development expenses:				
EryDex	$ 1,655	$ —	$ 1,655	100.0 %
Atuzaginstat (COR388)	153	1,400	(1,247)	(89.1) %
COR588	28	5,467	(5,439)	(99.5) %
NOV004	2,048	1,834	214	11.7 %
Other direct research costs	280	1,510	(1,230)	(81.5) %
Indirect research and development expenses:				
Personnel related (including stock-based compensation)	4,619	14,147	(9,528)	(67.3) %
Facilities and other research and development expenses	664	820	(156)	(19.0) %
Total research and development expenses	$ 9,447	$ 25,178	$ (15,731)	(62.5) %

Research and development expenses were $9.4 million for the year ended December 31, 2023, compared to $25.2 million for the year ended December 31, 2022, a decrease of $15.7 million. We anticipate our research and development expenses to increase significantly from current levels as we begin enrollment and proceed with the Phase 3 NEAT clinical trial.

The costs for EryDex development increased $1.7 million from prior year due to the start-up costs related to the Phase 3 NEAT clinical trial. This increase is primarily due to an increase in clinical trial costs of $1.6 million and a $0.1 million increase in Medtech and drug manufacturing costs.

The costs for atuzaginstat (COR388) development decreased $1.2 million from the prior year due to a decrease of $0.8 million in drug manufacturing costs and a decrease of $0.4 million related to consulting for atuzaginstat (COR388).

Our Phase 1 SAD/MAD trial was completed in the second quarter of 2022 for our compound COR588 in healthy participants in Australia. As a result, the costs for COR588 decreased by $5.4 million from the prior year. This decrease was primarily due to a $2.7 million decrease for non-clinical studies to support COR588, a $1.6 million decrease in drug manufacturing costs, a $1.0 million decrease in clinical trial costs, and a $0.1 million decrease in consulting expenses to support COR588.

As we sold our legacy protease inhibitor portfolio including COR388 and COR588 to Lighthouse in January 2023, we do not expect any additional expenses related to these legacy assets in 2024.

For the year ended December 31, 2023, the costs for NOV004 increased by $0.2 million primarily as due to the increase in drug manufacturing costs as we wrapped up the program. Due to the decision made on January 30, 2023 to align with our updated corporate strategy, we do not expect further NOV004 costs in 2024.

Additionally, other direct research costs decreased $1.2 million primarily due to the winddown of pipeline development of our two arginine gingipain inhibitors, COR788 and COR822, our 3CLpro inhibitor, COR803, COR852 and other preclinical research which were sold to Lighthouse in January 2023.

For the year ended December 31, 2023, we experienced a net decrease of $9.5 million in personnel related expenses due to a $5.2 million decrease in allocated stock-based compensation costs, a decrease of $1.6 million of severance incurred, and a decrease of $2.8 million related to reduced headcount year over year.

Facilities and other research and development expenses decreased $0.2 million for the year ended December 31, 2023 primarily due to a $0.2 million decrease in the purchase of non-clinical supplies, a $0.2 million decrease in facilities and rent expense, and a $0.2 million decrease in administration and depreciation expense, offset by a $0.4 million increase in regulatory and quality assurance consulting costs.

General and Administrative Expenses

General and administrative expenses decreased by $8.3 million to $17.7 million for the year ended December 31, 2023 from $26.0 million for the year ended December 31, 2022. The decrease in general and administrative expenses was primarily due to an overall decrease in personnel expenses of $8.2 million, which is made up of a $6.2 million decrease in allocated stock-based compensation expense, a $1.6 million decrease of severance incurred, and a decrease of $0.3 million related to reduced headcount year over year. We also incurred a $1.3 million decrease in corporate insurance expenses, a net decrease of $0.2 million in legal, facilities, and other administrative expense due to our cost reductions efforts announced in the first quarter of 2023 and lower Director & Officers insurance premiums. This was partially offset by an increase of $1.2 million in business development consulting related to the EryDel transaction and a $0.2 million increase in audit, tax, and other professional fees year over year.

Goodwill Impairment Charge

As of September 30, 2022, we conducted an impairment analysis of our goodwill that resulted from the purchase of Novosteo, Inc. in May 2022. That assessment included a qualitative assessment of deteriorating macro-economic conditions, including inflationary pressures, rising interest rates, and the continuing decline in our market capitalization from the date of acquisition. This qualitative assessment indicated that our goodwill was potentially impaired. To determine the extent, if any, by which our goodwill was impaired, we conducted additional quantitative analyses which resulted in our fair value being significantly below our current carrying value. As a result of the analyses, we recorded a non-cash goodwill impairment charge of $0.8 million for the year ended December 31, 2022. There have been no additional impairment charges since the prior period as there were no impairment indicators detected as part of the qualitative assessment for the EryDel goodwill that remained on the books as of December 31, 2023.

Intangible Asset Impairment Charge

As of March 31, 2023, we conducted an impairment analysis of our intangible asset IPR&D that resulted from the purchase of Novosteo, Inc. in May 2022. To determine the extent, if any, by which our IPR&D intangible asset was impaired, we conducted a quantitative analysis which resulted in our fair value being significantly below our current carrying value due to the assumptions changing as a result of the decision to hold this asset for sale in January 2023. As a result of the analyses, we recorded a non-cash intangible asset IPR&D impairment charge of $5.9 million for the year ended December 31, 2023. There have been no additional impairment charges related to EryDel as there were no impairment indicators detected as part of the qualitative assessment for the EryDex IPR&D that remained on the books as of December 31, 2023.

Fair Value Adjustment for Contingent Consideration

For the year ended December 31, 2023, we recorded a fair value adjustment for contingent consideration which resulted in a $1.6 million charge primarily due to the passage of time related to the contingent consideration earn-outs resulting from the EryDel Acquisition.

Fair Value Adjustment for Long-term Debt

For the year ended December 31, 2023, we recorded a fair value adjustment for long-term debt which resulted in a $0.3 million charge primarily due to the passage of time and the interest accrued for the debt with the EIB.

Interest Income

For the year ended December 31, 2023, interest income increased by $2.4 million as compared to the year ended December 31, 2022. The increase was due to increased yields on our investment portfolio which were partially offset by decreased average balances.

Other Expense

Other expense decreased by $0.9 million for the year ended December 31, 2023, primarily due to $0.6 million lower of unrealized losses resulting from changes in foreign exchange rates, as well as a $0.2 million decrease related to the San Diego lease impairment loss and loss on disposal of fixed assets incurred during the year ended December 31, 2022.

Income Tax

There was no change in the income tax benefit as we recorded a $0.2 million income tax benefit for the year ended December 31, 2023 as a result of the quantitative and qualitative analysis that concluded in the NOV004 asset being fully impaired and written off.

Liquidity, Capital Resources and Plan of Operations

We have incurred cumulative net losses and negative cash flows from operations since our inception and anticipate we will continue to incur net losses for the foreseeable future. As of December 31, 2023, we had an accumulated deficit of $319.6 million. To date we have funded our operations primarily from the sales of our equity securities. As of December 31, 2023, we had cash, cash equivalents and investments of $75.1 million. Based on our current business plans, we believe that our existing capital resources will be sufficient to fund our projected operating requirements for at least the next twelve months from the date of the issuance of the accompanying consolidated financial statements. Our cash, cash equivalents, and marketable debt securities are held in a variety of deposit accounts, interest-bearing accounts, corporate bond securities, U.S government securities, debt securities in government-sponsored entities, and money market funds. Cash in excess of immediate requirements is invested with a view toward liquidity and capital preservation, and we seek to minimize the potential effects of concentration and credit risk.

Capital Resources

Our primary use of cash is to fund operating expenses, which consist primarily of research and development expenditures related to EryDex, the initiation and continuation of the Phase 3 NEAT clinical trial, and general and administrative expenditures. Cash used to fund operating expenses is impacted by the timing of when we pay these expenses, as reflected in the change in our outstanding accounts payable and accrued expenses.

In January 2023 we out-licensed our legacy protease inhibitors to Lighthouse and terminated our license for NOV004. We also intend to concentrate our efforts on development of EryDex. Accordingly, we cannot estimate the actual amounts necessary to successfully complete the development and commercialization of any drug candidates or whether, or when, we may achieve profitability.

We may continue to require additional capital to develop our drug candidates and fund operations for the foreseeable future. We may seek to raise capital through private or public equity or debt financings, collaborative or other arrangements with other companies, or through other sources of financing. Adequate additional funding may not be available to us on acceptable terms or at all. Our failure to raise capital as and when needed could have a negative impact on our financial condition and our ability to pursue our business strategies. Following our acquisition of EryDel, we anticipate that we will need to raise substantial additional capital, the requirements of which will depend on many factors, including:

- the progress, costs, trial design, results of and timing of our Phase 3 NEAT clinical trial and any potential future trials;
- the outcome, costs and timing of seeking and obtaining FDA and any other regulatory approvals;
- payment of future milestones to EryDel shareholders and payments to the EIB in respect of obligations under the EIB Facility;
- the number and characteristics of drug candidates that we pursue;
- our ability to maintain, expand and defend the scope of our intellectual property portfolio, including the amount and timing of any payments we may be required to make, or that we may receive, in connection with the licensing, filing, prosecution, defense and enforcement of any patents or other intellectual property rights;
- our need and ability to retain management and hire scientific and clinical personnel;
- the effect of competing drugs and drug candidates and other market developments;
- our need to implement additional internal systems and infrastructure, including financial and reporting systems; and
- the economic and other terms, timing of and success of any collaboration, licensing or other arrangements into which we may enter in the future.

If we raise additional funds by issuing equity securities, our stockholders will experience dilution. Any future debt financing into which we enter may impose upon us additional covenants that restrict our operations, including limitations on our ability to incur liens or additional debt, pay dividends, repurchase our common stock, make certain investments and engage in certain merger, consolidation or asset sale transactions. Any debt financing or additional equity that we raise may contain terms that are not

favorable to us or our stockholders. If we are unable to raise additional funds when needed, we may be required to delay, reduce, or terminate some or all of our development programs and clinical trials. We may also be required to sell or license to other rights to our drug candidates in certain territories or indications that we would prefer to develop and commercialize ourselves.

Our ability to raise additional capital may be adversely impacted by potential worsening global economic conditions and the recent disruptions to, and volatility in, the credit and financial markets in the United States and worldwide. However, based on our current business plans, we believe that our existing cash, cash equivalents and investments will be sufficient to fund our planned operations, which would include anticipated clinical and development activities related to EryDel's lead asset through completion of the Phase 3 NEAT clinical trial, and into 2026, but does not include any costs or cash expenditures associated with any additional potential asset acquisition.

Summary Statement of Cash Flows

The following table sets forth the primary sources and uses of cash and cash equivalents for each of the periods presented below (in thousands):

	Year Ended December 31,	
	2023	2022
Net cash (used in) provided by:		
Operating activities	$ (18,292)	$ (44,038)
Investing activities	(5,758)	18,002
Financing activities	143	707
Effect of exchange rate changes on cash	80	184
Net decrease in cash and cash equivalents	$ (23,827)	$ (25,145)

Operating Activities

Net cash used in operating activities was $18.3 million for the year ended December 31, 2023. Cash used in operating activities in the year ended December 31, 2023 was primarily due to our net loss for the period of $31.4 million, which included non-cash expenses of $11.2 million, including $5.2 million in stock-based compensation, and a net decrease in accounts payable and accrued expenses and other current liabilities of $2.3 million, offset by increases in our current assets of $4.2 million.

Net cash used in operating activities was $44.0 million for the year ended December 31, 2022. Cash used in operating activities in the year ended December 31, 2022 was primarily due to our net loss for the period of $51.7 million, which included non-cash expenses of $17.5 million, including $16.6 million in stock-based compensation, and a net decrease in accounts payable and accrued expenses and other current liabilities of $12.6 million and increases in our current assets of $2.8 million.

Investing Activities

Cash used in investing activities was $5.8 million in the year ended December 31, 2023, primarily related to the purchase of investments of $113.8 million, offset by maturities of investments of $111.2 million, transaction costs related to the EryDel acquisition net of cash acquired of $2.1 million, the advance of the note receivable to EryDel prior to the close of the acquisition of $1.0, and the purchase of equipment of $0.2 million.

Cash provided by investing activities was $18.0 million in the year ended December 31, 2022, primarily related to the purchase of equipment of $0.1 million, purchase of investments of $75.0 million, offset by maturities of investments of $82.5 million and cash acquired from the Novosteo Acquisition of $10.6 million.

Financing Activities

Cash provided by financing activities was $0.1 million in the year ended December 31, 2023, which consisted of net proceeds from the exercise of stock options in the period.

Cash provided by financing activities was $0.7 million in the year ended December 31, 2022, which consisted of proceeds from the issuance of common stock in connection with an open market sales agreement, net of issuance costs as well as proceeds from the exercise of options.

Contractual Obligations and Commitments

Material contractual obligations arising in the normal course of business primarily consist of operating leases, drug manufacturing, preclinical and clinical contract obligations. As of December 31, 2023, our operating lease payment obligations were $0.4 million, of which $0.1 million is expected to be paid within 12 months and the remainder thereafter. See Note 6 to the consolidated financial statements for amounts outstanding for operating leases on December 31, 2023.

We enter into contracts in the normal course of business with third-party contract organizations for clinical trials, non-clinical studies and testing, manufacturing, and other services and products for operating purposes. The amount and timing of the payments under these contracts varies based upon the timing of the services. We have recorded accrued expense of approximately $1.0 million in our consolidated balance sheet for expenditures incurred by these vendors as of December 31, 2023. We have approximately $17.1 million in cancellable future operating expense commitments based on existing contracts as of December 31, 2023. These obligations will be satisfied in the normal course of business, but generally no longer than 12 months. As of December 31, 2023, the fair value of the EIB loan is $13.4 million and it is recorded as long-term debt on the consolidated balance sheet at Fair Value. As of December 31, 2023, the fair value of long-term contingent consideration on our books for the earnout related to the EryDel Acquisition is $53.6 million and the short-term portion is $4.1 million, refer to Note 3 to the consolidated financial statements for further details.

Indemnification

As permitted under Delaware law and in accordance with our bylaws, we indemnify our officers and directors for certain events or occurrences while the officer or director is or was serving in such capacity. We are also party to indemnification agreements with our officers and directors. We believe the fair value of the indemnification rights and agreements is minimal. Accordingly, we have not recorded any liabilities for these indemnification rights and agreements as of December 31, 2023 and December 31, 2022.

Recent Accounting Pronouncements

See Note 2 to our consolidated financial statements included in Part II, Item 8, “Financial Statements and Supplementary Data,” of this Annual Report on Form 10-K for a description of recent accounting pronouncements applicable to our business.

Item 7A. Quantitative and Qualitative Disclosures About Market Risk.

We are a smaller reporting company as defined by Rule 12b-2 of the Securities Exchange Act of 1934, as amended, or the Exchange Act, and are not required to provide the information required under this item.

Item 8. Financial Statements and Supplementary Data.

Quince Therapeutics, Inc.
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REPORT OF INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM

Shareholders and Board of Directors
Quince Therapeutics, Inc.
South San Francisco, California

Opinion on the Consolidated Financial Statements

We have audited the accompanying consolidated balance sheets of Quince Therapeutics, Inc. (the "Company") as of December 31, 2023 and 2022, the related consolidated statements of operations and comprehensive loss, stockholders' equity, and cash flows for each of the years then ended, and 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 at December 31, 2023 and 2022, and the results of its operations and its cash flows for each of 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 in accordance with the standards of the PCAOB. Those standards require that we plan and perform the audit 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.

Critical Audit Matter

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: (1) relates to accounts or disclosures that are material to the consolidated financial statements and (2) involved our especially challenging, subjective, or complex judgments. The communication of a critical audit matter 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 separate opinions on the critical audit matter or on the accounts or disclosures to which it relates.

Valuation of Contingent Consideration and Intangible Assets Related to In-process Research and Development (IPR&D)

As disclosed in Notes 15 and 16 to the consolidated financial statements, on October 20, 2023, the Company completed its acquisition of EryDel S.p.A., which the Company accounted for as a business combination. The acquisition date fair value of the consideration transferred was approximately $66.9 million, which included $56.1 million of contingent consideration based on achieving certain milestones. The Company estimated the fair value of the contingent consideration using a probability-weighted discounted cash flow model. In addition, as a result of the acquisition, the Company acquired IPR&D with a fair value of $60.6 million, which was determined using the Multi-Period Excess Earnings Method under the income approach. Management used significant assumptions including probability rates and discount rates in determining the fair values of the contingent consideration and IPR&D, as well as certain significant assumptions in its revenue projections in determining the fair value of the IPR&D.

We identified the valuation of contingent consideration and intangible assets related to IPR&D at the acquisition date as a critical audit matter due to the significant management judgment and subjectivity required in determining their fair values. Auditing management's assumptions related to the probability rates and discount rates for the valuations of the contingent consideration and IPR&D and certain significant assumptions used in the revenue projections for the IPR&D valuation involved especially subjective auditor

judgment due to the nature and extent of audit effort required to address this matter, including the involvement of professionals with specialized skills or knowledge.

The primary procedures we performed to address this critical audit matter included:

- Evaluating the reasonableness of certain significant assumptions used in the revenue projections for the IPR&D valuation by comparing these assumptions with relevant industry or market data and historical selling prices of similar products.
- For the valuations of the contingent consideration and IPR&D, utilizing valuation professionals with specialized skill and knowledge to assist in (i) assessing the appropriateness of the valuation methodologies used by management, (ii) evaluating the reasonableness of the probability rates and discount rates used by management, and (iii) developing a range of independent estimates of probability rates and discount rates and comparing those to the rates used by management.

/s/ BDO USA, P.C.
We have served as the Company's auditor since 2018.
San Jose, California
April 1, 2024

QUINCE THERAPEUTICS, INC.
CONSOLIDATED BALANCE SHEETS
(in thousands except share and per share data)

	December 31, 2023	December 31, 2022
ASSETS		
Current assets:		
Cash and cash equivalents	$ 20,752	$ 44,579
Short-term investments	54,307	45,602
Prepaid expenses and other current assets	2,381	3,567
Total current assets	77,440	93,748
Assets held for sale	10	—
Property and equipment, net	234	393
Operating lease right-of-use assets	385	291
Long-term investments	—	3,578
Goodwill	17,625	—
Intangible asset	63,672	5,900
Other assets	8,456	—
Equity investments in Lighthouse Pharmaceuticals, Inc.	78	—
Total assets	$ 167,900	$ 103,910
LIABILITIES AND STOCKHOLDERS' EQUITY		
Current liabilities:		
Accounts payable	$ 2,033	$ 570
Short-term contingent consideration	4,103	—
Accrued expenses and other current liabilities	3,436	2,499
Total current liabilities	9,572	3,069
Long-term debt	13,429	—
Long-term operating lease liabilities	321	—
Long-term contingent consideration	53,603	—
Deferred tax liabilities	5,304	248
Other long-term liabilities	587	—
Total liabilities	82,816	3,317
Commitments and contingencies (See Note 8)		
Stockholders' equity:		
Preferred stock, $0.001 par value, 10,000,000 authorized (100,000 shares of which are designated as Series A Junior Participating Preferred Stock), no shares issued and outstanding as of December 31, 2023 and 2022, respectively	—	—
Common stock, $0.001 par value, 100,000,000 shares authorized, 42,973,215 and 36,136,480 issued and outstanding as of December 31, 2023 and 2022, respectively	43	36
Additional paid in capital	401,638	389,105
Accumulated other comprehensive income (loss)	3,047	(289)
Accumulated deficit	(319,644)	(288,259)
Total stockholders' equity	85,084	100,593
Total liabilities and stockholders' equity	$ 167,900	$ 103,910

See accompanying notes to the consolidated financial statements

QUINCE THERAPEUTICS, INC.
CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS
(in thousands except for share and per share amounts)

	Year Ended December 31,	
	2023	2022
Operating expenses:		
Research and development	$ 9,447	$ 25,178
General and administrative	17,695	26,012
Goodwill impairment charge	—	825
Intangible asset impairment charge	5,900	—
Fair value adjustment for contingent consideration	1,578	—
Total operating expenses	34,620	52,015
Loss from operations	(34,620)	(52,015)
Fair value adjustment for long-term debt	(338)	—
Interest income	3,478	1,068
Other expense, net	(102)	(997)
Net loss before income tax benefit	(31,582)	(51,944)
Income tax benefit	197	284
Net loss	(31,385)	(51,660)
Other comprehensive income (loss):		
Foreign currency translation adjustments	2,789	248
Unrealized gain (loss) on available-for-sale securities	547	(458)
Total comprehensive loss	$ (28,049)	$ (51,870)
Net loss per share - basic and diluted	$ (0.84)	$ (1.54)
Weighted average shares of common stock outstanding - basic and diluted	37,237,149	33,496,534

See accompanying notes to the consolidated financial statements

QUINCE THERAPEUTICS, INC.
CONSOLIDATED STATEMENTS OF STOCKHOLDERS' EQUITY
(in thousands except share amounts)

	Common Stock		Additional Paid in Capital	Accumulated Other Comprehensive Income / (Loss)	Accumulated Deficit	Shareholders' Equity
	Shares	Amount				
Balance January 1, 2022	30,074,412	$ 30	$ 355,234	$ (79)	$ (236,599)	$ 118,586
Issuance of common stock in connection with open market sales agreement, net of issuance costs of $19	51,769	—	608	—	—	608
Issuance of common stock on exercise of stock options and vesting of restricted stock units	490,299	—	148	—	—	148
Stock based compensation	—	—	16,618	—	—	16,618
Share issuance in connection with acquisition of Novosteo, Inc.	5,520,000	6	16,497	—	—	16,503
Foreign currency translation adjustment	—	—	—	248	—	248
Unrealized gain (loss) on available-for-sale investments	—	—	—	(458)	—	(458)
Net loss	—	—	—	—	(51,660)	(51,660)
Balance December 31, 2022	36,136,480	$ 36	$ 389,105	$ (289)	$ (288,259)	$ 100,593
Issuance of common stock on exercise of stock options and vesting of restricted stock units	374,713	—	167	—	—	167
Restricted stock award forfeiture	(63,293)	—	(18)	—	—	(18)
Stock based compensation	—	—	5,220	—	—	5,220
Share issuance in connection with acquisition of EryDel S.p.A.	6,525,315	7	7,164	—	—	7,171
Foreign currency translation adjustment	—	—	—	2,789	—	2,789
Unrealized gain (loss) on available-for-sale investments	—	—	—	547	—	547
Net loss	—	—	—	—	(31,385)	(31,385)
Balance December 31, 2023	42,973,215	$ 43	$ 401,638	$ 3,047	$ (319,644)	$ 85,084

See accompanying notes to the consolidated financial statements

QUINCE THERAPEUTICS, INC.
CONSOLIDATED STATEMENTS OF CASH FLOWS
(in thousands)

	Year Ended December 31,	
	2023	2022
Cash flows from operating activities		
Net Loss	$ (31,385)	$ (51,660)
Adjustments to reconcile net loss to net cash used in operating activities:		
Non-cash rent expense	—	(29)
Stock based compensation	5,220	16,618
Depreciation and amortization	322	204
Impairment loss on operating lease	66	136
Gain on sale of Legacy Assets	(78)	—
Loss on disposal of fixed assets	37	94
Change in the fair value of contingent consideration liabilities	1,578	—
Change in fair value of EIB loan	338	—
Non-cash goodwill impairment charge	—	825
Non-cash intangibles impairment charge	5,900	—
Amortization of discount on available-for-sale investments	(2,019)	(113)
Change in deferred tax liabilities due to acquisition of Novosteo, Inc.	(248)	(284)
Changes in operating assets and liabilities, net of acquisitions:		
Prepaid expenses and other current assets	4,246	2,580
Other assets	(9)	194
Accounts payable	(288)	(5,943)
Accrued expenses and other current liabilities	(1,972)	(6,660)
Net cash used in operating activities	(18,292)	(44,038)
Cash flow from investing activities:		
Purchase of investments	(113,781)	(75,021)
Proceeds from maturities of investments	111,209	82,493
Cash acquired from Novosteo, Inc.	—	10,593
Advancement of notes receivable	(1,000)	—
Cash paid in acquisition of EryDel S.p.A. net of cash acquired	(2,116)	—
Proceeds from disposal of assets	90	70
Purchase of property and equipment	(160)	(133)
Net cash provided by (used in) investing activities	(5,758)	18,002
Cash flows from financing activities:		
Payments of finance leases	(6)	(49)
Proceeds from issuance of common stock upon exercise of stock options	149	148
Proceeds from issuance of common stock in connection with open market sales agreement, net of issuance costs	—	608
Net cash provided by financing activities	143	707
Effect of exchange rate changes on cash	80	184
Net decrease in cash and cash equivalents	(23,827)	(25,145)
Cash and cash equivalents at beginning of period	44,579	69,724
Cash and cash equivalents at end of period	$ 20,752	$ 44,579
Supplemental disclosures of non-cash information:		
Net assets acquired of EryDel S.p.A. in exchange for common stock	$ 63,732	$ —
Net assets acquired of Novosteo, Inc. in exchange for common stock	$ —	$ 16,503
Right-of-use assets obtained in exchange for new operating lease liabilities	$ —	$ 411
Right-of-use asset and financing lease liability reduction as a result of lease modification	$ (70)	$ —
Right-of-use asset and operating lease liability reduction as a result of lease modification	$ —	$ (640)

See accompanying notes to the consolidated financial statements

QUINCE THERAPEUTICS, INC.
NOTES TO CONSOLIDATED FINANCIAL STATEMENTS

Note 1. Organization

Description of Business

Effective August 1, 2022, Cortexyme Inc. changed its name to Quince Therapeutics, Inc (the "Company"). The Company was incorporated in the State of Delaware in June 2012 and is headquartered in South San Francisco, California.

From its inception, the Company has been focused on novel therapeutic approaches to improve the lives of patients diagnosed with Alzheimer's and other degenerative diseases. The predecessor company, Cortexyme, Inc. was initially founded on the seminal discovery of the presence of Porphyromonas gingivalis, and its secreted toxic virulence factor proteases, called gingipains, in the relevant brain areas of both Alzheimer's and Parkinson's disease patients. In May 2022, the Company completed the acquisition of Novosteo focused on targeted therapeutics to treat rare skeletal diseases, bone fractures and injury. In 2023, the Company decided to discontinue the internal development of NOV004, which was acquired in the acquisition of Novosteo, and terminated the related license.

On October 20, 2023, the Company completed the acquisition of EryDel, a privately held, late-stage biotechnology company with a Phase 3 lead asset, EryDex, that targets the potential treatment of a rare neurodegenerative disease, A-T.

Novosteo Acquisition

On May 9, 2022, the Company entered into an Agreement and Plan of Merger and Reorganization (the "Merger Agreement") with Novosteo, Quince Merger Sub I, Inc., a Delaware corporation and a wholly owned subsidiary of the Company, Quince Merger Sub II, LLC, a Delaware limited liability company and a wholly owned subsidiary of Company, Novosteo, and Fortis Advisors LLC, a Delaware limited liability company, solely in its capacity as the securityholders' representative. The transaction closed on May 19, 2022. Pursuant to the terms of the Merger Agreement, at the closing of the Novosteo Acquisition, each share of capital stock of Novosteo that was issued and outstanding immediately prior to the Effective Time was automatically cancelled and converted into the right to receive 0.0911 shares of common stock, par value $0.001 per share, of the Company. The Company issued 5,520,000 shares and assumed 507,108 outstanding Novosteo options after conversion with the awards, retaining the same vesting and other terms and conditions as in effect immediately prior to consummation of the Novosteo Acquisition.

Pursuant to the Merger Agreement, upon the terms and subject to the conditions set forth therein, Merger Sub I merged with and into Novosteo (the "First Merger"), with Novosteo as the surviving entity in the First Merger (the "First Step Surviving Corporation"). Immediately following the First Merger, the First Step Surviving Corporation merged with and into Merger Sub II, with Merger Sub II surviving the Acquisition. Merger Sub II was renamed Novosteo, LLC and is a wholly-owned single member limited liability corporation.

Sale of Legacy Portfolio

On January 27, 2023, we sold our legacy small molecule protease inhibitor portfolio, including COR588, COR388, COR852, and COR803, pursuant to an asset purchase agreement with Lighthouse Pharmaceuticals, Inc., (the "Purchaser" or "Lighthouse") an entity co-founded by Casey Lynch, former chief executive officer of Cortexyme. Lighthouse is a variable interest entity but the Company is not the primary beneficiary.

Upon the consummation of the transaction, we received shares of common stock of Purchaser ("Common Stock") equal to seven and a half percent (7.5%) of the currently issued and outstanding Common Stock. The issuance is governed by a Stock Issuance Agreement entered into by us and the Purchaser on January 27, 2023 (the "Stock Agreement").

Pursuant to the terms of the asset purchase agreement, we are eligible to receive milestone payments up to $150 million on a product by product basis for the achievement of certain regulatory approvals and global net sales thresholds. Additionally, we are eligible to receive certain sales-based royalty payments on a product by product basis, ranging from high single-digit to mid-teens of annual net sales related to the two existing clinical stage programs, and low single-digit royalties for the preclinical programs, and certain sublicense income on a product by product basis, either in addition to milestone payments and royalties prior to Phase 2 initiation for COR588 or COR388, or in lieu of milestones payments and royalties after initiation of Phase 2 for COR588 or COR388 or for the preclinical programs.

EryDel Acquisition

On October 20, 2023, we completed our acquisition of EryDel, a privately held, late-stage biotechnology company with a Phase 3 lead asset, EryDex, that targets the potential treatment of a rare neurodegenerative disease, A-T.

The EryDel Acquisition was completed pursuant to that certain Stock Purchase Agreement, dated as of July 21, 2023. Pursuant to the terms of the Purchase Agreement, the Company issued 6,525,315 shares of its common stock to the EryDel Shareholders. Up to an additional 725,037 shares of the Company's common stock may be issued to the EryDel Shareholders upon the first anniversary of the closing of the EryDel Acquisition. The EryDel Shareholders have a contingent right to receive up to an aggregate of $485.0 million in potential cash payments, comprised of up to $5.0 million upon the achievement of a specified development milestone, $25.0 million at NDA acceptance by the FDA, up to $60.0 million upon the achievement of specified approval milestones, and up to $395.0 million upon the achievement of specified on market and sales milestones, with no royalties paid to EryDel. EryDel is a variable interest entity and the Company is the primary beneficiary and sole shareholder. Refer to Note 15 Business Combination for additional details.

Liquidity and Capital Resources

The Company has incurred losses and negative cash flows from operations since inception and expects to continue to generate operating losses for the foreseeable future. As of December 31, 2023, the Company had an accumulated deficit of $319.6 million. Since inception through December 31, 2023, the Company has funded operations primarily with the net proceeds from the issuance of convertible promissory notes, from the issuance of redeemable convertible preferred stock, from the net proceeds from the IPO and from the net proceeds from the PIPE Financing. As of December 31, 2023, the Company had cash, cash equivalents, and short-term investments of $75.1 million, which it believes will be sufficient to fund its planned operations for a period of at least 12 months from the date of the issuance of the accompanying consolidated financial statements.

Management expects to incur additional losses in the future to fund the Company's operations and conduct product research and development and may need to raise additional capital to fully implement its business plan. The Company may raise additional capital through the issuance of equity securities, debt financings or other sources including out-licensing or partnerships, in order to further implement its business plan. However, if such financing is not available when needed and at adequate levels, the Company will need to reevaluate its operating plan and may be required to delay the development of its product candidates.

Note 2. Summary of Significant Accounting Policies

Basis of Consolidation

The accompanying consolidated financial statements include the accounts of Quince Therapeutics, Inc. and its wholly owned subsidiaries. All intercompany balances and transactions have been eliminated upon consolidation.

Basis of Presentation

The accompanying consolidated financial statements and the notes thereto have been prepared in accordance with accounting principles GAAP pursuant to the instructions of the SEC on Form 10-K through the rules and interpretive releases of the SEC under federal securities law.

Use of Estimates

The preparation of the Company’s consolidated financial statements in conformity with GAAP requires management to make estimates and assumptions that affect the reported amounts of assets, liabilities, and expenses, as well as related disclosure of contingent assets and liabilities. The most significant estimates used in the Company’s consolidated financial statements relate to the determination of the fair value of stock-based awards and other issuances, determination of the fair value of identifiable assets and liabilities in connection with the acquisition of Novosteo, Inc. and EryDel S.p.A., including associated intangible assets and goodwill, contingent consideration, accruals for research and development costs, useful lives of long-lived assets, stock-based compensation and related assumptions, the incremental borrowing rate for leases and income tax uncertainties, including a valuation allowance for deferred tax assets, eligibility of expenses for the Australia research and development refundable tax credits, impairment of intangible assets or goodwill; and contingencies. The Company bases its estimates on historical experience and on various other market specific and other relevant assumptions that it believes to be reasonable under the circumstances, the results of which form the basis for making judgments about the carrying values of assets and liabilities that are not readily apparent from other sources. Actual results could differ materially from the Company’s estimates.

Foreign Currency Translation and Transactions

The functional currency of the Company's wholly-owned subsidiaries are the Australian Dollar and the Euro. The Company's financial results and financial position are translated into U.S. dollars using exchange rates at balance sheet dates for assets and liabilities and using average exchange rates for income and expenses. The resulting translation differences are presented as a separate component of accumulated other comprehensive loss, as a separate component of equity.

Foreign currency transactions are translated into the functional currencies using the exchange rates prevailing at the dates of the transactions. Foreign exchange gains and losses, resulting from the settlement of such transactions and from the re-measurement of monetary assets and liabilities denominated in foreign currencies using exchange rates at balance sheet date and non-monetary assets and liabilities using historical exchange rates, are recognized in the consolidated statements of operations and comprehensive income.

Risk and Uncertainties

The Company's future results of operations involve a number of risks and uncertainties. Factors that could affect the Company's future operating results and cause actual results to vary materially from expectations include, but are not limited to, uncertainty of results of clinical trials and reaching milestones, uncertainty of regulatory approval of the Company's drug candidates, uncertainty of market acceptance of the Company's drug candidates, competition from substitute products and larger companies, securing and protecting proprietary technology, strategic relationships and dependence on key individuals and sole source suppliers. The Company's drug candidates will require approvals from the FDA and comparable foreign regulatory agencies prior to commercial sales in their respective jurisdictions. There can be no assurance that any drug candidate will receive the necessary approvals.

Segments

The Company operates and manages its business as one reportable and operating segment, which is the business of developing and commercializing therapeutics. The Company's chief executive officer, who is the chief operating decision maker, reviews financial information on an aggregate basis for purposes of allocating and evaluating financial performance. All long-lived assets are maintained in Italy.

Business Combinations

The Company evaluates acquisitions of assets and other similar transactions to assess whether or not the transaction should be accounted for as a business combination or asset acquisition by first applying a screen to determine if substantially all of the fair value of the gross assets acquired is concentrated in a single identifiable asset or group of similar identifiable assets. If the screen is met, the transaction is accounted for as an asset acquisition. If the screen is not met, further determination is required as to whether or not the Company has acquired inputs and processes that have the ability to create outputs, which would meet the requirements of a business.

The Company accounts for business combinations using the acquisition method pursuant to the FASB ASC Topic 805. This method requires, among other things, that results of operations of acquired companies are included in the Company's financial results beginning on the respective acquisition dates, and that identifiable assets acquired and liabilities assumed are recognized at fair value as of the acquisition date. Intangible assets acquired in a business combination are recorded at fair value using one of three valuation approaches, the income approach, the market approach or the cost approach. The Company reviewed the three valuation approaches and determined the income approach was the most appropriate model to approximate fair value for both the Novosteo and EryDel Acquisitions. The income approach model requires assumptions about the timing and amount of future net cash flows, the cost of capital and terminal values from the perspective of a market participant. Any excess of the fair value of consideration transferred (the "Purchase Price") over the fair values of the net assets acquired is recognized as goodwill. The fair value of identifiable assets acquired and liabilities assumed in certain cases may be subject to revision based on the final determination of fair value during a period of time not to exceed 12 months from the acquisition date. Legal costs, due diligence costs, business valuation costs and all other acquisition-related costs are expensed when incurred.

Intangible Assets

Intangible assets with a definite useful life are amortized on a straight-line basis over the estimated useful life of the related assets. Intangible assets with an indefinite useful life are not amortized. Intangible assets acquired in a business combination that are used in research and development activities (regardless of whether they have an alternative future use) shall be considered indefinite lived until the completion or abandonment of the associated research and development efforts. Intangible assets acquired in a business combination are initially recorded at fair value. During the period that those assets are considered indefinite lived, they shall not be amortized but shall be tested for impairment. Once the research and development efforts are completed or abandoned, the entity shall determine the useful life of the assets. An intangible asset shall be tested for impairment annually and more frequently if events or

changes in circumstances indicate that it is more likely than not that the asset is impaired. The Company first assesses qualitative factors to determine whether it is more likely than not that the fair value of the intangible asset is less than its carrying amount, If that is the case, the Company performs a quantitative impairment test, and, if the carrying amount of the Company exceeds its fair value, then the Company will recognize an impairment charge for the amount by which its carrying amount exceeds its fair value, not to exceed the carrying amount of the intangible asset. Qualitative factors to be considered include but are not limited to:

- Cost factors such as increases in raw materials, labor, or other costs that have a negative effect on future expected earnings and cash flows.
- Legal/regulatory factors or progress and results of clinical trials.
- Other relevant entity-specific events such as changes in management, key personnel, strategy, or customers; contemplation of bankruptcy; or litigation that could affect significant inputs used to determine the fair value of the indefinite-lived intangible asset.
- Industry and market considerations such as a deterioration in the environment in which an entity operates, an increased competitive environment.
- Macroeconomic conditions such as deterioration in general economic conditions, limitations on accessing capital, fluctuations in foreign exchange rates, or other developments in equity and credit markets that could affect significant inputs used to determine the fair value of the indefinite-lived intangible asset.

Goodwill

Goodwill represents the excess of the purchase price over the fair value of the net assets acquired as of the acquisition date. Goodwill has an indefinite useful life and is not amortized. The Company reviews its goodwill for impairment at least annually or whenever events or changes in circumstances indicate that the carrying amount of the Company may exceed its fair value. The Company first assesses qualitative factors to determine whether it is more likely than not that the fair value of the Company is less than its carrying amount, including goodwill. If that is the case, the Company performs a quantitative impairment test, and, if the carrying amount of the Company exceeds its fair value, then the Company will recognize an impairment charge for the amount by which its carrying amount exceeds its fair value, not to exceed the carrying amount of the goodwill.

Contingent Consideration

The Company determines the acquisition date fair value of contingent consideration using a discounted cash flow method, with significant inputs that are not observable in the market and thus represents a Level 3 fair value measurement as defined in ASC Topic 820, Fair Value Measurement. The significant inputs in the Level 3 measurement not supported by market activity included our probability assessments of expected future cash flows related to the Company's acquisition of EryDel in October 2023, during the contingent consideration period, appropriately discounted considering the uncertainties associated with the earnout obligation, and calculated in accordance with the terms of the definitive agreement. The liabilities for the contingent consideration are established at the time of the acquisition and will be evaluated on a quarterly basis based on additional information as it becomes available. Any change in the fair value adjustment is recorded in the earnings of that period. During the year ended December 31, 2023, the Company recorded a $1.6 million adjustment to increase the fair value of its contingent consideration related to the acquisition of EryDel. The adjustment is reflected within operating loss on the consolidated statement of operations and comprehensive loss. Changes in the fair value of the contingent consideration obligations may result from changes in probability assumptions with respect to the likelihood of achieving the various contingent payment obligations. Significant increases or decreases in the inputs noted above in isolation would result in a significantly lower or higher fair value measurement.

Cash, Cash Equivalents and Investments

The Company considers all highly liquid investments with maturities of three months or less when purchased to be cash equivalents. Cash equivalents include marketable securities. Management determines the appropriate classification of its investments in debt securities at the time of purchase and at the end of each reporting period. Investments with original maturities beyond three months at the date of purchase and which mature at, or less than twelve months from the balance sheet date are classified as short-term investments. Investments with a maturity beyond twelve months from the balance sheet date are classified as long-term investments. Collectively, cash equivalents, short-term investments and long-term investments are considered available-for-sale and are recorded at fair value. Unrealized gains and losses are recorded as a component of other comprehensive loss in the consolidated statements of operations and included as a separate component of consolidated statements of stockholders’ equity (deficit). Realized gains and losses are included in interest income in the consolidated statements of operations and comprehensive loss.

Premiums (discounts) are amortized (accreted) over the life of the related investment as an adjustment to yield using the straight-line interest method. Dividend and interest income are recognized when earned. These amounts are recorded in "interest income" in the consolidated statements of operations and comprehensive loss.

Property and Equipment, Net

Property and equipment are stated at cost and reduced by accumulated depreciation. Depreciation expense is recognized using the straight-line method over the estimated useful lives of the respective assets. Depreciation and amortization begin at the time the asset is placed in service. Maintenance and repairs are charged to expense as incurred, and improvements are capitalized. When assets are retired or otherwise disposed of, the cost and accumulated depreciation are removed from the consolidated balance sheet and any resulting gain or loss is reflected in operations in the period realized.

The useful lives of property and equipment are as follows:

Computer equipment	3 years
Lab equipment	2.5 to 5 years
Finance lease right of use assets	Shorter of estimated useful life or lease term
Leasehold improvement	Shorter of estimated useful life or lease term
Office furniture	4 to 5 years

Concentration of Credit Risk

Cash equivalents, short-term and long-term investments are financial instruments that potentially subject the Company to concentrations of credit risk. The Company invests in money market funds, repurchase agreements, treasury bills and notes, government bonds, and corporate notes. The Company limits its credit risk associated with cash equivalents, short-term and long-term investments by placing them with banks and institutions it believes are highly credit worthy and in highly rated investments. However, cash balances in excess of Federal Deposit Insurance Corporation (FDIC) insured limit of $0.3 million are at risk.

Impairment of Long-Lived Assets

The Company reviews long-lived assets, including property and equipment, for impairment whenever events or changes in business circumstances indicate that the carrying amount of the assets may not be fully recoverable. An impairment charge would be recorded when estimated undiscounted future cash flows expected to result from the use of the asset and its eventual disposition are less than its carrying amount. Impairment, if any, is assessed using discounted cash flows or other appropriate measures of fair value.

The Company recognized impairment charges of $0.2 million related to the San Diego lease impairment loss and loss on disposal of fixed assets for the year ended December 31, 2022. The Company recognized an impairment charge $0.1 million for the Purdue lease the year ended December 31, 2023.

Leases

The Company determines if an arrangement includes a lease at inception. Right-of-use lease assets and lease liabilities are recognized based on the present value of the future minimum lease payments over the lease term at the commencement date. The right-of-use lease asset includes any lease payments made and excludes lease incentives. Incremental borrowing rate is used in determining the present value of future payments. The Company applies a portfolio approach to the property leases to apply an incremental borrowing rate to leases with similar lease terms. The lease terms may include options to extend or terminate the lease. The Company recognizes the options to extend the lease as part of the right-of-use lease assets and lease liabilities only if it is reasonably certain that the option would be exercised. Lease expense for minimum lease payments is recognized on a straight-line basis over the non-cancelable lease term.

Research and Development Expenses

Research and development costs are expensed as incurred. Research and development expenses consist primarily of personnel costs for the Company's research and product development employees. Also included are non-personnel costs such as professional fees payable to third parties for preclinical and clinical studies and research services, laboratory supplies and equipment maintenance, product licenses, and other consulting costs. The Company estimates preclinical and clinical study and research expenses based on the services performed, pursuant to contracts with CROs that conduct and manage preclinical and clinical studies and research services on its behalf. Expenses related to clinical studies are based on estimates of the services received and efforts

expended pursuant to contracts with many research institutions, clinical research organizations and other service providers that conduct and manage clinical studies on the Company's behalf. The financial terms of these agreements are subject to negotiation and vary from contract to contract and may result in uneven payment flows. Generally, these agreements set forth the scope of work to be performed at a fixed fee or unit price. Payments under the contracts are mainly driven by time and materials incurred by these service providers. Payments made to third parties under these arrangements in advance of the performance of the related services by the third parties are recorded as prepaid expenses until the services are rendered. Expenses related to clinical studies are generally recorded based on the timing of when services that have been performed on the Company's behalf by the service providers, clinical trial budgets and in accordance with the contracts and related amendments. The determination of timing involves reviewing open contracts and purchase orders, communicating with applicable personnel to identify the timing of when services that have been performed on the Company's behalf and estimating the level of service performed and the associated cost incurred for the service when the Company has not yet been invoiced or otherwise notified of actual cost. The Company periodically confirms the accuracy of estimates with the service providers and makes adjustments if necessary. Examples of estimated clinical expenses include:

- fees paid to CROs in connection with clinical studies;
- fees paid to investigative sites in connection with clinical studies;
- fees paid to contract manufacturers in connection with the production of clinical study materials; and
- fees paid to vendors in connection with preclinical development activities.

If the actual timing of the performance of services or the level of effort varies from the original estimates, the Company will adjust the prepaid or accrual accordingly. Payments associated with licensing agreements to acquire exclusive licenses to develop, use, manufacture and commercialize products that have not reached technological feasibility and do not have alternate commercial use are expensed as incurred.

Patent Costs

The Company has no historical data to support a probable future economic benefit for the arising patent applications, filing and prosecution costs. Therefore, patent costs are expensed as incurred.

Stock-Based Compensation

The Company accounts for stock-based compensation arrangements with employees in accordance with ASC 718, Compensation—Stock Compensation. Stock-based awards granted include stock options with service-based vesting. ASC 718 requires the recognition of compensation expense, using a fair value-based method, for costs related to all stock-based payments. The Company's determination of the fair value of stock options with service-based vesting on the date of grant utilizes the Black-Scholes option-pricing model and is impacted by its common stock price as well as other variables including: but not limited to, expected term that options will remain outstanding, expected common stock price volatility over the term of the option awards, risk-free interest rates and expected dividends. The fair value of a stock-based award is recognized over the period during which an optionee is required to provide services in exchange for the option award, known as the requisite service period (usually the vesting period) on a straight-line basis. Stock-based compensation expense is recognized based on the fair value determined on the date of grant and is reduced for forfeitures as they occur. Stock options exercised are issued new shares of our common stock.

Income Taxes

The Company accounts for income taxes under the asset and liability method. Current income tax expense or benefit represents the amount of income taxes expected to be payable or refundable for the current year. Deferred income tax assets and liabilities are determined based on differences between the consolidated financial statement reporting and tax bases of assets and liabilities and net operating loss and credit carryforwards and are measured using the enacted tax rates and laws that will be in effect when such items are expected to reverse. Deferred income tax assets are reduced, as necessary, by a valuation allowance when management determines it is more likely than not that some or all of the tax benefits will not be realized.

The Company accounts for uncertain tax positions in accordance with ASC 740-10, Accounting for Uncertainty in Income Taxes. The Company assesses all material positions taken in any income tax return, including all significant uncertain positions, in all tax years that are still subject to assessment or challenge by relevant taxing authorities. Assessing an uncertain tax position begins with the initial determination of the position's sustainability and is measured at the largest amount of benefit that is greater than fifty percent likely of being realized upon ultimate settlement. As of each balance sheet date, unresolved uncertain tax positions must be reassessed, and the Company will determine whether (i) the factors underlying the sustainability assertion have changed and (ii) the amount of the recognized tax benefit is still appropriate. The recognition and measurement of tax benefits requires significant judgment. Judgments concerning the recognition and measurement of a tax benefit might change as new information becomes available.

The Company includes any penalties and interest expense related to income taxes as a component of other expense, net and interest expense, net, as necessary.

Comprehensive Loss

The Company is required to report all components of comprehensive loss, including net loss, in the consolidated financial statements in the period in which they are recognized. Comprehensive loss is defined as a change in equity of a business enterprise during a period, resulting from transactions and other events and circumstances from non-owner sources. The Company had an unrealized gain and loss from its available-for sale securities and cumulative translation adjustment during the years ended December 31, 2023 and December 31, 2022, respectively, which are considered other comprehensive loss.

Net Loss per Share

Basic net loss per share is calculated by dividing the net loss by the weighted-average number of common shares outstanding during the period, without consideration for potentially dilutive securities. Diluted net loss per share is computed by dividing the net loss by the weighted-average number of common shares and common share equivalents of potentially dilutive securities outstanding for the period. For purposes of the diluted net loss per share calculation and common stock options are considered to be potentially dilutive securities. Because the Company reported a net loss for the years ended December 31, 2023 and December 31, 2022, and the inclusion of the potentially dilutive securities would be antidilutive, diluted net loss per share is the same as basic net loss per share for both periods.

Recent Accounting Pronouncements Adopted

Financial Instruments—Credit Losses: In June 2016, the FASB issued ASU 2016-13, Financial Instruments—Credit Losses (Topic 326): Measurement of Credit Losses on Financial Instruments which amends the principles around the recognition of credit losses by mandating entities incorporate an estimate of current expected credit losses when determining the value of certain assets. The guidance also amends reporting around allowances for credit losses on available-for-sale marketable securities. In November 2019, the FASB issued ASU 2019-10, Financial Instruments—Credit Losses (Topic 326), Derivatives and Hedging (Topic 815) and Leases (Topic 842): Effective Dates, which established that a one-time determination of the effective date for ASU 2016-13 would be based on the Company's SEC reporting status as of November 15, 2019. The Company was a "smaller reporting company" as defined by Item 10 of Regulation S-K, and therefore, ASU 2016-13 is effective for fiscal years beginning after December 15, 2022, including interim periods within those fiscal years. This guidance helps to provide financial statement users with more decision-useful information about the expected credit losses on financial instruments and other commitments to extend credit held by a reporting entity at each reporting date. To achieve this objective, the amendments in Topic 326 replace the incurred loss impairment methodology in current U.S. GAAP with a methodology that reflects expected credit losses and requires consideration of a broader range of reasonable and supportable information to inform credit loss estimates. The Company has adopted the new guidance as of January 1, 2023, and it did not have a material impact on its consolidated financial statements and related disclosures.

For available-for-sale debt securities in an unrealized loss position, the Company first assesses whether it intends to sell, or it is more likely than not that it will be required to sell the security before recovery of its amortized cost basis. If either of the criteria regarding intent or requirement to sell is met, the security's amortized cost basis is written down to fair value and recognized in interest and other income, net in the statement of operations and comprehensive loss. If neither criteria is met, the Company evaluates whether the decline in fair value is related to credit-related factors or other factors. In making this assessment, management considers the extent to which fair value is less than amortized cost, any changes to the rating of the security by a rating agency, and adverse conditions specifically related to the security, among other factors. Credit-related impairment losses, limited by the amount that the fair value is less than the amortized cost basis, are recorded through an allowance for credit losses in interest and other income, net.

Any unrealized losses from declines in fair value below the amortized cost basis as a result of non-credit factors are recognized in accumulated other comprehensive loss, net of tax as a separate component of stockholders' equity, along with unrealized gains. Realized gains and losses and declines in fair value, if any, on available-for-sale securities are included in interest and other income, net in the statement of operations and comprehensive loss.

For purposes of identifying and measuring credit-related impairments, the Company's policy is to exclude applicable accrued interest from both the fair value and amortized cost basis of the related security. The Company has elected to write-off uncollectible accrued interest receivable balances in a timely manner, which is defined by the Company as when interest due becomes 90 days delinquent. The accrued interest write-off will be recorded by reversing interest income. Accrued interest receivable is recorded in other current assets on the balance sheets.

Recent Accounting Pronouncements Not Yet Adopted

The following are new accounting pronouncements that the Company is evaluating for future impacts on its consolidated financial statements:

Improvements to Income Tax Disclosures (ASC 740); In December 2023, the FASB issued ASU No. 2023-09, "Improvements to Income Tax Disclosures." This ASU establishes new income tax disclosure requirements in addition to modifying and eliminating certain existing requirements. Under this ASU, entities must consistently categorize and provide greater disaggregation of information in the rate reconciliation. They must also further disaggregate income taxes paid. The ASU is effective in December 2024 under a prospective approach. Early adoption is permitted. Adoption of this ASU is not expected to have a material impact on the Company's consolidated financial statements.

Accounting Standard Update 2023-07, Segment Reporting (Topic 280): Improvements to Reportable Segment Disclosures ("ASU 2023-07"): In November 2023, the FASB issued ASU 2023-07, which is intended to improve reportable segment disclosure requirements, primarily through additional disclosures about significant segment expenses, including for single reportable segment entities. The standard is effective for fiscal years beginning after December 15, 2023, and interim periods within fiscal years beginning after December 15, 2024, with early adoption permitted. The amendments should be applied retrospectively to all prior periods presented in the financial statements. The Company is evaluating the disclosure requirements related to the new standard.

All other newly issued accounting pronouncements not yet effective have been deemed either immaterial or not applicable.

Note 3. Fair Value Measurements

The fair value of the Company's financial instruments reflects the amounts that the company estimates would receive in connection with the sale of an asset or pay in connection with the transfer of a liability in an orderly transaction between market participants at the measurement date (exit price). The Company discloses and recognizes the fair value of the assets and liabilities using a hierarchy that prioritizes the inputs to valuation techniques used to measure fair value. The hierarchy gives the highest priority to valuations based upon unadjusted quoted prices in active markets for identical assets or liabilities (Level 1 measurements) and the lowest priority to valuations based upon unobservable inputs that are significant to the valuation (Level 3 measurements). The guidance establishes three levels of the fair value hierarchy as follows:

Level 1 - Inputs that reflect unadjusted quoted prices in active markets for identical assets or liabilities that the Company has the ability to access at the measurement date.

Level 2 - Inputs other than quoted prices that are observable for the assets or liability either directly or indirectly, including inputs in markets that are not considered to be active.

Level 3 - Inputs that are unobservable. Assets and liabilities measured at fair value are classified in their entirety based on the lowest level of input that is significant to the fair value measurement.

The Company's financial instruments are carried in the accompanying consolidated balance sheets at amounts that approximate fair value.

The Company's assessment of the significance of a particular input to the fair value measurement in its entirety requires management to make judgments and consider factors specific to the asset or liability. The Company recognizes transfers between levels of the fair value hierarchy as of the end of the reporting period. There were no transfers within the hierarchy during the years ended December 31, 2023 and December 31, 2022.

The Company elected the fair value option for the EIB Loan assumed as part of the EryDel Acquisition. The Company adjusted the EIB Loan to fair value through the change in fair value of debt in the accompanying consolidated statements of operations and comprehensive loss. Subsequent unrealized gains and losses on items for which the fair value option is elected are reported in earnings. The Company will breakout any change in value due to credit loss in accumulated other comprehensive loss. For the year ended December 31, 2023 there was no change in value due to credit loss.

Financial assets and liabilities subject to fair value measurements on a recurring basis and the level of inputs used in such measurements by major security type as of December 31, 2023 and 2022 are presented in the following tables (in thousands):

	Fair Value Measurements at December 31, 2023			
	Total	Level 1	Level 2	Level 3
Assets:				
Money market funds	$ 4,285	$ 4,285	$ —	$ —
Certificates of Deposit	729	—	729	—
Government and agency notes	68,524	3,971	64,553	—
Total assets	$ 73,538	$ 8,256	$ 65,282	$ —
Liabilities:				
Accrued earnout	57,706	—	—	57,706
Long-term debt	13,429	—	—	13,429
Total liabilities	$ 71,135	$ —	$ —	$ 71,135

	Fair Value Measurements at December 31, 2022			
	Total	Level 1	Level 2	Level 3
Money market funds	$ 10,988	$ 10,988	$ —	$ —
Certificates of Deposit	6,102	—	6,102	—
Repurchase Agreements	9,000	—	9,000	—
Corporate notes	12,411	—	12,411	—
Government and agency notes	50,766	—	50,766	—
Municipal notes	506	—	506	—
Total	$ 89,773	$ 10,988	$ 78,785	$ —

The Company classifies corporate notes, certificates of deposit, repurchase agreements, municipal notes, and government and agency notes as Level 2 investments as the Company uses quoted prices for similar assets sourced from certain third-party pricing services. The third-party pricing services generally utilize industry standard valuation models for which all significant inputs are observable, either directly or indirectly, to estimate the price or fair value of the securities. The primary input generally includes reported trades of or quotes on the same or similar securities. The Company does not make additional judgments or assumptions made to the pricing data sourced from the third-party pricing services.

Contingent Consideration

The following table reflects the changes in present value of acquisition related accrued earnouts of contingent consideration liability using significant unobservable inputs (Level 3) for the year ended December 31, 2023 follows:

(in thousands)	
Beginning Balance as of January 1, 2023	$ —
Acquisition date fair value of contingent consideration	56,128
Change in fair value of contingent consideration	1,578
Ending Balance as of December 31, 2023	$ 57,706

The following table summarizes the quantitative information including the unobservable inputs related to the Company's acquisition related accrued earnout as of December 31, 2023:

Quantitative Information about Level 3 Fair Value Measurements

(in thousands)	**Fair Value at December 31, 2023**	**Valuation Technique**	**Unobservable Input**	**Range (Input Used)**
Accrued earnout	$ 57,706	Expected present value	Probability of achieving earnout objectives per the purchase agreement	0% - 100%

Long-term Debt

The following table presents the changes in the fair value of the Level 3 EIB Debt:

(in thousands)	
Beginning Balance as of January 1, 2023	$ —
Acquisition of EIB Debt	12,564
Change in fair value	338
Due to foreign currency translation	527
Ending Balance as of December 31, 2023	$ 13,429

The following table summarizes the quantitative information including the unobservable inputs related to the Company's acquisition related long term debt as of December 31, 2023:

Quantitative Information about Level 3 Fair Value Measurements

(in thousands)	Fair Value at December 31, 2023	Valuation Technique	Unobservable Input	Discount Rate (Input Used)
EIB loan	$ 13,429	Expected present value	Credit quality of company and credit spreads for comparable debt	13%

Note 4: Cash, Cash Equivalents and Investments

The following tables categorize the fair values of cash, cash equivalents, short-term investments and long-term investments measured at fair value on a recurring basis on our balance sheets (in thousands):

	December 31,	
	2023	2022
Cash and cash equivalents:		
Cash	$ 1,521	$ 3,986
Money market funds	4,285	10,988
Repurchase agreements	—	9,000
Government and agency notes	14,946	20,605
Total cash and cash equivalents	$ 20,752	$ 44,579
Short-term investments:		
Certificates of deposit	$ 729	$ 5,390
Municipal notes	—	506
Corporate notes	—	12,411
Government and agency notes	53,578	27,295
Total short-term investments	$ 54,307	$ 45,602
Long-term investments		
Certificates of deposit	$ —	$ 712
Government and agency notes	—	2,866
Total long-term investments	$ —	$ 3,578

The investments are classified as available-for-sale securities. As of December 31, 2023, the weighted average remaining contractual maturities of available-for-sale securities was approximately 2 months. At December 31, 2023 and 2022, the unrealized gain (loss) activity related to the Company’s available-for-sale securities is included in the Company’s accumulated other comprehensive loss. There were no significant realized gains or losses recognized on the sale or maturity of available-for-sale securities for the years ended December 31, 2023 and 2022 and as a result, the Company did not reclassify any amounts out of accumulated other comprehensive loss. Based on the Company’s review of its available-for-sale securities, the Company has a limited number of available-for-sale securities in insignificant loss positions as of December 31, 2023. No other-than-temporary impairments on these securities were recognized for the years ended as of December 31, 2023 and 2022.

For available-for-sale debt securities in an unrealized loss position, the Company first assesses whether it intends to sell, or it is more likely than not that it will be required to sell the security before recovery of its amortized cost basis. If either of the criteria regarding intent or requirement to sell is met, the security's amortized cost basis is written down to fair value and recognized in interest and other income, net in the statement of operations and comprehensive loss. If neither criteria is met, the Company evaluates whether the decline in fair value is related to credit-related factors or other factors. In making this assessment, management considers the extent to which fair value is less than amortized cost, any changes to the rating of the security by a rating agency, and adverse conditions specifically related to the security, among other factors. Credit-related impairment losses, limited by the amount that the fair value is less than the amortized cost basis, are recorded through an allowance for credit losses in interest and other income, net.

Any unrealized losses from declines in fair value below the amortized cost basis as a result of non-credit factors are recognized in accumulated other comprehensive loss, net of tax as a separate component of stockholders' equity, along with unrealized gains. Realized gains and losses and declines in fair value, if any, on available-for-sale securities are included in interest and other income, net in the statement of operations and comprehensive loss.

For purposes of identifying and measuring credit-related impairments, the Company's policy is to exclude applicable accrued interest from both the fair value and amortized cost basis of the related security. The Company has elected to write-off uncollectible accrued interest receivable balances in a timely manner, which is defined by the Company as when interest due becomes 90 days delinquent. The accrued interest write-off will be recorded by reversing interest income. Accrued interest receivable is recorded in other current assets on the balance sheets.

The following table summarizes the available-for-sale securities (in thousands):

	Fair Value Measurements at December 31, 2023			
	Amortized Cost	Unrealized Gains	Unrealized Losses	Fair Value
Money market funds	$ 4,285	$ —	$ —	$ 4,285
Certificates of Deposit	735	—	(6)	729
Government and agency notes	68,528	13	(17)	68,524
Total cash equivalents and investments	$ 73,548	$ 13	$ (23)	$ 73,538
Classified as:				
Cash equivalents (original maturities within 90 days)				$ 19,231
Short-term investments (maturities within 1 year)				54,307
Total cash equivalents and investments				$ 73,538

	Fair Value Measurements at December 31, 2022			
	Amortized Cost	Unrealized Gains	Unrealized Losses	Fair Value
Money market funds	$ 10,988	$ —	$ —	$ 10,988
Certificates of Deposit	6,237	1	(136)	6,102
Repurchase Agreements	9,000	—	—	9,000
Corporate notes	12,575	—	(164)	12,411
Government and agency notes	51,020	4	(258)	50,766
Municipal notes	510	—	(4)	506
Total cash equivalents and investments	$ 90,330	$ 5	$ (562)	$ 89,773
Classified as:				
Cash equivalents (original maturities within 90 days)				$ 40,593
Short-term investments (maturities within 1 year)				45,602
Long-term investments (maturities beyond 1 year)				3,578
Total cash equivalents and investments				$ 89,773

The table below summarizes the unrealized losses of the Company's investments in debt securities measured at fair value as of December 31, 2023 (in thousands):

	Less than twelve months		Twelve months or greater		Total	
	Fair value	Gross unrealized loss	Fair value	Gross unrealized loss	Fair value	Gross unrealized loss
Certificates of deposit	$ —	$ —	$ 729	$ (6)	$ 729	$ (6)
Government and agency notes	3,966	—	2,975	(17)	6,941	(17)
Total cash equivalents and investments	$ 3,966	$ —	$ 3,704	$ (23)	$ 7,670	$ (23)

There were no transfers between Levels 1, 2 or 3 for the period presented.

The table below summarizes the contractual maturities of the Company's investments in debt securities measured at fair value as of December 31, 2023 (in thousands):

	Maturities by Period				
	Total	Less Than 1 Year	1-5 Years	6-10 Years	More Than 10 Years
Fair value of debt securities	$ 54,307	$ 54,307	$ —	$ —	$ —

Note 5: Balance Sheet Components

Prepaid Expenses and Other Current Assets

Prepaid expenses and other current assets consisted of the following (in thousands):

	December 31,	
	2023	2022
Prepaid expenses	$ 365	$ 223
Prepaid insurance	809	977
Prepaid research and development expenses	133	1,088
Australia research and development refundable tax credit	—	1,003
Short-term Italian research and development refundable tax credit	993	—
Other current assets	81	276
Total prepaid expenses and other current assets	$ 2,381	$ 3,567

EryDel is eligible to obtain an R&D tax credit as companies in Italy that invest in eligible research and development activities, regardless of the legal form and economic sector in which they operate, can benefit from a R&D tax credit. Such tax credits can only be used to offset payments of certain taxes and contributions (e.g., social contributions, VAT payables, registration fees, income and withholding taxes and all other tax-related items that companies usually pay monthly). The Company recognized reductions to R&D expense of $0.2 million and $0 for the years ended December 31, 2023 and 2022, respectively.

Cortexyme Australia, Pty, Ltd, a wholly-owned subsidiary of the company is eligible to obtain a cash refund from the Australian Taxation Office for eligible R&D expenditures under the Australian R&D Tax Incentive Program (the “Australian Tax Incentive”). The Australian Tax Incentive is recognized as a reduction to R&D expense when there is reasonable assurance that the relevant expenditure has been incurred, the amount can be reliably measured and that the Australian Tax Incentive will be received. The Company recognized reductions to R&D expense of $0 and $0.6 million for the years ended December 31, 2023 and 2022, respectively. The Company received a refundable tax credit of $0.5 million in the year ended December 31, 2023, which reduced prepaid expenses and other current assets by $0.5 million as of December 31, 2023.

Novosteo Pty, Ltd, a wholly-owned subsidiary of Novosteo, LLC, is eligible to obtain a cash refund from the Australian Taxation Office for eligible R&D expenditures under the Australian Tax Incentive as well. The Company received a refundable tax credit of $0.5 million in the first quarter of 2023, which reduced prepaid expenses and other current assets by $0.5 million as of March 31, 2023.

Other Assets

Other assets consisted of the following (in thousands):

	December 31, 2023	December 31, 2022
VAT receivable	$ 3,463	$ —
Long-term Italian research and development refundable tax credit	4,993	—
Total other assets	$ 8,456	$ —

Assets Held for Sale

Assets held for sale consist of the following (in thousands):

	December 31, 2023	December 31, 2022
Assets held for sale	$ 10	$ —
Total assets held for sale	$ 10	$ —

Property and equipment, net

Property and equipment, net consisted of the following (in thousands):

	December 31, 2023	December 31, 2022
Computer equipment	$ 36	$ 18
Computer software	30	—
Lab equipment	486	415
Finance lease right of use assets	—	124
Leasehold improvement	36	21
Office furniture	153	—
Less: accumulated amortization and depreciation	(507)	(185)
Property and equipment, net	$ 234	$ 393

Depreciation and amortization expense for property and equipment was $0.3 million and $0.2 million for the years ended December 31, 2023 and 2022, respectively.

Accrued Expenses and Other Current Liabilities

Accrued expenses and other current liabilities consisted of the following (in thousands):

	December 31, 2023	December 31, 2022
Personnel expenses	$ 2,340	$ 1,130
Professional fees	211	234
Research and development expenses	564	497
Current portion of operating lease liabilities	64	377
Current portion of finance lease liability	—	76
Other	257	185
Total accrued expenses and other current liabilities	$ 3,436	$ 2,499

Below is the severance accrual activity included in the personnel expenses in the above table related to a cost reduction program during the years ended December 31, 2023 and December 31, 2022 (in thousands):

	For the year ended December 31, 2023	2022
Beginning accrued severance	$ —	$ —
Incurred during the period	770	3,942
Severance paid during the period	(429)	(3,942)
Ending accrued severance	$ 341	$ —

In response to the reprioritization of the Company's pipeline following the decision to discontinue internal development of NOV004 and to pursue out-licensing opportunities, the Board approved a cost reduction program to reorganize operations and allow continued support for the needs of the business. Under the cost reduction program, the Company lowered headcount through a reduction in workforce. The Company recognized the severance of $0.3 million and related expenses of $0.1 million over the requisite employment obligation period. The reduction in force was completed in April 2023.

On August 4, 2023, the Company entered into a transition and separation agreement with Karen Smith, M.D., Ph.D., (the "Separation Agreement") in connection with Dr. Smith's transition and departure from the Company as the Company's Chief Medical Officer, effective as of September 1, 2023. Pursuant to the Separation Agreement, the Company is required to pay cash severance, equal to her annual salary, in the aggregate amount of $0.5 million, of which $0.1 million was recognized during the year ended December 31, 2023. The severance is paid on regular payroll schedule through the third quarter of 2024. Additionally, pursuant to the Separation Agreement, the Company paid an additional cash bonus severance payment equal to 100% of Dr. Smith's target annual bonus opportunity for 2023 on a prorated basis, an additional cash severance payment equal to 12 months' of the monthly premiums for health care continuation benefits, and provided for 50% accelerated vesting with respect to Dr. Smith's equity award. The acceleration of 612,141 options and 54,757 RSAs resulted in a stock-based compensation expense of approximately $0.1 million.

Note 6. Leases

Real Estate Operating Leases

In June 2022, the Company entered into a Sublease Agreement to rent office space in South San Francisco, California. The Sublease Agreement commenced on June 18, 2022 and ended on November 30, 2023. The total payments under the term of the lease were approximately $0.3 million. The Company paid a security deposit of $17,000 which is included in Prepaid Expenses and Other Current Assets on the December 31, 2023 consolidated balance sheets and the deposit was refunded in January 2024. At the commencement of the lease, the Company recorded an operating lease right of use asset and liability of $0.3 million. On October 31, 2023, the Company signed a lease agreement for a smaller office space in South San Francisco, California. The total payments under the new lease are expected to be approximately $22,000 for the year. The lease commenced on November 30, 2023 and will end on November 30, 2024.

In October 2022, the Company entered into a lease agreement to rent space in West Lafayette, Indiana. The lease agreement amended the original lease to transfer liability to the Company due to the Novosteo Acquisition. The lease agreement is for 15 months, which commenced on October 1, 2022 and ended on December 31, 2023. The total payments under the term of the lease were approximately $0.2 million. At the commencement of the lease, the Company recorded an operating lease right of use asset and liability of $0.1 million.

In December 2022, the Company entered into an amendment to the lease agreement of the rental space in West Lafayette to rent additional space in the same facility under the same terms as its existing facility lease except the terms of payment. Under the terms of the amendment, the Company will pay rent monthly for the additional space. The Company recorded an operating lease right of use asset and liability of $10,000.

In February 2023, as a result of the decision to discontinue internal development of NOV004 and to pursue out-licensing opportunities, the Company entered into a sublease agreement as the lessor for the majority of the West Lafayette facility. The lease commenced on March 17, 2023 and ended on December 31, 2023. The sublessee paid the Company a security deposit of $6,000 which is included in Accrued expenses and other current liabilities on the December 31, 2023 consolidated balance sheet. Under the terms of the sublease, the Company is entitled to receive a total rental income that is expected to offset rent expense of $57,000. As a result of this decision and the sublease agreement, the Company recorded an impairment loss of approximately $66,000 which is included in other expense, net per the consolidated statement of operations and comprehensive loss for the year ended December 31, 2023.

In October 2023, as part of the EryDel Acquisition the Company acquired operating leases in which the Company recorded an operating lease right of use asset and liability in total of $0.4 million. This includes a lease agreement renting office space in Bresso, in the Province of Milan, Italy. The Lease Agreement commenced on September 1, 2016, and will end on August 31, 2028. At acquisition date, the Company recorded an operating lease right of use asset and liability of $0.1 million. This also includes a lease agreement renting office space in Medolla, in the Province of Modena, Italy. The Lease Agreement commenced on June 18, 2018, with a duration of 12 years, until January 31, 2030. At acquisition date, the Company recorded an operating lease right of use asset and liability of $0.2 million. The acquired lease agreements also included other operating leases two of which are car leases and the other a printing press which in total the Company recorded an operating lease right of use asset and liability of $31,000.

In January 2024, the above-mentioned Lease Agreement for the office space in Medolla (Italy) was renegotiated. The new Lease Agreement commenced on February 1, 2024, and will end on January 31, 2036, substituting the Lease Agreement commenced in June 2018.

The Company recognizes lease expense on a straight-line basis over the term of its operating lease. As of December 31, 2023, total future rent expense from all real estate operating leases of $0.4 million will be recognized over the remaining term of 29 to 73 months on a straight-line basis over the respective lease period.

Clinical Equipment Financing Lease

As part of the Novosteo Acquisition, the Company acquired a financing lease for certain lab equipment. The Company recognizes the amortization expense in research and development expenses in the consolidated statements of operations and comprehensive loss and recognizes expense on a straight-line basis starting when the equipment is placed into service until the end of the remaining contract term of 18 months. Amortization expense of the financing lease right of use asset for the year ended December 31, 2023 was $6,000. Amortization expense of the financing lease right of use asset for the year ended December 31, 2022 was $50,000.

In February 2023, as a result of the decision to discontinue internal development of NOV004 and to pursue out-licensing opportunities, the Company exercised its purchase option for the financed equipment in order to resell and this equipment is currently held on consignment and is included in assets held for sale on the December 31, 2023 consolidated balance sheet. As a result of this action, the Company reduced the Finance lease ROU asset and Finance lease liability by approximately $70,000.

Supplemental balance sheet information related to leases as follows (in thousands except lease terms and discount rates):

	December 31, 2023	December 31, 2022
Operating lease right of use asset, net	$ 385	$ 291
Short-term operating lease liability	64	377
Long-term operating lease liability	321	—
	$ 385	$ 377
Finance lease right of use asset	—	124
Finance lease accumulated amortization	—	(50)
Total finance lease right of use asset, net	$ —	$ 74
Weighted average remaining lease term		
Operating leases	5.4 years	0.9 years
Finance leases	—	1.0 year
Weighted average discount rate		
Operating leases	7.95%	5.71%
Finance leases	—%	4.45%

Year ended December 31,	Operating Lease
2024	91
2025	91
2026	88
2027	81
2028	69
Thereafter	48
Total lease payments	469
Less: imputed interest	(83)
Total remaining lease liability	$ 385

Lease costs for the years ended December 31, 2023 and 2022 were approximately:

	Years ended December 31,	
	2023	2022
Lease costs:		
Finance lease amortization of right of use assets	$ 6	$ 50
Operating lease costs	266	572
Short-term lease costs	96	75
Total lease costs	$ 368	$ 697

Note 7. Long-term Debt

In connection with the acquisition of EryDel on October 20, 2023, the Company assumed an unsecured line of credit between EryDel and the European Investment Bank (the "EIB Loan"). The EIB Loan was amended and restated as of the acquisition date. The EIB Loan provides for maximum borrowings of 30.0 million euro through four tranches; tranche A, 3.0 million euro; tranche B, 7.0 million euro; tranche C, 10.0 million euro; and tranche D, 10.0 million euro. Each tranche is subject to conditions precedent related to the Company's business and capitalization. The unused portions of each tranche may be canceled by the Company at any time, subject to a cancellation fee. As of December 31, 2023, only tranches A and B have been drawn. All amounts due under tranche A and B are payable on their maturity date of August 2026. Tranche C and D are payable in equal installments of principal together with all amounts outstanding under the tranches on the repayment dates specified in the relevant Disbursement Offer. The first Repayment Date of tranche C shall fall not earlier than twelve months from the Disbursement Date of such tranche. The last Repayment Date of tranche C and tranche D shall fall not later than 5 years from the Disbursement Date of tranche C and tranche D, respectively. The EIB Loan bears interest at fixed rates for each tranche and is payable on the maturity date for each Tranche. The fixed rates range from 7.0% to 9.0% per annum. As of December 31, 2023, principal of 10.0 million euros ($11.0 million) was outstanding on the EIB Loan and it is recorded as Long-term debt on the consolidated balance sheet at fair value with imputed interest of 9.0% included.

The Company may voluntarily prepay, in whole or in part with a prepayment premium. In the event of an occurrence of an event of default or a change in control, as specified in the Debt Agreement, the Company will be required to prepay the outstanding EIB Debt.

The Debt Agreement includes a provision for additional remuneration to be paid in addition to interest. The amount of additional remuneration to be paid is equal to 2.5% of revenue up to 125.0 million euros, plus 1.85% of revenue between 125.0 and 250.0 million euros, plus 1.0% of revenue in excess of 250.0 million euros, multiplied by a varying percentage based on how many tranches have been drawn. The varying percentage is equal to 30.0% in the event tranche A has been drawn, 50.0% in the event tranche A and B have been drawn, 80.0% in the event tranche A, B and C have been drawn, and 100.0% in the event all four tranches have been drawn. The additional remuneration is payable for seven years, during the period January 1, 2026, through December 31, 2032. In the event of an occurrence of an event of default or prepayment, the Company may be required to pay an additional remuneration buyout fee.

The Company elected to account for the EIB Loan at fair value, which requires the EIB Loan to be recorded at fair value at issuance and at the end of each reporting period. Gains or losses upon remeasurement are to be recorded in other income (expense) in the consolidated statements of operations and comprehensive income. The Company presents separately in other comprehensive income the portion of the total change in the fair value of the EIB Loan that results from a change in instrument-specific credit risk. The EIB Loan's fair value at the date it was assumed adjusted its carrying value based on using a discounted cash flow analysis with a discount rate based on a yield curve that was adjusted for credit rating. The change in fair value at December 31, 2023 was determined using a discounted cash flow analysis discounted at the market yield. The significant inputs used to measure the market yield at December 31, 2023 relative to the date the EIB Loan was assumed was the change in credit quality of the Company, the change in credit spreads for comparable debt instruments, and the change in the risk-free rate. As of December 31, 2023, the fair value of the EIB Loan is valued at $13.4 million, which includes a fair value adjustment of $0.3 million and foreign currency translation of $0.5 million, from the amount recorded at fair value on the date of the EryDel Acquisition of $12.6 million.

Future minimum principal payments, as of December 31, 2023 are as follows (in thousands):

Year Ending December 31,	Amount
2024	$ —
2025	—
2026	11,053
2027 and thereafter	—
Total future minimum payments	11,053
Imputed interest	2,376
Total Debt as of December 31, 2023	$ 13,429

Note 8. Commitments and Contingencies

Legal Matters

The Company's industry is characterized by frequent claims and litigation, including claims regarding intellectual property. As a result, the Company may be subject to various legal proceedings from time to time. The results of any future litigation cannot be predicted with certainty, and regardless of the outcome, litigation can have an adverse impact on the Company because of defense and settlement costs, diversion of management resources and other factors. Management is not aware of any pending or threatened litigation.

Indemnification

As permitted under Delaware law and in accordance with the Company's bylaws, the Company indemnifies its officers and directors for certain events or occurrences while the officer or director is or was serving in such capacity. The Company is also party to indemnification agreements with its directors. The Company believes the fair value of the indemnification rights and agreements is minimal. Accordingly, the Company has not recorded any liabilities for these indemnification rights and agreements as of December 31, 2023 and 2022.

Contingencies

From time to time, the Company may have certain contingent liabilities that arise in the ordinary course of its business activities. The Company accrues a liability for such matters when it is probable that future expenditures will be made, and such expenditures can be reasonably estimated.

Note 9. Equity Incentive Plans

The Company operates three stock plans as of December 31, 2023.

- 2019 Equity Incentive Plan (Quince)
- 2019 Equity Incentive Plan (Novosteo)
- 2022 Inducement Plan (Quince)

2019 Equity Incentive Plan (Quince)

On December 4, 2014, the Company's stockholders approved the 2014 Stock Plan ("2014 Plan"), and on April 25, 2019 amended, restated and re-named the 2014 Plan as the 2019 Equity Incentive Plan (the "Quince 2019 Plan"), which became effective as of May 7, 2019, the day prior to the effectiveness of the registration statement filed in connection with the IPO. The remaining shares available for issuance under the 2014 Plan were added to the shares reserved for issuance under the Quince 2019 Plan.

The Quince 2019 Plan provides for the grant of stock options (including incentive stock options and non-qualified stock options), stock appreciation rights, restricted stock, RSUs, performance units, and performance shares to the Company's employees, directors, and consultants. The maximum aggregate number of shares that may be issued under the Quince 2019 Plan is 10,036,489 shares of the Company's common stock. In addition, the number of shares available for issuance under the Quince 2019 Plan will be annually increased on the first day of each of its fiscal years beginning with fiscal 2020, by an amount equal to the least of (i) 2,146,354 shares of common stock; (ii) 4% of the outstanding shares of its common stock as of the last day of its immediately preceding fiscal year; and (iii) such other amount as the Company's Board of Directors may determine.

The Quince 2019 Plan may be amended, suspended or terminated by the Company's Board of Directors at any time, provided such action does not impair the existing rights of any participant, subject to stockholder approval of any amendment to the Quince 2019 Plan as required by applicable law or listing requirements. Unless sooner terminated by the Company's Board of Directors, the 2019 Plan will automatically terminate on April 23, 2029.

Stock Options

Stock options under the 2019 Plan may be granted for periods of up to 10 years and at prices no less than 100% of the estimated fair value of the shares on the date of grant as determined by the board of directors. If, at the time of grant, the optionee directly owns stocks representing more than 10% of the voting power of all our outstanding capital stock, the exercise price for these options must be at least 110% of the fair value of the underlying common stock. Stock options granted to employees and non-employees generally have a maximum term of ten years and vest over four years from the vesting commencement date, of which 25%

vest on the one-year anniversary of the vesting commencement date, and 75% vest in equal monthly installments over the remaining three years or monthly vesting over 3 to 4 years. We may grant options with different vesting terms from time to time. Unless an employee's or non-employee's termination is due to cause, disability or death, upon termination of service, any unexercised vested options will be forfeited at the end of the three months from the termination date or expiration of the option, whichever is earlier.

Activity for service-based stock options under the Quince 2019 Plan is as follows:

	Number of Options and Unvested Shares	Weighted Average Exercise Price	Weighted average remaining contractual life (years)	Aggregate intrinsic value
				(In thousands)
Balance at December 31, 2021	**5,571,293**	$ 28.70	8.26	$ 15,687
Options granted	2,051,058	8.13	—	—
Options exercised	(102,152)	1.45	—	—
Options cancelled / forfeited	(4,200,488)	29.30	—	—
Balance at December 31, 2022	**3,319,711**	$ 16.07	4.77	$ 65
Options granted	3,346,958	1.03	—	—
Options exercised	(258,705)	0.42	—	—
Options cancelled / forfeited	(2,140,786)	16.52	—	—
Balance at December 31, 2023	**4,267,178**	$ 5.00	8.85	$ 197
Options vested and expected to vest as of December 31, 2023	4,267,178	5.00	8.85	197
Options exercisable at December 31, 2023	1,109,464	$ 14.09	7.32	$ 34

Aggregate intrinsic value represents the difference between the Company's estimated fair value of its common stock as of their respective balance sheet dates and the exercise price of outstanding options. The total intrinsic value of the Quince 2019 Plan options exercised was $0.3 million and $1.4 million for the years ended December 31, 2023 and 2022, respectively. The weighted-average grant date fair value of options granted during the years ended December 31, 2023 and 2022 was $0.82 and $6.05 per share, respectively. The total estimated grant date fair value of options vested during each of the years ended December 31, 2023 and 2022 was $11.1 million and $31.8 million, respectively.

In 2023 and 2022 , the Company recognized $2.6 million and $11.4 million, respectively, of stock-based compensation expense related to options granted to employees and non-employees. The compensation expense is allocated on a departmental basis, based on the classification of the option holder. No income tax benefits have been recognized in the consolidated statement of operations and comprehensive loss for stock-based compensation arrangements. As of December 31, 2023, total unamortized employee stock-based compensation was $4.0 million, which is expected to be recognized over the remaining estimated vesting period of 2.55 years.

Restricted Stock Units

RSUs are share awards that entitle the holder to receive freely tradable shares of the Company's common stock upon vesting. The fair value of RSUs is based upon the closing sales price of the Company's common stock on the grant date. RSUs granted to employees generally vest over a 2–4 year period.

The following table summarizes activity under the Company's RSUs from the Quince 2019 Plan and related information:

	Restricted Stock Units Outstanding	
	Number of Shares	Weighted Average Grant Date Fair Value
Unvested - December 31, 2022	**30,876**	**$ 4.30**
RSUs granted	—	—
RSUs vested	(10,200)	4.30
RSUs cancelled	(19,188)	4.30
Unvested - December 31, 2023	**1,488**	**$ 4.30**

The fair value of the RSUs is determined on the grant date based on the fair value of the Company's common stock. The fair value of the RSUs is recognized as expense ratably over the vesting period of two years. The total grant date fair value of the

RSUs vested during the years ended December 31, 2023 and 2022 was $44,000 and $1,700,000, respectively. The aggregate intrinsic value of the shares of the RSUs vested during the years ended December 31, 2023 and December 31, 2022 were $11,000 and $1.1 million, respectively.

For the years ended December 31, 2023 and 2022, the Company recognized stock-based compensation expense of $37,000 and $1,337,000, respectively, related to these RSUs. As of December 31, 2023, the total unamortized stock-based compensation related to RSUs was $4,000, which is expected to be recognized over the remaining estimated vesting period of 0.17 years.

2019 Equity Incentive Plan (Novosteo)

On May 19, 2022, in accordance with the term of the Merger Agreement, the Company assumed the 2019 Novosteo Equity Incentive Plan (the "2019 Novosteo Plan"). The 2019 Novosteo Plan provides for the grant of stock options (including incentive stock options and non-qualified stock options), stock appreciation rights, restricted stock, RSUs, performance units, and performance shares to the Novosteo legacy employees. On the closing date, each outstanding Novosteo stock option granted under Novosteo's equity compensation plans was converted into a corresponding stock option with the number of shares underlying such option and the applicable exercise price adjusted based and adjusted into the right to purchase 0.0911 shares of common stock. Each such converted stock option will continue to be subject to substantially the same terms and conditions as applied to the corresponding Novosteo stock option prior to the Acquisition. The maximum aggregate number of shares that may be issued under the 2019 Novosteo Plan is 544,985 shares of the Company's common stock.

The 2019 Novosteo Plan may be amended, suspended or terminated by the Board at any time, provided such action does not impair the existing rights of any participant, subject to stockholder approval of any amendment to the 2019 Novosteo Plan as required by applicable law or listing requirements. Unless sooner terminated by the Board, the 2019 Novosteo Plan will automatically terminate on May 20, 2029.

Stock options under the 2019 Novosteo Plan may be granted for periods of up to 10 years and at prices no less than 100% of the estimated fair value of the shares on the date of grant as determined by the board of directors. If, at the time of grant, the optionee directly owns stocks representing more than 10% of the voting power of all our outstanding capital stock, the exercise price for these options must be at least 110% of the fair value of the underlying common stock. Stock options granted to employees and non-employees generally have a maximum term of ten years and vest over four years from the vesting commencement date, of which 25% vest on the one-year anniversary of the vesting commencement date, and 75% vest in equal monthly installments over the remaining three years or monthly vesting over 3 to 4 years. We may grant options with different vesting terms from time to time. Unless an employee's or non-employee's termination is due to cause, disability or death, upon termination of service, any unexercised vested options will be forfeited at the end of the three months from the termination date or expiration of the option, whichever is earlier.

Activity for service-based stock options under the 2019 Novosteo Plan is as follows:

	Number of Options and Unvested Shares	Weighted Average Exercise Price	Weighted average remaining contractual life (years)	Aggregate intrinsic value
				(In thousands)
Balance at December 31, 2021	—	$ —	—	$ —
Options assumed	507,648	0.55	—	—
Options exercised	—	—	—	—
Options cancelled / forfeited	(4,543)	0.55	—	—
Balance at December 31, 2022	**503,105**	$ 0.55	9.23	$ 44
Options granted	—	—	—	—
Options exercised	(105,808)	0.55	—	40
Options cancelled / forfeited	(86,866)	0.55	—	—
Balance at December 31, 2023	**310,431**	$ 0.55	8.23	$ 155
Options vested and expected to vest as of December 31, 2023	310,431	0.55	8.23	155
Options exercisable as of December 31, 2023	135,812	$ 0.55	8.23	$ 68

For the years ended December 31, 2023 and 2022, the Company recognized stock-based compensation expense of $275,000 and $245,000, respectively, related to options granted to employees and non-employees for the 2019 Novosteo plan. The compensation expense is allocated on a departmental basis, based on the classification of the option holder. No income tax benefits have been recognized in the consolidated statement of operations and comprehensive loss for stock-based compensation arrangements. As of December 31, 2023, total unamortized employee stock-based compensation was $0.4 million, which is expected to be recognized over the remaining estimated vesting period of 2.23 years.

The total aggregate intrinsic value of the Novosteo 2019 Plan options exercised was $427,000 and $0 for the years ended December 31, 2023 and 2022. The weighted-average grant date fair value of options granted during the years ended December 31, 2023 and 2022 was $0 and $2.51 per share, respectively. The total estimated grant date fair value of options vested during the years ended December 31, 2023 and 2022 was $0.3 million and $0.3 million, respectively.

On May 19, 2022, in accordance with the term of the Merger Agreement, the Company assumed a number of RSAs agreements with certain employees. Each outstanding Novosteo RSA was converted into a corresponding RSA with the number of shares underlying such RSA adjusted into 0.0911 shares of common stock. Each such converted RSA will continue to be subject to substantially the same terms and conditions as applied to the corresponding Novosteo RSA prior to the Acquisition.

Restricted Stock Awards

	Restricted Stock Awards Outstanding	
	Number of Shares	Weighted Average Grant Date Fair Value
Unvested - December 31, 2022	**427,401**	**$ 3.30**
RSAs issued	—	—
RSAs vested	(188,344)	3.30
RSAs cancelled	(63,293)	3.30
Unvested - December 31, 2023	**175,764**	**$ 9.90**

For the years ended December 31, 2023 and 2022, the Company recognized stock-based compensation expense of $496,000 and $338,000, respectively, related to restricted stock awards. The compensation expense is allocated on a departmental basis, based on the classification of the option holder. No income tax benefits have been recognized in the consolidated statement of operations and comprehensive loss for stock-based compensation arrangements. As of December 31, 2023, total unamortized employee stock-based compensation was $0.6 million, which is expected to be recognized over the remaining estimated vesting period of 1.74 years.

2022 Inducement Plan

On May 9, 2022, the Company's board of directors approved 4,000,000 shares of the Registrant’s common stock that may be offered or issued under the Quince Therapeutics, Inc. 2022 Inducement Plan. The 2022 Inducement Plan was adopted by the independent members of the Board without stockholder approval pursuant to Rule 5635(c)(4) of the Nasdaq Listing Rules. In accordance with rule awards under those plans may only be made to an employee who has not previously been an employee or member of the Board or of any board of directors of any parent or subsidiary of the Company, or following a bona fide period of non-employment by the Company or a parent or subsidiary, if he or she is granted such award in connection with his or her commencement of employment with the Company or a subsidiary and such grant is an inducement material to his or her entering into employment with the Company or such subsidiary. The terms and conditions of the 2022 Inducement Plan are substantially similar to those of the Quince 2019 Plan.

Options under the 2022 Inducement Plan may be granted for periods of up to 10 years at prices no less than 100% of the estimated fair value of the shares on the date of grant as determined by the board of directors. Options granted to employees may have different performance goals or other vesting provisions (including continued employment) in accordance with Award Agreement. Unless an employee's termination service is due to disability or death, upon termination of service, any unexercised vested options will be forfeited at the end of the three months or expiration of the option, whichever is earlier.

Activity for service-based stock options under the 2022 Inducement Plan is as follows:

	Number of Options and Unvested Shares	Weighted Average Exercise Price	Weighted average remaining contractual life (years)	Aggregate intrinsic value
				(In thousands)
Balance at December 31, 2021	—	$ —	—	$ —
Options granted	3,744,255	2.98	—	—
Options exercised	—	—	—	—
Options cancelled / forfeited	(2,000)	2.98	—	—
Balance at December 31, 2022	**3,742,255**	$ 2.98	9.39	$ —
Options granted	—	—	—	—
Options exercised	—	—	—	—
Options cancelled / forfeited	(1,408,949)	2.98	—	—
Balance at December 31, 2023	**2,333,306**	$ 2.98	8.39	$ —
Options vested and expected to vest as of December 31, 2023	2,333,306	2.98	8.39	—
Options exercisable as of December 31, 2023	923,599	$ 2.98	8.39	$ —

For the years ended December 31, 2023 and 2022, the Company recognized stock-based compensation expense of $1,802,000 and $1,293,000, respectively, related to options granted to employees and non-employees for the 2022 Inducement plan. The compensation expense is allocated on a departmental basis, based on the classification of the option holder. No income tax benefits have been recognized in the consolidated statement of operations and comprehensive loss for stock-based compensation arrangements. As of December 31, 2023, total unamortized employee stock-based compensation was $3.2 million, which is expected to be recognized over the remaining estimated vesting period of 2.39 years.

The total aggregate intrinsic value of the 2022 Inducement Plan options exercised was $0 for both the years ended December 31, 2023 and 2022. The weighted-average grant date fair value of options granted during the years ended December 31, 2023 and 2022 was $0.00 and $2.26 per share, respectively. The total estimated grant date fair value of options vested during the years ended December 31, 2023 and 2022 was $2.1 million and $0, respectively.

Stock-Based Compensation Expense

The following table summarizes employee and non-employee stock-based compensation expense for the years ended December 31, 2023 and 2022 and the allocation within the consolidated statements of operations and comprehensive loss (in thousands):

	2023	2022
General and administrative expense	$ 4,003	$ 10,225
Research and development expense	1,217	6,393
Total stock-based compensation	$ 5,220	$ 16,618

The Company estimates the fair value of its service-based stock option awards utilizing the Black-Scholes option pricing model, which is dependent upon several variables, such as expected term, volatility, risk-free interest rate, and expected dividends. Each of these inputs is subjective and generally requires significant judgment to determine. Stock-based compensation is measured at the grant date based on the fair value of the award and is recognized as expense, over the requisite service period, which is generally the vesting period of the respective award. The Company recognizes compensation on a straight-line basis over the requisite vesting period for each award. Forfeitures are recognized as they occur. The following weighted average assumptions were used to calculate the fair value of stock-based compensation for the years ended December 31, 2023 and 2022:

	2023	2022
Expected volatility	106.36%	89.98%
Expected dividend yield	— %	— %
Expected term (in years)	6.22	6.23
Risk-free interest rate	4.01%	2.67%

Expected Term — The Company has opted to use the "simplified method" for estimating the expected term of options, whereby the expected term equals the arithmetic average of the vesting term and the original contractual term of the option (generally 10 years). The expected term was estimated using the simplified method for employee stock options since the Company does not have adequate historical exercise data to estimate the expected term.

Expected Volatility—Due to the Company's limited operating history and a lack of company specific historical and implied volatility data, the Company has based its estimate of expected volatility on the historical volatility of its own stock and the stock of companies within its defined peer group. The historical volatility data was computed using the daily closing prices for the selected companies' shares during the equivalent period of the calculated expected term of the stock-based awards.

Risk-Free Interest Rate — The risk-free rate assumption is based on the U.S. Treasury instruments with maturities similar to the expected term of the Company's stock options.

Expected Dividend — The Company has not issued any dividends in its history and does not expect to issue dividends over the life of the options and therefore has estimated the dividend yield to be zero.

Fair value of Common Stock — The board of directors uses the closing price of stock on the date of grant to determine the fair value. The board of directors intends all options granted to be exercisable at a price per share not less than the estimated per share fair value of common stock underlying those options on the date of grant.

Employee Stock Purchase Plan

On April 24, 2019, the Company's Board of Directors adopted its 2019 Employee Stock Purchase Plan ("2019 ESPP"), which was subsequently approved by the Company's stockholders and became effective on May 7, 2019, the day immediately prior to the effectiveness of the registration statement filed in connection with the IPO. The 2019 ESPP is intended to qualify as an "employee stock purchase plan" within the meaning of Section 423 of the Code for U.S. employees. In addition, the 2019 ESPP authorizes grants of purchase rights that do not comply with Section 423 of the Code under a separate non-423 component for non-U.S. employees and certain non-U.S. service providers. The Company has reserved 1,494,530 shares of common stock for issuance under the 2019 ESPP. In addition, the number of shares reserved for issuance under the 2019 ESPP will be increased automatically on the first day of each fiscal year for a period of up to ten years, starting with the 2020 fiscal year, by a number equal to the lesser of: (i) 536,589 shares; (ii) 1% of the shares of common stock outstanding on the last day of the prior fiscal year; or (iii) such lesser number of shares determined by the Company's Board of Directors. The 2019 ESPP is expected to be implemented through a series of offerings under which participants are granted purchase rights to purchase shares of the Company's common stock on specified dates during such offerings. The Company has not yet approved an offering under the 2019 ESPP.

Note 10. Common Stock

Equity Transactions

On December 23, 2021, the Company entered into an Open Market Sales Agreement, with Jefferies, whereby the Company may sell up to $150.0 million in aggregate proceeds of common stock from time to time, through Jefferies as our sales agent. During the years ended December 31, 2023 and 2022, the Company sold zero and 51,769 shares of common stock, respectively, under this agreement and received net proceeds of $0 and $0.6 million, respectively.

Common Stock

The Company had reserved shares of common stock for future issuance as follows:

	December 31,	
	2023	2022
Options issued and outstanding under the Quince 2019 Stock Plan	4,267,178	3,319,711
Shares available for issuance under Quince 2019 Stock Plan	4,005,784	3,747,309
Shares available for issuance under the Employee Stock Purchase Plan	1,494,530	1,133,165
Options issued and outstanding under the Novosteo 2019 Plan	310,431	503,105
Shares available for issuance under Novosteo 2019 Plan	246,797	41,880
Options issued and outstanding under the 2022 Inducement Plan	2,333,306	3,742,255
Shares available for issuance under 2022 Inducement Plan	1,666,694	257,745
Total	14,324,720	12,745,170

The Company is authorized to issue 100,000,000 shares of common stock with a par value of $0.001 per share. Each share of common stock is entitled to one vote. The holders of common stock are also entitled to receive dividends whenever funds are legally available and when and if declared by the board of directors, subject to the prior rights of holders of any preferred stock that may be outstanding at the time. The Company has never declared any dividends on common stock. As of December 31, 2023 and 2022, the Company had 42,973,215 and 36,136,480 shares of common stock issued and outstanding, respectively.

In addition, in April 2023, we implemented the Rights Agreement, also called a “poison pill,” that may have the effect of discouraging or preventing a change of control by, among other things, making it uneconomical for a third party to gain control of us through open market accumulation of shares without paying all stockholders an appropriate control premium or without the consent of our board of directors. The Rights will expire on April 5, 2024, unless the Rights are earlier redeemed or exchanged by the Company.

Note 11. Related Party Transactions

David Lamond, Chairperson of the Board of Quince Therapeutics, Inc. was a director and an equity holder in Novosteo which Quince acquired on May 19, 2022. The shares of Novosteo beneficially owned by Mr. Lamond were automatically cancelled and converted into the right to receive shares of Quince common stock in accordance with the terms of the Merger Agreement. The respective boards of directors of Quince and Novosteo approved the Merger Agreement, and the Novosteo’s stockholders adopted the Merger Agreement upon recommendation of the Novosteo board of directors. Mr. Lamond was not part of either company's special committees that evaluated the Novosteo Acquisition and recused himself from board meetings where the Novosteo Acquisition was discussed.

Dirk Thye, M.D., Chief Executive Officer, is an investor in Morphimmune Inc. and Philip Low, Ph.D, a former Board member of Quince Therapeutics, Inc., is a co-founder and Board member of Morphimmune Inc. During the year ended December 31, 2023, the Company sold certain lab equipment to Morphimmune Inc. for $80,000 as well as signed a sublease with Morphimmune as the sublessee with total payments of approximately $57,000 for the lease term of March 17, 2023 through December 31, 2023.

Note 12. Income taxes

The components of the Company's loss before income taxes were as follows (in thousands):

	Year ended December 31,	
	2023	2022
United States	$ (27,375)	$ (48,191)
International	(4,207)	(3,753)
Total	$ (31,582)	$ (51,944)

The components of the Company's benefit for income taxes were as follows:

(in thousands)	Year ended December 31, 2023	2022
Current expense (benefit):		
Federal	$ —	$ —
State	—	—
Foreign	61	—
Total current expense (benefit):	61	—
Deferred expense (benefit):		
Federal	(248)	284
State	—	—
Foreign	(10)	—
Total deferred expense (benefit):	(258)	284
Total income tax expense (benefit)	$ (197)	$ 284

The provision for income taxes differs from the amount expected by applying the federal statutory rate to the loss before taxes as follows:

	Year ended December 31, 2023	2022
Federal statutory income tax rate	21.00 %	21.00 %
State income taxes	4.47	0.89
Income tax credits	1.07	1.49
Stock based compensation	(26.35)	(0.82)
Non-deductible expenses and others	(1.25)	(3.38)
Change in valuation allowance	1.68	(18.63)
	0.62 %	0.55 %

As of December 31, 2023 and 2022, the components of the Company's deferred tax assets are as follows (in thousands):

	Year ended December 31, 2023	2022
Deferred tax asset:		
Federal and State net operating loss carryforwards	$ 81,881	$ 49,481
Stock based compensation	2,401	9,667
Other accruals	527	515
Capitalized research and development expense	3,220	3,094
Tax credits	8,343	7,970
Disallowed interest expense carryforward	1,043	—
Gross deferred tax asset	97,415	70,727
Valuation allowance	(85,111)	(69,692)
Total deferred tax assets	12,304	1,035
Deferred tax liabilities:		
Property and equipment	—	(11)
Capitalized leases	—	(32)
IP R&D	(17,608)	(1,239)
Gross deferred tax liabilities	(17,608)	(1,282)
Net deferred tax liabilities	$ (5,304)	$ (247)

Deferred income taxes reflect the net tax effects of (a) temporary differences between the carrying amounts of assets and liabilities for financial reporting purposes and the amounts used for income tax purposes, and (b) operating losses and tax credit carryforwards.

The Company's accounting for deferred taxes involves the evaluation of a number of factors concerning the realizability of its net deferred tax assets. The Company primarily considered such factors as its history of operating losses, the nature of the Company's deferred tax assets, and the timing, likelihood and amount, if any, of future taxable income during the periods in which those temporary differences and carryforwards become deductible. At present, the Company does not believe that it is more likely than not that the deferred tax assets will be realized; accordingly, a full valuation allowance has been established and a deferred tax liability has been recorded as shown in the accompanying balance sheets. The valuation allowance increased by approximately $15.4 million and $11.1 million, respectively for the years ended December 31, 2023 and 2022.

At December 31, 2023, the Company has federal net operating loss carryforwards of approximately $238.4 million of which $222.6 million will not expire and $15.8 million begin expiring in 2034. The Company also has state net operating loss carryforwards of approximately $34.2 million which begin to expire in 2034. Additionally, the Company has federal tax credits of approximately $9.8 million which begin to expire in 2036 and state tax credits of approximately $2.9 million which do not expire.

At December 31, 2023, the Company has foreign net operating loss carryforwards, primarily in Italy, of approximately $121.1 million, which have no expiration date.

Use of the net operating loss and credit carryforwards may be subject to a substantial annual limitation due to the ownership change provisions of U.S. tax law and similar state provisions. The annual limitation may result in the expiration of net operating losses and credits before use.

Pursuant to the Code Sections 382 and 383, annual use of a company's U.S. NOL and research and development credit carryforwards may be limited if there is a cumulative change in ownership of greater than 50% within a three-year period. The amount of the annual limitation is determined based on the value of the Company immediately prior to the ownership change. Subsequent ownership changes may further affect the limitation in future years. If limited, the related tax asset would be removed from the deferred tax asset schedule with a corresponding reduction in the valuation allowance. The Company has completed such an analysis pursuant to Sections 382 and 383 in prior years which determined that ownership changes occurred on December 22, 2015 and May 13, 2019, which had no impact on the NOLs available to offset future income. The Company has rolled forward the analysis through December 31, 2023 and no additional ownership changes have occurred.

The Company follows the provisions of the FASB ASC 740-10, Accounting for Uncertainty in Income Taxes. ASC 740-10 prescribes a comprehensive model for the recognition, measurement, presentation and disclosure in the consolidated financial statements of uncertain tax positions that have been taken or expected to be taken on a tax return. It is the Company's policy to include penalties and interest related to income tax matters in income tax expense.

The Company is subject to taxation in the United States, Australia, and Italy. Because of the net operating loss and research credit carryforwards, all of the Company's tax years, from 2013 to 2023, remain open to U.S. federal, California, other state tax examinations. The Company's Australian subsidiaries remain open to examination from their inception to 2023. The Company's Italian subsidiary remain open to examination from their inception to 2023. The majority of our unrecognized tax benefits would not impact our effective tax rate due to a valuation allowance offsetting our deferred tax assets. The impact on our effective tax rate of recognizing unrecognized tax benefits is approximately $0.5 million. There were interest and penalties of $0.1 million and $0 accrued at December 31, 2023 and 2022, respectively. The Company does not expect that our uncertain tax positions will materially change in the next twelve months.

A reconciliation of the beginning and ending amount of unrecognized tax benefits is as follows (in thousands):

	Year ended December 31,	
	2023	2022
Beginning balance	$ 3,688	$ 3,249
Additions for tax positions taken in a prior year	200	—
Additions for tax positions taken in a current year	451	439
Ending balance	$ 4,339	$ 3,688

Note 13. Net Loss per Share

The following table sets forth the computation of basic and diluted net loss per share (in thousands except for share and per share amounts):

	December 31,	
	2023	2022
Numerator:		
Net loss	$ (31,385)	$ (51,660)
Denominator:		
Weighted average common shares outstanding	37,237,149	33,496,534
Net loss per share, basic and diluted	$ (0.84)	$ (1.54)

The following outstanding potentially dilutive securities were excluded from the computation of diluted net loss per share for the periods presented because including them would have been antidilutive:

	December 31,	
	2023	2022
Stock options issued and outstanding	6,910,915	7,565,071
Restricted stock units	1,488	30,876
Restricted stock awards	175,764	427,401
Total	7,088,167	8,023,348

Note 14. Employee Benefit Plan

The Company sponsors a 401(k) defined contribution plan for its US employees. This plan provides for pre-tax and post-tax contributions for all US employees. Employee contributions are voluntary. Employees may contribute up to 100% of their annual compensation to this plan, as limited by an annual maximum amount as determined by the Internal Revenue Service. The Company may match employee contributions, and may make profit sharing contributions, in amounts to be determined at the Company's sole discretion. The amount of contributions that the Company made to the 401(k) Plan during the years ended December 31, 2023 and 2022 was $0.1 million and $0.2 million, respectively.

The Company has defined benefit plans, regulated by the Italian laws in which the Company's non-US employees participate in. The benefits due to employees under the defined benefit plans are calculated based on the employee compensation and the duration of the employment relationship and are paid to the employee upon termination of the employment relationship or retirement. The costs of the defined benefit plans reported in the Company's consolidated statements of operations and comprehensive loss is determined by an actuarial calculation performed on an annual basis. The actuarial valuation is performed using the "Projected Unit Credit Method" based on the employees' expected date of separation or retirement.

Note 15. Business Combination

EryDel business combination

On October 20, 2023, the Company completed its acquisition of EryDel, a privately held, late-stage biotechnology company with a lead Phase 3 lead asset, EryDex, that targets the potential treatment of a rare neurodegenerative disease, A-T. The acquisition will drive Quinces' next stage of growth, as EryDel's proprietary drug-device combination technology platform and promising late-stage clinical asset represents an opportunity for the Company to expand into several debilitating rare diseases where chronic corticosteroid treatment is the standard of care – or could be in the absence of long-term corticosteroid toxicity. The Company accounted for this acquisition in accordance with ASC 805, Business Combinations, which requires the assets acquired and the liabilities assumed to be measured at fair value at the date of the acquisition. As part of the acquisition of EryDel, the Company recorded deferred tax liability of $5.1 million and uncertain tax position liability of $0.5 million against Goodwill.

The acquisition date fair value of the consideration transferred for EryDel was approximately $66.9 million, which consisted of the following (in thousands):

		Fair Value of Consideration
Cash	$	2,615
Quince Therapeutics common stock (7,250,352 shares)		7,164
Contingent consideration		56,128
Settlement of preexisting notes receivable		1,000
Fair value of total consideration transferred	$	66,907

The fair value of the Company's common stock was determined based on the closing market price of the Company's common stock of $0.989 per share on the acquisition date. The aggregate stock consideration consists of 6,525,315 shares of Company's common stock issued at closing and 725,037 shares of common stock (the "Indemnity Holdback Shares") withheld by the Company for general representations and warranties. The Indemnity Holdback Shares will be issued to the EryDel Shareholders upon the first anniversary of the closing of the acquisition, subject to reduction for any indemnification claims, if any. Any indemnification claims after the acquisition date will result in an adjustment to the consideration transferred if the indemnification claim is made before the end of the measurement period. Any indemnification claim after the end of the measurement period will be recognized in the Company's consolidated statements of operations and comprehensive loss. The Company has included the total fair value of the stock consideration within additional-paid-in capital and common stock.

The contingent consideration arrangement requires the Company to pay $485.0 million of additional consideration in cash, comprised of up to $5.0 million upon the achievement of a specified development milestone, $25.0 million at NDA acceptance, up to $60.0 million upon the achievement of specified approval milestones, and up to $395.0 million upon the achievement of specified on market and sales milestones. The Company estimated the fair value of the contingent consideration using a probability-weighted discounted cash flow model. This fair value measurement is based on significant inputs not observable in the market and thus represents a Level 3 measurement as defined in ASC 820. The key assumptions in applying the income approach are as follows: 15% discount rate, probability of achievement, 0% to 100%, each of the milestones and future revenues from commercialization over the contingent consideration period. No contingent consideration is payable unless and until the milestones are achieved. The fair value of each milestone after the acquisition is reassessed, with the subsequent change in fair value recorded in the Company's consolidated statements of operations and comprehensive loss. As of December 31, 2023, there were no significant changes in the range of outcomes for the contingent consideration recognized as a result of the EryDel acquisition.

Prior to the acquisition the Company had a preexisting relationship with EryDel. The Company had advanced $1.0 million to EryDel pursuant to a promissory note agreement. At the date of the acquisition, the Company had notes receivable due from EryDel of $1.0 million, including interest receivable of $1,300. As a result of the EryDel Acquisition, the promissory note between EryDel Italy and EryDel was effectively settled and recognized as additional consideration.

The following table summarizes the allocation of the consideration paid for EryDel to the estimated fair value of the assets acquired and liabilities assumed at the acquisition date, with the excess recorded to goodwill (in thousands):

Assets acquired:		Preliminary Purchase Price Allocation
Cash	$	560
Tax assets		10,187
Other current assets		644
Property and equipment, net		238
Operating lease right-of-use assets, net		383
Other non-current assets		14
Intangible assets		61,096
Goodwill		16,929
Total assets acquired		90,051
Liabilities assumed:		
Trade payables		(1,685)
Accrued expenses and other current liabilities		(2,943)
Debt, non-current		(12,564)
Other non-current liabilities		(854)
Deferred tax liability		(5,098)
Total liabilities assumed		(23,144)
Fair value of total consideration transferred	$	66,907

The purchase price allocation is preliminary and may change as a result of additional information obtained regarding assets acquired and liabilities assumed and revisions of estimates of fair values of intangible assets and related deferred tax assets and liabilities. The Company will finalize its valuation and the allocation of the purchase price, along with required retrospective adjustments, if any, within a year following the acquisition date.

The fair value of identifiable Acquired IPR&D intangible assets was $60.6 million. IPR&D was determined using the Multi-Period Excess Earnings Method ("MPEEM") under the income approach. MPEEM calculates the economic benefits by determining the income attributable to an intangible asset after the returns are subtracted for contributory assets such as working capital, assembled workforce, and fixed assets. The resulting after-tax net earnings were discounted at 16.6%, a rate commensurate with the risk inherent in the economic benefit projections of the assets. The probability-weighted, projected cash flows were calculated based on projections of revenues and expenses related to the asset and were assumed to extend through a multi-year projection period. The IPR&D has an indefinite useful life, and as such, is not amortized but rather tested for impairment at least annually.

The fair value of tradename intangible assets was $0.5 million. The tradename intangible assets were derived using the relief from royalties method under the income approach, utilizing royalty rate of 0.3%. This approach is used to estimate the cost savings that accrue for the owner of an intangible asset who would otherwise have to pay royalties or licensing fees on revenues earned through the use of the asset if they had not owned the rights to use the assets. The probability-weighted, net after-tax royalty savings are calculated for each year in the remaining economic life of the intangible asset and discounted to present value using a discount rate of 16.6%. The trademark has a useful life of 21 years. The trademark is amortized on a straight-line basis over the useful life.

The excess of the fair value of purchase consideration over the fair value of net tangible and identifiable intangible assets acquired was recorded as goodwill, which is primarily attributed to the assembled workforce and expanded global market opportunities. None of the goodwill is expected to be deductible for income tax purposes. Goodwill is not amortized but is tested for impairment at least annually, refer to Note 16 for this assessment.

The transaction costs associated with the acquisition were approximately $2.5 million, of which $2.3 million were recorded in general and administrative expenses and $0.2 million were recorded in research and development expenses in the consolidated statement of operations and comprehensive loss.

The Company has included the financial results of EryDel in the consolidated financial statements from the date of acquisition. From October 21, 2023 through December 31, 2023, the Company recognized no revenue and a net loss of $3.7 million attributable to EryDel.

The following unaudited pro forma information gives effect to the acquisition of EryDel as if it had been completed on January 1, 2022 (the beginning of the comparable prior reporting period), including pro forma adjustments primarily related to

amortization of acquired intangible assets, tax benefit from release of the valuation allowance and the inclusion of acquisition-related expenses reflected in the revenue and net loss (in thousands):

	For the year ended December, 31	
	2023	2022
Revenue	$ —	$ —
Net Loss	(40,265)	(67,580)

The 2023 supplemental pro forma earnings were adjusted to exclude $2.5 million of acquisition-related costs incurred in 2023, the 2022 pro forma earnings were adjusted to include these charges.

Novosteo business combination

On May 19, 2022, the Company completed the Novosteo Acquisition. Pursuant to the terms of the Merger Agreement, at the closing of the Novosteo Acquisition (the "Effective Time"), each share of capital stock of Novosteo (the "Novosteo Capital Stock") that was issued and outstanding immediately prior to the Effective Time was automatically cancelled and converted into the right to receive 0.0911 shares of common stock, par value $0.001 per share, of the Company (the "Company Common Stock"). These shares included options to purchase an aggregate of 507,108 shares of the Company Common Stock upon conversion of the outstanding Novosteo options based on the Company Option Exchange Ratio (as defined in the Merger Agreement), with the awards retaining the same vesting and other terms and conditions as in effect immediately prior to consummation of the Acquisition. These options, as well as 519,216 unvested restricted shares were concluded to be post-combination expense and were excluded from purchase consideration.

The Company has included the financial results of Novosteo in the consolidated financial statements from the date of the Acquisition and recorded immaterial amounts of expenses and earnings since the period from May 19, 2022 through December 31, 2023. The transaction costs associated with the Acquisition were approximately $1.1 million and were recorded in general and administrative expense. The acquisition date fair value of the consideration transferred for Novosteo was approximately $16,502,587, which consisted of 5,000,784 shares at $3.30 per share.

The Company accounted for the Acquisition as a business combination in accordance with ASC 805. The Company applied the acquisition method, which requires the identifiable assets acquired and liabilities assumed be recorded at fair value with limited exceptions. The following table summarizes the fair values of the identifiable assets acquired and liabilities assumed as the final determination of the date of acquisition (in thousands):

	May 19, 2022
Identifiable assets acquired and liabilities assumed:	
Cash and cash equivalents	$ 10,593
Prepaid expenses and other current assets	1,040
ROU asset	124
Property and equipment	279
In-process Research and Development	5,900
Accounts payable and accrued liabilities	(1,726)
Deferred tax liabilities	(532)
Net assets acquired	15,678
Goodwill	$ 825

The final determination of the fair value of assets and liabilities have been completed within the one-year measurement period as required by ASC 805. As part of the valuation analysis, the fair value of the intangible assets was estimated by discounting forecasted risk adjusted cash flows at a rate that approximated the cost of capital of a market participant. Management's forecast of future cash flows was based on the income approach. Significant estimates, all of which are considered Level 3 inputs, were used in the fair value methodology, including the Company's forecast regarding its future operations and likeliness of obtaining approval to sell its products, as well as other market conditions. The Company recorded no measurement period adjustments for the years ended December 31, 2023 and 2022. All subsequent adjustments will be recorded to earnings.

The excess of the fair value of purchase consideration over the fair value of net tangible and identifiable intangible assets acquired was recorded as goodwill, which is primarily attributed to the assembled workforce and expanded market opportunities, for which there is no basis for U.S. income tax purposes. Goodwill amounts are not amortized but are rather tested for impairment at least annually, see Note 16 for this assessment. Goodwill is not deductible for tax purposes.

The Intangible asset balance above is attributable to in-process research and development with an indefinite useful life.

The amounts of Novosteo's net loss was $9.4 million included in the Company's consolidated statement of operations and comprehensive loss for the year ended year ended December 31, 2022. The amount of Novosteo's revenue was $0 for the year ended December 31, 2022. The unaudited pro forma revenue and net loss of the combined entity had the acquisition date been January 1, 2021 are as follows (in thousands):

	For the year ended December 31, 2022
Revenue	$ 262
Net loss	(52,592)

The 2022 supplemental pro forma earnings were adjusted to exclude $2.2 million of acquisition-related costs incurred in 2022, the 2021 pro forma earnings were adjusted to include these charges. The Company's consolidated statements of operations and comprehensive loss for the year ended December 31, 2022 include immaterial net revenue and net loss attributable to the Acquisition.

Note 16. Intangible Assets

EryDel Intangible Assets

The following table provides details of the carrying amount of the Company's indefinite-lived intangible asset (in thousands):

	As of December 31, 2023
Unamortized intangible assets:	
In-process research and development	$ 60,636
Impairment charge	—
Foreign currency translation adjustments	2,561
Balance as of December 31, 2023	$ 63,197

The following table provides details of the carrying amount of the Company's finite-lived intangible asset (in thousands, except useful life):

	Useful life	For the year ended December 31, 2023
Finite life intangible assets:		
Trade name	21 years	$ 460
Impairment charge		—
Intangible asset amortization		(4)
Foreign currency translation adjustments		19
Balance as of December 31, 2023		$ 475

The Company performs annual impairment reviews of its intangible assets during the fourth fiscal quarter or more frequently if appropriate. The Company did not incur any impairment losses related to its EryDel intangible assets during the year ended December 31, 2023.

Novosteo Intangible Assets

The intangible asset acquired as a result of the Novosteo Acquisition consists of in-process research and development ("IPR&D") related to NOV004, the Company's bone targeting molecule designed to accelerate fracture repair. The value of the IPR&D was determined using discounted probable future cash flows. Significant assumptions used in determining the value of the intellectual property include the initiation of clinical trials and NDA approval with respect to NOV004, probability of reaching various phases of development, costs and cost of goods sold, and the risk adjusted discount rate applied to the cash flows.

The following table provides details of the carrying amount of the Company's indefinite-lived intangible asset (in thousands):

	As of December 31, 2023
Unamortized intangible assets:	
In-process research and development	$ 5,900
Impairment charge	(5,900)
Balance as of December 31, 2023	$ —

In January 2023, the Company decided to discontinue the internal development of NOV004 and pursue out-licensing opportunities. As a result, several of the assumptions used in determining the initial fair value have changed including discount rate and expected cash flows and thus triggered the need for an interim impairment assessment as required under ASC 350. As a result, the fair value was determined to be significantly below its carrying value and the Company recognized an impairment charge of $5.9 million during the year ended December 31, 2023.

On December 22, 2023, the Company, following its prior decision to discontinue internal development of NOV004, approved that certain Mutual Termination of License Agreement (the “Termination Agreement”) by and between the Company and Purdue Research Foundation ("PRF") to terminate the License Agreement dated June 3, 2020, as amended on March 17, 2022, July 22, 2022, and June 23, 2023 (the “License Agreement”). Under the License Agreement, the Company obtained from PRF an exclusive worldwide license under certain bone fracture repair related patents and technology developed by Purdue University, including patents claiming NOV004 and related compounds and use of such compounds in the treatment of bone fractures.

Under the Termination Agreement, the License Agreement was terminated effective as of October 31, 2023. The Company agreed to reimburse PRF for certain fees and costs incurred in connection with the prosecution of the licensed patents prior to termination. The Company also agreed to assign to PRF certain documents and materials developed by the Company in connection with the development of the licensed product under the License Agreement, subject to the Company’s retained right to use such documents and materials for internal research purpose.

Goodwill

The following table summarizes the changes in the carrying amount of goodwill (in thousands):

Balance as of December 31, 2021	$ —
Additions[(a)]	825
Impairment charge	(825)
Balance as of December 31, 2022	—
Additions[(a)]	16,929
Foreign currency translation adjustments	696
Balance as of December 31, 2023	$ 17,625

(a) Goodwill additions related to the acquisition of Novosteo in second quarter of 2022 and the acquisition of EryDel in the fourth quarter of 2023 (see Note 15).

There was no amount related to goodwill related to the Novosteo acquisition reflected on the consolidated balance sheet for the Company as of December 31, 2022. In 2022, management performed an impairment evaluation of goodwill related to the Novosteo acquisition after assessing qualitative factors that indicated a possible impairment of goodwill. Under the qualitative assessment, management considers relevant events and circumstances including but not limited to macroeconomic conditions, industry and market considerations, overall Company performance and events directly affecting the Company. It was noted during our assessment that the Company's market capitalization was significantly below its carrying value and a further quantitative analysis was conducted to determine to the extent, if any, the Company's carrying value exceeded its fair value as of September 30, 2022. The quantitative analysis used fair value based on market capitalization adjusted for control premium based on market comparable transactions. This quantitative analysis resulted in the Company's fair value being significantly below its carrying value, resulting in a non-cash goodwill impairment charge of $0.8 million being recorded during the year ended December 31, 2022.

As part of the EryDel Acquisition, the Company recorded goodwill, the excess of the fair value of purchase consideration over the fair value of net tangible and identifiable intangible assets acquired. The Company performed its annual qualitative test for goodwill in the fourth quarter of 2023, the Company concluded that no impairment exists for the year ended December 31, 2023.

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 carried out an evaluation, under the supervision and with the participation of our management, including our Chief Executive Officer and Principal Financial Officer, of the effectiveness of our "disclosure controls and procedures" as of the end of the period covered by this Annual Report, pursuant to Rules 13a-15(b) and 15d-15(b) under the Exchange Act. In connection with that evaluation, our Chief Executive Officer and Principal Financial Officer concluded that our disclosure controls and procedures were effective and designed to provide reasonable assurance that the information required to be disclosed is recorded, processed, summarized and reported within the time periods specified in the SEC rules and forms as of December 31, 2023. For the purpose of this review, disclosure controls and procedures means controls and procedures designed to ensure that information required to be disclosed by us in the reports that we file or submit is recorded, processed, summarized and reported within the time periods specified in the SEC's rules and forms. These disclosure controls and procedures include, without limitation, controls and procedures designed to ensure that information required to be disclosed by us in the reports that we file or submit is accumulated and communicated to management, including our principal executive officer and principal financial officer, as appropriate to allow timely decisions regarding required disclosure.

In designing and evaluating the disclosure controls and procedures, our management recognized that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving the desired control objectives, and our management necessarily was required to apply its judgment in evaluating the cost-benefit relationship of possible controls and procedures.

Management's Report on Internal Control over Financial Reporting

Management is responsible for establishing and maintaining adequate internal control over financial reporting as defined in Rules 13a-15(f) and 15d-15(f) of the Exchange Act. Our management used the Committee of Sponsoring Organizations of the Treadway Commission Internal Control - Integrated Framework issued by the Committee of Sponsoring Organizations of the Treadway Commission (2013 framework), or the COSO framework, to evaluate the effectiveness of internal control over financial reporting. Management believes that the COSO framework is a suitable framework for its evaluation of financial reporting because it is free from bias, permits reasonably consistent qualitative and quantitative measurements of our internal control over financial reporting, is sufficiently complete so that those relevant factors that would alter a conclusion about the effectiveness of our internal control over financial reporting are not omitted and is relevant to an evaluation of internal control over financial reporting.

Management has assessed the effectiveness of our internal control over financial reporting as of December 31, 2023 and has concluded that such internal control over financial reporting is effective. On October 20, 2023, we completed our acquisition of EryDel, a privately held, late-stage biotechnology company with a Phase 3 lead asset, EryDex, that targets the potential treatment of a rare neurodegenerative disease, A-T. We are in the process of integrating EryDel into our internal control over financial reporting. In accordance with SEC Staff guidance permitting a company to exclude an acquired business from management's assessment of the effectiveness of internal controls over financial reporting for the year in which the acquisition is completed, management has excluded the business that we acquired from our assessment of the effectiveness of internal control over financial reporting as of December 31, 2023. The business that we acquired represents approximately $91.9 million of the Company's total assets and $4.0 million of the Company's total operating loss for the year-ended December 31, 2023.

This Annual Report on Form 10-K does not include an attestation report of our independent registered public accounting firm. Our independent registered public accounting firm will not be required to formally attest to the effectiveness of our internal control over financial reporting as long as we are a smaller reporting company pursuant to the provisions of Rule 12b-2 of the Exchange Act.

Changes in Internal Control over Financial Reporting

There have been no changes in our internal control over financial reporting (as defined in Rules 13a-15(f) and 15d-15(f) under the Exchange Act) during our fourth quarter ended December 31, 2023, that have materially affected, or are reasonably likely to materially affect our internal control over financial reporting.

Item 9B. Other Information

None

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 this item will be included in our 2024 Proxy Statement under the caption "Proposal One: Election of Directors," which will be filed with the SEC within 120 days after the end of the fiscal year to which this report relates and is incorporated herein by reference.

Code of Business Conduct and Ethics

We have adopted a Code of Business Conduct and Ethics that applies to all of the members of our board of directors, officers and employees. Information regarding our Code of Business Conduct and Ethics required by this item will be contained in our 2024 Proxy Statement under the caption "Code of Business Conduct and Ethics" and is hereby incorporated by reference. The full text of our Code of Business Conduct and Ethics is posted on the Investor Relations section of our website, which is located at https://ir.quincetx.com/investor-relations, by clicking on "Governance Documents" in the "Governance" section of our website. We intend to satisfy the disclosure requirement under Item 5.05 of Form 8 K regarding amendment to, or waiver from, a provision of our Code of Business Conduct and Ethics by posting such information on our website at the location specified above.

Item 11. Executive Compensation

The information required by this item will be included in our 2024 Proxy Statement under the captions "Director Compensation," "Executive Compensation," which will be filed with the SEC within 120 days after the end of the fiscal year to which this report relates and is incorporated herein by reference.

Item 12. Security Ownership of Certain Beneficial Owners and Management And Related Stockholder Matters

The information required in this item will be included in our 2024 Proxy Statement under the captions "Security Ownership of Certain Beneficial Owners and Management" and "Equity Compensation Plan Information," which will be filed with the SEC within 120 days after the end of the fiscal year to which this report relates and is incorporated herein by reference.

Item 13. Certain Relationships and Related Transactions, and Director Independence

The information required in this item will be included in our 2024 Proxy Statement under the captions "Review, Approval or Ratification of Transactions with Related Parties" and "Independence of Directors," which will be filed with the SEC within 120 days after the end of the fiscal year to which this report relates and is incorporated herein by reference.

Item 14. Principal Accountant Fees and Services

Our independent registered public accounting firm is BDO USA, P.C., Chicago, Illinois, PCAOB Auditor ID 243.

The information required in this item will be included in our 2024 Proxy Statement under the caption "Independent Registered Public Accounting Firm Fees and Services," which will be filed with the SEC within 120 days after the end of the fiscal year to which this report relates and is incorporated herein by reference.

PART IV

Item 15. Exhibits and Consolidated Financial Statement Schedules

(a) The following documents are filed as part of this report:

1. Consolidated Financial Statements

See Index to Consolidated Financial Statements in Part II Item 8 of this Annual Report on Form 10-K.

2. Consolidated Financial Statement Schedules

All schedules are omitted because they are not applicable or the required information is shown in the consolidated financial statements or notes thereto.

3. Exhibits

The documents listed in the Exhibit Index are incorporated by reference or are filed with this report, in each case as indicated therein (numbered in accordance with Item 601 of Regulation S-K).

Item 16. Form 10-K Summary

None.

Exhibit Index

Exhibit No.	Exhibit title	Incorporated by reference				Filed or furnished herewith
		Form	File No.	Exhibit No.	Filing date	
2.1	Agreement and Plan of Merger and Reorganization, dated as of May 9, 2022 by and among Cortexyme,Inc., Novosteo Inc., Quince Merger Sub I, Inc., Quince Merger Sub II, LLC and Fortis Advisors LLC	8-K	001-38890	2.1	5/12/2022	
2.2	Stock Purchase Agreement, dated as of July 21, 2023 by and among Quince Therapeutics, Inc., EryDel Italy, Inc., EryDel S.p.A., certain holders and managers set forth on Schedule II thereto, and Shareholder Representative Services LLC, as the stockholder representative	8-K	001-38890	2.1	7/21/2023	
3.1	Amended and Restated Certificate of Incorporation	8-K	001-38890	3.1	5/13/2019	
3.2	Certificate of Amendment to the registrant's Certificate of Incorporation, effective August 1, 2022	8-K	001-38890	3.1	8/1/2022	
3.3	Amended and Restated Bylaws	8-K	001-38890	3.2	8/1/2022	
3.4	Certificate of Designation of Series A Junior Participating Preferred Stock	8-K	001-38890	3.1	4/5/2023	
4.1	Specimen Stock Certificate	S-1	333-230853	4.1	4/29/2019	
4.2	Amended and Restated Investors' Rights Agreement, dated May 23, 2018 by an among the Registrant and certain of its stockholders	S-1	333-230853	4.2	4/12/2019	
4.3	Description of Securities	10-K	001-36276	4.3	3/1/2021	
4.4	Rights Agreement dated as of April 5, 2023, between Quince Therapeutics, Inc. and American Stock Transfer & Trust Company, LLC	8-K	001-38890	4.1	4/5/2023	
10.1+	Employment Offer Letter, by and between Cortexyme, Inc. and Brendan Hannah, dated May 9,2022	10-Q	001-38890	10.2	8/9/2022	
10.2+	Employment Offer Letter, by and between Cortexyme, Inc. and Dirk Thye, dated May 9,2022	10-Q	001-38890	10.4	8/9/2022	
10.3+	Offer Letter between Quince Therapeutics, Inc. and Charles Ryan, dated as of August 1, 2023	10-Q	001-38890	10.3	11/14/2023	
10.4+	Form of Indemnification Agreement between Cortexyme, Inc. and each of its officers and directors	S-1/A	333-230853	10.2	4/29/2019	
10.5+	2014 Stock Plan, as amended as of November 28, 2018, and related forms of stock award agreements	S-1	333-230853	10.3	4/12/2019	
10.6+	2019 Equity Incentive Plan and forms of stock award agreements thereunder					X
10.7+	2019 Employee Stock Purchase Plan	S-1/A	333-230853	10.5	4/29/2019	
10.8+	Executive Incentive Bonus Plan	S-1	333-230853	10.6	4/12/2019	
10.9+	Cortexyme, Inc. 2022 Inducement Plan	S-8	333-265109	99.1	5/20/2022	
10.10+	Forms of Stock Option Award Agreement, Notice of Stock Option Grant and Exercise Notice under Cortexyme, Inc. 2022 Inducement Plan	S-8	333-265109	99.2	5/20/2022	
10.11+	Forms of Restricted Stock Unit Award Agreement and Notice of Restricted Stock Unit Grant Cortexyme, Inc. 2022 Inducement Plan	S-8	333-265109	99.3	5/20/2022	
10.12+	Novosteo Inc. 2019 Equity Incentive Plan	S-8	333-265109	99.4	5/20/2022	

10.13+	Executive Change in Control and Severance Agreement by and between Cortexyme, Inc. and Brendan Hannah, dated May 19, 2022	10-Q	001-38890	10.10	8/9/2022	
10.14+	Transition and Separation Agreement between Quince Therapeutics, Inc. and Karen Smith, dated as of August 4, 2023	10-Q	001-38890	10.2	11/14/2023	
10.15+	Executive Change in Control and Severance Agreement by and between Cortexyme, Inc. and Dirk Thye, dated May 19, 2022	10-Q	001-38890	10.12	8/9/2022	
10.16+	Executive Change in Control and Severance Agreement between Quince Therapeutics, Inc. and Charles Ryan, dated as of September 1, 2023	10-Q	001-38890	10.4	11/14/2023	
10.17	Sublease Agreement, by and between Cortexyme, Inc. and ICON Clinical Research LLC, dated as of May 5, 2022	10-Q	001-38890	10.14	8/9/2022	
10.18	Consent to Sublease, by and between Cortexyme, Inc. and ICON Clinical Research LLC, dated as of June 8, 2022	10-Q	001-38890	10.15	8/9/2022	
10.19	Sub-Sublease Agreement by and between Cortexyme, Inc. and Verily Life Sciences LLC, dated June 18, 2018.	10-Q	333-230853	10.1	4/12/2019	
10.20	Amendment No. 1 to Sub-Sublease by and between Cortexyme, Inc. and Verily Life Sciences LLC dated April 2, 2019.	10-Q	001-38890	10.1	8/9/2019	
10.21	Second Amendment to Sub-Sublease by and between Cortexyme, Inc. and Verily Life Sciences LLC dated May 26, 2020	10-Q	001-38890	10.1	8/14/2020	
10.22	Third Amendment to Sub-Sublease by and between Cortexyme, Inc. and Verily Life Sciences LLC dated July 15,2021	10-Q	001-38890	10.7	8/6/2021	
10.23	Outside Director Compensation Policy adopted April 9,2019; Amended and Restated: June 7, 2022	10-Q	001-38890	10.17	8/9/2022	
10.24	Open Market Sales Agreement℠ dated December 23, 2021, by and between Cortexyme, Inc. and Jefferies LLC	8-K	001-38890	10.1	12/23/2021	
10.25	Lighthouse Purchase Agreement	10-Q	001-38890	10.1	5/15/2023	
10.26**	Accession, Amendment and Restatement Agreement to the Finance Contract relating to the Finance Contract dated 24 July 2020, as amended from time to time, by and between the Company, EryDel Italy, Inc., EryDel US, Inc., EryDel USA, Inc. EryDel S.p.A, and the European Investment Bank, dated as of October 20, 2023					X
10.27**	Autonomous First Demand Guarantee (*Garanzia Autonoma a Prima Richiesta*) by and between the Company, EryDel Italy, Inc., EryDel US, Inc., EryDel S.p.A, and the European Investment Bank, dated as of October 20, 2023					X
21.1	List of subsidiaries					X
23.1	Consent of Independent Registered Public Accounting Firm					X
24.1	Power of Attorney (incorporated by reference to the signature page of this Annual Report on Form 10-K)					X
31.1	Certification of Principal Executive Officer pursuant to Rules 13a-14(a) and Rule 15d-14(a) of the Exchange Act					X

31.2	Certification of Principal Financial Officer pursuant to Rules 13a-14(a) and Rule 15d-14(a) of the Exchange Act	X
32.1#	Certification of Principal Executive Officer pursuant to Rule 13a-14(b) of the Exchange Act and 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002	X
32.2#	Certification of Principal Financial Officer pursuant to Rule 13a-14(b) of the Exchange Act and 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002	X
97.1	Incentive Compensation Recoupment Policy	X
101.INS	Inline XBRL Instance Document	X
101.SCH	Inline XBRL Taxonomy Extension Schema Document	X
101.CAL	Inline XBRL Taxonomy Extension Calculation Linkbase Document	X
101.DEF	Inline XBRL Taxonomy Extension Definition Linkbase Document	X
101.LAB	Inline XBRL Taxonomy Extension Label Linkbase Document	X
101.PRE	Inline XBRL Taxonomy Extension Presentation Linkbase Document	X
104	The cover page from this Annual Report on Form 10-K, formatted in Inline XBRL	

+ Management contract or compensatory plan or arrangement.

** Portions of this exhibit have been redacted pursuant to Item 601(b)(10) of Regulation S-K as the Registrant has determined that (i) the omitted information is not material and (ii) the omitted material is of the type that the Registrant treats as private or confidential.

In accordance with Item 601(b)(32)(ii) of Regulation S-K and SEC Release Nos. 33-8238 and 34-47986, Final Rule: Management's Reports on Internal Control Over Financial Reporting and Certification of Disclosure in Exchange Act Periodic Reports, the certifications furnished in Exhibits 32.1 and 32.2 hereto are deemed to accompany this Annual Report on Form 10-K and will not be deemed "filed" for purpose of Section 18 of the Exchange Act. Such certifications will not be deemed to be incorporated by reference into any filing under the Securities Act or the Exchange Act, except to the extent that the registrant specifically incorporates it by reference.

SIGNATURES

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

Quince Therapeutics, Inc.

Date: April 1, 2024

By: /s/ Dirk Thye
Dirk Thye

Chief Executive Officer

POWER OF ATTORNEY

Each person whose signature appears below constitutes and appoints Dirk Thye and Brendan Hannah, and each of them, as his true and lawful attorney-in-fact and agent, with full power of substitution and resubstitution, for him or her and in his or her name, place and stead, in any and all capacities, to sign any and all amendments to this Annual Report on Form 10-K, and to file the same, with all exhibits thereto, and other documents in connection therewith, with the Securities and Exchange Commission, granting unto said attorneys-in-fact and agents, and each of them, full power and authority to do and perform each and every act and thing requisite and necessary to be done in connection therewith, as fully to all intents and purposes as he or she might or could do in person, hereby ratifying and confirming all that said attorneys-in-fact and agents, or any of them, or their or his substitutes, may lawfully do or cause to be done by virtue thereof.

Pursuant to the requirements of the Securities Exchange Act of 1934, as amended, this Report has been signed below by the following persons on behalf of the Registrant in the capacities and on the dates indicated.

Name	Title	Date
/s/ Dirk Thye **Dirk Thye**	Chief Executive Officer, and Director (Principal Executive Officer)	April 1, 2024
/s/ Brendan Hannah **Brendan Hannah**	Chief Business Officer and Chief Operating Officer (Principal Financial and Accounting Officer)	April 1, 2024
/s/ David A. Lamond **David A. Lamond**	Director	April 1, 2024
/s/ Margaret McLoughlin **Margaret McLoughlin, Ph.D.**	Director	April 1, 2024
/s/ Una Ryan **Una Ryan, OBE Ph.D.**	Director	April 1, 2024
/s/ Christopher J. Senner **Christopher J. Senner**	Director	April 1, 2024
/s/ Luca Benatti **Luca Benatti**	Director	April 1, 2024
/s/ Rajiv Patni **Rajiv Patni**	Director	April 1, 2024
/s/ June Bray **June Bray**	Director	April 1, 2024