

**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, 2025

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: 001-41855

**LEXEO THERAPEUTICS, INC.**

(Exact name of Registrant as specified in its Charter)

**Delaware**

(State or other jurisdiction of  
incorporation or organization)

**345 Park Avenue South, Floor 6**

**New York, New York**

(Address of principal executive offices)

**85-4012572**

(I.R.S. Employer  
Identification No.)

**10010**

(Zip Code)

**Registrant's telephone number, including area code: (212) 547-9879**

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 \$0.0001	LXEO	The Nasdaq Global Market

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	<input type="checkbox"/>	Accelerated filer	<input type="checkbox"/>
Non-accelerated filer	<input checked="" type="checkbox"/>	Smaller reporting company	<input checked="" type="checkbox"/>
Emerging growth company	<input checked="" type="checkbox"/>		

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

As of June 30, 2025, the last day of the registrant's most recently completed second fiscal quarter, the aggregate market value of the voting and non-voting common equity held by non-affiliates of the registrant was approximately \$216.5 million.

The number of shares of Registrant's common stock outstanding as of March 27, 2026 was 74,087,063.

**DOCUMENTS INCORPORATED BY REFERENCE**

Portions of the Registrant's definitive Proxy Statement relating to the 2026 Annual Meeting of Stockholders are incorporated herein by reference in Part III of this Annual Report on Form 10-K to the extent stated herein. Such proxy statement will be filed with the Securities and Exchange Commission within 120 days of the registrant's fiscal year ended December 31, 2025.

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## SPECIAL NOTE REGARDING FORWARD-LOOKING STATEMENTS

This Annual Report on Form 10-K, or Annual Report, contains forward-looking statements that involve risks and uncertainties. We make such forward-looking statements pursuant to the safe harbor provisions of the Private Securities Litigation Reform Act of 1995 and other federal securities laws. All statements other than statements of historical facts contained in this Annual Report, including statements regarding our future results of operations and financial position, business strategy, development plans, planned preclinical studies and clinical trials, future results of clinical trials, expected research and development costs, regulatory strategy, timing and likelihood of success, as well as plans and objectives of management for future operations, are forward-looking statements. In some cases, you can identify forward-looking statements by terms such as “may,” “will,” “should,” “expect,” “plan,” “anticipate,” “could,” “intend,” “target,” “project,” “estimate,” “believe,” “predict,” “potential” or “continue” or the negative of these terms or other similar expressions intended to identify statements about the future. Forward-looking statements contained in this Annual Report include, without limitation, statements about:

- the timing, progress and results of our preclinical studies and clinical trials of our product candidates, including statements regarding the timing of initiation and completion of studies or trials and related preparatory work, the period during which the results of the trials will become available and our research and development programs;
- the timing of our planned investigational new drug, or IND, submissions, initiation of planned clinical trials and timing of expected clinical results for LX2006 and LX2020, if applicable, and our other future product candidates;
- the timing for receipt and announcement of data from our preclinical studies and clinical trials;
- the timing of any submission of filings for regulatory approval of and our ability to obtain and maintain regulatory approvals for LX2006, LX2020 and any other product candidates;
- the impact of public health crises and other adverse global economic conditions on our operations and the potential disruption in the operations and business of third-party manufacturers, contract research organizations, or CROs, other service providers, and collaborators with whom we conduct business;
- our ability to identify patients with the diseases treated by our product candidates, and to enroll patients in trials;
- the beneficial characteristics, safety, efficacy and therapeutic effects of our product candidates;
- the ability of our preclinical studies and clinical trials to demonstrate safety and efficacy of our product candidates, and other positive results;
- our expectations regarding the size of the patient populations, market acceptance and opportunity for and clinical utility of our product candidates, if approved for commercial use;
- our manufacturing capabilities and strategy, including the scalability and commercial viability of our manufacturing methods and processes;
- our reliance on third party manufacturing partners to comply with significant regulations with respect to manufacturing our products;
- our expectations regarding the scope of any approved indication for LX2006, LX2020 or any other product candidate;
- our ability to successfully commercialize our product candidates, if approved;
- our ability to leverage our platform to identify and develop future product candidates;
- our estimates of our expenses, ongoing losses, future revenue, capital requirements and our need for or ability to obtain additional funding before we can expect to generate any revenue from product sales;
- our ability to establish or maintain collaborations or strategic relationships and any expected benefits related thereto;
- our ability to identify, recruit and retain key personnel;
- our reliance upon intellectual property licensed from third parties and our ability to obtain such licenses on commercially reasonable terms or at all;

- our ability to protect and enforce our intellectual property position for our product candidates, and the scope of such protection;
- our financial performance;
- our competitive position and the development of and projections relating to our competitors or our industry;
- our estimates regarding future revenue, expenses and needs for additional financing;
- our anticipated use of our cash, cash equivalents, and investments balances and any proceeds from the ATM Program (as defined below);
- the impact of current and future laws and regulations; and
- our expectations regarding the time during which we will be an emerging growth company under the Jumpstart Our Business Startups Act of 2012, or JOBS Act.

We caution you that the foregoing list does not contain all of the forward-looking statements made in this Annual Report. We have based these forward-looking statements largely on our current expectations and projections about our business, the industry in which we operate and financial trends that we believe may affect our business, financial condition, results of operations and prospects, and these forward-looking statements are not guarantees of future performance or development. These forward-looking statements speak only as of the date of this Annual Report and are subject to a number of risks, uncertainties and assumptions described in the section titled “Item 1A. Risk Factors” and elsewhere in this Annual Report. Because forward-looking statements are inherently subject to risks and uncertainties, some of which cannot be predicted or quantified and some of which are beyond our control, you should not rely on these forward-looking statements as predictions of future events. Although we believe that we have a reasonable basis for each forward-looking statement contained in this Annual Report, the events and circumstances reflected in our forward-looking statements may not be achieved or occur and actual results could differ materially from those projected in the forward-looking statements. Except as required by applicable law, we do not plan to publicly update or revise any forward-looking statements contained herein, whether as a result of any new information, future events or otherwise. In addition, statements that “we believe” and similar statements reflect our beliefs and opinions on the relevant subject. These statements are based upon information available to us as of the date of this Annual Report, and while we believe such information forms a reasonable basis for such statements, such information may be limited or incomplete, and our statements should not be read to indicate that we have conducted an exhaustive inquiry into, or review of, all potentially available relevant information. These statements are inherently uncertain, and you are cautioned not to unduly rely upon these statements.

## PART I

### Item 1. Business.

#### *Overview*

We are a clinical stage genetic medicine company dedicated to reshaping heart health by applying pioneering science to fundamentally change how cardiovascular diseases are treated. We are advancing a portfolio of therapeutic candidates that take aim at the underlying genetic causes of conditions, including Friedreich ataxia, or FA, cardiomyopathy, plakophilin-2, or PKP2, arrhythmogenic cardiomyopathy, and other devastating diseases with high unmet need.

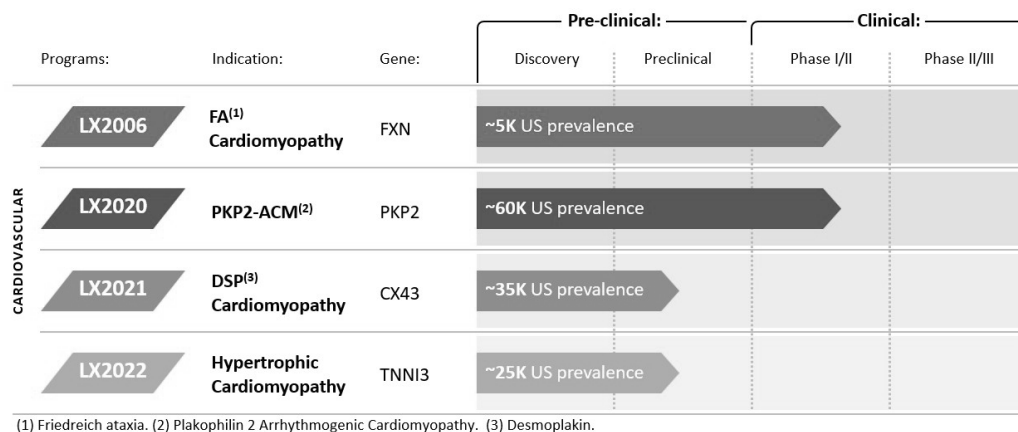
Our most advanced cardiovascular product candidate, LX2006 for the treatment of FA cardiomyopathy, is currently being evaluated in SUNRISE-FA, our ongoing Phase 1/2 clinical trial and in a Cornell investigator-initiated trial. In October 2025, we reported positive interim clinical data demonstrating an encouraging safety profile and evidence of meaningful cardiac and functional benefit. Further, interim clinical data presented in a late-breaker presentation at the American College of Cardiology (ACC) Annual Meeting in March 2026 continue to show sustained or deepening improvements across both cardiac and neurologic measures of FA. In February 2026, we submitted the final registrational trial design and statistical analysis plan for the SUNRISE-FA 2 study to the U.S. Food and Drug Administration (FDA) following a Type B meeting. We expect to receive final feedback from the FDA in the second quarter of 2026 and plan to initiate a registrational study in the first half of 2026.

Our second most advanced cardiovascular product candidate, LX2020 for the treatment of arrhythmogenic cardiomyopathy, or ACM, caused by mutations in the PKP2 gene, referred to as PKP2-ACM, is currently being evaluated in HEROIC-PKP2, an ongoing Phase 1/2 clinical trial. In January 2026, we announced positive interim data from the HEROIC Phase 1/2 clinical trial. Across dose cohorts, LX2020 was generally well tolerated and led to robust transduction, increased PKP2 protein expression, and clinically meaningful improvement or stabilization in measures of arrhythmia burden in the majority of participants. We plan to share additional safety and clinical efficacy data for LX2020 in the fourth quarter of 2026, once all participants in the high dose cohorts reach at least twelve months of follow up.

Each of our gene therapy candidates utilizes the vector construct, dose and route of administration that we believe will result in the most favorable biodistribution and safety profile for our product candidate for each disease. Our most advanced programs use the AAVrh10 vector due to its high transduction efficiency in myocardial cells, potential for lower toxicity given the opportunity to utilize lower doses compared to other well-established AAV serotypes, and low pre-existing immunity.

## Our pipeline

Utilizing a stepwise, capital-efficient development approach, we are leveraging early proof-of-concept functional and biomarker data to advance a deep and diverse pipeline of cardiovascular programs for larger-rare and prevalent indications. We retain exclusive worldwide development and commercialization rights to all of our product candidates and programs.



We are developing a number of disease-modifying gene therapy candidates to treat larger-rare cardiovascular diseases that have high unmet need and no approved treatments that address the underlying genetic cause of the disease. These programs include:

- LX2006* is an AAVrh10-based gene therapy candidate designed to intravenously deliver a functional frataxin, or FXN, gene for the treatment of FA cardiomyopathy, which is the most common cause of mortality in patients with FA and affects approximately 5,000 patients in the United States. *LX2006* is designed to promote the expression of the protein frataxin to restore normal mitochondrial function and energy production in myocardial cells. *LX2006* has received Rare Pediatric Disease, Orphan Drug, Fast Track, Regenerative Medicine Advanced Therapy (RMAT), and Breakthrough Therapy designations from the FDA, and Orphan Medicinal Product designation from the European Commission. *LX2006* has also been selected by the FDA to participate in its Chemistry, Manufacturing, and Controls (CMC) Development and Readiness Pilot (CDRP) program, which is intended to facilitate expedited CMC development of investigational therapies with expedited clinical development timeframes via an increased level of communication with the FDA on CMC development.
- LX2020* is an AAVrh10-based gene therapy candidate designed to intravenously deliver a functional *PKP2* gene to cardiac muscle for the treatment of PKP2-ACM. *PKP2* mutations are associated with approximately 75% of all genetic cases of ACM, and we estimate they affect approximately 60,000 patients in the United States. *PKP2* mutations can cause replacement of heart muscle with fibrotic tissue and fatty deposits and severe abnormal heart rhythms, or arrhythmias, that cause cardiac dysfunction and can result in sudden cardiac death. *LX2020* is designed to increase desmosomal PKP2 protein levels, reassemble desmosomes and restore myocardial cell function. *LX2020* has received Fast Track and Orphan Drug designations from the FDA and Orphan Medicinal Product designation from the European Commission.
- LX2021* is a gene therapy candidate we are developing to intravenously deliver the coding sequence for the functional connexin 43, or Cx43, protein for a group of inherited cardiac muscle disorders associated with a high risk of sudden death, including ACM and certain forms of dilated cardiomyopathy. We believe restoring the Cx43 protein can potentially treat multiple genetic causes of ACM because the cardiac loss of Cx43 is a molecular deficit generally observed in all ACM patient populations. Our *LX2021* program is initially targeting Desmoplakin, or DSP, cardiomyopathy, a distinct form of ACM as well as a certain form of dilated cardiomyopathy, with a prevalence that may be as high as 4% of all inherited dilated cardiomyopathies and 4% of all ACMs, impacting approximately 30,000 to 35,000 patients in the United States.

- *LX2022* is a gene therapy candidate we are developing to intravenously deliver a functional *TNNI3* gene to myocardial cells to treat a distinct form of hypertrophic cardiomyopathy, or HCM, due to mutations in the *TNNI3* gene. Mutations in the *TNNI3* gene often result in left ventricular hypertrophy and restrictive cardiomyopathy, leading to arrhythmias and heart failure. With an estimated prevalence of 1 in 500 people in the United States, HCM is one of the most common forms of genetic cardiomyopathy and is caused by mutations that affect the cardiac sarcomere in approximately 75% of cases. It is estimated that as many as 25,000 patients in the United States and 34,000 patients in the European Union are affected by HCM caused by mutations in the *TNNI3* gene.

Additionally, we are seeking business development opportunities for our portfolio of candidates targeting APOE4-associated Alzheimer's disease, including LX1001 which has completed a Phase 1/2 clinical trial.

## **Our Approach**

### *Our technology approach*

Our most advanced programs use the AAVrh10 vector due to its high transduction efficiency in myocardial cells, higher ratio of cardiac to liver biodistribution, potentially lower toxicity given the ability to utilize lower doses, compared to other well-established AAV serotypes, and lower pre-existing immunity. The AAVrh10 vector has been shown to be capable of transducing myocardial cells based on preclinical research and ongoing clinical studies. We believe these results demonstrate that among currently available, commonly used serotypes, AAVrh10 is an efficient vector for delivery and expression of transgenes for the treatment of the cardiovascular diseases that we are currently targeting. We have observed organ-specific biodistribution advantages for our AAVrh10 vector compared to AAV9, a well-known serotype that has been evaluated in several clinical trials, including one cardiovascular clinical trial. For example, we have observed vector distribution of AAVrh10 in cardiac tissue of non-human primates, or NHPs, that is two times greater than for AAV9. Additionally, our preliminary studies in Yucatan minipigs have shown that AAVrh10 has approximately 1.5 times greater biodistribution in the heart than AAV9. In addition, preclinical studies conducted by Weill Cornell Medical College, or Weill Cornell Medicine, have demonstrated that systemic administration of AAVrh10 promotes a ratio of cardiac-to-liver biodistribution that is more favorable than what is known of other commonly used vector serotypes.

### *Our disease area strategy*

Our cardiovascular gene therapy programs are designed to have the following characteristics:

- *Indications that may be effectively treated by gene therapy.* We select targets that correspond to populations with a specific genetic profile and clearly defined disease phenotype.
- *Indications with the potential to demonstrate early evidence of meaningful clinical benefit.* We pursue clearly defined biomarkers and functional endpoints that can provide early proof-of-mechanism and inform clinical development decisions, including the potential to seek accelerated approval pathways.
- *Present opportunity to address high unmet medical need.* We are focused on genetically defined cardiovascular diseases where there are no currently approved treatments or where we believe our therapeutic candidates will have a meaningful improvement relative to existing standards of care.
- *Significant market opportunity.* We pursue indications with significant commercial opportunities beyond those typically associated with gene therapy companies targeting rare monogenic diseases.
- *Targets that have established proof-of-concept.* We have leveraged our relationships with academic institutions to in-license product candidates with established proof-of-concept in relevant preclinical models that closely resemble the clinical phenotype we are pursuing.

### *Our manufacturing approach*

We are developing gene therapy candidates for larger-rare and prevalent disease patient populations that require a high-quality process that can produce vectors in relatively large quantities while utilizing traditional biologics manufacturing infrastructure. We utilize a baculovirus/Sf9 expression system to manufacture our gene therapy candidates. Our manufacturing platform is designed to infect Sf9 cells at high densities in suspension cell culture with both an AAVrh10 and baculovirus containing the transgene. The output is coupled with a chromatography-based purification process which allows for efficient AAV purification, resulting in higher yields and fewer empty AAV capsids than traditional plasmid HEK adherent cell culture approaches.

We believe our manufacturing process enables us to efficiently pursue our goal of targeting larger-rare and prevalent patient populations. Historically, manufacturing challenges, largely driven by the quantity of vector required to pursue large commercial opportunities have limited the utility of gene therapy for large patient populations. We believe our proprietary Sf9 baculovirus process will allow us to produce vectors at the necessary scale to support the patient populations we are targeting at a cost-of-goods profile similar to what the pharmaceutical marketplace has seen with biologics. To date, we have developed a highly reproducible and scalable 200L GMP AAV production process using the Sf9-baculovirus system, which does not require costly plasmids. Purification is also a straightforward 2-column process. The overall process delivers over 1E15 vg/L of purified AAVrh.10 product that contains less than 25% empty capsids.

In May 2025, we presented new data at the 28th American Society of Gene & Cell Therapy (ASGCT) Annual Meeting on AAV manufacturing optimization via our Sf9-baculovirus process. Data presentations reviewed the high purity and potency of our yields with improved scalability of production and reduced cost.

In October 2025, we shared CMC data for LX2006, which were submitted to the FDA to support analytical comparability between the HEK293 manufacturing process and the Sf9-baculovirus manufacturing platform. These data demonstrate similar frataxin expression *in vitro* between the two processes as measured by a quantitative relative potency assay in cardiomyocytes. In November 2025, the FDA approved this analytical comparability report establishing comparability between LX2006 HEK293 and Sf9 manufacturing processes, endorsing use of the optimized, Sf9 final commercial manufacturing process for LX2006 in the planned pivotal study and clearing comparability requirements to begin dosing patients.

In March 2026, we conducted an initial CMC Development and Readiness Program meeting with the FDA for LX2006, during which the agency reiterated its support for a flexible validation approach including potential for reduced process performance qualification runs and concurrent process validation. We are proceeding with validation and commercial manufacturing readiness activities to support the Biologic License Application (BLA) submission for LX2006.

Based on our approach, we expect that our existing partnerships can supply material for all of our currently ongoing and planned clinical trials as well as potential commercial production of some of our programs. We will own the intellectual property created by our manufacturing process development activities or have the ability to license it and will maintain the option to transfer the process to other CDMOs in the future and/or to our own potential facility to ensure ongoing redundancy and reliability.

## **Our cardiovascular gene therapy programs**

### ***LX2006 for the treatment of FA cardiomyopathy***

#### *Overview of FA*

FA is a genetic, progressive, and degenerative multi-system disorder with a prevalence of 1:50,000 or approximately 5,000 people in the United States. It is estimated that approximately 80% of these patients will develop FA cardiomyopathy. FA is caused by a mutation in the *FXN* gene that disrupts the normal production of the protein frataxin, which is critical to the function of mitochondria in a cell and to the maintenance of cardiac function. The most common FA phenotype presents with significantly reduced frataxin levels compared to healthy individuals, however levels of frataxin in the cardiac tissue of FA patients is unknown. The frataxin deficiency in the mitochondria of myocardial cells in FA patients causes disruption of normal iron-sulfur cluster biosynthesis leading to mitochondrial dysfunction and deficient energy production.

The neurologic disease and cardiac disease are two distinct manifestations of FA. The disease is inherited in an autosomal recessive manner, where both inherited genes are abnormal, and symptoms usually begin in childhood. Absence of fully functional frataxin leads to damage to peripheral nerves and the parts of the brain that control movement and balance, leading to neurological symptoms that include impaired muscle coordination, or ataxia, that worsen over time. Initial symptoms may include unsteady posture, frequent falling, and progressive difficulty in walking due to impaired ability to coordinate voluntary movements. Affected individuals often develop slurred speech, hearing loss, scoliosis, diabetes, characteristic foot deformities, and an irregular curvature of the spine. The typical age of onset of neurological symptoms is five to 15 years old. As the disease progresses, patients typically experience various heart conditions, including thickening of the heart muscle, or hypertrophic cardiomyopathy, and arrhythmias. Hypertrophic cardiomyopathy, fibrosis, heart failure and arrhythmias are the cause of death in approximately two-thirds of FA patients. Typical onset of the cardiac disease is at 15 to 30 years old.

By the time the cardiac disease of FA emerges, the neurologic disease is generally significantly advanced and may not be amenable to gene therapy. There are currently no approved treatments for the cardiac manifestations of FA. *Skyclarys*, the only approved treatment for FA, is an Nrf2 activator which targets only the CNS component of the disease. As a result, patients with FA have significant unmet need for a therapy to treat cardiovascular complications associated with the disease.

### Our approach: LX2006

We are developing LX2006 as an AAVrh10-based gene therapy delivered intravenously for the treatment of FA cardiomyopathy. LX2006 is designed to deliver the *FXN* gene under the transcriptional control of the CAG promoter, a strong synthetic promoter frequently used in viral vectors. LX2006 utilizes AAVrh10 based on its favorable cardiac affinity and vector distribution profile observed in preclinical studies, as compared to AAV9. LX2006 is designed to transfer the *FXN* gene to myocardial cells and increase frataxin levels in the mitochondria. The increase in frataxin levels in the mitochondria is intended to restore mitochondrial function and energy production in cardiac myocytes.

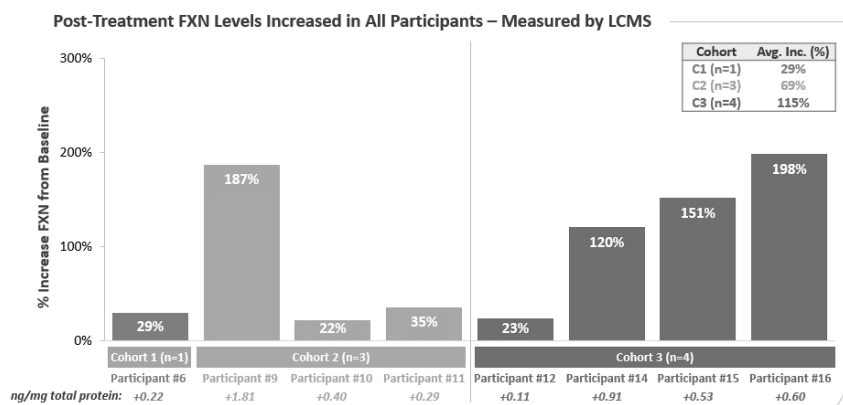
### Lexeo-sponsored LX2006 clinical development and trial design

LX2006 is currently being evaluated in an ongoing, multi-site Phase 1/2 clinical trial, or SUNRISE-FA, in patients with FA cardiomyopathy. SUNRISE-FA is a first-in-human, 52-week, dose-ascending, open-label trial of LX2006 in patients who have FA cardiomyopathy. LX2006 is administered as a one-time intravenous infusion with prednisone utilized for immune suppression. The trial consists of three dose cohorts. The cohort 1 dose level is  $1.8 \times 10^{11}$  vg/kg, the cohort 2 dose level is  $5.6 \times 10^{11}$  vg/kg, and the cohort 3 dose level is  $1.2 \times 10^{12}$  vg/kg. There is a long-term follow-up for patients who receive LX2006 to monitor ongoing safety for a total of five years, per FDA requirement.

### Cardiac biopsy results from SUNRISE-FA trial

In April 2025, we provided an interim clinical update, which included frataxin protein expression from eight participants in SUNRISE-FA that had undergone cardiac biopsies. These data demonstrated increase in frataxin protein expression versus baseline at three months in all participants as measured by liquid chromatography-mass spectrometry, or LCMS. Dose-dependent increases were observed across cohorts on average, with 115% mean increase in the high-dose cohort 3 (n=4). To our knowledge, SUNRISE-FA is the first clinical trial to evaluate frataxin levels in the target organ of FA cardiomyopathy patients.

### Increased frataxin expression across all participants evaluated at 3-months post treatment



### Additional ongoing clinical trial of AAVrh10.hFXN (LX2006 at Lexeo)

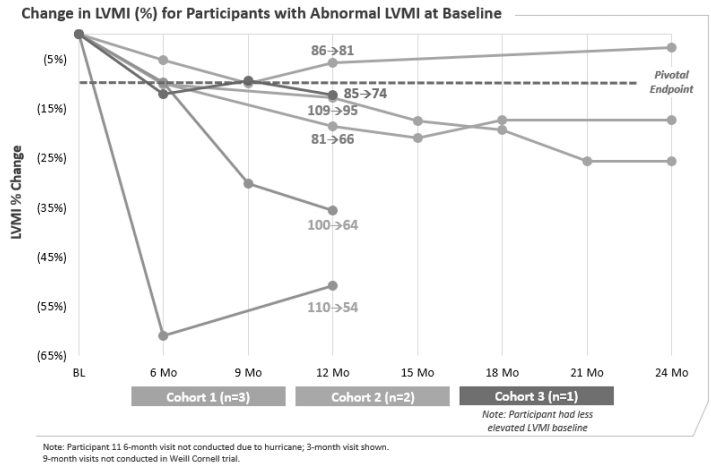
AAVrh10.hFXN, which we refer to as LX2006, is currently being evaluated in an additional ongoing investigator-initiated, single-site clinical trial conducted by Weill Cornell Medicine (NCT05302271). Both the Weill Cornell investigator-initiated clinical study and our SUNRISE-FA clinical study use drug product manufactured at Weill Cornell Medicine utilizing the same process with identical doses in each study's cohort 1 and cohort 2. In April 2024 we announced a license agreement with Cornell University for intellectual property rights including current and future clinical data from the ongoing Weill Cornell Medicine investigator-initiated trial of LX2006.

### Cardiac biomarker data from ongoing clinical trials of LX2006

In October 2025, we provided an interim clinical update, which included baseline characteristics and safety data from the 17 treated participants across the two studies, and clinical efficacy data from the 16 participants who had reached at least six months follow-up as of that time. These data showed sustained or deepening improvements in the majority of participants across both cardiac and neurologic measures of FA.

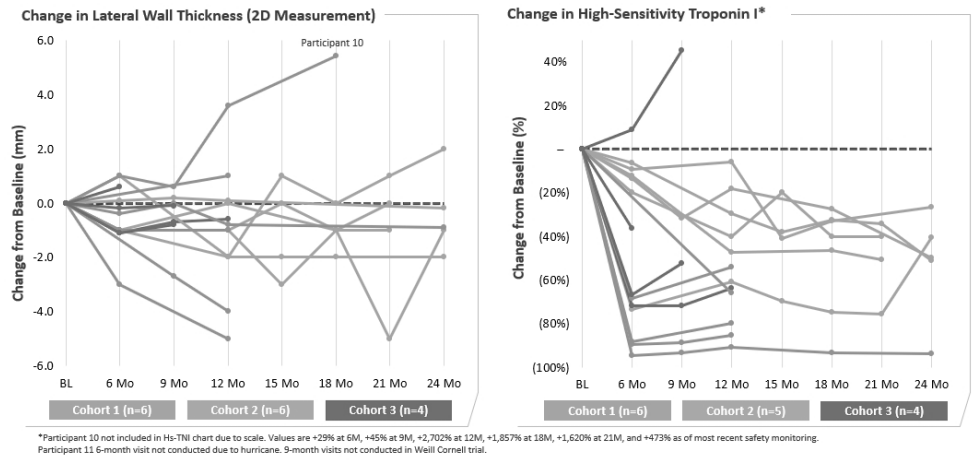
Improvements in LVMI among participants with abnormal baseline LVMI (n=6). Five of six participants achieved >10% by 12-month visit or sooner and six of six participants achieved LVMI measurements within the normal range as of latest visit. A 18% mean improvement in LVMI was observed at 6-month visit and a 23% mean improvement in LVMI was observed at 12-month visit, exceeding 10% FDA-aligned threshold for pivotal study. In the mid- and high-dose cohorts (n=3), a 28% mean improvement in LVMI was observed at 6-month visit and a 33% mean improvement in LVMI was observed at 12-month visit, suggesting dose-dependent improvement. Among participants with normal baseline LVMI (n=10), the majority demonstrated LVMI improvement or stabilization over time.

**Participants with abnormal LVMI at baseline, LVMI % change**



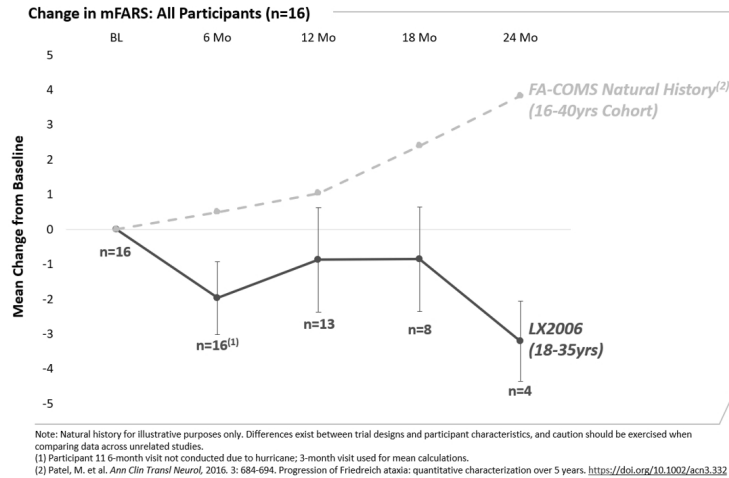
Improvements in secondary cardiac biomarkers. Fourteen of sixteen participants achieved >25% reduction in high-sensitivity troponin I from baseline at latest visit. Fourteen of sixteen participants achieved reduction or stabilization in LWT from baseline at latest visit.

**All participants, improvements in LWT and high-sensitivity troponin I**



Improvements in mFARS as demonstrated by a 2.0-point mean improvement from baseline at latest visit across all participants. Eleven of sixteen participants achieved reduction or stabilization in mFARS from baseline at latest visit.

## All participants, functional improvement over time using mFARS



In March 2026, we shared updated interim clinical data for LX2006 at the ACC Annual Meeting. Phase 1/2 interim clinical data presented at the ACC Annual Meeting continue to show sustained or deepening improvements across both cardiac and neurologic measures of FA, including statistically significant improvement in mean mFARS scores for LX2006-treated participants compared to a propensity-matched control cohort from the UNIFAI natural history study.

### *Safety profile of LX2006 from ongoing clinical trials of LX2006*

Treatment with LX2006 has been generally well tolerated with no Grade 3 treatment-related SAEs to date, no clinically significant complement activation, minimal, transient liver function test (LFT) elevations, and no signs of frataxin over-expression observed in cardiac tissue. One previously disclosed, possibly treatment-related Grade 2 event of asymptomatic myocarditis observed one year after dosing. No participants discontinued from either study.

### *Regulatory Updates*

In July 2025, we announced Breakthrough Therapy designation based on interim clinical data from Phase 1/2 trials showing clinically meaningful improvements in cardiac biomarkers and functional measures. We also announced that LX2006 was selected for FDA Chemistry, Manufacturing, and Controls Development and Readiness Pilot (CDRP) program, created to facilitate CMC registrational readiness and support faster patient access.

In October 2025, in response to our questions regarding the possibility of a faster path to a Biologics License Application, or BLA, the FDA indicated openness to a BLA submission for accelerated approval that includes clinical data from the ongoing Phase 1/2 studies of LX2006 pooled with new clinical data to be generated in the planned pivotal study. The FDA also previously agreed to evaluate the co-primary endpoint of LVMI at a time point earlier than 12 months. Collectively, we believe this regulatory feedback has the potential to reduce the size and length of the planned pivotal study, possibly accelerating the overall timeline to BLA submission.

In February 2026, we submitted the final registrational trial design and statistical analysis plan for the SUNRISE-FA 2 study to the FDA following a Type B meeting. The proposed open-label study is generally consistent with previously communicated guidance on key design elements, such as study size, duration, patient population, and the LVMI primary endpoint, and incorporates FDA feedback on minimizing bias, including physician selection bias of study participants. We expect to receive final feedback from the FDA in the second quarter of 2026 and plan to provide a more detailed update at that time. Initiation of the SUNRISE-FA 2 study is expected in the first half of 2026.

## *LX2020 for the treatment of ACM caused by PKP2 mutations*

### *Overview of ACM*

ACM is a genetic heart disease primarily characterized by myocardial cell loss and the replacement of heart muscle with fibrotic tissue and fatty deposits. ACM can result from mutations in several desmosomal genes. These genetic mutations impair the structure and function of cardiac desmosomes, which are membrane protein complexes engaged in cell-to-cell adhesion and the structural integrity of the ventricular myocardium. Lack of functioning cardiac desmosomes can lead to myocardial cell death and fibrosis, heart dysfunction, rhythm abnormalities, and sudden death. Standard of care may include antiarrhythmics, implantable cardioverter-defibrillators, and ablation procedures; however, none of these therapies address the underlying cause of myocardial dysfunction and ACM.

We estimate that ACM has a prevalence of approximately 130,000 patients in the United States and estimate that over half of all ACM patients have a genetic form of the disease with five desmosomal genes accounting for nearly all of the identified genetic causes of ACM. We have initially targeted the *PKP2* gene because mutations in this gene are the most commonly known genetic cause of ACM. We believe that mutations in the *PKP2* gene are associated with approximately 75% of all genetic cases of ACM, resulting in approximately 60,000 patients affected by the *PKP2* mutation in the United States. Most familial cases of the disease have an autosomal dominant pattern of inheritance, meaning one copy of an altered gene in each cell is sufficient to cause the disorder. Since having only one functioning copy of the *PKP2* gene is insufficient to produce the wild-type phenotype, this results in a phenomenon in genetics known as haploinsufficiency.

Symptoms of the disease can include palpitations, dizziness, heart failure and sudden death. Abnormal function of the right ventricle, fatty or fibrotic infiltrates in the myocardium, abnormal electrocardiogram, or ECG, arrhythmias, or a family history of ACM can all lead physicians to diagnose the disease. No effective treatments for PKP2-ACM exist.

### *Our approach: LX2020*

We are developing LX2020 as an AAVrh10-based gene therapy candidate for the treatment of PKP2-ACM. LX2020 is designed to intravenously deliver a functional *PKP2* gene to cardiac muscle to increase desmosomal PKP2 protein levels and restore myocardial cell function. We believe that by delivering a functional *PKP2* gene, LX2020 has the potential to address the underlying cause of PKP2-ACM for many patients and have a significant effect on lifespan by reassembling the cardiac desmosomes, preventing cardiac arrhythmias and preventing or treating cardiac dysfunction.

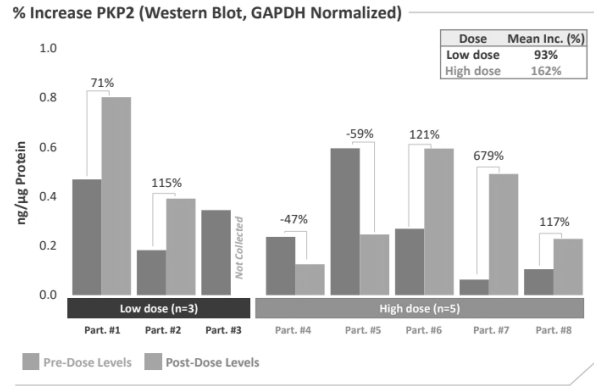
### *LX2020 clinical development and trial design*

LX2020 is currently being evaluated in HEROIC-PKP2, a first-in-human, 52-week, open-label, dose-ascending multicenter trial to determine the safety and tolerability of LX2020 in patients with PKP2-ACM. Preliminary efficacy measures include myocardial protein expression, biomarkers measuring cardiac structure and function, and arrhythmia burden. Key inclusion criteria include: patients aged 18-65 years with a confirmed diagnosis of ACM with either 2010 Task Force Criteria or 2020 International Criteria for ACM; documented PKP2 mutation; existing implantable cardioverter defibrillator (ICD) that is MRI compatible; and a minimum threshold of PVCs over a 24-hour period. LX2020 is administered as a one-time intravenous infusion to patients in at least two ascending-dose cohorts of three patients each, with the potential for cohort expansion. Prednisone and rapamycin are utilized for immune suppression. We have completed enrollment and dosing in this trial, with ten participants dosed: three in the low dose cohort 1 ( $2 \times 10^{13}$  vg/kg), three in the high dose cohort 2 ( $6 \times 10^{13}$  vg/kg), and four in the dose-expansion high dose cohort 3 ( $6 \times 10^{13}$  vg/kg). There is a long-term follow-up for patients who receive LX2020 to monitor ongoing safety for a total of five years, per FDA requirement.

### *Cardiac biopsy results from HEROIC-PKP2 trial*

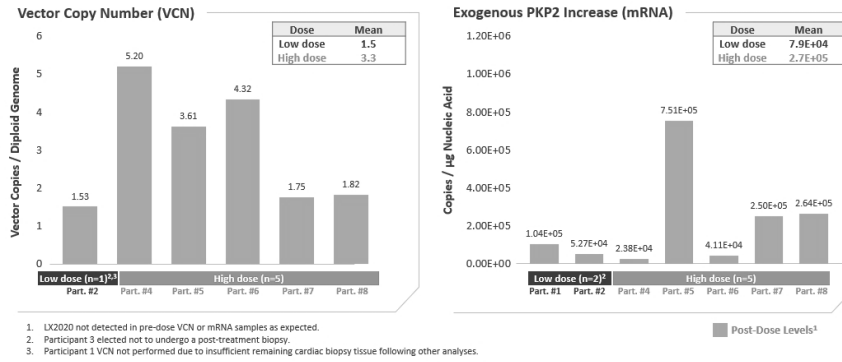
In January 2026, we provided an interim clinical update, which included PKP2 protein expression from participants in HEROIC-PKP2 with post-treatment cardiac biopsies (n=7). The data demonstrated dose-dependent transduction and protein expression, with mean increase in PKP2 protein of 93% in low-dose (n=2) and 162% in high dose (n=5) by western blot, assessed at 3 months post dosing.

## Increases in PKP2 protein expression



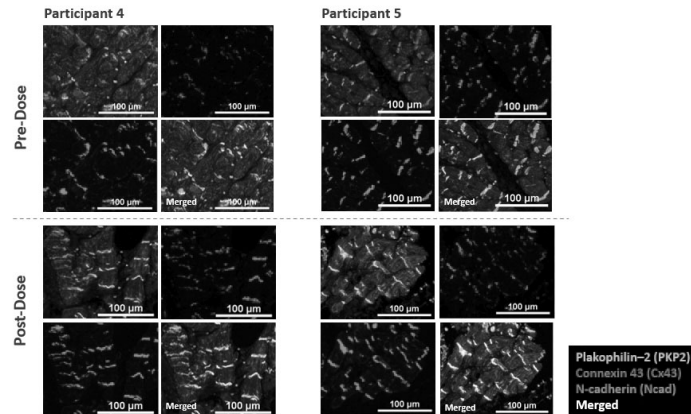
Additionally, dose-dependent increases were observed in mean vector copy number and exogenous mRNA in all participants.

## LX2020 transduction and transcription observed in all participants



Additionally, in two participants at the high dose, immunofluorescence demonstrates appropriate co-localization of PKP2 in desmosome. Following LX2020 administration, PKP2 protein colocalizes with Cx43, a gap junction protein, and Ncad, a desmosomal adhesion protein, within cardiac intercalated discs.

## Immunofluorescence demonstrates appropriate co-localization of PKP2 in desmosome

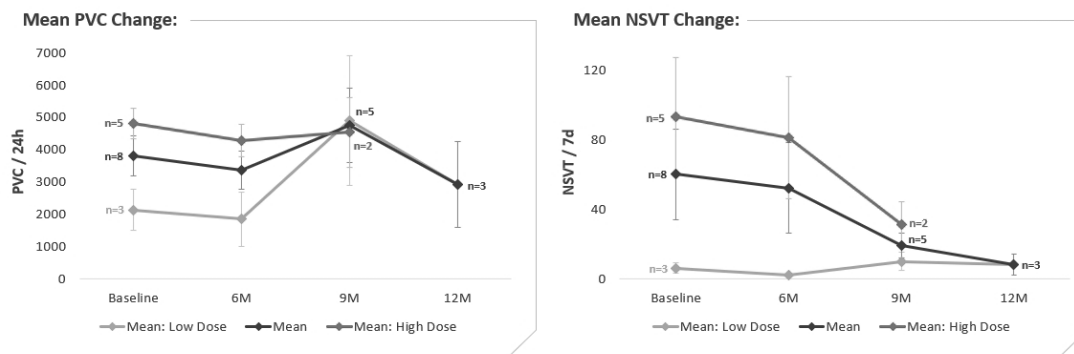


### Cardiac biomarker data from HEROIC-PKP2

In January 2026, we provided an interim clinical data update, which included baseline characteristics and safety data from the ten participants dosed, including three participants in cohort 1 at the low dose and seven participants in cohorts 2 and 3 at the high dose. Clinical efficacy data were inclusive of those participants with at least 6 months of follow-up (n=8).

Arrhythmia burden stabilized or improved in majority of participants with dose-dependent response in NSVT and PVCs. NSVT reduced or stabilized in the majority of participants, with a 22% mean improvement in high-dose cohorts at latest visit (n=5). PVCs reduced or stabilized in the majority of participants, with a 14% mean improvement in high-dose cohorts at latest visit.

#### Reductions in arrhythmia burden over time



Additionally, four of five participants in high-dose cohorts report improvement relative to baseline on the Patient Global Impression of Change, or PGIC, scale, a patient-reported outcome measure. Participants stable across other clinical measures including QRS duration, T-wave inversion, RVEF and New York Heart Association, or NYHA, Class.

### Safety profile of LX2020 from HEROIC-PKP2

Across all participants dosed, LX2020 has been generally well-tolerated to date with no clinically significant complement activation. Elevations in liver function tests (LFT) observed in seven participants at the high dose, treated successfully with re-introduction of low-dose prednisone in five participants and increased prednisone and sirolimus in two participants per the trial protocol. All elevations resolved without complication, hospitalization or other treatment. One Grade 3 serious adverse event of sustained ventricular tachycardia (VT) was observed three months after dosing in a single participant at the high dose and assessed as possibly treatment related. This event is consistent with the natural course of PKP2-ACM and its known clinical manifestations. The participant was successfully treated with anti-arrhythmic medication and discharged with no additional intervention required. No participants discontinued from the HEROIC-PKP2 Phase 1/2 study.

Additionally, we have initiated SNAPSHOT-PKP2, a natural history study designed to evaluate PKP2-ACM disease progression up to two years retrospectively and over twelve months prospectively. The study will enroll up to 20 patients in the United States.

We anticipate sharing additional safety and clinical efficacy data for LX2020 in the fourth quarter of 2026, once all participants in the high dose cohorts reach at least twelve months of follow up.

### ***LX2021 for the treatment of DSP cardiomyopathy***

*LX2021* is a gene therapy candidate we are developing to intravenously deliver the coding sequence for the functional Cx43 protein for a group of inherited cardiac muscle disorders associated with a high risk of sudden death, including ACM and certain forms of dilated cardiomyopathy, which are typically due to abnormalities or deficits in cardiac desmosomes. Cx43 is an integral protein component of gap junctions and functionally allows small molecules and ions to flow directly between cells to allow for electrical synchronization of muscle contraction. In patients with heart disease, including heart failure, Cx43 is often relocated in the lateral walls of myocardial cells. As a result, it is significantly reduced at cardiac muscle cell junctions, especially in ACM populations. Restoring Cx43 protein to cardiac muscle cell-cell junctions can potentially treat multiple genetic causes of ACM because the cardiac loss of Cx43 is a molecular deficit generally observed in all ACM patient populations. DSP is a structural protein critical for force transmission in heart muscle. Mutations in DSP cause a distinct form of ACM and a certain form of dilated cardiomyopathy, known as DSP cardiomyopathy. Unlike many forms of ACM that predominantly affect the right side of the heart, DSP cardiomyopathy frequently affects the left side of the heart. The prevalence of DSP cardiomyopathy is unknown but may be as high as 4% of all inherited dilated cardiomyopathies and 4% of ACM, impacting up to 35,000 individuals in the U.S. Given the role of Cx43 in other forms of heart failure, we are also evaluating *LX2021* for the treatment of additional indications beyond DSP cardiomyopathy.

We have conducted a preclinical study in a cardiac-specific DSP loss-of-function mouse model, or DSP-cKO mice, which is a genetic model of ACM. DSP-cKO mice develop molecular, histological and physiological features of ACM and ultimately die prematurely from heart failure. In this study, we observed that severely diseased mice treated with AAV-based gene therapy expressing Cx43 display fewer arrhythmias, have improved cardiac mechanical function, and experience an almost two-fold increase in lifespan when compared to untreated DSP-cKO adult mice. A single dose of an AAV-based gene therapy candidate representing Cx43 was administered intravenously to the cardiac-specific DSP-cKO mice, at four to six weeks of age. DSP-cKO mice are a genetic model of ACM harboring severe structural disease and loss of Cx43. RNA analysis of the treated mice revealed that cardiac-specific Cx43 gene delivery increased Cx43 RNA levels. Additionally, the Cx43-treated DSP-cKO mice showed a re-expression of mechanical junction proteins in their hearts compared to controls. Essential basement membrane proteins, including N-cadherin, as well as desmosomal proteins, PKP2 and junction plakoglobin, were also restored to cell-cell junctions in Cx43-treated DSP-cKO hearts compared to end-stage untreated DSP-cKO hearts, which had limited localization of junctional proteins at cell-cell junctions. These findings further validate the ability of Cx43 to resurrect the cardiac mechanical muscle junction complex in diseased hearts.

### ***LX2022 for the treatment of HCM caused by TNNI3 mutations***

*LX2022* is a gene therapy candidate we are developing to intravenously deliver a functional *TNNI3* gene to myocardial cells to treat a distinct form of HCM due to mutations in the *TNNI3* gene. With an estimated prevalence of 1 in 500 people in the United States, HCM is one of the most common forms of genetic cardiomyopathy caused by mutations that affect the cardiac sarcomere in approximately 75% of cases. It is estimated that as many as 25,000 patients in the United States are affected by HCM caused by mutations in the gene *TNNI3*. The *TNNI3* gene encodes troponin I, an essential protein in the thin filament of the sarcomere, which is involved in cardiac contraction and relaxation. Mutations in the gene often result in left ventricular hypertrophy and restrictive cardiomyopathy, leading to arrhythmias and heart failure.

Our preclinical data from an A157V *TNNI3* murine model, a model which results in cardiac dysfunction, was generated to validate the delivery of an AAV-based therapy candidate expressing human *TNNI3* regulated by a cardiac-specific promoter. At four weeks post injection, the level and specificity of *TNNI3* gene expression were evaluated in the liver, skeletal muscle, and heart tissue. As *TNNI3* was regulated by a cardiac-specific promoter, expression of *TNNI3* was only detected in the heart tissue. Additionally, retro-orbital injection of AAV9-hTNNI3 expressing A157V mutation was performed on three-month-old mice euthanized four weeks later. The hearts were dissected and immunostained. Immunostaining revealed robust expression of human *TNNI3* in the cardiomyocyte after treatment with AAV-based *TNNI3*. Moreover, high magnification imaging demonstrated the incorporation of *TNNI3* within the cardiac sarcomere. Based on these preclinical findings, we believe that AAV-based therapy expressing *TNNI3* can be administered to achieve transgene expression in heart tissue.

### **Outside of Core Focus Area: APOE4-associated Alzheimer's disease programs**

In addition to our cardiac gene therapy programs, we have a portfolio of approaches aimed at treating the genetics underlying Alzheimer's disease. We believe precision therapies, particularly those focused on the underlying genetics of Alzheimer's disease, may have a substantial impact on this treatment landscape. Importantly, we believe gene therapy allows for a unique approach to treating the genetics of Alzheimer's disease by delivering a therapeutic which acts upstream of both the amyloid- $\beta$  and tau-driven pathology of Alzheimer's disease. This treatment strategy is designed to impact multiple pathways, as opposed to most other treatments in development which target a single mechanism of Alzheimer's disease.

In our lead Alzheimer's disease program, LX1001, we are initially targeting homozygous APOE4-associated Alzheimer's disease patients by administering AAVrh10 containing the *APOE2* gene. Our approach to treating Alzheimer's disease is predicated on the belief that expressing the protective APOE2 in the CNS of *APOE4* homozygous patients will halt or slow the progression of Alzheimer's disease. We believe these patients represent an ideal target for gene therapy because the *APOE4* homozygous profile is the most common genetic driver of Alzheimer's disease.

Our current gene therapy programs for the treatment of Alzheimer's disease are:

- *LX1001*: a clinical-stage AAVrh10-based gene therapy candidate that is designed to express the protective APOE2 protein in the CNS of *APOE4* homozygous patients.
- *LX1021*: an early-stage AAVrh10-based gene therapy candidate that is designed to express the Christchurch-modified APOE2 protein in the CNS of *APOE4* homozygous patients. The Christchurch mutation has been observed to protect patients against Alzheimer's disease even in the presence of significant amyloid pathology.
- *LX1020*: an early-stage AAVrh10-based gene therapy candidate that is designed to express the protective APOE2 protein in the CNS of *APOE4* homozygous patients, while concurrently delivering miRNA to suppress the expression of the APOE4 protein.

LX1001 has been evaluated in an open-label, dose-ranging Phase 1/2 clinical trial in patients who are *APOE4* homozygous patients with clinical diagnoses ranging from mild cognitive impairment to mild or moderate dementia due to Alzheimer's disease. All participants had evidence of amyloid plaque by PET scan and/or CSF biomarkers consistent with Alzheimer's disease at baseline. The trial evaluated four ascending dose cohorts ( $1.4 \times 10^{10}$  vg/ml,  $4.4 \times 10^{10}$  vg/ml,  $1.4 \times 10^{11}$  vg/ml and  $1.4 \times 10^{14}$  total genomes), with the dose for each patient in the first three cohorts determined based on CSF volume measured by MRI and patients in the fourth cohort receiving a fixed dose. Fifteen patients were enrolled in the study across the four dose cohorts. The primary objective of the trial was to evaluate the safety of LX1001 administered to the CNS via injection between cervical vertebrae 1 and 2, or intracisternal injection, and to establish the maximum tolerated dose. The trial was also designed to evaluate the conversion of the CSF from the *APOE4* homozygous profile to an *APOE4/E2* profile. Additional secondary endpoints included CSF biomarkers, including A $\beta$ 42, total tau, and phosphorylated tau, amyloid PET scan, structural MRI imaging and cognitive tests. Corticosteroids were utilized for immune suppression. Participants will continue to be evaluated for ongoing safety in a long-term follow-up study, per FDA requirement.

We have completed the Phase 1/2 trial of LX1001 and shared preliminary results across all four dose cohorts in a late-breaking oral presentation at the Clinical Trials on Alzheimer's Disease Conference in October 2024. In January 2025 we announced that we are seeking partnering opportunities to continue development.

## Competition

The biotechnology and pharmaceutical industries are characterized by rapidly changing technologies, significant competition and a strong emphasis on intellectual property. This is also true for the development and commercialization of treatments for cardiovascular and neurodegenerative diseases such as FA and PKP2-ACM and broadly across gene therapies. While we believe that our management and scientific team's deep expertise in gene therapy provides us with competitive advantages, we face competition from several sources, including large and small biopharmaceutical companies, government agencies and academic and private research institutions. Not only must we compete with other companies that are focused on gene therapy technology, but any product candidates that we successfully develop and commercialize will compete with existing therapies, to the extent applicable, and new therapies that may become available in the future.

Drug development, particularly in the gene therapy field, is highly competitive and subject to rapid and significant technological advancements. A significant unmet medical need exists in each of the indications that we are targeting, and it is likely that additional drugs will become available in the future for the treatment of these diseases.

We are aware that our competitors are developing product candidates for the treatment of diseases that our product candidates will target. With respect to LX2006, we are aware of clinical stage gene therapy programs in development at Solid Biosciences Inc. and those being developed in collaborations between Voyager Therapeutics, Inc. and Neurocrine Biosciences, Inc. Among other treatment modalities for FA, we are aware that Larimar Therapeutics, Inc. is developing a clinical stage product candidate, CTI-1601, and that PTC Therapeutics, Inc. is evaluating next steps for vatiquinone following a Complete Response Letter on their new drug application to the FDA. Reata Pharmaceuticals, Inc.'s omaveloxolone (Skyclarys) was approved by the FDA in 2023, and Biogen Inc. acquired Reata Pharmaceuticals, Inc. for approximately \$7.3 billion in the same year and is currently commercializing Skyclarys.

With respect to LX2020, both Rocket Pharmaceuticals Inc., or Rocket, and Tenaya Therapeutics Inc. are developing an AAV-based gene therapy candidate designed to deliver a functional *PKP2* gene to patients with PKP2-ACM.

Many of our existing or potential competitors have substantially greater financial, technical and human resources than we do and significantly greater experience in the discovery and development of product candidates, as well as in obtaining regulatory approvals of those product candidates in the United States and in foreign countries.

These competitors also compete with us in recruiting and retaining qualified scientific and management personnel and establishing clinical trial sites and patient registration for clinical trials, as well as in acquiring technologies complementary to, or necessary for, our programs. Mergers and acquisitions in the pharmaceutical and biotechnology industries may result in even more resources being concentrated among a smaller number of our competitors. Smaller or early-stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies.

We will face competition from other drugs or from other non-drug products and treatments currently approved or that will be approved in the future in the cardiovascular and neurology field, including for the treatment of diseases and diseases in the therapeutic categories we intend to target. Therefore, our ability to compete successfully will depend largely on our ability to:

- develop, manufacture and commercialize drugs that are superior to other products in the market;
- demonstrate through our clinical trials that our product candidates are differentiated from existing and future therapies;
- attract qualified scientific, product development and commercial personnel;
- obtain patent or other proprietary protection for our medicines;
- obtain required regulatory approvals;
- obtain coverage and adequate reimbursement from, and negotiate competitive pricing with, third-party payors; and
- successfully collaborate with pharmaceutical companies in the discovery, development and commercialization of new medicines.

The availability of our competitors' products could limit the demand, and the price we are able to charge, for any product candidate we develop. The inability to compete with existing or subsequently introduced drugs would have an adverse impact on our business, financial condition and prospects. In addition, the reimbursement structure of currently approved or future gene therapies by other companies could impact the anticipated reimbursement structure of our gene therapies, if approved, and our business, financial condition, results of operations and prospects.

Established pharmaceutical companies may invest heavily to accelerate discovery and development of novel compounds or to in-license novel compounds that could make our product candidates less competitive. In addition, any new product that competes with an approved product must demonstrate compelling advantages in efficacy, convenience, tolerability and safety in order to overcome price competition and to be commercially successful. Accordingly, our competitors may succeed in obtaining patent protection, discovering, developing, receiving regulatory and marketing approval for, or commercializing, drugs before we do, which would have an adverse impact on our business and results of operations.

### **Intellectual property**

We actively seek to protect our proprietary technology, inventions, and other intellectual property that is commercially important to the development of our business by a variety of means, such as seeking, maintaining, and defending patent rights, whether developed internally or licensed from third parties. We also may rely on trade secrets and know-how relating to our proprietary technology platform, on continuing technological innovation and on in-licensing opportunities to develop, strengthen and maintain the strength of our position in the field of gene therapy that may be important for the development of our business. We also intend to seek patent protection or rely upon trade secret rights to protect other technologies that may be used to discover and validate targets, and that may be used to manufacture and develop novel gene therapy products. We are a party to license agreements that give us rights to use specific technologies in our gene therapy candidates and in manufacturing our products. Additional regulatory protection may also be afforded through data exclusivity, market exclusivity and patent term extensions where available.

As of December 31, 2025, we in-licensed four U.S. patents, 16 pending U.S. non-provisional patent applications, one pending PCT application, eight foreign patents and 106 pending foreign applications.

In regard to our LX2006 product candidate, we in-license from Cornell University and Adverum Biotechnologies, Inc., or Adverum, two U.S. patents, five pending U.S. non-provisional patent applications, five foreign patents granted in India, Mexico, South Africa, and New Zealand and nine pending foreign applications pending in such jurisdictions as Australia, Brazil, Canada, Eurasia, Europe, Hong Kong, Israel and Mexico. Not accounting for any patent term adjustment and assuming that all annuity and/or maintenance fees are paid timely, these patents, if granted, will expire in 2033. These patents and pending patent applications disclose and/or contain composition-of-matter claims to an AAV vector encoding *FXN*, or a fragment thereof, and disclose and/or contain claims to methods of producing and methods of treatment using the AAV *FXN* vector. Cornell University co-owns these patents and patent applications with Institut National de la Santé et de la Recherche Médicale, Centre National de la Recherche Scientifique, Université de Strasbourg, Université Paris-Saclay and Assistance Publique-Hôpitaux de Paris.

We have rights to additional patent applications that we co-own with Cornell University, and which also relate to our LX2006 cardiac FA program. We in-license Cornell University's interest in one pending U.S. non-provisional patent application and 14 pending foreign applications in such jurisdictions as Australia, Brazil, Canada, China, Eurasia, Europe, Hong Kong, Israel, India, Japan, Korea, Mexico, New Zealand, and South Africa. Not accounting for any patent term adjustment and assuming that all annuity and/or maintenance fees are paid timely, if granted, patents issuing from these applications will expire in 2043. These pending applications disclose and/or contain composition-of-matter claims to a pharmaceutical dosage form of viral vectors encoding *FXN*, and disclose and/or contain claims to methods of producing and methods of treatment using the pharmaceutical dosage form.

In regard to our LX2020 product candidate, we in-license from The Regents of the University of California one pending U.S. non-provisional patent application and 17 pending foreign applications in such jurisdictions as Australia, Brazil, Canada, China, Colombia, Eurasia, Europe, Hong Kong, Israel, India, Japan, Korea, Mexico, New Zealand, Singapore and South Africa. Not accounting for any patent term adjustment and assuming that all annuity and/or maintenance fees are paid timely, if granted, patents issuing from these applications will expire in 2042. These pending applications disclose and/or contain composition-of-matter claims to a vector encoding human PKP2, and disclose and/or contain claims to methods of producing and methods of treatment using the PKP2 vector.

In regard to our LX2021 product candidate, we in-license from The Regents of the University of California two U.S. patents, three pending U.S. non-provisional patent applications, two foreign patents granted in Australia and Europe, respectively, and nine pending foreign applications pending in Australia, Canada, China, Europe, Israel, and Japan. Not accounting for any patent term adjustment and assuming that all annuity and/or maintenance fees are paid timely, if granted, these patents and patent applications will expire in 2038. These pending patent applications disclose and/or contain composition-of-matter claims to a vector encoding a human Cx43 polypeptide, and disclose and/or contain claims to methods of producing and methods of treatment using the Cx43 vector.

In regard to our LX2022 product candidate, we in-license from The Regents of the University of California one pending U.S. non-provisional patent application. Not accounting for any patent term adjustment and assuming that all annuity and/or maintenance fees are paid timely, if granted, patent applications claiming the priority to this patent application, will expire in 2040. This pending U.S. non-provisional patent application discloses and/or contains composition-of-matter claims to a vector encoding a human *TNNI3* gene, and discloses and/or contains claims to methods of producing and methods of treatment using the *TNNI3* vector.

In regard to our LX1020 product candidate, we in-license from Cornell University one pending U.S. non-provisional application and 16 pending foreign applications pending in Australia, Brazil, Canada, China, Colombia, Europe, Hong Kong, Israel, Japan, Korea, Mexico, New Zealand, Singapore and South Africa. Not accounting for any patent term adjustment and assuming that all annuity and/or maintenance fees are paid timely, if granted, these patent applications will expire in 2040. We also in-license from Cornell an additional pending PCT application related to our LX1020 product candidate. Not accounting for any patent term adjustment and assuming that all annuity and/or maintenance fees are paid timely, if granted, patents claiming priority to the pending PCT application will expire in 2045. The pending patent applications disclose and/or contain composition-of-matter claims to a vector encoding human APOE2 protein and encoding one or more RNAi nucleic acid sequences for inhibition of APOE4 mRNA, and disclose and/or contain claims to methods of producing and methods of treatment using the APOE2+/APOE4- vector.

In regard to our LX1021 product candidate, we in-license from Cornell University two pending U.S. non-provisional application, one foreign patent granted in Australia, and 13 pending foreign applications pending in Brazil, Canada, China, Europe, Hong Kong, Israel, Japan, Korea, Mexico, New Zealand, Singapore and South Africa. Not accounting for any patent term adjustment and assuming that all annuity and/or maintenance fees are paid timely, if granted, these patent applications will expire in 2040. The patent and pending patent applications disclose and/or contain composition-of-matter claims to a vector encoding a mutated human apolipoprotein E protein, and disclose and/or contain claims to methods of producing and methods of treatment using the mutated human apolipoprotein E vector.

We also in-license from Cornell additional patent applications, which relate to our LX1021 product candidate. We in-license from Cornell University one pending U.S. non-provisional application and 12 pending foreign applications pending in such jurisdictions as Australia, Brazil, Canada, China, Europe, Israel, Japan, Korea, Mexico, New Zealand, Singapore and South Africa. Not accounting for any patent term adjustment and assuming that all annuity and/or maintenance fees are paid timely, if granted, the pending patent applications will expire in 2044. The pending patent applications disclose and/or contain composition-of-matter claims to a vector encoding a mutated human apolipoprotein E protein, and disclose and/or contain claims to methods of producing and methods of treatment using the mutated human apolipoprotein E vector.

In regard to our LX1001 product candidate, we co-own pending patent applications with Cornell University. We in-license Cornell University's interest in one pending U.S. non-provisional patent application and 13 pending foreign applications pending in such jurisdictions as Australia, Brazil, Canada, China, Europe, Hong Kong, Israel, Japan, Korea, Mexico, New Zealand, Singapore and South Africa. Not accounting for any patent term adjustment and assuming that all annuity and/or maintenance fees are paid timely, if granted, the pending patent applications will expire in 2043. The pending patent applications disclose and/or contain composition-of-matter claims to a pharmaceutical dosage form of viral vectors encoding human APOE2, and discloses and/or contains claims to methods of producing and methods of treatment using the pharmaceutical dosage form.

#### ***License, research collaboration and sponsored research agreements***

##### *First license agreement with Cornell University*

In May 2020, we entered into a license agreement with Cornell University, or the Cornell First License Agreement, pursuant to which we obtained a sublicensable, worldwide license under certain patents to make, use, and sell products that are covered by a valid claim of a licensed patent, are based on the transferred IND from Cornell University, use any licensed materials, or are produced using a licensed method, and to practice certain licensed technology, in all cases, for all human and non-human prophylactic and therapeutic uses. In July 2022, we entered into Amendment No. 1 to the Cornell First License Agreement that updated fees and royalties payable by us under the Cornell First License Agreement. In September 2022, we entered into Amendment No. 2 to the Cornell First License Agreement that clarified patent prosecution and maintenance obligations and added certain inventions and patents to the list of inventions and patent rights covered under the Cornell First License Agreement. The technology under the license includes portfolios for APOE, Alzheimer's disease, and Anti-Tau, although our license is not restricted by such indications. The license is exclusive with respect to certain patents and non-exclusive with respect to other patents. Additionally, under the Cornell First License Agreement, Cornell University assigned to us an IND for the use of AAVrh10.hAPOE2 vector to treat *APOE4* homozygous patients who are at risk of having or have Alzheimer's disease. Cornell University reserved the rights to publish and disseminate information about inventions included in the licensed technology and licensed patents, and to use, and allow other nonprofit institutions to use, the patents and technology for educational and research purposes.

We are obligated to diligently proceed with the development, manufacture, and sale of licensed products, to raise certain amounts within specified time frames, to achieve certain development milestones within specified time frames, to meet agreed minimum-spend requirements, and to meet other diligence obligations. If we fail to perform these obligations, Cornell University could terminate the Cornell First License Agreement in full or in part in specified circumstances, subject to certain rights for us to extend these time frames.

Under the Cornell First License Agreement, we paid Cornell University an upfront payment of approximately \$0.15 million and entered into a purchase agreement for approximately \$0.6 million of convertible preferred securities. We are obligated to pay Cornell University an annual license maintenance fee ranging from the low four digits to the mid five digits, increasing annually until such time that we are commercially selling a licensed product. Under the Cornell First License Agreement, we are obligated to pay Cornell University up to \$8.4 million for each portfolio upon the achievement of specific clinical and regulatory milestones. We are obligated to pay Cornell University a flat royalty in the mid-single-digits based on net sales of a licensed product in a country, which royalty rate increases by one percent if the licensed product is an orphan drug, subject to a reduction upon the expiration of valid claims in licensed patents and certain reductions for third-party licenses. In addition, in certain specific instances where sales are made by a sublicensee, the royalty rate increases by an amount in the low to mid-single digits. If the royalties are below certain agreed amounts, we are required to pay Cornell University minimum annual royalties ranging from low-six digits to low-seven digits. The royalty term continues for each licensed product on a country-by-country basis beginning on the first commercial sale of such licensed product and ending on the latest of (a) expiration or invalidation of the last valid claim in the licensed patent, (b) the expiration of regulatory exclusivity, and (c) the month of the first commercial launch of a generic equivalent in such country. We are also obligated to pay Cornell University a percentage of sublicensing fees in the low-double digits to mid-double digits.

We may terminate the Cornell First License Agreement or any portfolio thereunder at any time upon ninety (90)-days' advance written notice to Cornell University. Cornell University may terminate the Cornell First License Agreement if we commit a material breach and fail to cure such breach within a specified cure period after written notice or if we challenge the validity of a licensed patent. Upon expiration of the royalty term of a given licensed product in a country, the license becomes non-exclusive and royalty-free. Upon termination of the Cornell First License Agreement, all licenses and rights granted by either party will terminate, although we will have a period of time to sell off any remaining licensed product.

#### *Second license agreement with Cornell University*

In May 2020, we entered into a second license agreement with Cornell University, or the Cornell Second License Agreement, pursuant to which we obtained a sublicensable, worldwide license under certain patents to make, use, and sell products that are covered by a valid claim of a licensed patent, are based on the transferred IND from Cornell University, use any licensed materials, or are produced using a licensed method, and to practice certain licensed technology, in all cases, for all human and non-human prophylactic and therapeutic uses. In January 2022, we entered into Amendment No. 1 to the Cornell Second License Agreement, whereby we paid Cornell a material transfer fee and a license fee totaling \$30,000, and Cornell added certain materials to the list of original materials subject to the Cornell Second License Agreement. In July 2022, we entered into Amendment No. 2 to the Cornell Second License Agreement that updated fees and royalties payable by us under the Cornell Second License Agreement. The technology under the license includes a portfolio for FA, although our license is not restricted by such indications. The license is exclusive with respect to certain patents and non-exclusive with respect to other patents. Cornell University reserved the rights to publish and disseminate information about inventions included in the licensed technology and licensed patents, and to use, and allow other nonprofit institutions to use, the patents and technology for educational and research purposes.

We are obligated to diligently proceed with the development, manufacture, and sale of licensed products, to raise certain amounts within specified time frames, to achieve certain development milestones within specified time frames, to meet agreed minimum-spend requirements, and to meet other diligence obligations. If we fail to perform these obligations, Cornell University could terminate the Cornell Second License Agreement in full or in part in specified circumstances, subject to certain rights for us to extend these time frames.

Under the Cornell Second License Agreement, we paid Cornell University an upfront payment of approximately \$0.15 million and entered into a purchase agreement for approximately \$0.6 million of convertible preferred securities. We are obligated to pay Cornell University an annual license maintenance fee ranging from the low four digits to the mid five digits, increasing annually until such time that we are commercially selling a licensed product. Under the Cornell Second License Agreement, we are obligated to pay Cornell University up to \$4.3 million for two portfolios and up to \$0.6 million for a third portfolio upon the achievement of specific clinical and regulatory milestones. Upon submitting our IND application for LX2006 to the FDA in the first quarter of 2022, we achieved the first clinical milestone under the Cornell Second License Agreement, and we paid \$0.1 million to Cornell University in the second quarter of 2022 in connection with this milestone. We are obligated to pay Cornell University a flat royalty in the mid-single-digits based on net sales of a licensed product in a country, which royalty rate increases by one percent if the licensed product is an orphan drug, subject to a reduction upon the expiration of valid claims in licensed patents and certain reductions for third-party licenses. In addition, in certain specific instances where sales are made by a sublicensee, the royalty rate increases by an amount in the low to mid-single digits. If the royalties are below certain agreed amounts, we are required to pay Cornell University minimum annual royalties ranging from low six digits to low seven digits. The royalty term continues for each licensed product on a country-by-country basis beginning on the first commercial sale of such licensed product and ending on the latest of (a) expiration or invalidation of the last valid claim in the licensed patent, (b) the expiration of regulatory exclusivity, and (c) the month of the first commercial launch of a generic equivalent in such country. We are also obligated to pay Cornell University a percentage of sublicensing fees ranging in the low double digits.

We may terminate the Cornell Second License Agreement or any portfolio thereunder at any time upon ninety (90)-days' advance written notice to Cornell University. Cornell University may terminate the Cornell Second License Agreement if we commit a material breach and fail to cure such breach within a specified cure period after written notice or if we challenge the validity of a licensed patent. Upon expiration of the royalty term of a given licensed product in a country, the license becomes non-exclusive and royalty-free. Upon termination of the Cornell Second License Agreement, all licenses and rights granted by either party will terminate, although we will have a period of time to sell off any remaining licensed product.

### *Third license agreement with Cornell University*

In April 2024, we entered into a third license agreement with Cornell University, or the Cornell Third License Agreement, pursuant to which we obtained certain rights for FA cardiomyopathy, including an exclusive license to practice under certain patent rights generated in animal studies conducted by Cornell University on our behalf and a non-exclusive license to know-how concerning a gene therapy for FA cardiomyopathy and current and future data generated in an ongoing Cornell University investigator-initiated Phase 1A trial of a gene therapy candidate AAVrh10.hFXN, known as LX2006 at Lexeo, to treat FA cardiomyopathy. Both licenses are worldwide and cover products with human and non-human prophylactic and therapeutic uses. Cornell University has also granted us a right of reference to its IND application for a gene therapy for FA cardiomyopathy.

We are obligated to diligently proceed with the development, manufacture, and sale of licensed products, to raise certain amounts within specified time frames, to achieve certain development milestones within specified time frames, to meet agreed minimum-spend requirements, and to meet other diligence obligations. If we fail to perform these obligations, Cornell University could terminate the Cornell Third License Agreement in full or in part in specified circumstances, subject to certain rights for us to extend these time frames.

Under the Cornell Third License Agreement, we paid a license issue fee and an initial data transfer fee to Cornell University totaling \$0.6 million. Additionally, we will be paying an annual data transfer fee of \$50,000 until data is no longer being gathered. We have agreed to pay annual license maintenance fees ranging from \$2,500 to \$25,000 until such time we commercialize a licensed product. In addition, we will pay Cornell University up to an aggregate of \$2.1 million in regulatory milestones and up to an aggregate of \$100 million in commercial milestones, plus low single digit royalties on net sales.

In March 2025, we entered into a Research Collaboration Agreement with Cornell University on behalf of Weill Cornell Medicine, or the 2025 Cornell Collaboration Agreement, in connection with the Cornell Third License Agreement entered into in April 2024. Under the 2025 Cornell Collaboration Agreement, we committed to fund scientific research at Weill Cornell Medicine related to intellectual property and know-how licensed to us pursuant to the Cornell Third License Agreement. Cornell University reserved the rights to publish and disseminate information about the results generated from Cornell University's investigator's conduct of the research.

Under the Cornell Third License Agreement, we committed to provide funding for research projects at Weill Cornell Medicine of \$500,000 per year over a two-year period. The total budget for the research under the 2025 Cornell Collaboration Agreement is approximately \$1.1 million, which will be credited against our research funding commitment under the Cornell Third License Agreement.

With respect to each Weill Cornell Medicine invention and joint invention and related joint results that is either an improvement to, or is dominated by, the patent rights under the Cornell Third License Agreement or is specifically designed for a licensed product under the Cornell Third License Agreement, or an Improvement, and for which we have made an election to obtain such inventions, the Cornell Third License Agreement will be amended to include license grants to such Improvements following payment of the relevant amendment fee in the low five digits. With respect to each Weill Cornell Medicine invention and joint invention and related joint results that are not Improvements, we have the first option to negotiate for a royalty-bearing, worldwide license under such intellectual property to develop, make, have made, use, offer for sale, sell, have sold and import products on commercially reasonable terms.

The 2025 Cornell Collaboration Agreement has a two-year term and may be terminated earlier by either party upon a material breach that remains uncured for 60 days following written notice.

### *License agreement with Adverum*

In January 2021, we entered into a license agreement with Adverum, which we amended in February 2022 pursuant to the First Amendment to the Adverum Agreement, such agreement as amended, the Amended Adverum Agreement. Pursuant to the Amended Adverum Agreement, we obtained an exclusive, sublicensable, worldwide license under certain patents, know-how, and other intellectual property relating to viral vector technology for gene therapy applications for the treatment of FA cardiomyopathy.

We are responsible for the development, manufacture, and commercialization of gene therapy products that consist of a specific nucleic acid sequence that is delivered by a specific gene therapy, or the Products. We are obligated to use commercially reasonable efforts to develop, obtain regulatory approval for, and commercialize the Products.

Under the Amended Adverum Agreement, we paid Adverum a \$7.5 million upfront payment. We are obligated to pay Adverum up to \$17.5 million upon the achievement of specified development and regulatory milestones, including a \$3.5 million development milestone that was achieved in the first quarter of 2023, and up to \$49 million in commercialization and sales milestones for the Products. We are obligated to pay Adverum tiered royalties ranging from high single-digits to sub teens based on annual aggregate worldwide net sales of Products, subject to reductions upon the expiration of valid claims in licensed patents and third-party licenses. The royalty term continues for each Product on a country-by-country basis beginning on the first commercial sale of such Product and ending on the latest of (a) expiration of the last valid claim in the licensed patent that covers the manufacture, use, or sale of the Product in such country, (b) the expiration of all regulatory and data exclusivity in such country, and (c) ten years after the first commercial sale of such Product in such country.

The Amended Adverum Agreement will expire, unless earlier terminated, on the expiration of the last royalty term for a Product in a particular country. We have the right to terminate the Amended Adverum Agreement at any time upon one-hundred twenty days' advance written notice to Adverum. In addition, subject to certain conditions, either we or Adverum may terminate the Amended Adverum Agreement upon the insolvency of the other or if the other party commits a material breach of the agreement and fails to cure such breach within a specified cure period after written notice is provided. Additionally, Adverum may terminate the Amended Adverum Agreement if we challenge the validity of any licensed patents. Upon expiration of the Amended Adverum Agreement, the license becomes royalty-free, irrevocable and perpetual. Upon termination of the Amended Adverum Agreement, all licenses, sublicenses, and rights granted by either party will terminate.

On December 9, 2025, Eli Lilly and Company, or Lilly, announced the acquisition of Adverum pursuant to a tender offer by Lilly's wholly owned subsidiary to purchase all outstanding shares of Adverum common stock. Accordingly, Lilly assumed all of Adverum's obligations and responsibilities under the Amended Adverum Agreement after the acquisition.

*First license agreement with The Regents of University of California, San Diego*

Stelios Therapeutics, Inc., or Stelios, which we acquired in August 2021, is successor-in-interest to ARVC Therapeutics, Inc., or ARVC Therapeutics, under a worldwide license agreement entered into by ARVC Therapeutics and The Regents of the University of California, on April 23, 2020, or the UCSD First License Agreement, pursuant to which they obtained a license to certain intellectual property related to gene therapies for ARVC. The UCSD First License Agreement relates to our development efforts for our LX2021 program. Pursuant to the UCSD First License Agreement, we obtained an exclusive, sublicensable, worldwide license under certain patents to make, use, sell, offer for sale and import services, methods, composition and products that incorporate or are developed using any licensed invention or licensed methods, or is covered by a valid claim of a licensed patent, or a Product, in all cases for all diagnostic and therapeutic uses. The Regents of University of California, San Diego, or UCSD, reserved the right to practice the relevant invention and licensed patents for educational and research purposes, to publish and disseminate information about the relevant invention and licensed patents, and to allow other nonprofit institutions to use, publish, or disseminate information about the relevant inventions and licensed patents for educational and research purposes.

Under the UCSD First License Agreement, we are obligated to achieve certain development milestones within specified time frames, to use commercially reasonable efforts to diligently develop, manufacture, and sell Products, and to meet agreed minimum-spend requirements. If we fail to perform any of these obligations, UCSD could either terminate the UCSD First License Agreement or convert the exclusive license to a nonexclusive license, subject to certain rights for us to extend these time frames.

The UCSD First License Agreement required Stelios to pay one-time up-front non-refundable cash fees of \$20,000. We are obligated to pay annual license maintenance fees in the mid-four digits to low-five digits, increasing until such time that we are commercially selling a Product. We are also obligated to pay up to \$4.75 million upon the achievement of specific development and commercialization milestones for the first Product and low- to mid-single digit royalties based on aggregate net sales, subject to certain reductions for third-party licenses. The royalty term continues until the expiration of the UCSD First License Agreement. Under the UCSD First License Agreement, if the royalties are below certain agreed amounts, we are required to pay UCSD minimum annual royalties ranging from low- to mid-five digits. We are also obligated to pay UCSD a percentage of sublicensing fees ranging in the low double digits. In the event that we assign the UCSD First License Agreement, we will be obligated to pay an assignment fee that will be determined based on certain aspects of the assignment.

The UCSD First License Agreement will expire, unless earlier terminated, on the expiration of the last claim of any licensed patent or patent applications in a particular country. We have the right to terminate the UCSD First License Agreement at any time upon sixty (60)-days' written notice to UCSD. UCSD may terminate the UCSD First License Agreement if we commit a material breach, if we fail to meet certain specified milestones within the prescribed time periods, if we are delinquent on any report or payment or provide an intentionally misleading report, if we fail to diligently develop and commercialize the licensed products, or if we challenge the validity of a licensed patent, in each case only if we fail to cure such problem within a specified cure period after written notice. Upon termination of the UCSD First License Agreement, all licenses and rights granted by UCSD to us will terminate.

*Second license agreement with The Regents of University of California, San Diego*

Stelios, which we acquired in August 2021, entered into a worldwide license agreement in August 2020 with The Regents of the University of California, or the UCSD Second License Agreement, pursuant to which they obtained a license to certain intellectual property and materials related to gene therapies for HCM. The UCSD Second License Agreement relates to our development efforts for our LX2022 programs. Pursuant to the UCSD Second License Agreement, we obtained an exclusive, sublicensable, worldwide license under certain patents to make, use, sell, offer for sale and import services, methods, composition and products that incorporate or are developed using any licensed invention or licensed methods or is covered by a valid claim of a licensed patent, or a Product, and a non-exclusive license to use nonpublic technical information, or Technology, in all cases for all diagnostic and therapeutic uses. UCSD reserved the rights to practice the relevant invention, Technology and licensed patents for educational and research purposes, to publish and disseminate information about the relevant invention, Technology and licensed patents, and to allow other nonprofit institutions to use, publish, or disseminate information about the relevant invention, Technology and licensed patents for educational and research purposes.

Under the UCSD Second License Agreement, we are obligated to achieve certain development milestones within specified time frames and to use commercially reasonable efforts to diligently develop, manufacture, and sell Products. If we fail to perform any of these obligations, UCSD could either terminate the UCSD Second License Agreement or convert the exclusive license to a nonexclusive license subject to certain rights for us to extend these time frames.

The UCSD Second License Agreement required Stelios to pay one-time up-front non-refundable cash fees of \$20,000. We are obligated to pay annual license maintenance fees in the mid-four digits to low-five digits, increasing until such time that we are commercially selling a Product. We are also obligated to pay up to \$2.4 million upon the achievement of certain development and commercialization milestones for the first Product and low-single digit royalties based on aggregate net sales, subject to certain reductions for third-party licenses. The royalty term continues until the expiration of the UCSD Second License Agreement. We are also obligated to pay UCSD a percentage of sublicensing fees ranging in the low double digits.

On November 14, 2025, the Company entered into an amended and restated license agreement with UCSD, which amended, restated, and superseded the UCSD Second License Agreement in its entirety (the "Amended and Restated UCSD Second License Agreement"). The Amended and Restated UCSD Second License Agreement maintained the Company's prior exclusive worldwide license to materials and intellectual property related to a gene therapy for hypertrophic cardiomyopathy, replaced Stelios with the Company as licensee, and exclusively in-licensed additional materials and intellectual property related to the LX2022 program. The economic terms in the Amended and Restated UCSD Second License Agreement are substantially similar to the terms of the UCSD Second License Agreement.

The Amended and Restated UCSD Second License Agreement will expire, unless earlier terminated, on the expiration of the last claim of any licensed patent or patent applications in a particular country. We have the right to terminate the Amended and Restated UCSD Second License Agreement at any time upon sixty (60)-days' written notice to UCSD. UCSD may terminate the Amended and Restated UCSD Second License Agreement if we commit a breach, if we fail to meet certain specified milestones within the prescribed time periods, if we are delinquent on any report or payment, if we are not diligently developing or commercializing Products in accordance with our diligence obligations, if we provide any intentionally false report, or if we challenge the validity of a licensed patent, in each case only if we fail to cure such problem within a specified cure period after written notice. In the event that we assign the Amended and Restated UCSD Second License Agreement, we will be obligated to pay an assignment fee that will be determined based on the acquisition price. Upon termination of the Amended and Restated UCSD Second License Agreement, all licenses and rights granted by UCSD to us will terminate.

*Third license agreement with The Regents of University of California, San Diego*

In October 2021, we entered into a worldwide license agreement with UCSD, or the UCSD Third License Agreement, and collectively with the UCSD First License Agreement and the UCSD Second License Agreement, The UCSD License Agreements, pursuant to which we obtained a license to materials and intellectual property related to a gene therapy for ARVC. The UCSD Third License Agreement relates to our development efforts for our LX2020 program. Pursuant to the UCSD Third License Agreement, we obtained an exclusive, sublicensable, worldwide license under certain patents to make, use, and sell, offer for sale and import services, methods, composition and products that incorporate or are developed using any licensed invention or licensed methods, or is covered by a valid claim of a licensed patent, or a Product, and an exclusive license to use nonpublic technical information, or Technology, in all cases for all diagnostic and therapeutic uses. UCSD reserved the rights to practice the relevant invention, Technology and licensed patents for educational and research purposes, to publish and disseminate information about the relevant invention, Technology and licensed patents, and to allow other nonprofit institutions to use, publish, or disseminate information about the relevant invention, Technology and licensed patents for educational and research purposes.

Under the UCSD Third License Agreement, we are obligated to achieve certain development milestones within specified time frames, to use commercially reasonable efforts to diligently develop, manufacture, and sell licensed products, and to meet agreed minimum-spend requirements. If we fail to perform any of these obligations, UCSD could either terminate the UCSD Third License Agreement or convert the exclusive license to a nonexclusive license subject to certain rights for us to extend these time frames.

The UCSD Third License Agreement required us to pay a one-time up-front non-refundable cash fee of \$20,000. We are obligated to pay annual license maintenance fees in the mid-four digits to low-five digits, increasing until such time that we are commercially selling a Product. We are also obligated to pay up to \$4.0 million upon the achievement of specified development and commercialization milestones for the first Product and low- to mid-single digit royalties based on aggregate net sales, subject to certain reductions for third-party licenses. The royalty term continues until the expiration of the UCSD Third License Agreement. If the royalties are below certain agreed amounts, we are required to pay UCSD minimum annual royalties ranging from low- to mid-five digits. We are also obligated to pay UCSD a percentage of sublicensing fees ranging in the low double digits.

The UCSD Third License Agreement will expire, unless earlier terminated, on the expiration of the last claim of any licensed patent or patent applications in a particular country. We have the right to terminate the UCSD Third License Agreement at any time upon sixty (60)-days' written notice to the UCSD. UCSD may terminate the UCSD Third License Agreement if we commit a material breach, if we fail to meet certain specified milestones within the prescribed time periods, if we are delinquent on any report or payment, if we are not diligently developing or commercializing Products in accordance with our diligence obligations, if we provide any intentionally false report, or if we challenge the validity of a licensed patent, in each case only if we fail to cure such problem within a specified cure period after written notice. In the event that we assign the UCSD Third License Agreement or we undergo a change of control, we will be obligated to pay a flat low-six digit fee or a fee that will be determined based on the acquisition price. Upon termination of the UCSD Third License Agreement, all licenses and rights granted by The Regents of UCSD to us will terminate.

*Sponsored research agreements with The Regents of University of California, San Diego*

In connection with the UCSD License Agreements, on December 3, 2021, we entered into a sponsored research agreement with UCSD for our LX2020 and LX2021 programs, or First UCSD SRA, which was subsequently amended on April 5, 2023 and August 31, 2023, and another sponsored research agreement with UCSD for our LX2022 program, or Second UCSD SRA, which was subsequently amended on April 19, 2023. We refer to these agreements, as amended, as the Amended UCSD SRAs. UCSD reserved the rights to publish and disseminate information about the results generated from UCSD's investigator's conduct of the research. The total costs to be invoiced to us under the terms of the Amended UCSD SRAs are approximately \$5.6 million. Under the terms of the Amended UCSD SRAs, we have the first rights to obtain non-exclusive or exclusive, sublicensable, royalty-bearing, perpetual and transferable worldwide licenses to any inventions generated by UCSD or UCSD's interest in any inventions generated jointly by The Regents of UCSD and us, and we retain the rights to any inventions generated solely by us.

On April 13, 2024, we entered into a third sponsored research agreement with UCSD, or the Third UCSD SRA, for our LX2022 program in connection with the UCSD Second License Agreement. Under the terms of the Third UCSD SRA, we have the first rights to obtain non-exclusive or exclusive, sublicensable, royalty-bearing, perpetual and transferable worldwide licenses in any resulting inventions owned by UCSD or resulting inventions jointly owned between us and UCSD, and we retain the rights to any resulting inventions owned by us. The Third UCSD SRA has a two-year term and may be terminated early by us at any time upon the giving of thirty (30)-days' written notice to UCSD. The costs to be invoiced to us over the term of the Third UCSD SRA are \$0.7 million, and we may incur additional costs of \$0.6 million under the Third UCSD SRA if certain study objectives are met.

On April 13, 2024, we entered into another amendment to the Second UCSD SRA for our LX2022 program that extended the term of the Second UCSD SRA to December 2024.

On April 19, 2024 and September 27, 2024, we entered into amendments, respectively, to the First UCSD SRA for our LX2021 program in connection with the UCSD First License Agreement. We refer to the First UCSD SRA, as amended, as the Amended First UCSD SRA. The Amended First UCSD SRA extends the term of the First UCSD SRA to December 2026 and provides for additional research and development studies and expenses. The total costs to be invoiced to us under the Amended First UCSD SRA are \$0.7 million.

## **Government regulation**

Government authorities in the United States at federal, state and local levels, as well as in foreign countries, extensively regulate, among other things, the research, development, testing, manufacture, quality control, import, export, safety, effectiveness, labeling, packaging, storage, distribution, recordkeeping, approval, advertising, promotion, marketing, post-approval monitoring and post-approval reporting of biologics, including gene therapies, such as those we are developing. Generally, before a new biologic 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, authorized, or cleared by the applicable regulatory authority. We, along with third-party contractors, will be required to navigate the various preclinical, clinical and commercial approval requirements of the governing regulatory agencies of the countries in which we wish to conduct studies or seek approval or licensure of our product candidates.

### ***U.S. biologics regulation***

In the United States, biological products are subject to regulation under the U.S. Federal Food, Drug, and Cosmetic Act, or FDCA, and the Public Health Service Act, or PHSA, and their implementing regulations and other federal, state, local and foreign statutes and regulations. Failure to comply with the applicable United States requirements at any time during the product development process, approval process or following approval may subject an applicant to administrative actions or judicial sanctions. These actions and sanctions could include, among other actions, the FDA's refusal to approve pending applications, withdrawal of an approval, license revocation, a clinical hold, untitled or warning letters, voluntary or mandatory 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 fines or penalties.

Our product candidates must be approved by the FDA through the BLA the process which is required by the FDA before biological product candidates may be marketed in the United States and generally involves the following:

- completion of extensive preclinical laboratory tests and animal studies performed in accordance with applicable regulations, including the FDA's good laboratory practices, or GLPs, regulations;
- submission to the FDA of an IND application, which must become effective before human clinical trials may begin;
- approval by an Institutional Review Board, or IRB, or ethics committee at each clinical site before the trial is commenced;
- performance of adequate and well-controlled human clinical trials in accordance with applicable IND regulations, the FDA's current Good Clinical Practices, or cGCPs, and other clinical trial-related regulations to establish the safety, purity and potency of the proposed biological product candidate for its intended purpose;
- preparation of and submission to the FDA of a BLA that contains sufficient data to demonstrate substantial evidence of effectiveness;
- a determination by the FDA within 60 days of its receipt of a BLA to file the application for review;
- payment of user fees for FDA review of the BLA;
- satisfactory completion of an FDA pre-license inspection of the manufacturing facility or facilities at which the proposed product will be produced to assess compliance with current good manufacturing practices, or cGMPs, and to assure that the facilities, methods and controls are adequate to ensure and preserve the biological product's identity, strength, quality and purity, and of selected clinical investigation sites to assess compliance with the cGCPs;
- satisfactory completion of an FDA Advisory Committee review, if applicable; and
- FDA review and approval, or licensure, of a BLA to permit commercial marketing of the product for particular indications for use in the United States.

### ***Preclinical and clinical development***

Prior to beginning the first clinical trial with a product candidate, the product candidate must undergo rigorous preclinical testing. Preclinical studies include laboratory evaluation of product chemistry and formulation, as well as *in vitro* and animal studies to assess safety and in some cases to establish a rationale for therapeutic use. The conduct of preclinical studies is subject to federal and state regulations and requirements, including GLP regulations for safety/toxicology studies.

An IND sponsor must submit 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, to the FDA as part of an IND application to the FDA. An IND application is a request for authorization from the FDA to administer an IND product to humans. The central focus of an IND submission is on the general investigational plan and the protocol or protocols for preclinical studies and clinical trials. The IND application also includes results of animal and *in vitro* studies assessing the toxicology, pharmacokinetics, pharmacology and pharmacodynamic characteristics of the product, chemistry, manufacturing and controls information, and any available human data or literature to support the use of the investigational product. An IND application must become effective before human clinical trials may begin. The IND application automatically becomes effective 30 days after receipt by the FDA, unless the FDA, within the 30-day period, raises concerns or questions about the proposed clinical trial. In such a case, the IND application may be placed on clinical hold and the IND sponsor and the FDA must resolve any outstanding concerns or questions before the clinical trial can begin. Submission of an IND application therefore may or may not result in FDA authorization to begin a clinical trial. Additionally, the review of information in an IND submission may prompt FDA to, among other things, scrutinize existing INDs and could generate requests for information or clinical holds on other product candidates or programs.

Clinical trials involve the administration of the investigational product to human subjects under the supervision of qualified investigators, generally physicians not employed by or under the sponsor's control, in accordance with cGCPs, which include the requirement that all research subjects provide their informed consent for their participation in any clinical study. Clinical trials are conducted under protocols detailing, among other things, the objectives of the trial, dosing procedures, subject selection and exclusion criteria and the parameters to be used in monitoring safety and the effectiveness criteria to be evaluated. A separate submission to the existing IND application must be made for each successive clinical protocol conducted during product development and for any subsequent protocol amendments. Furthermore, an independent IRB for each site proposing to conduct the clinical trial must review and approve the plan for any clinical trial and its informed consent form before the clinical trial begins at that site, and must monitor the study until completed to ensure that the risks to individuals participating in the clinical trial are minimized and are reasonable in relation to anticipated benefits.

A sponsor who wishes to conduct a clinical trial outside of the United States may, but need not, obtain FDA authorization to conduct the clinical trial under an IND application. If a foreign clinical trial is not conducted under an IND application, the sponsor may submit data from the clinical trial to the FDA in support of a BLA. The FDA will accept a well-designed and well-conducted foreign clinical trial not conducted under an IND application if the foreign data are applicable to the United States population and medical practice, the trial was performed by clinical investigators of recognized competence, the trial was conducted in accordance with cGCP requirements, and the data may be considered valid without the need for an on-site inspection by the FDA or the FDA is able to validate the data through an onsite inspection if deemed necessary.

For purposes of BLA approval of a product candidate, human clinical trials are typically conducted in three sequential phases that may overlap or be combined:

- *Phase 1.* For gene therapies in general, the investigational product is initially introduced into patients with the target disease or condition. These studies are designed to test the safety, dosage tolerance, absorption, metabolism and distribution of the investigational product in humans, the side effects associated with increasing doses, and, if possible, to gain early evidence on effectiveness.
- *Phase 2.* The investigational product is administered to a limited patient population to evaluate the preliminary efficacy, optimal dosages and dosing schedule and to identify possible adverse side effects and safety risks.
- *Phase 3.* The investigational product is administered to an expanded patient population to further evaluate dosage, to provide statistically significant evidence of clinical efficacy and to further test for safety, generally at multiple geographically dispersed clinical trial sites. These clinical trials are intended to establish the overall risk/benefit ratio of the investigational product and to provide an adequate basis for product approval.

When these phases overlap or are combined, the trials may be referred to as Phase 1/2 or Phase 2/3.

In some cases, the FDA may require, or companies may voluntarily pursue, additional clinical trials after a product is approved to gain more information about the product. These so-called Phase 4 studies may be made a condition to approval of the BLA. Concurrent with clinical trials, companies may complete additional animal studies and develop additional information about the characteristics of the product candidate, and must 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 product candidate and, among other things, must develop methods for testing the identity, strength, potency, quality and purity of the final product. Additionally, appropriate packaging must be selected and tested and stability studies must be conducted to demonstrate that the product candidate does not undergo unacceptable deterioration over its shelf life.

During all phases of clinical development, regulatory agencies require extensive monitoring and auditing of all clinical activities, clinical data and clinical study investigators. Written IND safety reports must be promptly submitted to the FDA and the investigators for serious, unexpected and suspected adverse events, any findings from other studies, tests in laboratory animals or in vitro testing that suggest a significant risk for human subjects, or any clinically important increase in the rate of a serious, and unexpected suspected adverse reaction over that listed in the protocol or investigator brochure. The sponsor must submit an IND safety report within 15 calendar days after the sponsor determines that the information qualifies for reporting. The sponsor also must notify the FDA of any unexpected fatal or life-threatening suspected adverse reaction within seven calendar days after the sponsor's initial receipt of the information. The FDA or the sponsor may suspend a clinical study at any time on various grounds, including a finding that the research patients or patients are being exposed to an unacceptable health risk. Similarly, an IRB can suspend or terminate approval of a clinical study at its institution if the clinical study is not being conducted in accordance with the IRB's requirements or if the biological product candidate has been associated with unexpected serious harm to patients. Some studies also include oversight by an independent group of qualified experts organized by the clinical study sponsor, known as a data safety monitoring board, which provides authorization for whether or not a study may move forward at designated checkpoints based on access to certain data from the study and may recommend halting the clinical trial if it determines that there is an unacceptable safety risk for subjects or other grounds, such as no demonstration of efficacy. There are also requirements governing the reporting of ongoing clinical trials and completed clinical trial results to public registries. Sponsors of clinical trials of FDA-regulated products, including biologics, are required to register and disclose certain clinical trial information, which is publicly available at [www.clinicaltrials.gov](http://www.clinicaltrials.gov).

### ***BLA submission and review***

Assuming successful completion of all required testing in accordance with all applicable regulatory requirements, the results of product development, preclinical studies and clinical trials are submitted to the FDA as part of a BLA requesting approval to market the product for one or more indications. FDA approval of a BLA must be obtained before a biologic may be marketed in the United States. The BLA must include all relevant data available from pertinent preclinical studies and clinical trials, including negative or ambiguous results as well as positive findings, together with detailed information relating to the product's chemistry, manufacturing, controls and proposed labeling, among other things. The submission of a BLA requires payment of a substantial application user fee to the FDA, unless a waiver or exemption applies. No user fees are assessed on BLAs for products designated as orphan drugs, unless the product also includes a non-orphan indication. The FDA reviews all submitted BLAs before it accepts them for filing, and may request additional information rather than accepting the BLA for filing. The FDA has 60 days from the applicant's submission of a BLA to either issue a refusal to file letter or accept the BLA for filing, indicating that it is sufficiently complete to permit substantive review.

Once a BLA has been accepted for filing, the FDA's goal is to review standard applications within ten months after it accepts the application for filing, or, if the application qualifies for priority review, six months after the FDA accepts the application for filing. In both standard and priority reviews, the review process can be significantly extended by FDA requests for additional information or clarification. The FDA reviews a BLA to determine, among other things, whether a product is safe, pure and potent for its intended use, and whether the facility in which it is manufactured, processed, packed or held meets standards designed to ensure and preserve the product's identity, safety, strength, quality, potency and purity. The FDA may convene an advisory committee, typically a panel that includes clinicians and other experts, to provide clinical insight on applications for novel products or products which present difficult questions of safety or efficacy. The advisory committee will provide a recommendation as to whether the application should be approved and under what conditions, if any. The FDA is not bound by the recommendations of an advisory committee, but it considers such recommendations when making decisions on approval. Before approving a BLA, the FDA will typically inspect the facility or facilities where the product is manufactured. 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 a BLA, the FDA will typically inspect one or more clinical sites to assure compliance with cGCPs. If the FDA determines that the application, manufacturing process or manufacturing facilities are not acceptable, it will outline the deficiencies in the submission and often will request additional testing or information. Notwithstanding the submission of any requested additional information, the FDA ultimately may decide that the application does not satisfy the regulatory criteria for approval.

After the FDA evaluates a BLA and conducts inspections of manufacturing facilities where the investigational product and/or its drug substance will be manufactured, 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 indicates that the review cycle of the application is complete, and the application will not be approved in its present form. A Complete Response Letter will usually describe all of the deficiencies that the FDA has identified in the BLA, except that where the FDA determines that the data supporting the application are inadequate to support approval, the FDA may issue the Complete Response Letter without first conducting required inspections, testing submitted product lots and/or reviewing proposed labeling. In issuing the Complete Response Letter, the FDA may recommend actions that the applicant might take to place the BLA in condition for approval, including requests for additional information or clarification, which may include the potential requirement for additional preclinical studies or clinical trials or additional manufacturing activities. If a Complete Response Letter is issued, the applicant may either resubmit the BLA, addressing all of the deficiencies identified in the letter, or withdraw the application or request an opportunity for a hearing. The FDA may delay or refuse approval of a BLA if applicable regulatory criteria are not satisfied, require additional testing or information and/or require post-marketing testing and surveillance to monitor the safety or efficacy of a product. Further, FDA's "real time" release of newly issued Complete Response Letters associated with withdrawn or abandoned applications, if applicable to any of our product candidates, can materially impact our business and competitive advantage.

If regulatory approval of a product is granted, such approval will be granted for particular indications and may entail limitations on the indicated uses for which such product may be marketed. For example, the FDA may approve the BLA with a Risk Evaluation and Mitigation Strategy, or REMS, to ensure the benefits of the product outweigh its risks. A REMS is a safety strategy to manage a known or potential serious risk associated with a product and to enable patients to have continued access to such medicines by managing their safe use, and could include medication guides, physician communication plans, or elements to assure safe use, such as restricted distribution methods, patient registries and other risk minimization tools. The FDA also may condition approval on, among other things, changes to proposed labeling or the development of adequate controls and specifications. Once approved, the FDA may withdraw the product approval if compliance with pre- and post-marketing requirements is not maintained or if problems occur after the product reaches the marketplace. The FDA may require one or more Phase 4 post-market studies and surveillance to further assess and monitor the product's safety and effectiveness after commercialization, and may limit further marketing of the product based on the results of these post-marketing studies.

#### ***Expedited development and review programs***

The FDA offers a number of expedited development and reviews programs for qualifying product candidates. The Fast Track program is intended to expedite or facilitate the process for developing new products that meet certain criteria. Specifically, product candidates are eligible for Fast Track designation if they are intended to treat a serious or life-threatening disease or condition and demonstrate the potential to address unmet medical needs for the disease or condition. Fast Track designation applies to the combination of the product candidate and the specific indication for which it is being studied. The sponsor of a Fast Track designated product candidate has opportunities for frequent interactions with the FDA review team during product development and, once a BLA is submitted, the product candidate may be eligible for priority review. A Fast Track designated product candidate may also be eligible for rolling review, where the FDA may consider for review sections of the BLA on a rolling basis before the complete application is submitted, if the sponsor provides a schedule for the submission of the sections of the BLA, the FDA agrees to accept sections of the BLA and determines that the schedule is acceptable, and the sponsor pays any required user fees upon submission of the first section of the BLA. The sponsor can request the FDA to designate the product candidate for Fast Track status any time before receiving BLA approval, but ideally no later than the pre-BLA meeting.

A product candidate intended to treat a serious or life-threatening disease or condition may also be eligible for Breakthrough Therapy designation to expedite its development and review. A product candidate may receive Breakthrough Therapy designation if preliminary clinical evidence indicates that the product candidate, alone or in combination with one or more other drugs or biologics, may demonstrate substantial improvement over available therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development. The designation includes all of the Fast Track program features, as well as more intensive FDA interaction and guidance beginning as early as Phase 1 and an organizational commitment to expedite the development and review of the product candidate, including involvement of senior managers.

As part of the 21st Century Cures Act, Congress amended the FDCA to facilitate an efficient development program for, and expedite review of regenerative medicine therapies, which include cell therapies, therapeutic tissue engineering products, human cell and tissue products, and combination products using any such therapies or products. Gene therapies, including genetically modified cells that lead to a durable modification of cells or tissues may meet the definition of a “regenerative medicine therapy.” A product candidate may be eligible for RMAT designation if it meets the following criteria: (1) it is a regenerative medicine therapy; (2) it is intended to treat, modify, reverse, or cure a serious or life-threatening disease or condition; and (3) preliminary clinical evidence indicates that it has the potential to address unmet medical needs for such a disease or condition. A sponsor may request that the FDA designate a product candidate as an RMAT concurrently with or at any time after the submission of an IND. A BLA for a product candidate that has received RMAT designation may be eligible for priority review or accelerated approval through (1) surrogate or intermediate endpoints reasonably likely to predict long-term clinical benefit or (2) reliance upon data obtained from a meaningful number of sites. Benefits of such designation also include early interactions with FDA to discuss any potential surrogate or intermediate endpoint to be used to support accelerated approval. A product candidate with RMAT designation that is granted accelerated approval and is subject to post-approval requirements may fulfill such requirements through the submission of clinical evidence, clinical studies, patient registries, or other sources of real world evidence, such as electronic health records; the collection of larger confirmatory data sets; or post-approval monitoring of all patients treated with such therapy prior to its approval.

Any marketing application for a biologic submitted to the FDA for approval, including a product with a Fast Track designation and/or Breakthrough Therapy designation, may be eligible for other types of FDA programs intended to expedite the FDA review and approval process, such as priority review. A product is eligible for priority review if it is designed to treat a serious or life threatening disease or condition and, if approved, would provide a significant improvement in safety and effectiveness compared to available therapies. For original BLAs, priority review designation means the FDA’s goal is to take action on the marketing application within six months of the 60-day filing date.

Additionally, a product candidate may be eligible for accelerated approval if it is designed to treat a serious or life-threatening disease or condition and demonstrates an effect on a surrogate endpoint that is reasonably likely to predict clinical benefit, or on a clinical endpoint that can be measured earlier than irreversible morbidity or mortality, or IMM, that is reasonably likely to predict an effect on IMM or other clinical benefit, taking into account the severity, rarity, or prevalence of the condition and the availability or lack of alternative treatments. As a condition of accelerated approval, the FDA will generally require the sponsor to perform adequate and well-controlled post-marketing clinical studies with due diligence to verify and describe the anticipated effect on IMM or other clinical benefit. The FDA may withdraw approval of a product or indication approved under accelerated approval if, for example, the confirmatory trial fails to verify the predicted clinical benefit of the product. In addition, for products being considered for accelerated approval, the FDA generally requires, unless otherwise informed by the agency, pre-approval of all advertising and promotional materials intended for dissemination or publication, which could adversely impact the timing of the commercial launch of the product. The Food and Drug Omnibus Reform Act also made several changes to the FDA’s authorities and its regulatory framework, including, among other changes, reforms to the accelerated approval pathway, such as requiring the FDA to specify conditions for post-approval study requirements and setting forth procedures for the FDA to withdraw a product on an expedited basis for non-compliance with post-approval requirements.

Fast Track designation, Breakthrough Therapy designation, RMAT designation, and priority review do not change the standards for approval but may expedite the development or approval process. Even if a product qualifies for one or more of these programs, the FDA may later decide that the product no longer meets the conditions for qualification or decide that the time period for FDA review or approval will not be shortened.

#### ***Orphan Drug designation***

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

If a product that has Orphan Drug designation subsequently receives the first FDA approval for the disease or condition for which it has such designation, the product is entitled to orphan drug exclusive approval (or exclusivity), which means that the FDA may not approve any other applications, including a full BLA, to market the same product for the same indication for seven years, except in limited circumstances, such as a showing of clinical superiority to the product with orphan drug exclusivity by means of greater effectiveness, greater safety or providing a major contribution to patient care or if the holder of the orphan drug exclusivity cannot assure the availability of sufficient quantities of the orphan drug to meet the needs of patients with the disease or condition for which the product was designated. Orphan drug exclusivity does not prevent the FDA from approving a different drug or biologic for the same disease or condition, or the same drug or biologic for a different disease or condition. Among the other benefits of Orphan Drug designation are tax credits for certain research and a waiver of the BLA application fee.

A designated orphan drug may not receive orphan drug exclusivity if it is approved for a use that is broader than the indication for which it received Orphan Drug designation. In addition, exclusive marketing rights in the United States may be lost if the FDA later determines that the request for designation was materially defective or if the manufacturer is unable to assure sufficient quantities of the product to meet the needs of patients with the rare disease or condition. In view of the court decision in *Catalyst Pharms., Inc. v. Becerra*, 14 F.4th 1299 (11th Cir. 2021), in January 2023, the FDA published a notice in the Federal Register to clarify that while the agency complies with the court's order in *Catalyst*, FDA intends to continue to apply its longstanding interpretation of the regulations to matters outside of the scope of the *Catalyst* order – that is, the agency will continue tying the scope of orphan-drug exclusivity to the uses or indications for which a drug is approved, which permits other sponsors to obtain approval of a drug for new uses or indications within the same orphan designated disease or condition that have not yet been approved. The Consolidated Appropriations Act of 2026, signed into law in February 2026, codified this longstanding FDA interpretation of the Orphan Drug Act.

In June 2024, the U.S. Supreme Court overruled the *Chevron* doctrine, which gives deference to regulatory agencies' statutory interpretations in litigation against federal government agencies, such as the FDA, where the law is ambiguous. This landmark Supreme Court decision may invite various stakeholders to bring lawsuits against the FDA to challenge longstanding decisions and policies, which could lead to uncertainties in the industry. Further, changes in the leadership of the FDA and other federal agencies under the Trump administration may lead to new policies, changes in the regulations, or disruptions to the operations of federal agencies, any of which may impact our clinical development plans.

#### ***Rare Pediatric Disease designation and priority review vouchers***

Under the FDCA, as amended, the FDA incentivizes the development of product candidates that meet the definition of a “rare pediatric disease,” defined to mean a serious or life-threatening disease in which the serious or life-threatening manifestations primarily affect individuals aged from birth to 18 years and the disease affects fewer than 200,000 individuals in the United States or affects 200,000 or more in the United States and for which there is no reasonable expectation that the cost of developing and making in the United States a drug for such disease or condition will be received from sales in the United States of such drug. The sponsor of a product candidate for a rare pediatric disease may be eligible for a voucher that can be used to obtain a priority review for a subsequent human drug or biologic application after the date of approval of the rare pediatric disease drug product. A sponsor may request Rare Pediatric Disease designation from the FDA prior to the submission of its BLA. A Rare Pediatric Disease designation does not guarantee that a sponsor will receive a priority review voucher, or PRV, upon approval of its BLA. Moreover, a sponsor who chooses not to submit a Rare Pediatric Disease designation request may nonetheless receive a PRV upon approval of their marketing application if they request such a voucher in their original marketing application and meet all of the eligibility criteria. If a PRV is received, it may be sold or transferred an unlimited number of times. The Consolidated Appropriations Act of 2026 extended the rare pediatric disease PRV program to September 30, 2029, which means the FDA may not award any PRVs under this program after September 30, 2029, unless Congress takes further action to extend the program.

### ***Pediatric information and pediatric exclusivity***

Under the Pediatric Research Equity Act, or PREA, certain BLAs and certain supplements to a BLA must contain data to assess the safety and efficacy of the product for the claimed indications in all relevant pediatric subpopulations and to support dosing and administration for each pediatric subpopulation for which the product is safe and effective. The FDA may grant deferrals for the submission of pediatric data or full or partial waivers. The Food and Drug Administration Safety and Innovation Act, or FDASIA, amended the FDCA to require that a sponsor who is planning to submit a marketing application for a product that includes a new active ingredient, new indication, new dosage form, new dosing regimen or new route of administration submit an initial Pediatric Study Plan, or PSP, within 60 days of an end-of-Phase 2 meeting or, if there is no such meeting, as early as practicable before the initiation of the Phase 3 or Phase 2/3 study. The initial PSP must include an outline of the pediatric study or studies that the sponsor plans to conduct, including study objectives and design, age groups, relevant endpoints and statistical approach, or a justification for not including such detailed information, and any request for a deferral of pediatric assessments or a full or partial waiver of the requirement to provide data from pediatric studies along with supporting information. The FDA and the sponsor must reach an agreement on the PSP. A sponsor can submit amendments to an agreed-upon initial PSP at any time if changes to the pediatric plan need to be considered based on data collected from preclinical studies, early phase clinical trials and/or other clinical development programs. A biological product can also obtain pediatric market exclusivity in the United States. Pediatric exclusivity, if granted, adds six months to existing exclusivity periods. This six-month exclusivity, which runs from the end of other exclusivity protection, may be granted based on the voluntary completion of a pediatric study in accordance with an FDA-issued "Written Request" for such a study.

### ***Post-approval requirements***

Any products manufactured or distributed by us pursuant to FDA approvals are subject to pervasive and continuing regulation by the FDA, including, among other things, requirements relating to record-keeping, reporting of adverse experiences, periodic reporting, product sampling and distribution, and advertising and promotion of the product. As part of the manufacturing process, the manufacturer is required to perform certain tests on each lot of the product before it is released for distribution. After a BLA is approved for a biological product, the product also may be subject to official lot release. If the product is subject to official release by the FDA, the manufacturer submits samples of each lot of product to the FDA together with a release protocol showing a summary of the history of manufacture of the lot and the results of all of the manufacturer's tests performed on the lot. The FDA also may perform certain confirmatory tests on lots of some products before releasing the lots for distribution by the manufacturer. In addition, the FDA conducts laboratory research related to the regulatory standards on the safety, purity, potency and effectiveness of biologics. 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 user fee requirements, under which the FDA assesses an annual program fee for each product identified in an approved BLA. Biologic manufacturers and their subcontractors are required to register their establishments with the FDA and certain state agencies, and are subject to periodic unannounced inspections by the FDA and certain state agencies for compliance with cGMPs, which impose certain procedural and documentation requirements upon us and our third-party manufacturers. Changes to the manufacturing process are strictly regulated, and, depending on the significance of the change, may require prior FDA approval before being implemented. FDA regulations also require investigation and correction of any deviations from cGMPs and impose reporting requirements upon us and any third-party manufacturers that we may decide to use. Manufacturers and other parties involved in the drug supply chain for prescription drug products must also comply with product tracking and tracing requirements and for notifying the FDA of counterfeit, diverted, stolen and intentionally adulterated products or products that are otherwise unfit for distribution in the United States. Accordingly, manufacturers must continue to expend time, money and effort in the area of production and quality control to maintain compliance with cGMPs and other aspects of regulatory compliance.

The FDA may withdraw 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 adverse events 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 studies to assess new safety risks; or imposition of distribution restrictions or other restrictions under a REMS program. Other potential consequences include, among other things:

- restrictions on the marketing or manufacturing of a product, complete withdrawal of the product from the market or product recalls;
- fines, warning or untitled letters or holds on post-approval clinical studies;
- refusal of the FDA to approve pending applications or supplements to approved applications, or suspension or revocation of existing product approvals;
- product seizure or detention, or refusal of the FDA to permit the import or export of products;

- consent decrees, corporate integrity agreements, debarment or exclusion from federal healthcare programs;
- mandated modification of promotional materials and labeling and the issuance of corrective information;
- the issuance of safety alerts, Dear Healthcare Provider letters, press releases and other communications containing warnings or other safety information about the product; or
- injunctions or the imposition of civil or criminal penalties.

The FDA closely regulates the marketing, labeling, advertising and promotion of biologics. A company can make only those claims relating to safety and efficacy, purity and potency that are approved by the FDA and in accordance with the provisions of the approved label. However, companies may share truthful and not misleading information that is otherwise consistent with a product's FDA-approved labeling. The FDA and other agencies actively enforce the laws and regulations prohibiting the promotion of off-label uses. Failure to comply with these requirements can result in, among other things, adverse publicity, warning letters, corrective advertising and potential civil and criminal penalties. Physicians may prescribe legally available products for uses that are not described in the product's labeling and that differ from those tested by us and approved by the FDA. Such off-label uses are common across medical specialties. Physicians may believe that such off-label uses are the best treatment for many patients in varied circumstances. The FDA does not regulate the behavior of physicians in their choice of treatments. The FDA does, however, restrict manufacturer's communications on the subject of off-label use of their products.

### ***Biosimilars and reference product exclusivity***

The Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act, or collectively, the ACA, includes a subtitle called the Biologics Price Competition and Innovation Act, or BPCIA, which created an abbreviated approval pathway for biologics that are biosimilar to or interchangeable with an FDA-approved reference biological product. To date, a number of biosimilars have been licensed under the BPCIA, and numerous biosimilars have been approved in Europe. The FDA has issued several guidance documents outlining an approach to review and approval of biosimilars.

Biosimilarity, which requires that there be no clinically meaningful differences between the biological product and the reference product in terms of safety, purity and potency, can be shown through analytical studies, animal studies and a clinical study or studies. Interchangeability requires that a product is biosimilar to the reference product and the product must demonstrate that it can be expected to produce the same clinical results as the reference product in any given patient and, for products that are administered multiple times to an individual, the biologic and the reference biologic may be alternated or switched after one has been previously administered without increasing safety risks or risks of diminished efficacy relative to exclusive use of the reference biologic. Complexities associated with the larger, and often more complex, structures of biologics, as well as the processes by which such products are manufactured, pose significant hurdles to the implementation of the abbreviated approval pathway that are still being worked out by the FDA.

Under the BPCIA, an application for a biosimilar product may not be submitted to the FDA until four years following the date that the reference product was first licensed by the FDA. In addition, the approval of a biosimilar product may not be made effective by the FDA until 12 years from the date on which the reference product was first licensed. During this 12-year period of exclusivity, another company may still market a competing version of the reference product if the FDA approves a full BLA for the competing product containing that applicant's own preclinical data and data from adequate and well-controlled clinical trials to demonstrate the safety, purity and potency of its product. The BPCIA also created certain exclusivity periods for biosimilars approved as interchangeable products. At this juncture, it is unclear whether products deemed "interchangeable" by the FDA will, in fact, be readily substituted by pharmacies, which are governed by state pharmacy law.

The BPCIA is complex and continues to be interpreted and implemented by the FDA. In addition, government proposals have sought to reduce the 12-year reference product exclusivity period. Other aspects of the BPCIA, some of which may impact the BPCIA exclusivity provisions, have also been the subject of recent litigation. As a result, the ultimate impact and implementation of the BPCIA are subject to significant uncertainty.

### ***Regulation of companion diagnostics***

If an *in vitro* diagnostic, which is regulated by the FDA as a medical device, is essential to the safe and effective use of a therapeutic and is not available on the market, then the FDA generally will require approval or clearance of that diagnostic, known as a companion diagnostic, at the same time that the FDA approves the therapeutic product. In August 2014, the FDA issued final guidance clarifying the requirements that will apply to approval of therapeutic products and *in vitro* companion diagnostics. According to the guidance, an unapproved or uncleared companion diagnostic device used to make treatment decisions in clinical trials of a drug generally will be considered an investigational medical device unless it is employed for an intended use for which the device is already approved or cleared. If used to make critical treatment decisions, such as patient selection, the diagnostic device generally will be considered a significant risk device under the FDA's Investigational Device Exemption, or IDE, regulations. The sponsor of the diagnostic device will be required to comply with the IDE regulations for clinical studies involving the investigational diagnostic device. According to the guidance, if a diagnostic device and a drug are to be studied together to support their respective approvals, both products can be studied in the same clinical trial, if the trial meets both the requirements of the IDE regulations and the IND regulations. The guidance provides that depending on the details of the clinical trial protocol, the investigational product(s), and subjects involved, a sponsor may seek to submit an IDE alone (e.g., if the drug has already been approved by FDA and is used consistent with its approved labeling), or both an IND and an IDE.

Pursuing FDA approval/clearance of an *in vitro* companion diagnostic would require either a pre-market notification, also called 510(k) clearance, or a pre-market approval, or PMA, or a *de novo* classification for that diagnostic. The review of companion diagnostics involves coordination of review with the FDA's Center for Devices and Radiological Health. Once cleared or approved, the companion diagnostic must adhere to post-marketing requirements including the requirements of FDA's quality system regulation, medical device reporting, recalls and corrections along with product marketing requirements and limitations. Companion diagnostic manufacturers are subject to unannounced FDA inspections at any time during which the FDA will conduct an audit of the product(s) and the company's facilities for compliance with its authorities.

#### ***510(k) clearance process***

To obtain 510(k) clearance, a pre-market notification is submitted to the FDA demonstrating that the proposed device is substantially equivalent to a previously cleared 510(k) device or a device that was in commercial distribution before May 28, 1976 for which the FDA has not yet required the submission of a PMA application. The FDA's 510(k) clearance process may take three to 12 months from the date the application is submitted and filed with the FDA, but may take longer if FDA requests additional information, among other reasons. In some cases, the FDA may require clinical data to support substantial equivalence. In reviewing a pre-market notification submission, the FDA may request additional information, which may significantly prolong the review process. Notwithstanding compliance with all these requirements, clearance is never assured. After a device receives 510(k) clearance, any subsequent modification of the device that could significantly affect its safety or effectiveness, or that would constitute a major change in its intended use, will require a new 510(k) clearance or require a PMA. In addition, the FDA may make substantial changes to industry requirements, including which devices are eligible for 510(k) clearance, which may significantly affect the process.

#### ***De novo classification process***

If a new medical device does not qualify for the 510(k) pre-market notification process because no predicate device to which it is substantially equivalent can be identified, the device is automatically classified into Class III. The Food and Drug Administration Modernization Act of 1997 established a different route to market for low to moderate risk medical devices that are automatically placed into Class III due to the absence of a predicate device, called the "Request for Evaluation of Automatic Class III Designation," or the *de novo* classification process. This process allows a manufacturer whose novel device is automatically classified into Class III to request down-classification of its medical device into Class I or Class II on the basis that the device presents low or moderate risk, rather than requiring the submission and approval of a PMA. If the manufacturer seeks reclassification into Class II, the manufacturer must include a draft proposal for special controls that are necessary to provide a reasonable assurance of the safety and effectiveness of the medical device. The FDA may reject the reclassification petition if it identifies a legally marketed predicate device that would be appropriate for a 510(k) or determines that the device is not low to moderate risk and requires PMA or that general controls would be inadequate to control the risks and special controls cannot be developed. Obtaining FDA marketing authorization, *de novo* down-classification, or approval for medical devices is expensive and uncertain, and may take several years, and generally requires significant scientific and clinical data.

### ***PMA process***

The PMA process, including the gathering of clinical and nonclinical data and the submission to and review by the FDA, can take several years or longer. The applicant must prepare and provide the FDA with reasonable assurance of the device's safety and effectiveness, including information about the device and its components regarding, among other things, device design, manufacturing, and labeling. PMA applications are subject to an application fee. In addition, PMAs for medical devices must generally include the results from extensive preclinical and adequate and well-controlled clinical trials to establish the safety and effectiveness of the device for each indication for which FDA approval is sought. In particular, for a diagnostic, the applicant must demonstrate that the diagnostic produces reproducible results. As part of the PMA review, the FDA will typically inspect the manufacturer's facilities for compliance with the Quality Management System Regulation, which imposes extensive testing, control, documentation, and other quality assurance and GMP requirements.

### ***Foreign regulation***

In order to market any product outside of the United States, we would need to comply with numerous and varying regulatory requirements of other countries and jurisdictions regarding quality, safety, and efficacy and governing, among other things, clinical trials, marketing authorization, commercial sales and distribution of our products. Whether or not we obtain FDA approval for a product, we would need to obtain the necessary approvals by the comparable foreign regulatory authorities before we can commence clinical trials or marketing of the product in foreign countries and jurisdictions. Although many of the issues discussed above with respect to the United States apply similarly in the context of the European Union, the approval process varies between countries and jurisdictions and can involve additional product testing and additional administrative review periods. The time required to obtain approval in other countries and jurisdictions might differ from and be longer than that required to obtain FDA approval. Regulatory approval in one country or jurisdiction does not ensure regulatory approval in another, but a failure or delay in obtaining regulatory approval in one country or jurisdiction may negatively impact the regulatory process in others.

### ***Other healthcare laws and compliance requirements***

Pharmaceutical companies are subject to additional healthcare regulation and enforcement by the federal government and by authorities in the states and foreign jurisdictions in which they conduct their business, particularly once they have a commercialized product that is reimbursable by third-party payor programs. Such laws include, without limitation: the U.S. federal Anti-Kickback Statute, the civil False Claims Act, the federal Health Insurance Portability and Accountability Act of 1996, or HIPAA, and similar foreign, federal and state fraud and abuse, transparency and privacy laws.

- The U.S. federal Anti-Kickback Statute prohibits, among other things, persons and entities from knowingly and willfully soliciting, receiving, offering or paying remuneration, to induce, or in return for, either the referral of an individual, or the purchase or recommendation of an item or service for which payment may be made under any federal healthcare program. The term remuneration has been interpreted broadly to include anything of value, including stock options. There are a number of statutory exceptions and regulatory safe harbors protecting some common activities from prosecution, but they are drawn narrowly, and practices that involve remuneration, such as consulting agreements, that may be alleged to be intended to induce prescribing, purchasing or recommending may be subject to scrutiny if they do not qualify for an exception or safe harbor. Our practices may not in all cases meet all of the criteria for protection under a statutory exception or regulatory safe harbor. 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. Violations are subject to civil and criminal fines and penalties for each violation, plus up to three times the remuneration involved, imprisonment, and exclusion from government healthcare programs. In addition, a claim including items or services resulting from a violation of the U.S. federal Anti-Kickback Statute constitutes a false or fraudulent claim for purposes of the civil False Claims Act.
- Civil and criminal false claims laws, and civil monetary penalty laws, including the civil False Claims Act, which can be enforced through civil whistleblower or qui tam actions, prohibit, among other things, individuals or entities from knowingly presenting, or causing to be presented, claims for payment to the federal government, including federal healthcare programs, that are false or fraudulent. For example, the civil False Claims Act prohibits any person or entity from knowingly presenting, or causing to be presented, a false claim for payment to the federal government or knowingly making, using or causing to be made or used a false record or statement material to a false or fraudulent claim to the federal government.

- HIPAA created additional federal civil and criminal liability for, among other things, knowingly and willfully executing 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. Similar to the U.S. federal Anti-Kickback Statute, a person or entity can be found guilty of violating HIPAA without actual knowledge of the statute or specific intent to violate it.
- HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act of 2009, or HITECH, and their respective implementing regulations, impose certain requirements on HIPAA covered entities, which include certain healthcare providers, healthcare clearing houses and health plans, and individuals and entities that provide services on their behalf that involve individually identifiable health information, known as business associates, relating to the privacy, security and transmission of individually identifiable health information, as well as their covered subcontractors. HITECH also created new tiers of civil monetary penalties, amended HIPAA to make civil and criminal penalties directly applicable to business associates, and gave state attorneys general new authority to file civil actions for damages or injunctions in federal courts to enforce the federal HIPAA laws and seek attorneys' fees and costs associated with pursuing federal civil actions.
- The U.S. federal Physician Payments Sunshine Act requires certain manufacturers of drugs, devices, biologics and medical supplies for which payment is available under Medicare, Medicaid or the Children's Health Insurance Program, with specific exceptions, to annually report to the Centers for Medicare and Medicaid Services, or CMS, information related to payments and other transfers of value made to physicians (defined to include doctors, dentists, optometrists, podiatrists and chiropractors), certain other licensed 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.

We may also be subject to additional similar U.S. state and foreign law equivalents of each of the above federal laws, such as anti-kickback and false claims laws which may apply to sales or marketing arrangements and claims involving healthcare items or services reimbursed by non-governmental third-party payors, including private insurers, or that apply regardless of payor, state laws which require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government, state and local laws which require pharmaceutical companies to report information related to payments and other transfers of value to physicians and other healthcare providers or marketing expenditures, state laws which require the reporting of information related to product pricing, state and local laws requiring the registration of pharmaceutical sales representatives, and state and foreign laws governing the privacy and security of health information which, in some cases, differ from each other in significant ways, and may not have the same effect, thus complicating compliance efforts. If our operations are found to be in violation of any of such laws or any other governmental regulations that apply, we may be subject to penalties, including, without limitation, significant civil, criminal and administrative penalties, damages, fines, exclusion from government-funded healthcare programs, such as Medicare and Medicaid or similar programs in other countries or jurisdictions, integrity oversight and reporting obligations to resolve allegations of non-compliance, disgorgement, imprisonment, contractual damages, reputational harm, diminished profits and the curtailment or restructuring of our operations.

#### ***Coverage and reimbursement***

Significant uncertainty exists as to the coverage and reimbursement status of any pharmaceutical or biological product for which we obtain regulatory approval in the future. Sales of any product, if approved, depend, in part, on the extent to which such product will be covered by third-party payors, such as federal, state and foreign government healthcare programs, commercial insurance and managed healthcare organizations, and the level of reimbursement, if any, for such product by third-party payors. Decisions regarding whether to cover any of our product candidates, if approved, the extent of coverage and amount of reimbursement to be provided are made on a plan-by-plan basis. Further, no uniform policy for coverage and reimbursement exists in the United States, and coverage and reimbursement can differ significantly from payor to payor.

Third-party payors often rely upon Medicare coverage policy and payment limitations in setting their own reimbursement rates, but also have their own methods and approval process apart from Medicare determinations. As a result, the coverage determination process is often a time-consuming and costly process that will require us to provide scientific and clinical support for the use of our product candidates to each payor separately, with no assurance that coverage and adequate reimbursement will be applied consistently or obtained in the first instance.

Furthermore, it is possible that one or more of our product candidates may not be considered medically necessary or cost effective. A decision by a third-party payor not to cover any product candidates we may develop could reduce physician utilization of such product candidates once approved and have a material adverse effect on our sales, results of operations and financial condition.

For products administered under the supervision of a physician, obtaining coverage and adequate reimbursement may be particularly difficult because of the higher prices often associated with such drugs. Additionally, separate reimbursement for the product itself or the treatment or procedure in which the product is used may not be available, which may impact physician utilization.

In addition, the U.S. government, state legislatures and foreign governments have continued implementing cost-containment programs, including price controls, restrictions on coverage and reimbursement and requirements for substitution of generic products. Third-party payors are increasingly challenging the prices charged for medical products and services, examining the medical necessity and reviewing the cost-effectiveness of pharmaceutical or biologics, medical devices and medical services, in addition to questioning safety and efficacy. Adoption of price controls and cost-containment measures, and adoption of more restrictive policies in jurisdictions with existing controls and measures, could further limit sales of any product that receives approval. Decreases in third-party reimbursement for any product or a decision by a third party not to cover a product could reduce physician usage and patient demand for the product. In addition, companion diagnostic tests require coverage and reimbursement separate and apart from the coverage and reimbursement for their companion pharmaceutical or biological products. Similar challenges to obtaining coverage and reimbursement, applicable to pharmaceutical or biological products, will apply to companion diagnostics.

Outside the United States, ensuring adequate coverage and payment for any biological candidates we may develop 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 regulatory marketing approval for a product and may require us to conduct a clinical trial that compares the cost effectiveness of any product candidates we may develop to other available therapies. The conduct of such a clinical trial could be expensive and result in delays in our commercialization efforts. In the European Union, pricing and reimbursement schemes vary widely from country to country. Some countries provide that products may be marketed only after a reimbursement price has been agreed. Some countries may require the completion of additional studies that compare the cost-effectiveness of a particular product candidate to currently available therapies (so called health technology assessments) in order to obtain reimbursement or pricing approval. For example, the European Union 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. European Union 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 European Union 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 European Union. The downward pressure on healthcare costs in general, particularly prescription products, 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 European Union 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 reform**

The United States and some foreign jurisdictions are considering or have enacted a number of reform proposals to change the healthcare system. There is significant interest in promoting changes in healthcare systems with the stated goals of containing healthcare costs, improving quality or expanding access. In the United States, the pharmaceutical industry has been a particular focus of these efforts and has been significantly affected by federal and state legislative initiatives, including those designed to limit the pricing, coverage and reimbursement of pharmaceutical and biopharmaceutical products, especially under government-funded healthcare programs, and increased governmental control of drug pricing.

The ACA, which was enacted in 2010, substantially changed the way healthcare is financed by both governmental and private insurers in the United States, and significantly affected the pharmaceutical industry. In addition, other legislative and regulatory changes have been proposed and adopted in the United States since the ACA was enacted:

- The Budget Control Act of 2011 and subsequent legislation, among other things, created measures for spending reductions by Congress that include aggregate reductions of Medicare payments to providers of 2% per fiscal year, which remain in effect through 2032. Due to the Statutory Pay-As-You-Go Act of 2010, estimated budget deficit increases resulting from the American Rescue Plan Act of 2021, and subsequent legislation, Medicare payments to providers will be further reduced starting in 2025 absent further legislation. The U.S. American Taxpayer Relief Act of 2012 further reduced Medicare payments to several types of providers and increased the statute of limitations period for the government to recover overpayments to providers from three to five years.

- On April 13, 2017, CMS published a final rule that gives states greater flexibility in setting benchmarks for insurers in the individual and small group marketplaces, which may have the effect of relaxing the essential health benefits required under the ACA for plans sold through such marketplaces.
- On May 23, 2019, CMS published a final rule to allow Medicare Advantage Plans the option of using step therapy for Part B drugs beginning January 1, 2020.

Additionally, there has been heightened governmental scrutiny over the manner in which manufacturers set prices for their marketed products, which has resulted in several U.S. presidential executive orders, Congressional inquiries and proposed and enacted federal legislation designed to, among other things, bring more transparency to drug pricing, reduce the cost of prescription drugs under Medicare, and review the relationship between pricing and manufacturer patient programs. The Inflation Reduction Act of 2022, or the IRA, includes several provisions that may impact our business to varying degrees, including provisions that reduce the out-of-pocket spending cap for Medicare Part D beneficiaries from \$7,050 to \$2,000 starting in 2025, thereby effectively eliminating the coverage gap; impose new manufacturer financial liability on certain drugs under Medicare Part D, allow the U.S. government to negotiate Medicare Part B and Part D price caps for certain high-cost drugs and biologics without generic or biosimilar competition; require companies to pay rebates to Medicare for certain drug prices that increase faster than inflation; and delay until January 1, 2032 the implementation of the U.S. Department of Health and Human Services, or the HHS, rebate rule that would have limited the fees that pharmacy benefit managers can charge. The IRA permits HHS to implement many of these provisions through guidance, as opposed to regulation, for the initial years. Only high-expenditure single-source drugs that have been approved for at least 7 years (11 years for single-source biologics) can qualify for negotiation, with the negotiated price taking effect two years after the selection year. For 2026, the first year in which negotiated prices become effective, CMS selected 10 high-cost Medicare Part D drugs in 2023, negotiations began in 2024, and the negotiated maximum fair price for each drug has been announced. CMS has selected 15 additional Medicare Part D drugs for negotiated maximum fair pricing in 2027. For 2028, up to an additional 15 drugs, which may be covered under either Medicare Part B or Part D, will be selected, and for 2029 and subsequent years, up to 20 additional Part B or Part D drugs will be selected. HHS has and will continue to issue and update guidance as these programs are implemented. Various industry stakeholders have initiated lawsuits against the federal government asserting that the price negotiation provisions of IRA are unconstitutional. Additionally, the One Big Beautiful Bill Act (OBBBA), which was signed into law in July 2025, includes provisions that are expected to impact the U.S. healthcare system in various ways, including cuts to Medicaid and introducing new requirements for Medicaid coverage. The current administration also issued executive orders focused on decreasing prescription drug prices, including directing the Secretary of Health and Human Services to establish a mechanism through which American patients can buy drugs directly from manufacturers who sell at a most-favored-nation price and directing the U.S. Trade Representative and Secretary of Commerce to take action to ensure foreign countries are not engaged in practices that purposefully and unfairly undercut market prices and drive price hikes in the United States. In November 2025, CMS announced a voluntary initiative called the GENEROUS Model (GENErating cost Reductions fOr U.S. Medicaid Model) to introduce the option of most-favored-nation pricing to the Medicaid program, whereby a drug manufacturer may voluntarily offer supplemental rebates to participating state Medicaid programs for a manufacturer's covered outpatient drugs. Government agreements with pharmaceutical companies and other government measures that use most-favored-nation pricing targets for prescription drugs, including the use of international pricing reference to set drug prices in the United States, or that increase generic drug and biosimilar entry sooner than expected, could materially harm the Company's business, including with respect to its ability to set adequate pricing for new drugs to recover its research and development costs. It is currently unclear how the ongoing judicial challenges as well as other legislative, executive, and administrative actions, including how the IRA will be implemented, will impact the pharmaceutical industry. The implementation of cost containment measures or other healthcare reforms may prevent us from being able to generate revenue, attain profitability, or commercialize our product candidates if approved.

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.

### **Employees and human capital resources**

As of December 31, 2025, we had 59 full-time employees. Of our 59 full-time employees, 41 are engaged in research and development activities. None of our employees are represented by labor unions or covered by collective bargaining agreements. We consider our relationship with our employees to be good.

Our human capital resources objectives include, as applicable, identifying, recruiting, retaining, incentivizing and integrating our employees. We believe our success depends on our ability to attract, retain, develop and motivate highly skilled personnel. In particular, we depend upon the personal efforts and abilities of the principal members of our senior management to partner effectively as a team, and to provide strategic direction, develop our business, manage our operations and maintain a cohesive and stable work environment. We also rely on qualified managers and skilled employees, such as scientists, medical professionals, engineers and laboratory technicians, with technical expertise in operations, scientific knowledge, engineering skills and quality management experience in order to operate our business successfully.

Our compensation program is designed to retain, motivate and, as needed, attract highly qualified employees. Accordingly, we use a mix of competitive base salary, performance-based equity compensation awards and other employee benefits.

### **Corporate Information**

In February 2017, we were formed as a Delaware limited liability company under the name LEXEO Therapeutics, LLC. In November 2020, we converted into a Delaware corporation and were renamed Lexeo Therapeutics, Inc. Our principal executive offices are located at 345 Park Avenue South, Floor 6, New York, New York 10010, and our telephone number is (212) 547-9879.

### **Available Information**

Our website address is [www.lexeotx.com](http://www.lexeotx.com). The information contained on, or accessible through, our website is not incorporated by reference into this Annual Report. Our Annual Report on Form 10-K, Quarterly Reports on Form 10-Q, Current Reports on Form 8-K and amendments to reports filed pursuant to Sections 13(a) and 15(d) of the Securities Exchange Act of 1934, as amended, or the Exchange Act, are filed with the SEC. Such reports and other information filed by us with the SEC are available free of charge on our website at [ir.lexeotx.com](http://ir.lexeotx.com) when such reports are available on the SEC's website. The SEC maintains an internet site that contains reports, proxy and information statements and other information regarding issuers that file electronically with the SEC at [www.sec.gov](http://www.sec.gov). The information contained on the websites referenced in this Annual Report is not incorporated by reference into this filing. Further, our references to website URLs are intended to be inactive textual references only.

### **Channels of Distribution**

We announce material information to the public through filings with the SEC, the investor relations page on our website, press releases, public conference calls, our LinkedIn account and webcasts in order to achieve broad, non-exclusionary distribution of information to the public and for complying with our disclosure obligations under Regulation FD. We encourage investors, the media, and others to follow the channels listed above and to review the information disclosed through such channels. Any updates to the list of disclosure channels through which we will announce information will be posted on the investor relations page on our website.

## Item 1A. Risk Factors

*Investing in our common stock involves a high degree of risk. You should consider carefully the risks and uncertainties described below, together with all of the other information in this Annual Report, including the section titled “Management’s Discussion and Analysis of Financial Condition and Results of Operations” and our financial statements and related notes included elsewhere in this Annual Report, before deciding whether to invest in our common stock. The risks and uncertainties described below are not the only ones we face. Additional risks and uncertainties that we are unaware of, or that we currently believe are not material, may also become important factors that affect us. If any of the following risks are realized, our business, financial condition, results of operations and prospects could be materially and adversely affected. In that event, the price of our common stock could decline, and you could lose part or all of your investment. We cannot provide assurance that any of the events discussed below will not occur.*

### Risk Factors Summary

Investing in our common stock involves a high degree of risk because our business is subject to numerous risks and uncertainties, as fully described below. The principal factors and uncertainties that make investing in our common stock risky include, among others:

- we have incurred significant losses since our inception, and we expect to incur significant net losses for the foreseeable future and may not be able to achieve or sustain revenue or profitability in the future;
- we have a limited operating history and have no products approved for commercial sale;
- if we are unable to raise capital when needed, we could be forced to curtail our planned operations and the pursuit of our growth strategy;
- raising additional capital may cause dilution to our stockholders, restrict our operations or require us to relinquish rights to our technologies or product candidates;
- our business is dependent on our ability to advance our current and future product candidates through preclinical studies and clinical trials, obtain marketing approval and ultimately commercialize them. If we are unable to or experience significant delays in doing so, our business will be materially harmed;
- we are developing novel gene therapy product candidates, which makes it difficult to predict the time, cost and potential success of product candidate development;
- because gene therapy is novel and the regulatory landscape that governs any product candidates we may develop is rigorous, complex, uncertain and subject to change, we cannot predict with certainty the geographic areas in which we could obtain regulatory approval or the time and cost of obtaining regulatory approval, if we receive it at all, for any product candidates we may develop;
- preclinical studies and clinical trials are expensive, time-consuming, difficult to design and implement and involve an uncertain outcome. Further, we may encounter substantial delays in completing the development of our product candidates;
- the regulatory approval processes of the FDA, EMA and comparable foreign authorities are lengthy, time-consuming and inherently unpredictable. If we are not able to obtain required regulatory approval for our product candidates, our business will be substantially harmed;
- success in preclinical studies or earlier clinical trials may not be indicative of results in future clinical trials;
- interim “top-line” and preliminary results from our clinical trials that we announce, publish or present from time to time may change as more patient data become available and are subject to audit and verification procedures that could result in material changes in the final data;
- our preclinical studies and clinical trials may fail to demonstrate the safety and efficacy of our product candidates, or serious adverse or unacceptable side effects may be identified during the development of our product candidates, which could prevent or delay regulatory approval and commercialization, increase our costs or necessitate the abandonment or limitation of the development of some of our product candidates. We may also identify safety and efficacy concerns after the approval of a product candidate which can result in negative consequences to our business and results of operations;

- some of the diseases we initially seek to treat have low prevalence and it may be difficult to identify and enroll patients with these diseases. If we experience delays or difficulties in the enrollment and/or maintenance of patients in clinical trials, our receipt of necessary regulatory approvals could be delayed or prevented;
- we may seek Orphan Drug designation or Rare Pediatric Disease designation for some of our product candidates and we may be unsuccessful, or may be unable to maintain the benefits associated with Orphan Drug designation, including the potential for market exclusivity, for product candidates for which we obtain Orphan Drug designation;
- Fast Track, Breakthrough Therapy, or Regenerative Medicine Advanced Therapy designation that we may receive from the FDA may not actually lead to a faster development or regulatory review or approval process, and does not assure FDA approval of our product candidates;
- we have received Rare Pediatric Disease designation from the FDA for LX2006 for the treatment of FA and we may seek such designation for future product candidates if Congress extends the rare pediatric disease priority review program. However, a marketing application for these product candidates, if approved, may not meet the eligibility criteria for a rare pediatric disease priority review voucher;
- we and our contract manufacturers are subject to significant regulation with respect to manufacturing our products. The third-party manufacturing facilities on which we rely, and any manufacturing facility that we may have in the future, may have limited capacity or fail to meet the applicable stringent regulatory requirements;
- gene therapies are novel, complex and difficult to manufacture. We could experience manufacturing problems that result in delays in the development or commercialization of our product candidates or otherwise harm our business;
- we depend on third-party suppliers for materials used in the manufacture of our product candidates, and the loss of these third-party suppliers or their inability to supply us with adequate materials could harm our business;
- even if any of our product candidates receive marketing approval, they may fail to achieve the degree of market acceptance by physicians, patients, third-party payors and others in the medical community necessary for commercial success;
- we rely on our collaborations with several leading academic institutions to conduct research and development for many of our pipeline programs, including conducting preclinical and IND-enabling studies for portions of our near-term future pipeline. Failure or delay of our academic partners to fulfill all or part of their respective obligations to us under our agreements, a breakdown in collaboration between the parties or a complete or partial loss of either of these relationships could materially harm our business;
- we intend to continue to rely on third parties to conduct a significant portion of our existing clinical trials and potential future clinical trials for product candidates, and those third parties may not perform satisfactorily, including failing to meet deadlines for the completion of such trials;
- if we are unable to obtain or protect intellectual property rights related to any of our product candidates, we may not be able to compete effectively in our market;
- changes in the FDA and other government agencies, regulatory actions and other actions under the current administration could hinder their ability to hire and retain key leadership and other personnel, or otherwise prevent new products and services from being developed or commercialized in a timely manner, which could negatively impact our business; and
- we may become subject to claims that we and our employees, consultants or independent contractors have wrongfully used or disclosed confidential information or trade secrets of third parties.

## Risks related to our financial position and capital needs

***We have incurred significant losses since our inception. We expect to incur losses over the next several years and may never achieve or maintain profitability.***

Since our inception, we have incurred significant net losses, and we expect to continue to incur significant expenses and operating losses for the foreseeable future. For the years ended December 31, 2025 and December 31, 2024, we incurred net losses of \$100.0 million and \$98.3 million, respectively, and we had an accumulated deficit of \$380.1 million as of December 31, 2025. We have primarily financed our operations with net proceeds from the October 2025 Financing Transactions (as defined below), May 2025 Private Placement (as defined below), the March 2024 Private Placement (as defined below), net proceeds raised in our IPO and the subsequent partial exercise of the underwriters' 30-day option to purchase additional shares of common stock, as well as sales of our convertible equity securities. We have no products approved for commercialization and have never generated any revenue from product sales.

We are still in the early clinical stages of development of our lead product candidates. We expect to continue to incur significant expenses and operating losses over the next several years. Our operating expenses and net losses may fluctuate significantly from quarter to quarter and year to year. We anticipate that our expenses will increase substantially as we:

- continue to advance the preclinical and clinical development of our product candidates and discovery programs;
- initiate and complete additional clinical trials of our current and future product candidates;
- seek regulatory approval for any product candidates that successfully complete clinical trials;
- continue to develop our gene therapy product candidate pipeline;
- scale up our clinical and regulatory capabilities;
- work with our third party manufacturing partners to produce material in accordance with cGMP for clinical trials or potential commercial sales;
- establish, either alone or with a third party, a commercialization infrastructure and scale up manufacturing and distribution capabilities to commercialize any product candidates for which we may obtain regulatory approval;
- adapt our regulatory compliance efforts to incorporate requirements applicable to marketed products;
- maintain, expand and protect our intellectual property portfolio and patent claims;
- hire additional clinical, quality control, regulatory, manufacturing, scientific and administrative personnel;
- add operational, financial and management information systems and personnel, including personnel to support our product development and planned future commercialization efforts; and
- incur additional legal, accounting and other expenses while operating as a public company.

To date, we have not generated any revenue from the commercialization of our product candidates. To become and remain profitable, we must succeed in developing and eventually commercializing product candidates that generate significant revenue. This will require us to be successful in a range of challenging activities, including completing preclinical testing and clinical trials of our product candidates, obtaining regulatory approval, and manufacturing, marketing and selling any product candidates for which we may obtain regulatory approval, as well as discovering and developing additional product candidates. We are only in the preliminary stages of most of these activities and all of our product candidates are in early clinical trials or preclinical development. We may never succeed in these activities and, even if we do, may never generate any revenue or revenue that is significant enough to achieve profitability.

Even if we achieve profitability, we may not be able to sustain or increase profitability on a quarterly or annual basis. Our failure to become and remain profitable would depress the value of our company and could impair our ability to raise capital, expand our business, maintain our development efforts, obtain product approvals, diversify our offerings or continue our operations. A decline in the value of our company could also cause you to lose all or part of your investment.

***We have a limited operating history and no history of commercializing products, which may make it difficult for an investor to evaluate the success of our business to date and to assess our future viability.***

We are a clinical stage genetic medicine company with a limited operating history. We commenced substantive business operations in 2020, and our operations to date have been largely focused on organizing and staffing our company, business planning, raising capital, entering into collaboration and license agreements for conducting preclinical and clinical research and development activities for our product candidates and gene therapy pipeline, and conducting clinical trials for our product candidates through CROs and other third parties. To date, we have not yet demonstrated our ability to successfully complete pivotal clinical trials, manufacture a product on a commercial scale or arrange for a third party to do so on our behalf, or conduct sales and marketing activities necessary for successful commercialization. Consequently, any predictions you make about our future success or viability may not be as accurate as they could be if we had a longer operating history or a history of successfully developing and commercializing products.

***We will need substantial additional funding to meet our financial obligations and to pursue our business objectives. If we are unable to raise capital when needed, we could be forced to curtail our planned operations and the pursuit of our growth strategy.***

We will require substantial future capital in order to complete planned and future clinical development for our lead product candidates, preclinical development for our other product candidates, and potential commercialization of these product candidates, if any are approved. We expect our spending levels to significantly increase in connection with our planned clinical trials of our lead product candidates. In addition, if we obtain marketing approval for any of our product candidates, we expect to incur significant expenses related to product sales, medical affairs, marketing, manufacturing and distribution. We also expect to incur additional costs associated with operating as a public company. Accordingly, we will need to obtain substantial additional funding in connection with our continuing operations. If we are unable to raise capital when needed or on acceptable terms, we would be forced to delay, reduce or eliminate certain of our clinical trials, our research and development programs or other operations.

As of December 31, 2025, we had cash, cash equivalents, and investments in U.S. Treasury securities of \$246.6 million. We believe that our cash, cash equivalents, and investments in U.S. Treasury securities balances will be sufficient to fund our operating expenses and capital requirements into 2028. This estimate is based on assumptions that may prove to be wrong, and we could use our available capital resources sooner than we expect. Changes may occur beyond our control that would cause us to consume our available capital before that time, including changes in and progress of our development activities, acquisitions of additional product candidates, and changes in regulation. Our future capital requirements will depend on many factors, including:

- the costs of and investment in ongoing and future development of our gene therapy product candidates;
- the scope, progress, costs and results of discovery, preclinical development, laboratory testing and clinical trials for our product candidates;
- the extent to which we develop, in-license or acquire other product candidates and technologies in our product candidate pipeline;
- the costs and timing of process development and manufacturing scale-up activities associated with our product candidates and other programs as we advance them through preclinical and clinical development;
- the number of, and development requirements for, product candidates that we may pursue;
- the costs, timing and outcome of regulatory review of our product candidates;
- our headcount growth and associated costs as we expand our research and development capabilities and establish a commercial infrastructure;
- the costs of establishing and maintaining commercial-scale cGMP manufacturing capabilities, either internally or with third parties;
- the costs and timing of future commercialization activities, including product manufacturing, marketing, sales and distribution, for any of our product candidates for which we receive marketing approval;
- the costs and timing of preparing, filing and prosecuting patent applications, maintaining and enforcing our intellectual property rights and defending any intellectual property-related claims;
- the revenue, if any, received from commercial sales of our product candidates for which we receive marketing approval;
- our ability to establish and maintain additional collaborations on favorable terms, if at all;

- the success of any collaborations that we may establish and our license agreements;
- the outcome of any legal proceedings involving us;
- the achievement of milestones or occurrence of other developments that trigger payments under our collaboration agreements or any additional collaboration agreements we may enter into; and
- the costs of operating as a public company.

We will require additional capital to achieve our business objectives. While the long-term economic impact of the ongoing geopolitical conflicts in Ukraine and the Middle East is difficult to assess or predict, each of these events has caused significant disruptions to the global financial markets and contributed to a general global economic slowdown. Furthermore, inflation rates, particularly in the United States, have increased recently to levels not seen in decades. Increased inflation may result in increased operating costs and may affect our operating budgets, specifically with respect to increased labor costs and associated difficulties in recruiting qualified personnel. In addition, the U.S. Federal Reserve has raised, and may further raise, interest rates in response to concerns about inflation. Increases in interest rates, especially if coupled with reduced government spending and volatility in financial markets, may further increase economic uncertainty and heighten these risks. If the disruptions and slowdown deepen or persist, we may not be able to access additional capital on favorable terms, or at all, which could in the future negatively affect our financial condition and we could be forced to curtail our planned operations and the pursuit of our growth strategy.

***Raising additional capital may cause dilution to our stockholders, restrict our operations or require us to relinquish rights to our technologies or product candidates.***

Until such time, if ever, as we can generate substantial revenue, we may finance our cash needs through a combination of equity offerings, government or private party grants, debt financings and license and collaboration agreements. We do not currently have any committed external source of funds. To the extent that we raise additional capital through the sale of equity or convertible debt securities, your ownership interest will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect your rights as a common stockholder. Debt financing and preferred equity financing, if available, may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends.

If we raise additional funds through collaborations, strategic alliances or marketing, distribution or licensing arrangements with third parties, we may be required to relinquish valuable rights to our technologies, future revenue streams or product candidates, grant licenses on terms that may not be favorable to us or commit to future payment streams. If we are unable to raise additional funds through equity or debt financings when needed, we may be required to delay, limit, reduce or terminate our product development or future commercialization efforts or grant rights to develop and market product candidates that we would otherwise prefer to develop and market ourselves.

#### **Risks related to the development of our product candidates**

***Our business is dependent on our ability to advance our current and future product candidates through preclinical studies and clinical trials, obtain marketing approval and ultimately commercialize them. If we are unable to or experience significant delays in doing so, our business will be materially harmed.***

We have invested a significant portion of our time and financial resources in the development of our product candidates and technology platforms. Our business is dependent on our ability to successfully complete preclinical and clinical development of, obtain regulatory approval for, and, if approved, successfully commercialize LX2006, LX2020 and any other product candidates in a timely manner.

Each of our product candidates and programs will require additional preclinical and/or clinical development, regulatory approval and significant marketing efforts, and we will be required to obtain manufacturing supply and expertise and to build a commercial organization or successfully outsource commercialization before we generate any revenue from product sales. We do not have any products that are approved for commercial sale, and we may never be able to develop or commercialize marketable products.

Our ability to generate revenue from our product candidates, which may not occur for several years, will depend heavily on the successful development, regulatory approval and eventual commercialization of our product candidates. The success of our lead product candidates, or any other product candidates that we develop or otherwise may acquire will depend on various factors, including:

- timely and successful completion of preclinical studies, including toxicology studies, biodistribution studies and minimally efficacious dose studies in animals, where applicable, under the FDA's GLPs;

- the availability or development of suitable animal disease models for nonclinical studies to enable us to proceed into clinical development or support the submission of a marketing application;
- effective IND applications from the FDA or comparable foreign applications that allow commencement of our planned clinical trials or future clinical trials for our product candidates;
- sufficiency of our financial and other resources to complete the necessary preclinical studies and clinical trials;
- successful enrollment and completion of clinical trials, including under the FDA's current cGCPs;
- establishment of our own manufacturing capabilities and/or arrangements with third-party manufacturers for our commercial manufacturing processes for any of our product candidates that receive regulatory approval;
- receipt of timely marketing approvals from applicable regulatory authorities;
- launch of commercial sales of products, if approved, whether alone or in collaboration with others;
- acceptance of the benefits and use of our products, including method of administration, if and when approved, by patients, the medical community and third-party payors, for their approved indications;
- the prevalence and severity of adverse events experienced with any of our product candidates;
- the availability, perceived advantages, cost, safety and efficacy of alternative therapies for any diseases for which we are developing our product candidates;
- our ability to produce our product candidates on a commercial scale;
- attainment and maintenance of patent, trademark and trade secret protection and regulatory exclusivity for our product candidates and otherwise protecting our rights in our intellectual property portfolio;
- maintenance of compliance with regulatory requirements such as cGMPs;
- attainment and maintenance of third-party coverage and adequate reimbursement for our product candidates and patients' willingness to pay out-of-pocket in the absence of such coverage and adequate reimbursement; and
- maintenance of a continued acceptable safety, tolerability and efficacy profile of our products following approval.

If we are not successful with respect to one or more of these factors in a timely manner or at all, we could experience significant delays or an inability to successfully commercialize the product candidates we develop, which would materially harm our business. If we do not receive marketing approvals for any product candidate we develop, we may not be able to continue our operations.

***We are developing novel gene therapy product candidates, which makes it difficult to predict the time, cost and potential success of product candidate development.***

Our future success depends on the successful development of a novel therapeutic approach. To date, very few products that utilize gene transfer have been approved in the United States or Europe. There have been a limited number of clinical trials using AAVrh10. Although gene therapies have been studied in human clinical trials for over 30 years, only a limited number of AAV-based gene therapy products have been approved by the FDA.

We cannot be certain that our AAVrh10-based gene therapy product candidates will successfully complete clinical trials or that any future product candidates utilizing this or other vector constructs will successfully complete preclinical studies or clinical trials. We may not be successful in developing product candidates that avoid triggering toxicities or other side effects in preclinical studies or clinical trials. Our intravenous and intrathecal routes of administration may cause unforeseen side effects or present other challenges. Any such results could impact our ability to develop a product candidate, including our ability to enroll patients in our clinical trials. As a result of these factors, it is more difficult for us to predict the time and cost of product candidate development, and we cannot predict whether the application of our approach to gene therapy, or any similar or competitive programs, will result in the identification, development, and regulatory approval of any product candidate, or that other gene therapy programs will not be considered better or more favorable. There can be no assurance that any development problems we experience in the future related to our current gene therapy product candidates or any of our research programs will not cause significant delays or unanticipated costs, or that such development problems can be solved. We may also experience delays and challenges in achieving sustainable, reproducible, and scalable production. Any of these factors may prevent us from completing our preclinical studies or clinical trials or commercializing any product candidates we may develop on a timely or profitable basis, if at all.

***Because gene therapy is novel and the regulatory landscape that governs any product candidates we may develop is rigorous, complex, uncertain and subject to change, we cannot predict with certainty the geographic areas in which we could obtain regulatory approval nor the time and cost of obtaining regulatory approval, if we receive it at all, for any product candidates we may develop.***

The regulatory requirements that will govern any novel gene therapy product candidates we develop are not entirely clear and are subject to change. The novel nature of our capsids makes it difficult to determine how long it will take or how much it will cost to obtain regulatory approvals for our product candidates in the United States, the European Union or other jurisdictions. Within the broader genetic medicine field, very few gene therapy products have received marketing authorization from the FDA or the European Medicines Agency, or EMA. Even with respect to gene therapies, the regulatory landscape is still developing. Regulatory requirements governing gene therapy products have changed frequently and will likely continue to change in the future, including with respect to those responsible for regulation of existing gene therapy products. For example, in 2016, the FDA established the Office of Tissues and Advanced Therapies, or OTAT, within the Center for Biologics Evaluation and Research, or CBER, to consolidate the review of gene therapy and related products, and to advise the CBER on its review. In March 2023, the FDA retitled the OTAT to the Office of Therapeutic Products, or OTP, and elevated OTP to a “Super Office” to meet its growing cell and gene therapy workload.

Our product candidates will need to meet safety and efficacy standards applicable to any new biologic being pursued for a given disease under the regulatory framework administered by the FDA. Although the FDA decides whether individual gene therapy protocols may proceed, the review process and determinations of other reviewing bodies, including IRBs, can impede or delay the initiation of a clinical trial.

The same applies in the European Union. The EMA’s Committee for Advanced Therapies, or CAT, is responsible for assessing the quality, safety and efficacy of advanced-therapy medicinal products. Advanced-therapy medicinal products include gene therapy medicines, somatic-cell therapy medicines and tissue-engineered medicines. The role of the CAT is to prepare a draft opinion on an application for marketing authorization for a gene therapy medicinal candidate that is submitted to the EMA. In the European Union, the development and evaluation of a gene therapy product must be considered in the context of the relevant EU guidelines. The EMA may issue new guidelines concerning the development and marketing authorization for gene therapy products and require that we comply with these new guidelines. This could mean that any gene therapy product candidate we may develop in the future could be required to comply with additional and/or more stringent gene therapy guidelines in the European Union.

Adverse developments in preclinical studies or clinical trials conducted by others in the field of gene therapy and gene regulation products may cause the FDA, the EMA and other regulatory bodies to revise the requirements for approval of any product candidates we may develop or limit the use of products utilizing gene regulation technologies, either of which could harm our business. In addition, the clinical trial requirements of the FDA, the EMA and other regulatory authorities and the criteria these regulators use to determine the safety and efficacy of a product candidate vary substantially according to the type, complexity, novelty, and intended use and market of the potential products. The regulatory approval process for product candidates such as ours can be more expensive and take longer than for other, better known, or more extensively studied pharmaceutical or other product candidates. Further, as we are developing novel potential treatments for diseases in which, in some cases, there is little clinical experience with potential new endpoints and methodologies, there is heightened risk that the FDA, the EMA or other regulatory bodies may not consider the clinical trial endpoints to provide clinically meaningful results, and the resulting clinical data and results may be more difficult to analyze. In addition, we may not be able to identify or develop appropriate animal disease models to enable or support planned clinical development. Any natural history studies that we may rely upon in our clinical development may not be accepted by the FDA, EMA or other regulatory authorities. Regulatory agencies administering existing or future regulations or legislation may not allow production and marketing of products utilizing gene regulation technology in a timely manner or under technically or commercially feasible conditions. In addition, regulatory action or private litigation could result in expenses, delays, or other impediments to our research programs or the commercialization of resulting products. Further, approvals by one regulatory agency may not be indicative of what other regulatory agencies may require for approval.

The regulatory review committees and advisory groups described above and any new guidelines they promulgate may lengthen the regulatory review process, require us to perform additional preclinical studies or clinical trials, increase our development costs, lead to changes in regulatory positions and interpretations, delay or prevent approval and commercialization of these treatment product candidates, or lead to significant post-approval limitations or restrictions. As we advance our research programs and develop future product candidates, we will be required to consult with these regulatory and advisory groups and to comply with applicable guidelines. If we fail to do so, we may be required to delay or discontinue development of any product candidates we identify and develop. These additional processes may result in a review and approval process that is longer than we otherwise would have expected. Delays as a result of an increased or lengthier regulatory approval process or further restrictions on the development of our product candidates can be costly and could negatively impact our ability to complete clinical trials and commercialize our current and future product candidates in a timely manner, if at all.

***Preclinical studies and clinical trials are expensive, time-consuming, difficult to design and implement and involve an uncertain outcome. Further, we may encounter substantial delays in completing the development of our product candidates.***

All of our product candidates are in preclinical or clinical development, and the risk of failure is high. The preclinical studies, clinical trials and manufacturing of our product candidates are, and the manufacturing and marketing of our products, if approved, will be, subject to extensive and rigorous review and regulation by numerous government authorities in the United States and in other countries where we may test and market our product candidates. Before obtaining regulatory approvals for the commercial sale of any of our product candidates, we must demonstrate through lengthy, complex and expensive preclinical testing and clinical trials that our product candidates are both safe and effective for use in each target disease. In particular, because our product candidates are subject to regulation as biologics, we will need to demonstrate that they are sufficiently safe and of sufficient purity and potency for use in their target diseases. Each product candidate must demonstrate an adequate risk-versus-benefit profile in its intended patient population and for its intended use.

Clinical testing is expensive, can take many years to complete and is subject to uncertainty. We cannot guarantee that any clinical trials will be initiated on schedule, conducted as planned or completed on schedule, if at all. To date, we are sponsoring clinical trials of LX2006 and LX2020, but we have not successfully completed any clinical trial that we have internally sponsored. Failure can occur at any time during the clinical trial process. Even if our ongoing and future clinical trials are completed as planned, we cannot be certain that their results will support the safety and effectiveness of our product candidates for their targeted diseases or support continued clinical development of such product candidates. Our future clinical trial results may not be successful.

In addition, even if such trials are successfully completed, we cannot guarantee that the FDA or foreign regulatory authorities will interpret the results as we do, and more trials could be required before we submit our product candidates for approval. This is particularly true for clinical trials in rare diseases, where the small patient populations make it difficult or impossible to conduct two traditional, adequate and well-controlled trials, and therefore the FDA or comparable foreign regulatory authorities are often required to exercise flexibility in approving therapies for such diseases. Moreover, results acceptable to support approval in one jurisdiction may be deemed inadequate by another regulatory authority to support regulatory approval in that other jurisdiction. To the extent that the results of the trials are not satisfactory to the FDA or foreign regulatory authorities for support of a marketing application, we may be required to expend significant resources, which may not be available to us, to conduct additional trials in support of potential approval of our product candidates.

We may experience delays in initiating and conducting clinical trials of our lead product candidates and we do not know whether our clinical trials will begin on time, need to be redesigned, recruit and enroll patients on time or be completed on schedule, or at all. Events that may prevent successful or timely completion of clinical development include:

- inability to generate sufficient preclinical, toxicology, or other *in vivo* or *in vitro* data to support the initiation of clinical trials;
- delays in sufficiently developing, characterizing or controlling a manufacturing process suitable for advanced clinical trials;
- delays in sourcing or qualifying ancillaries required for administration of our clinical drug product (such as vials, stoppers, or tubing);
- delays in developing suitable assays for screening patients for eligibility for trials with respect to certain product candidates;
- delays in reaching agreement with the FDA, EMA or other regulatory authorities as to the design or implementation of our clinical trials;
- failure to obtain regulatory approval to commence a clinical trial;
- failure to reach an agreement on acceptable terms with clinical trial sites or prospective CROs, the terms of which can be subject to extensive negotiation and may vary significantly among different clinical trial sites;
- inability to obtain IRB approval for each clinical trial site;
- inability to recruit suitable patients to participate in a clinical trial in a timely manner;
- failure to have patients complete a clinical trial or return for post-treatment follow-up;
- deviations by clinical trial sites, CROs or other third parties from trial protocol;
- failure to perform our planned clinical trials in accordance with the FDA's cGCP requirements, or applicable regulatory guidelines in other countries;
- inability to address patient-safety concerns that arise during the course of a trial, including occurrence of adverse events associated with the product candidate that are viewed to outweigh its potential benefits;
- failure to initiate a sufficient number of clinical trial sites; or
- delays in manufacturing sufficient quantities of a product candidate for use in clinical trials.

We may experience numerous unforeseen events during, or as a result of, clinical trials that could delay or prevent our ability to receive marketing approval or commercialize our product candidates or significantly increase the cost of such trials, including:

- we may experience changes in regulatory requirements or guidance, or receive feedback from regulatory authorities, that require us to modify the design of our clinical trials;
- clinical trials of our product candidates may produce negative or inconclusive results, and we may decide, or regulators may require us, to conduct additional clinical trials or halt development programs;
- the number of patients required for clinical trials of our product candidates may be larger than we anticipate, enrollment in these clinical trials may be slower than we anticipate, or participants may drop out of these clinical trials at a higher rate than we anticipate;
- our third-party contractors may fail to comply with regulatory requirements or meet their contractual obligations to us in a timely manner, or at all;
- we, our investigators or regulators may suspend or terminate clinical trials of our product candidates for various reasons, including non-compliance with regulatory requirements, a finding that our product candidates have undesirable side effects or other unexpected characteristics, or a finding that the participants are being exposed to unacceptable health risks;
- the cost of clinical trials of our product candidates may be greater than we anticipate, and we may not have funds to cover the costs;

- the supply or quality of our product candidates or other materials necessary to conduct clinical trials of our product candidates may be insufficient or inadequate;
- regulators may revise the requirements for approving our product candidates, or such requirements may not be as we anticipate; and
- any future collaborators that conduct clinical trials may face any of the above issues and may conduct clinical trials in ways they view as advantageous to them but that are suboptimal for us.

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

- incur unplanned costs;
- be delayed in obtaining marketing approval for our product candidates or not obtain marketing approval at all;
- obtain marketing approval in some countries and not in others;
- obtain marketing approval for diseases or patient populations that are not as broad as intended or desired;
- obtain marketing approval with labeling that includes significant use or distribution restrictions or safety warnings, including boxed warnings REMS;
- be subject to additional post-marketing testing requirements; or
- have the product removed from the market after obtaining marketing approval.

We could encounter delays if a clinical trial is suspended or terminated by us, by the IRBs of the institutions in which such trials are being conducted, by the Data Safety Monitoring Board for such trial or by the FDA, EMA or other regulatory authorities. Such authorities may impose such a suspension or termination due to a number of factors, including failure to conduct the clinical trial in accordance with regulatory requirements or our clinical protocols, inspection of the clinical trial operations or trial site by the FDA, EMA or other regulatory authorities resulting in the imposition of a clinical hold, unforeseen safety issues or adverse side effects, failure to demonstrate a benefit from using a drug, changes in governmental regulations or administrative actions or lack of adequate funding to continue the clinical trial.

All of our product candidates will require extensive clinical testing before we are prepared to submit a BLA, or marketing authorization application, or MAA, for regulatory approval. We cannot predict with any certainty if or when we might complete the clinical development for our product candidates and submit a BLA or MAA for regulatory approval of any of our product candidates or whether any such BLA or MAA will be approved. We may also seek feedback from the FDA, EMA or other regulatory authorities on our clinical development program, and the FDA, EMA or such regulatory authorities may not provide such feedback on a timely basis, or such feedback may not be favorable, which could further delay our development programs.

We cannot predict with any certainty whether or when we might complete a given clinical trial. If we experience delays in the commencement or completion of our clinical trials, or if we terminate a clinical trial prior to completion, the commercial prospects of our product candidates could be harmed, and our ability to generate revenues from our product candidates may be delayed or lost. In addition, any delays in our clinical trials could increase our costs, slow down the development and approval process and jeopardize our ability to commence product sales and generate revenues. Any of these occurrences may harm our business, financial condition and results of operations. In addition, many of the factors that cause, or lead to, a delay in the commencement or completion of clinical trials may also ultimately lead to the denial of regulatory approval of our product candidates.

***The regulatory approval processes of the FDA, EMA and comparable foreign authorities are lengthy, time-consuming and inherently unpredictable. If we are not able to obtain required regulatory approval for our product candidates, our business will be substantially harmed.***

The time required to obtain approval or other marketing authorizations by the FDA, EMA and comparable foreign authorities is unpredictable, and it typically takes many years following the commencement of clinical trials and depends upon numerous factors, including the substantial discretion of the regulatory authorities. In addition, approval policies, regulations, and the type and amount of clinical data necessary to gain approval may change during the course of a product candidate's clinical development and may vary among jurisdictions. We have not obtained regulatory approval for any product candidate, and it is possible that we may never obtain regulatory approval for any product candidates we may seek to develop in the future. Neither we nor any collaborator is permitted to market any of our biologic product candidates in the United States until we receive regulatory approval of a BLA from the FDA, and we cannot market any of our product candidates in the European Union until we receive approval for an MAA from the EMA, or other required regulatory approval in other countries.

Prior to obtaining approval to commercialize any product candidate in the United States or abroad, we must demonstrate with substantial evidence from well-controlled clinical trials, and to the satisfaction of the FDA or foreign regulatory agencies, that such product candidates are safe, effective and of sufficient purity for their intended uses. Results from preclinical studies and clinical trials can be interpreted in different ways. Even if we believe the preclinical or clinical data for our product candidates meet regulatory standards, such data may not be sufficient to support approval by the FDA and other regulatory authorities. The FDA may also require us to conduct additional preclinical studies or clinical trials for our product candidates either prior to or after approval, or it may object to elements of our clinical development programs, or require changes to our manufacturing approaches.

Of the large number of products in development, only a small percentage successfully complete the FDA or foreign regulatory approval processes and are commercialized. The lengthy approval and marketing authorization process as well as the unpredictability of future clinical trial results may result in our failing to obtain regulatory approval and marketing authorization to market our product candidates, which would significantly harm our business, financial condition, results of operations and prospects.

We have invested a significant portion of our time and financial resources in the development of our product candidates and technology platforms. Our business is dependent on our ability to successfully complete preclinical and clinical development of, obtain regulatory approval for, and, if approved, successfully commercialize LX2006, LX2020, and our other product candidates in a timely manner.

Even if we eventually complete clinical testing and receive approval of a BLA or foreign marketing application for any of our product candidates, the FDA, EMA or the applicable foreign regulatory agency may grant approval or other marketing authorization contingent on the performance of costly additional clinical trials, including post-marketing clinical trials. The FDA, EMA or the applicable foreign regulatory agency also may approve or authorize for marketing a product candidate for a more limited disease or patient population than we originally request, and the FDA, EMA or applicable foreign regulatory agency may not approve or authorize the labeling that we believe is necessary or desirable for the successful commercialization of a product candidate. Any delay in obtaining, or inability to obtain, applicable regulatory approval or other marketing authorization would delay or prevent commercialization of that product candidate and would materially adversely impact our business and prospects.

In addition, the FDA, EMA and other regulatory authorities may change their policies, issue additional regulations or revise existing regulations, or take other actions, which may prevent or delay approval of our future products under development on a timely basis. Such policy or regulatory changes could impose additional requirements upon us that could delay our ability to obtain approvals, increase the costs of compliance or restrict our ability to maintain any marketing authorizations we may have obtained. In June 2024, the Supreme Court overruled the *Chevron* doctrine, which had given deference to regulatory agencies' statutory interpretations of ambiguous regulations in litigation against federal government agencies, such as the FDA. The overruling of the *Chevron* doctrine may significantly increase the number of challenges brought by companies and other stakeholders against federal agencies such as the FDA and its longstanding decisions and policies, including the FDA's statutory interpretations of market exclusivities and the "substantial evidence" requirements for drug approvals, which could undermine the FDA's authority, lead to uncertainties in the industry, and disrupt the FDA's normal operations, any of which could delay the FDA's review of our regulatory submissions. We cannot predict the full impact of this decision, future judicial challenges brought against the FDA, or the nature or extent of government regulation that may arise from future legislation or administrative action.

Further, under the current administration, agency reorganization, government shutdown, lapse in government appropriation, voluntary departures at the FDA, and layoffs due to the reduction in force initiative and other measures implemented by the Department of Government Efficiency may impact the normal operations of the FDA as well as other federal agencies. FDA may lack adequate staff and resources to meet current review, approval, and inspection schedules, which could delay our anticipated timelines. In January 2025, an executive order entitled “Unleashing Prosperity Through Deregulation”, was issued which calls for at least 10 existing regulations to be repealed whenever an executive department or agency publicly proposes for notice and comment or otherwise promulgates a new regulation. Recent developments at the FDA include announcement of a plan to phase out animal testing for monoclonal antibodies and certain other drugs and the announcement of a new Commissioner’s National Priority Voucher program for companies supporting certain U.S. national health priorities and interests. To the extent our competitors are selected for this new voucher pilot program and obtain faster approval than us, our competitive position may be harmed. FDA has also increased its scrutiny of foreign drug manufacturing facilities and other contractors based in China, especially with respect to the transfer of biological materials, genetic data, and other sensitive data of American patients to parties located in China. FDA’s “real-time” release of newly issued Complete Response Letters associated with withdrawn or abandoned applications, if received for any of our product candidates, can materially impact our competitive advantage and intellectual property. It is unclear how our industry and our clinical programs will be impacted by the current government shutdown, policies and regulations implemented under the current administration and FDA leadership, or other executive orders. There is significant uncertainty in the industry about how federal agencies like the FDA will change in the coming years under the current administration. To the extent the agency reorganization and other agency changes lead to disruptions in FDA’s operations, our correspondence and regulatory review processes with the FDA may be materially delayed.

***Success in preclinical studies or earlier clinical trials may not be indicative of results in future clinical trials.***

Success in preclinical testing and early clinical trials does not ensure that later clinical trials will generate the same results or otherwise provide adequate data to demonstrate the efficacy and safety of a product candidate. Preclinical tests and Phase 1 and Phase 2 clinical trials are primarily designed to test safety, to study pharmacokinetics and pharmacodynamics and to understand the side effects of product candidates at various doses and schedules. Success in preclinical or animal studies and early clinical trials does not ensure that later larger-scale efficacy and safety trials will be successful, nor does it predict final results. For example, we may be unable to identify suitable animal disease models for our product candidates, which could delay or frustrate our ability to proceed into clinical trials or obtain marketing approval. In addition, the preclinical studies conducted by Stelios (an entity that we acquired in 2021) and The Regents of UCSD for our product candidates LX2021 and LX2022 employed an AAV9-based formulation and studies using this vector may not be predictive of future testing we intend to conduct using an AAVrh10-based formulation or other potential capsid serotypes.

Our product candidates may fail to show the desired safety and efficacy in clinical development despite positive results in preclinical studies or having successfully advanced through initial clinical trials. Furthermore, our currently ongoing and most future clinical trials involve or will involve a small patient population. Because of the small sample sizes studied in our trials thus far, the results of these trials may not be indicative of results of future clinical trials.

Additionally, some of our ongoing and planned clinical trials utilize, or may utilize, an “open-label” trial design. An “open-label” clinical trial is one where both the patient and investigator know whether the patient is receiving the investigational product candidate or either an existing approved drug or placebo. Most typically, open-label clinical trials test only the investigational product candidate and sometimes may do so at different dose levels. Open-label clinical trials are subject to various limitations that may exaggerate any therapeutic effect as patients in open-label clinical trials are aware when they are receiving treatment. Open-label clinical trials may be subject to a “patient bias” where patients perceive their symptoms to have improved merely due to their awareness of receiving an experimental treatment. In addition, open-label clinical trials may be subject to an “investigator bias” where those assessing and reviewing the physiological outcomes of the clinical trials are aware of which patients have received treatment and may interpret the information of the treated group more favorably given this knowledge. The results from an open-label trial may not be predictive of future clinical trial results when studied in a controlled environment with a placebo or active control.

Many companies in the pharmaceutical and biotechnology industries have suffered significant setbacks in late-stage clinical trials even after achieving promising results in preclinical testing and earlier-stage clinical trials. Data obtained from preclinical and clinical activities are subject to varying interpretations, which may delay, limit, or prevent regulatory approval. In addition, we may experience regulatory delays or rejections as a result of many factors, including changes in regulatory policy during the period of our product candidate development. Any such delays could negatively impact our business, financial condition, results of operations and prospects.

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

From time to time, we have and may continue to publish or present interim top-line or preliminary results from our clinical trials. Interim results from clinical trials that we may complete are subject to the risk that one or more of the clinical outcomes may materially change as patient enrollment continues and more patient data become available. Preliminary or top-line results also remain subject to audit and verification procedures that may result in the final data being materially different from the preliminary data we previously published. As a result, interim and preliminary data should be viewed with caution until the final data are available. Differences between preliminary or interim data and final data could significantly harm our business prospects and may cause the trading price of our common stock to fluctuate significantly.

***Our preclinical studies and clinical trials may fail to demonstrate the safety and efficacy of our product candidates, or serious adverse or unacceptable side effects may be identified during the development of our product candidates, which could prevent or delay regulatory approval and commercialization, increase our costs or necessitate the abandonment or limitation of the development of some of our product candidates. We may also identify safety and efficacy concerns after the approval of a product candidate which can result in negative consequences to our business and results of operations.***

Before obtaining regulatory approvals for the commercial sale of our product candidates, we must demonstrate through lengthy, complex and expensive preclinical testing and clinical trials that our product candidates are safe, effective and of sufficient purity for use in each target disease, and failures can occur at any stage of testing. Preclinical studies and clinical trials often fail to demonstrate safety or efficacy of the product candidate studied for the target disease. While we have developed our AAVrh10-mediated gene therapy product candidates to leverage the low seropositivity of AAVrh10, any gene therapy product based on viral vectors carries the risks of immunogenicity, elevated liver enzymes and insertional oncogenesis, which is the process whereby the insertion of a functional gene near a gene that is important in cell growth or division results in uncontrolled cell division, which could potentially enhance the risk of malignant transformation. In one of our preclinical studies of LX2006, we observed four cases of hepatocellular carcinoma, or HCC, in wild-type mice at 10 months post-treatment. Although data reported by the FDA Cellular, Tissue, and Gene Therapies Advisory Committee in September 2021 suggests that HCC observed in mice after AAV treatment is unlikely to translate to risks for humans, any future instances of HCC in our clinical trials could result in delays or the abandonment of our trials. Health authorities also ask that sponsors closely monitor the risk of elevated liver enzymes and abnormal liver ultrasound on a routine basis in patients participating in gene therapy clinical trials.

Possible adverse side effects that could occur with treatment with gene therapy products include an immunologic reaction early after administration, which, while not necessarily adverse to the patient’s health, could substantially limit the effectiveness of the treatment. For example, in previous third-party clinical trials involving AAV capsids for gene therapy, some subjects experienced the development of a T-cell antibody response, whereby after the vector is within the target cell types, the cellular immune response system triggers the removal of transduced cell types by activated T-cells. If any of our product candidates demonstrate a similar effect, we may decide or be required to perform additional preclinical studies or to halt or delay further clinical development of our product candidates.

In addition to side effects caused by the product candidate, the administration process or related procedures also can cause adverse side effects. Our APOE-associated Alzheimer’s disease product candidates are designed to be delivered via intracisternal administration. While the intracisternal method of administration has been available for some years, its use for gene therapies is new and no gene therapy is currently approved for this method of administration. Intracisternal administration may have greater risk and/or be perceived as having greater risk than more common methods of administration, such as intravenous injection. Other gene therapy product candidates in clinical development utilizing intracisternal delivery could also generate data that could adversely affect the clinical, regulatory or commercial perception of our product candidates.

If adverse events occur, either as a result of the product candidate or administration process, our clinical trials could be suspended or terminated. If we cannot demonstrate that any adverse events were not caused by the drug or administration process or related procedures, the FDA, EMA or foreign regulatory authorities could order us to cease further development of, or deny approval of, our product candidates for any or all targeted diseases. Even if we are able to demonstrate that serious adverse events are not product-related, such occurrences could affect patient recruitment or the ability of enrolled patients to complete the trial. Moreover, if we elect, or are required, to not initiate, delay, suspend or terminate any future clinical trial of any of our product candidates, the commercial prospects of such product candidates may be harmed and our ability to generate product revenues from any of these product candidates may be delayed or eliminated. Any of these occurrences may harm our ability to develop other product candidates, and may harm our business, financial condition and prospects significantly. Furthermore, negative results in our development of LX2006 or LX2020 could be interpreted as a failure to achieve proof of concept for our technology and result in the abandonment of other development programs.

In addition, gene therapy is still a relatively new approach to disease treatment and additional adverse side effects could develop. There also is the potential risk of delayed adverse events following exposure to gene therapy products due to persistent biologic activity of the genetic material or other components of products used to carry the genetic material. If our product candidates are associated with side effects in clinical trials or have characteristics that are unexpected, we may need to abandon their development or limit development to more narrow uses in which the side effects or other characteristics are less prevalent, less severe or more acceptable from a risk-benefit perspective. The FDA or an IRB may also require that we suspend, discontinue, or limit our clinical trials based on safety information, or that we conduct additional animal or human studies regarding the safety and efficacy of our product candidates which we have not planned or anticipated. Such findings could further result in regulatory authorities failing to provide marketing authorization for our product candidates or limiting the scope of the approved indication, if approved. Many product candidates that initially showed promise in early-stage testing have later been found to cause side effects that prevented further development of the product candidate.

Additionally, if one or more of our product candidates receives marketing approval, and we or others identify undesirable side effects caused by such products or the administration procedure, a number of potentially significant negative consequences could result, including:

- regulatory authorities may withdraw approvals of such product;
- regulatory authorities may require additional warnings on the labels;
- we may be required to create a medication guide outlining the risks of such side effects for distribution to patients or other requirements subject to a REMS;
- we could be sued and held liable for harm caused to patients;
- we may not be able to obtain or maintain third-party payor coverage and adequate reimbursement; and
- our reputation and physician or patient acceptance of our products may suffer.

There can be no assurance that we will resolve any issues related to any product-related adverse events to the satisfaction of the FDA or foreign regulatory agency in a timely manner or at all. Moreover, any of these events could prevent us from achieving or maintaining market acceptance of the particular product candidate, if approved, and could significantly harm our business, results of operations and prospects.

***Some of the diseases we initially seek to treat have low prevalence and it may be difficult to identify and enroll patients with these diseases. If we experience delays or difficulties in the enrollment and/or maintenance of patients in clinical trials, our receipt of necessary regulatory approvals could be delayed or prevented.***

Successful and timely completion of clinical trials will require that we enroll a sufficient number of patients. Patient enrollment, a significant factor in the timing of clinical trials, is affected by many factors, including the size and nature of the patient population and competition for patients with other trials. The rare genetic diseases which some of our product candidates are designed to target have low incidence and prevalence and may be difficult to diagnose. In particular, because we are focused on patients with specific genetic mutations, our ability to enroll eligible patients may be limited or enrollment may be slower than we anticipate. For example, we estimate that approximately 6,600 people in the United States have FA and that approximately 80% of these patients will develop the cardiac manifestation of FA, or FA cardiomyopathy, and accordingly it may be difficult for us to identify and timely recruit a sufficient number of eligible patients to conduct our clinical trials. While the patient population for LX2020, our program targeting PKP2-ACM, is significantly larger than FA, we may face challenges in identifying and recruiting eligible patients to conduct our clinical trial given competing clinical trials. Even for more prevalent conditions such as Alzheimer's disease, it may be difficult to recruit patients to clinical trials due to the number of approved products, difficulty identifying patients with the specific genotype we are studying, and the number of clinical trials being conducted in this indication.

Our trials may be subject to delays as a result of patient enrollment taking longer than anticipated or patient withdrawal. We may not be able to initiate or continue clinical trials for our product candidates if we are unable to locate and enroll a sufficient number of eligible patients to participate in these trials as required by the FDA, EMA or other foreign regulatory authorities. We cannot predict how successful we will be at enrolling subjects in future clinical trials. Subject enrollment is affected by other factors including:

- the severity of the disease under investigation;
- the eligibility criteria for the trial in question;

- the size of the patient population and process for identifying patients;
- the perceived risks and benefits of the product candidate under study;
- clinicians' and patients' perceptions as to the potential advantages of the product candidate being studied in relation to other available therapies, including any new drugs that may be approved for the diseases we are investigating;
- the availability of competing commercially available therapies and other competing therapeutic product candidates' clinical trials;
- the efforts to facilitate timely enrollment in clinical trials;
- the risk that patients enrolled in clinical trials will drop out of the clinical trials before completion of their treatment;
- the patient referral practices of physicians;
- the ability to monitor patients adequately during and after treatment; and
- the proximity and availability of clinical trial sites for prospective patients.

Our inability to enroll a sufficient number of patients for clinical trials would result in significant delays and could require us to abandon one or more clinical trials altogether. Enrollment delays in these clinical trials may result in increased development costs for our product candidates, which would cause the value of our company to decline and limit our ability to obtain additional financing. Furthermore, we rely on CROs and clinical trial sites to help ensure the proper and timely conduct of our clinical trials and we may have limited influence over their performance. For additional information, see the risk factor in this section under the heading “*We intend to continue to rely on third parties to conduct a significant portion of our existing clinical trials and potential future clinical trials for product candidates, and those third parties may not perform satisfactorily, including failing to meet deadlines for the completion of such trials.*”

Furthermore, even if we are able to enroll a sufficient number of patients for our clinical trials, we may have difficulty maintaining enrollment of such patients in our clinical trials.

***We may seek Orphan Drug designation or Rare Pediatric Disease designation for some of our product candidates and we may be unsuccessful, or may be unable to maintain the benefits associated with Orphan Drug designation, including the potential for market exclusivity, for product candidates for which we obtain Orphan Drug designation.***

Regulatory authorities in some jurisdictions, including the United States, may designate drugs or biologics intended to treat relatively small patient populations as orphan drug products. Under the Orphan Drug Act, the FDA may designate a drug or biologic as an orphan drug if it is intended to treat a rare disease or condition, which is generally defined as a patient population of fewer than 200,000 individuals in the United States, or a patient population of 200,000 or more in the United States where there is no reasonable expectation that the cost of developing the drug will be recovered from sales in the United States.

In the United States, Orphan Drug designation entitles a party to potential financial incentives such as tax advantages and user fee waivers. Opportunities for grant funding toward clinical trial costs may also be available for clinical trials of drugs or biologics for rare diseases, regardless of whether the drugs or biologics are designated for the orphan use. In addition, if a drug or biologic with an Orphan Drug designation subsequently receives the first marketing approval for the disease for which it has such designation, the product is entitled to a seven-year period of marketing exclusivity, which precludes the FDA from approving another marketing application for the same drug and disease for that time period, except in limited circumstances. If our competitors are able to obtain orphan drug exclusivity prior to us, for products that constitute the “same drug” and treat the same diseases as our product candidates, we may not be able to have competing products approved by the applicable regulatory authority for a significant period of time.

Similarly, in the European Union, the European Commission, upon the recommendation of the EMA’s Committee for Orphan Medicinal Products, grants Orphan Drug designation to promote the development of drugs that are intended for the diagnosis, prevention or treatment of life-threatening or chronically debilitating conditions and either the prevalence of the condition is not more than 5 in 10,000 persons in the European Union, or, without incentives, it is unlikely that sales of the drug in the European Union would be sufficient to justify the necessary investment in developing the drug. In each case, there must be no satisfactory method of diagnosis, prevention or treatment of the condition that has been authorized, or, if such a method exists, the product in question must be of significant benefit to those affected by such condition. In the European Union, Orphan Drug designation entitles a party to financial incentives such as reduction of fees or fee waivers.

We have obtained from the FDA Orphan Drug designation for LX2006 for the treatment of FA and for LX2020 for the treatment of PKP2-ACM. LX2006 and LX2020 have also received Orphan Medicinal Product designation from the European Commission. We may seek orphan designation for some or all of our product candidates in orphan indications in which there is a medically plausible basis for the use of these product candidates. However, we may be unsuccessful in obtaining Orphan Drug designation and may be unable to maintain the benefits associated with such designations. Even if we obtain orphan drug exclusivity for any of our product candidates, that exclusivity may not effectively protect those product candidates from competition because different drugs can be approved for the same condition, and orphan drug exclusivity does not prevent the FDA from approving the same or a different drug in another indication. Even after an orphan drug is granted orphan exclusivity and approved, the FDA can subsequently approve a later application for the same drug for the same condition before the expiration of the seven-year exclusivity period if the FDA concludes that the later drug is clinically superior in that it is shown to be safer in a substantial portion of the target populations, more effective or makes a major contribution to patient care. On August 3, 2017, Congress passed the FDA Reauthorization Act of 2017, or FDARA. FDARA, among other things, codified the FDA's preexisting regulatory interpretation, to require that a drug sponsor demonstrate the clinical superiority of an orphan drug that is otherwise the same as a previously approved drug for the same rare disease in order to receive orphan drug exclusivity. The statute supplants prior precedent holding that the Orphan Drug Act unambiguously requires that the FDA recognize the orphan exclusivity period regardless of a showing of clinical superiority. Moreover, in the Consolidated Appropriations Act of 2021, Congress clarified that the interpretation of orphan drug exclusivity codified in FDARA would apply in cases where the FDA issued an orphan designation before the enactment of FDARA but where product approval came after the enactment of FDARA. In addition, a designated Orphan Drug may not receive orphan drug exclusivity if it is approved for a use that is broader than the disease for which it received orphan designation. In *Catalyst Pharms., Inc. v. Becerra*, 14 F.4<sup>th</sup> 1299 (11th Cir. 2021), the court disagreed with the FDA's longstanding position that orphan drug exclusivity only applies to the approved use or indication within an eligible disease. However, in January 2023, the FDA published a notice in the Federal Register to clarify that while the agency complies with the court's order in *Catalyst*, the FDA intends to continue to apply its longstanding interpretation of the regulations to matters outside of the scope of the *Catalyst* order – that is, the agency will continue tying the scope of orphan-drug exclusivity to the uses or indications for which a drug is approved, which permits other sponsors to obtain approval of a drug for new uses or indications within the same orphan designated disease or condition that have not yet been approved. The Consolidated Appropriations Act of 2026, signed into law in February 2026, codified this longstanding FDA interpretation of the Orphan Drug Act. However, the overturn of the *Chevron* doctrine in *Loper Bright Enterprises v. Raimondo* may invite various stakeholders to bring lawsuits against the FDA to challenge longstanding decisions and policies, including regulatory exclusivities, which could lead to uncertainties in the industry. Further, changes in the leadership of the FDA and other federal agencies under the current administration may lead to new policies and changes in the regulations and operations of the FDA, which may impact our clinical development plans. We do not know if, when, or how the FDA, Congress, or future judicial challenges may change the orphan drug regulations and policies in the future, and it is uncertain how any changes might affect our business. Depending on what changes the FDA may make to its orphan drug regulations and policies, our business could be adversely impacted. Moreover, orphan drug-exclusive marketing rights in the United States may be lost if the FDA later determines that the request for designation was materially defective or if we are unable to manufacture sufficient quantities of the product to meet the needs of patients with the rare disease or condition. Orphan Drug designation neither shortens the development time or regulatory review time of a drug nor gives the drug any advantage in the regulatory review or approval process.

***Fast Track, Breakthrough Therapy, or Regenerative Medicine Advanced Therapy designation that we may receive from the FDA may not actually lead to a faster development or regulatory review or approval process, and does not assure FDA approval of our product candidates.***

We may seek Fast Track, Breakthrough Therapy or Regenerative Medicine Advanced Therapy, or RMAT, designation from the FDA for some or all of our product candidates, but we may be unable to obtain such designations or to maintain the benefits associated with such designations. The FDA's Fast Track, Breakthrough Therapy, and RMAT designation programs are intended to expedite the development of certain qualifying product candidates intended for the treatment of serious diseases and conditions. If a product candidate is intended for the treatment of a serious or life-threatening condition and preclinical or clinical data demonstrate the product's potential to address an unmet medical need for this condition, the sponsor may apply for FDA Fast Track designation.

A product candidate may be designated as a breakthrough therapy if it is intended, alone or in combination with one or more other drugs or biologics to treat a serious or life-threatening condition and preliminary clinical evidence indicates that the product candidate may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints. For product candidates that have been designated as breakthrough therapies, interaction and communication between the FDA and the sponsor of the trial can help to identify the most efficient path for clinical development while minimizing the number of patients placed in ineffective control regimens. Drugs and biologics designated as breakthrough therapies by the FDA may also be eligible for accelerated approval.

A product candidate may receive RMAT designation if it is a regenerative medicine therapy that is intended to treat, modify, reverse or cure a serious or life-threatening disease or condition, and preliminary clinical evidence indicates that the product candidate has the potential to address an unmet medical need for such condition. RMAT designation allows companies developing regenerative medicine therapies to work more closely and frequently with the FDA, and RMAT-designated product candidates may be eligible for priority review and accelerated approval. FDA has confirmed that gene therapies, including genetically modified cells, that lead to a sustained effect on cells or tissues may meet the definition of a regenerative medicine therapy. For product candidates that have received an RMAT designation, interaction and communication between the FDA and the sponsor of the trial can help to identify the most efficient path for clinical development while minimizing the number of patients placed in ineffective control regimens.

We have received Fast Track designation for LX2006 for the treatment of FA cardiomyopathy, and for LX2020 for the treatment of PKP2-ACM. We have also received Breakthrough Therapy designation for LX2006 for the treatment of FA cardiomyopathy as well as RMAT designation for LX2006 for the treatment of FA cardiomyopathy. LX2006 has also been selected by the FDA to participate in its CDRP program, which is intended to facilitate expedited CMC development of investigational therapies with expedited clinical development timeframes via an increased level of communication with the FDA on CMC development. While we may seek Fast Track, Breakthrough Therapy and/or RMAT designation, or participation in the CDRP program, for some or all of our product candidates, there is no guarantee that we will be successful in obtaining any such designation. Even if we do obtain such designation, we may not experience a faster development process, review or approval compared to conventional FDA procedures. A Fast Track, Breakthrough Therapy, or RMAT designation, or participation in the CDRP program, does not ensure that the product candidate will receive marketing approval or that approval will be granted within any particular time frame. In addition, the FDA may withdraw Fast Track, Breakthrough Therapy, or RMAT designation, or remove a product candidate from the CDRP program, if it believes that the designation is no longer supported by data from our clinical development program. Fast Track, Breakthrough Therapy and/or RMAT designation, or participation in the CDRP program, alone, does not guarantee qualification for the FDA's priority review procedures.

***We have received Rare Pediatric Disease designation from the FDA for LX2006 for the treatment of FA and we may seek such designation for future product candidates. However, a marketing application for these product candidates, if approved, may not meet the eligibility criteria for a rare pediatric disease priority review voucher.***

We have received Rare Pediatric Disease designation from the FDA for LX2006 for the treatment of FA and LX1004 for the treatment of CLN2 disease and we may seek Rare Pediatric Disease designation for future product candidates. The FDA defines "rare pediatric disease" as a (i) serious or life-threatening disease in which the serious or life-threatening manifestations primarily affect individuals aged from birth to 18 years, including age groups often called neonates, infants, children, and adolescents; and (ii) a rare disease or condition within the meaning of the Orphan Drug Act. Designation of a product candidate as a product for a rare pediatric disease does not guarantee that a marketing application for such product candidate will meet the eligibility criteria for a rare pediatric disease priority review voucher at the time the application is approved. Under the FDCA, we will need to request a rare pediatric disease priority review voucher in our original marketing application for our product candidates for which we have received Rare Pediatric Disease designation. The FDA may determine that a marketing application for any such product candidates, if approved, does not meet the eligibility criteria for a priority review voucher, including for the following reasons:

- the rare pediatric disease that received such designation no longer meets the definition of a "rare pediatric disease";
- the marketing application contains an active ingredient (including any ester or salt of the active ingredient) that has been previously approved in a marketing application;
- the marketing application is not deemed eligible for priority review;
- the marketing application does not rely on clinical data derived from studies examining a pediatric population and dosages of the product intended for that population (that is, if the marketing application does not contain sufficient clinical data to allow for adequate labeling for use by the full range of affected pediatric patients); or
- the marketing application is approved for a different adult indication than the rare pediatric disease for which our product candidates are designated.

The Consolidated Appropriations Act of 2026 extended the rare pediatric disease PRV program to September 30, 2029, which means the FDA may not award any PRVs under this program after September 30, 2029, unless Congress takes further action to extend the rare pediatric disease priority review program. As such, if we do not obtain approval of a marketing application for LX2006 in patients with FA on or before September 30, 2029, and if the PRV program is not extended by Congressional action, we may not receive a PRV.

***Where appropriate, we may seek approval from the FDA, EMA or comparable foreign regulatory authorities through the use of accelerated approval pathways. If we are unable to obtain such approval, we may be required to conduct additional preclinical studies or clinical trials beyond those that we contemplate, which could increase the expense of obtaining, and delay the receipt of, necessary marketing approvals. Even if we receive accelerated approval from the FDA, EMA or comparable regulatory authorities, if our confirmatory trials do not verify clinical benefit, or if we do not comply with rigorous post-marketing requirements, the FDA, EMA or such other regulatory authorities may seek to withdraw accelerated approval.***

Where possible, we may pursue accelerated development strategies in areas of high medical need. We may seek an accelerated approval pathway for one or more of our therapeutic product candidates from the FDA, EMA or comparable foreign regulatory authorities. Under the accelerated approval provisions in the FDCA and the FDA's implementing regulations, the FDA may grant accelerated approval to a therapeutic candidate that is designed to treat a serious or life-threatening condition, generally provides a meaningful therapeutic benefit over available therapies, and demonstrates an effect on a surrogate endpoint that is reasonably likely to predict clinical benefit or on a clinical endpoint that can be measured earlier than IMM that is reasonably likely to predict an effect on IMM or other clinical benefit. The FDA considers a clinical benefit to be a positive therapeutic effect that is clinically meaningful in the context of a given disease, such as IMM. For the purposes of accelerated approval, a surrogate endpoint is a marker, such as a laboratory measurement, radiographic image, physical sign, or other measure that is thought to predict clinical benefit, but is not itself a measure of clinical benefit. An intermediate clinical endpoint is a clinical endpoint that can be measured earlier than an effect on IMM that is reasonably likely to predict an effect on IMM or other clinical benefit. The accelerated approval pathway may be used in cases in which the advantage of a new product over available therapy may not be a direct therapeutic advantage, but is a clinically important improvement from a patient and public health perspective. If granted, accelerated approval is usually contingent on the sponsor's agreement to conduct, in a diligent manner, additional post-approval confirmatory studies to verify and describe the product's clinical benefit. If such post-approval studies fail to confirm the product's clinical benefit, the FDA may withdraw its approval of the product. In addition, the FDA currently requires, unless otherwise informed by the agency, pre-approval of promotional materials for products receiving accelerated approval, which could adversely impact the timing of the commercial launch of the product.

Prior to seeking accelerated approval, we would seek feedback from the FDA, EMA or comparable foreign regulatory authorities and would otherwise evaluate our ability to seek and receive such accelerated approval. However, there can be no assurance that after our evaluation of the feedback and other factors we will decide to pursue or submit a BLA for accelerated approval or any other form of expedited development, review or approval for LX2006 or any other product candidate. Similarly, there can be no assurance that after subsequent feedback from the FDA, EMA or comparable foreign regulatory authorities, we will continue to pursue or apply for accelerated approval or any other form of expedited development, review or approval, even if we initially decide to do so. Furthermore, if we decide to submit an application for accelerated approval, there can be no assurance that such application will be accepted or that any approval will be granted on a timely basis, or at all. The FDA, EMA or other comparable foreign regulatory authorities could also require us to conduct further studies prior to considering our application or granting approval of any type, including, for example, if other products are approved via the accelerated pathway and subsequently converted by FDA to full approval. A failure to obtain accelerated approval or any other form of expedited development, review or approval for our therapeutic candidate would result in a longer time period to commercialization of such therapeutic candidate, could increase the cost of development of such therapeutic candidate and could harm our competitive position in the marketplace.

***Priority review designation by the FDA may not lead to a faster regulatory review or approval process and, in any event, does not assure FDA approval of our product candidates.***

If the FDA determines that a product candidate is intended to treat a serious disease or condition and, if approved, would provide a significant improvement in the safety or effectiveness of the treatment, prevention, or diagnosis of such disease or condition, the FDA may designate the product candidate for priority review. A priority review designation means that the goal for the FDA to review a marketing application is six months from filing of the application, rather than the standard review period of ten months. We may request priority review for certain of our product candidates. The FDA has broad discretion with respect to whether or not to grant priority review status to a product candidate, so even if we believe a particular product candidate is eligible for such designation or status, the FDA may disagree and decide not to grant it. Moreover, a priority review designation does not necessarily mean a faster regulatory review process or necessarily confer any advantage with respect to approval compared to conventional FDA procedures. Receiving priority review from the FDA does not guarantee approval within the six-month review cycle or thereafter.

***We may expend our limited resources to pursue a particular product candidate and fail to capitalize on product candidates that may be more profitable or for which there is a greater likelihood of success.***

Because we have limited financial and management resources, we must focus on development programs and product candidates that we identify for specific diseases. As such, currently we are primarily focused on the development of our current pipeline of product candidates. As a result, we may forego or delay pursuit of opportunities with other product candidates. Our resource allocation decisions may cause us to fail to capitalize on viable commercial products or profitable market opportunities. Our spending on current and future development programs and product candidates for specific diseases may not yield any commercially viable products. If we do not accurately evaluate the commercial potential or target market for a particular product candidate, we may relinquish valuable rights to that product candidate through collaboration, licensing or other royalty arrangements in cases in which it would have been more advantageous for us to retain sole development and commercialization rights to such product candidate.

***We may not be successful in our efforts to build a pipeline of additional product candidates.***

Our business model is centered on developing product candidates targeting patient populations that place significant burden on society and are most amenable to our genetic medicine approach. We are targeting diseases that have seen limited penetration of precision medicine and where we believe there is significant opportunity for gene therapy to play a role as a key therapeutic option. We aim to select, develop and advance product candidates that we believe will have a high probability of technical and regulatory success through development into commercialization. We may not be able to continue to identify and develop new product candidates. Even if we are successful in continuing to build our pipeline, the potential product candidates that we identify may not be suitable for clinical development. For example, they may be shown to have side effects or other characteristics that indicate that they are unlikely to be drugs that will receive marketing approval and achieve market acceptance. If we do not successfully develop and commercialize product candidates based upon our approach, we will not be able to obtain product revenue in future periods, which likely would result in significant harm to our financial position and adversely affect our stock price.

***If we are unable to successfully validate, develop and obtain regulatory approval for companion diagnostic tests for our product candidates that require or would commercially benefit from such tests, or experience significant delays in doing so, we may not realize the full commercial potential of these product candidates.***

In connection with the clinical development of our product candidates for certain indications, we intend to work with collaborators to develop or obtain access to *in vitro* companion diagnostic tests to identify patient subsets within a disease category who may derive selective and meaningful benefit from our product candidates. To be successful, we or our collaborators will need to address a number of scientific, technical, regulatory and logistical challenges. The FDA and comparable foreign regulatory authorities regulate *in vitro* companion diagnostics as medical devices and, under that regulatory framework, will likely require the conduct of clinical trials to demonstrate the safety and effectiveness of any diagnostics we may develop, which we expect will require separate regulatory clearance or approval prior to commercialization. The FDA generally will require approval or clearance of the diagnostic at the same time that the FDA approves the therapeutic product if the FDA determines that safe and effective use of a therapeutic product depends on an *in vitro* companion diagnostic. The clearance or approval of a companion diagnostic as part of the product label will also limit the use of the product candidate to patients who have met the screening criteria tested for by the companion diagnostic.

We intend to rely on third parties for the design, development and manufacture of companion diagnostic tests for our therapeutic product candidates that may require such tests. If we enter into such collaborative agreements, we will be dependent on the sustained cooperation and effort of our future collaborators in developing and obtaining approval for these companion diagnostics. It may be necessary to resolve issues such as selectivity/specificity, analytical validation, reproducibility, or clinical validation of companion diagnostics during the development and regulatory approval processes. Moreover, even if data from preclinical studies and early clinical trials appear to support development of a companion diagnostic for a product candidate, data generated in later clinical trials may fail to support the analytical and clinical validation of the companion diagnostic. We and our future collaborators may encounter difficulties in developing, obtaining regulatory approval for, manufacturing and commercializing companion diagnostics similar to those we face with respect to our therapeutic candidates themselves, including issues with achieving regulatory clearance or approval, production of sufficient quantities at commercial scale and with appropriate quality standards, and in gaining market acceptance. If we are unable to successfully develop companion diagnostics for these product candidates, or experience delays in doing so, the development of these product candidates may be adversely affected, these product candidates may not obtain marketing approval, and we may not realize the full commercial potential of any of these product candidates that obtain marketing approval. As a result, our business, results of operations and financial condition could be materially harmed. In addition, a diagnostic company with whom we contract may decide to discontinue selling or manufacturing the companion diagnostic test that we anticipate using in connection with development and commercialization of our product candidates or our relationship with such diagnostic company may otherwise terminate. We may not be able to enter into arrangements with another diagnostic company to obtain supplies of an alternative diagnostic test for use in connection with the development and commercialization of our product candidates or do so on commercially reasonable terms, which could adversely affect and/or delay the development or commercialization of our product candidates.

## Risks related to the manufacturing of our product candidates

***We and our contract manufacturers are subject to significant regulation with respect to manufacturing our products. The third-party manufacturing facilities on which we rely, and any manufacturing facility that we may have in the future, may have limited capacity or fail to meet the applicable stringent regulatory requirements.***

We currently have relationships with a limited number of suppliers for the manufacturing of all components of our product candidates. However, if we experience slowdowns or problems with our manufacturing partners and are unable to establish or scale our internal manufacturing capabilities, we will need to continue to contract with manufacturers that can produce the preclinical, clinical and commercial supply of our products. Each supplier may require licenses to manufacture such components if such processes are not owned by the supplier or in the public domain and we may be unable to license such intellectual property rights on reasonable commercial terms or to transfer or sublicense the intellectual property rights we may have with respect to such activities.

All entities involved in the preparation of therapeutics for clinical trials or commercial sale, including our existing contract manufacturers for components of our product candidates, are subject to extensive regulation. Components of a finished therapeutic product approved for commercial sale or used in late-stage clinical trials in the United States and European Union must be manufactured in accordance with cGMP. These regulations govern manufacturing processes and procedures (including recordkeeping) and the implementation and operation of quality systems to control and assure the quality of investigational products and products approved for sale. Poor control of production processes can lead to the introduction of adventitious agents or other contaminants, or to inadvertent changes in the properties or stability of our product candidates that may not be detectable in final product testing. We or our contract manufacturers must supply all necessary documentation in support of a BLA or an MAA on a timely basis. Our potential manufacturing facilities and quality systems and the facilities and quality systems of some or all of our third-party contractors must pass a pre-approval inspection for compliance with the applicable regulations as a condition of regulatory approval of our product candidates or any of our other potential products. In addition, the regulatory authorities may, at any time, audit or inspect a manufacturing facility involved with the preparation of our product candidates or our other potential products or the associated quality systems for compliance with the regulations applicable to the activities being conducted, and they could put a hold on one or more of our clinical trials if the facilities of our contract manufacturing organizations, or CMOs, do not pass such audit or inspections. If these facilities do not pass a pre-approval plant inspection, FDA approval of the products will not be granted.

The regulatory authorities also may, at any time following approval of a product for sale, inspect or audit our manufacturing facilities or those of our third-party contractors. If any such inspection or audit identifies a failure to comply with applicable regulations or if a violation of our product specifications or applicable regulations occurs independent of such an inspection or audit, we or the relevant regulatory authority may require remedial measures that may be costly and/or time-consuming for us or a third party to implement and that may include the temporary or permanent suspension of a clinical trial or commercial sales or the temporary or permanent closure of a facility. Any such remedial measures imposed upon us or third parties with whom we contract could harm our business.

If we or any of our third-party manufacturers fail to maintain regulatory compliance, the FDA can impose regulatory sanctions including, among other things, refusal to approve a pending application for a new drug product or biologic product, or revocation of a pre-existing approval. As a result, our business, financial condition and results of operations may be harmed. Additionally, if supply from one approved manufacturer is interrupted, there could be a significant disruption in commercial supply. An alternative manufacturer would need to be qualified through a BLA and/or an MAA supplement which could result in further delay. The regulatory agencies may also require additional studies if a new manufacturer is relied upon for commercial production. Switching manufacturers may involve substantial costs and is likely to result in a delay in our desired clinical and commercial timelines.

These factors could cause the delay of clinical trials, regulatory submissions, required approvals or commercialization of our product candidates, cause us to incur higher costs and prevent us from commercializing our products successfully, if approved. Furthermore, if our suppliers fail to meet contractual requirements, and we are unable to secure one or more replacement suppliers capable of production at a substantially equivalent cost, our clinical trials may be delayed, or we could lose potential revenue.

***Gene therapies are novel, complex and difficult to manufacture. We could experience manufacturing problems that result in delays in the development or commercialization of our product candidates or otherwise harm our business.***

The manufacture of gene therapy products is technically complex and necessitates substantial expertise and capital investment. Production difficulties caused by unforeseen events may delay the availability of material for our clinical studies.

We rely on third-party manufacturers to manufacture our product candidates for preclinical studies and clinical trials. The manufacturers of pharmaceutical products must comply with strictly enforced cGMP requirements, state and federal regulations, as well as foreign requirements when applicable. Any failure by us or our CMOs to adhere to or document compliance to such regulatory requirements could lead to a delay or interruption in the availability of our product candidate materials for clinical trials or enforcement action from the FDA, EMA or foreign regulatory authorities. If we or our manufacturers fail to comply with the requirements of the FDA, EMA or other regulatory authority, sanctions could be imposed on us, including clinical holds, fines, injunctions, civil penalties, delays, suspension or withdrawal of approvals, license revocation, seizures or recalls of product candidates or products, operating restrictions and criminal prosecutions, any of which could significantly and adversely affect supplies of our product candidates.

There can be no assurances that our third-party manufacturers will be able to meet our timetable and requirements. If any third party with whom we contract fails to perform its obligations, we may be forced to either manufacture the materials ourselves, for which we may not have the capabilities or resources, or enter into an agreement with a different third-party manufacturer, which we may not be able to do on reasonable terms, if at all. In either scenario, our clinical trials and future commercial supply could be delayed significantly as we establish alternative supply sources. In some cases, the technical skills required to manufacture our product candidates may be unique or proprietary to the original third-party manufacturer and we may have difficulty, or there may be contractual restrictions prohibiting us from, transferring such skills to a back-up or alternate supplier, or we may be unable to transfer such skills at all. In addition, if we are required to change manufacturers for any reason, we will be required to verify that the new manufacturer maintains facilities and procedures that comply with quality standards and with all applicable regulations. We will also need to verify, such as through a manufacturing comparability study, that any new manufacturing process will produce our product candidates according to the specifications previously submitted to the FDA or another regulatory authority. The delays associated with the verification of a new manufacturer could negatively affect our ability to develop product candidates or commercialize our products, if approved, in a timely manner or within budget.

If we are unable to arrange for alternative third-party manufacturing sources on commercially reasonable terms or in a timely manner, we may be delayed in the development of our product candidates. Our dependence upon others for the manufacture of our product candidates may also adversely affect our future profit margins and our ability to commercialize any product candidates that receive regulatory approval on a timely and competitive basis.

Our product candidates require processing steps that are more complex than those required for most chemical pharmaceuticals. Moreover, unlike chemical pharmaceuticals, the physical and chemical properties of a biologic such as our modified virus generally cannot be fully characterized. As a result, assays of the finished product may not be sufficient to ensure that the product will perform in the intended manner. Although we believe that the manufacture of our product candidates may be simplified due to their shared raw materials and other similarities, we cannot be certain that this will be the case and we may be required to develop manufacturing methods that ultimately differ significantly between product candidates, which would require that we invest substantial time and capital to develop suitable manufacturing methods. Our program materials are manufactured using technically complex processes requiring specialized equipment and facilities, highly specific raw materials, cell types and reagents, and other production constraints. Our production process also requires a number of highly specific raw materials, cell types and reagents with limited suppliers. Even though we aim to have backup supplies of raw materials, cell types and reagents whenever possible, we cannot be certain they will be sufficient if our primary sources are unavailable. A shortage of a critical raw material, cell line, or reagent, or a technical issue during manufacturing may lead to delays in clinical development or commercialization plans. We are particularly susceptible to any shortages, delays or our inability to obtain suitable raw materials for our lead product candidates. Any changes in the manufacturing of components of the raw materials we use could result in unanticipated or unfavorable effects in our manufacturing processes, resulting in delays.

In addition, if any of our product candidates obtain approval, the FDA, EMA and other regulatory authorities may require us to submit samples of any lot of any approved product together with the protocols showing the results of applicable tests at any time. Under some circumstances, the FDA, EMA or other regulatory authorities may require that we not distribute a lot until the agency authorizes its release. Slight deviations in the manufacturing process, including those affecting quality attributes and stability, may result in unacceptable changes in the product that could result in lot failures or product recalls. Lot failures or product recalls could cause us to delay product launches or clinical trials, which could be costly to us and otherwise harm our business, financial condition, results of operations and prospects.

***We depend on third-party suppliers for materials used in the manufacture of our product candidates, and the loss of these third-party suppliers or their inability to supply us with adequate materials could harm our business.***

We rely on third-party suppliers for the materials and components required for the production of our product candidates. Our dependence on these third-party suppliers and the challenges we may face in obtaining adequate supplies of materials involve several risks, including limited control over pricing, availability, and delivery schedules. There is substantial demand and limited supply for certain of the raw materials used to manufacture gene therapy products. As a small company, our negotiation leverage is limited, and we may get lower priority than our competitors that are larger than we are. We cannot be certain that our suppliers will continue to provide us with the quantities of these raw materials within the timelines that we require or satisfy our anticipated specifications and quality requirements. Any supply interruption in limited or sole-sourced raw materials could materially harm our ability to manufacture our product candidates until a new source of supply, if any, could be identified and qualified. We may be unable to find a sufficient alternative supply channel in a reasonable time or on commercially reasonable terms. Any performance failure on the part of our suppliers could delay the development and potential commercialization of our product candidates, including limiting supplies necessary for clinical trials and regulatory approvals, which would have a material adverse effect on our business.

***Any contamination or interruption in our manufacturing process, shortages of raw materials or failure of our suppliers of viruses to deliver necessary components could result in delays in our clinical development or marketing schedules.***

Given the nature of gene therapy manufacturing, there is a risk of contamination occurring during the manufacturing process. Any contamination could adversely affect our ability to produce product candidates on schedule and could, therefore, harm our results of operations and cause reputational damage. Some of the raw materials required in our manufacturing process are derived from biologic sources. Such raw materials are difficult to procure and may be subject to contamination or recall. A material shortage, contamination, recall or restriction on the use of biologically derived substances in the manufacture of our product candidates could adversely impact or disrupt the commercial manufacturing or the production of clinical material, which could adversely affect our development timelines and our business, financial condition, results of operations and prospects.

***Changes in methods of product candidate manufacturing or formulation may result in additional costs or delay.***

As product candidates proceed through preclinical studies to late-stage clinical trials towards potential approval and commercialization, it is common that various aspects of the development program, such as manufacturing methods and formulation, are altered along the way in an effort to optimize processes and product characteristics. Such changes carry the risk that they will not achieve our intended objectives. Any such changes could cause our product candidates to perform differently and affect the results of planned clinical trials or other future clinical trials conducted with the materials manufactured using altered processes. Such changes may also require additional testing, FDA notification or FDA approval. This could delay the initiation and completion of clinical trials, require the conduct of bridging clinical trials or the repetition of one or more clinical trials, increase clinical trial costs, delay approval of our product candidates and jeopardize our ability to commence sales and generate revenue. In addition, we may be required to make significant changes to our upstream and downstream processes across our pipeline, which could delay the development of our future product candidates. Regulatory agencies, and in particular the FDA and EMA, have demonstrated increased caution in their regulation of gene therapies, including increased scrutiny related to CMC issues. This increased regulatory scrutiny around gene therapy CMC may result in us being required to conduct additional preclinical studies or clinical trials with respect to any of our product candidates, which may result in delays and increased costs in the development or commercialization of our product candidates and ultimately could lead to the failure to obtain approval for any gene therapy product.

#### **Risks related to the commercialization of our product candidates**

***Even if any of our product candidates receive marketing approval, they may fail to achieve the degree of market acceptance by physicians, patients, third-party payors and others in the medical community necessary for commercial success.***

If any of our product candidates receive marketing approval, they may nonetheless fail to gain sufficient market acceptance by physicians, patients, third-party payors and others in the medical community. If our product candidates do not achieve an adequate level of acceptance, we may not generate significant revenue and we may not become profitable. The degree of market acceptance of our product candidates, if approved for commercial sale, will depend on a number of factors, including:

- their efficacy, safety and potential advantages compared to alternative treatments;
- our ability to offer our products for sale at competitive prices;
- their convenience and ease of administration compared to alternative treatments;

- product labeling or product insert requirements of the FDA or foreign regulatory authorities, including any limitations or warnings contained in a product's approved labeling, including any boxed warning or REMS;
- the willingness of the target patient population to try new treatments, such as gene therapy as a novel modality for treatment of our target indications and of physicians to prescribe these treatments;
- our ability to hire and retain a sales force in the United States;
- the strength of marketing and distribution support;
- the availability of coverage and adequate reimbursement for our product candidates, once approved, from third-party payors and government authorities;
- the prevalence and severity of any side effects; and
- any restrictions on the use of our products together with other medications.

***Negative public opinion of gene therapy and increased regulatory scrutiny of gene therapy and genetic research may adversely impact the development or commercial success of our current and future product candidates.***

Our product candidates involve introducing genetic material into a patient's cells via intrathecal and intravenous administration. The clinical and commercial success of our potential products will depend in part on public acceptance of the use of gene therapy and gene regulation for the prevention or treatment of human diseases. Public attitudes may be influenced by claims that gene therapy and gene regulation are unsafe, unethical or immoral, and consequently, our products may not gain the acceptance of the public or the medical community. Adverse public attitudes may adversely impact our ability to enroll clinical trials. Moreover, our success will depend upon physicians prescribing, and their patients being willing to receive, treatments that involve the use of product candidates we may develop in lieu of, or in addition to, existing treatments with which they are already familiar and for which greater clinical data may be available.

More restrictive government regulations or negative public opinion would have a negative effect on our business or financial condition and may delay or impair the development and commercialization of our product candidates or demand for any products once approved. In recent years, sponsors of other clinical trials involving gene therapies have announced imposition of clinical holds by the FDA to evaluate safety issues arising during the trials. Among the risks in any gene therapy product based on viral vectors are the risks of immunogenicity, elevated liver enzymes and insertional oncogenesis. If any of our vectors demonstrate a similar effect, we may decide or be required to halt or delay further clinical development of any product candidates that utilize that vector. Adverse events in our or others' clinical trials, even if not ultimately attributable to our product candidates, and the resulting publicity could result in increased governmental regulation, unfavorable public perception, potential regulatory delays in the testing or approval of our product candidates, stricter labeling requirements for those product candidates that are approved and a decrease in demand for any such product candidates. The risk of cancer remains a concern for gene therapy, and we cannot assure that it will not occur in any of our planned or future clinical trials or in any clinical trials conducted by other companies. In addition, there is the potential risk of delayed adverse events following exposure to gene therapy products due to persistent biological activity of the genetic material or other components of products used to carry the genetic material. In addition, for our regulated gene replacement therapy product candidates which require that the expression of a therapeutic transgene be tightly regulated, we may inadvertently cause overexpression, which could lead to numerous issues, including safety and toxicity concerns. Furthermore, one of our regulatory gene replacement therapy candidates, LX1020, requires the insertion of miRNA targets into the viral genome, which is a technology that to our knowledge is not present in any approved gene therapy products. If any such adverse events occur, commercialization of our product candidates or further advancement of our clinical trials could be halted or delayed, which would have a negative impact on our business and operations.

***The affected populations for our other product candidates may be smaller than we or third parties currently project, which may affect the addressable markets for our product candidates.***

We currently focus our research and product development on several indications that are larger-rare diseases. However, our projections of the number of people who have the diseases we are seeking to treat, as well as the subset of people with these diseases who have the potential to benefit from treatment with our product candidates, are estimates based on our knowledge and understanding of these diseases. These estimates may prove to be incorrect and new studies may further reduce the estimated incidence or prevalence of these diseases. The number of patients in the United States, the European Union and elsewhere may turn out to be lower than expected, may not be otherwise amenable to treatment with our product candidate or patients may become increasingly difficult to identify and access, all of which would adversely affect our business, financial condition, results of operations and prospects.

The total addressable market opportunity for our product candidates will ultimately depend upon a number of factors, including the diagnosis and treatment criteria included in the final label, if approved for sale in specified indications, acceptance by the medical community, patient access and product pricing and reimbursement. Incidence and prevalence estimates are frequently based on information and assumptions that are not exact and may not be accurate, and the methodology is forward-looking and potentially speculative. The process we have used in developing an estimated incidence and prevalence range for the indications we are targeting has involved collating limited data from multiple sources. Accordingly, the incidence and prevalence estimates included in this Annual Report should be viewed in that context. Further, the data and statistical information used in this Annual Report, including estimates derived from them, may differ from information and estimates made by our competitors or from current or future studies conducted by independent sources.

***We face significant competition from other biotechnology and pharmaceutical companies, and our operating results will suffer if we fail to compete effectively.***

Drug development, particularly in the gene therapy field, is highly competitive and subject to rapid and significant technological advancements. As a significant unmet medical need exists in the cardiovascular disease area, there are several large and small pharmaceutical companies focused on delivering therapeutics for the treatment of these diseases, including those that we are initially targeting. It is likely that additional drugs will become available in the future for the treatment of our target diseases.

We are aware that our competitors are developing product candidates for the treatment of diseases that our product candidates will target. With respect to LX2006, we are aware of clinical stage gene therapy programs in development at Solid Biosciences Inc. and those being developed in collaborations between Voyager Therapeutics, Inc. and Neurocrine Biosciences, Inc. Among other treatment modalities for FA, we are aware that Larimar Therapeutics, Inc. is developing a clinical stage product candidate, CTI-1601, and that PTC Therapeutics, Inc. is evaluating next steps for vatiquinone following a Complete Response Letter on their new drug application to the FDA. Reata Pharmaceuticals, Inc.'s omaveloxolone (Skyclarys) was approved by the FDA in 2023, and Biogen Inc. acquired Reata Pharmaceuticals, Inc. for approximately \$7.3 billion in the same year and is currently commercializing Skyclarys.

With respect to LX2020, both Rocket Pharmaceuticals Inc., or Rocket, and Tenaya Therapeutics Inc. are developing an AAV-based gene therapy candidate designed to deliver a functional *PKP2* gene to patients with PKP2-ACM.

Many of our existing or potential competitors have substantially greater financial, technical and human resources than we do and significantly greater experience in the discovery and development of product candidates, as well as in obtaining regulatory approvals of those product candidates in the United States and in foreign countries. Our current and potential future competitors may also have significantly more experience commercializing drugs, particularly gene therapy and other biologics, that have been approved for marketing. Mergers and acquisitions in the pharmaceutical and biotechnology industries could result in even more resources being concentrated among a small number of our competitors.

We will face competition from other drugs or from other non-drug products currently approved or that will be approved in the future in the cardiac and neurology fields, including for the treatment of diseases and diseases in the therapeutic categories we intend to target. Therefore, our ability to compete successfully will depend largely on our ability to:

- develop and commercialize drugs that are advantageous as compared to other products in the market;
- demonstrate through our clinical trials that our product candidates are differentiated from existing and future therapies;
- attract qualified scientific, product development and commercial personnel;
- obtain patent or other proprietary protection for our product candidates;

- obtain required regulatory approvals;
- obtain coverage and adequate reimbursement from, and negotiate competitive pricing with, third-party payors; and
- successfully collaborate with other pharmaceutical companies in the discovery, development and commercialization of new medicines.

The availability of our competitors' products could limit the demand, and the price we are able to charge, for any product candidate we develop. The inability to compete with existing or subsequently introduced drugs would have an adverse impact on our business, financial condition and prospects. In addition, the reimbursement structure of approved gene therapies by other companies could impact the anticipated reimbursement structure of our gene therapies, if approved, and our business, financial condition, results of operations and prospects.

Established pharmaceutical companies may invest heavily to accelerate discovery and development of novel compounds or to in-license novel compounds that could make our product candidates less competitive. In addition, any new product that competes with an approved product must demonstrate compelling advantages in efficacy, convenience, tolerability and safety in order to overcome price competition and to be commercially successful. Accordingly, our competitors may succeed in obtaining patent protection, discovering, developing, receiving regulatory and marketing approval for, or commercializing, drugs before we do, which would have an adverse impact on our business and results of operations.

***Any product candidates for which we intend to seek approval as biologic products may face competition sooner than anticipated.***

The Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act, includes a subtitle called the Biologics Price Competition and Innovation Act of 2009, or BPCIA, which created an abbreviated approval pathway for biologics that are biosimilar to or interchangeable with an FDA-licensed reference biological product. Under the BPCIA, an application for a biosimilar product may not be submitted to the FDA until four years following the date that the reference product was first licensed by the FDA. In addition, the approval of a biosimilar product may not be made effective by the FDA until 12 years from the date on which the reference product was first licensed. During this 12-year period of exclusivity, another company may still market a competing version of the reference product if the FDA approves a full BLA for the competing product containing the sponsor's own preclinical data and data from adequate and well-controlled clinical trials to demonstrate the safety, purity and potency of its product. The law is complex and is still being interpreted and implemented by the FDA. As a result, its ultimate impact, implementation and meaning are subject to uncertainty. While it is uncertain when such processes intended to implement BPCIA may be fully adopted by the FDA, any such processes could have an adverse effect on the future commercial prospects for our biologics.

There is a risk that any of our product candidates approved as a biological product under a BLA would not qualify for the 12-year period of exclusivity or that this exclusivity could be shortened due to congressional action or otherwise, or that the FDA will not consider our product candidates to be reference products for competing products, potentially creating the opportunity for generic competition sooner than anticipated. Other aspects of the BPCIA, some of which may impact the BPCIA exclusivity provisions, have also been the subject of recent litigation. Moreover, the extent to which a biosimilar, once approved, will be substituted for any one of our reference products in a way that is similar to traditional generic substitution for non-biologics is not yet clear, and will depend on a number of marketplace and regulatory factors that are still developing. If competitors are able to obtain marketing approval for biosimilars referencing our product candidates, if approved, our products may become subject to competition from such biosimilars, with the attendant competitive pressure and potential adverse consequences.

***The success of our product candidates will depend significantly on coverage and adequate reimbursement or the willingness of patients, commercial and government payors to pay for these procedures.***

We believe our success depends on obtaining and maintaining coverage and adequate reimbursement for our product candidates, including LX2006 and LX2020, and the extent to which patients will be willing to pay out-of-pocket for such products, in the absence of reimbursement for all or part of the cost. In the United States and in other countries, patients who are provided medical treatment for their conditions generally rely on third-party payors to reimburse all or part of the costs associated with their treatment. The availability of coverage and adequacy of reimbursement for our products by third-party payors, including government health care programs (e.g., Medicare, Medicaid, TRICARE), managed care providers, private health insurers, health maintenance organizations and other organizations is essential for most patients to be able to afford medical services and pharmaceutical products such as our product candidates. Third-party payors often rely upon Medicare coverage policy and payment limitations in setting their own coverage and reimbursement policies. However, decisions regarding the extent of coverage and amount of reimbursement to be provided are made on a payor-by-payor basis. One payor's determination to provide coverage for a drug product does not assure that other payors will also provide coverage, and adequate reimbursement.

The principal decisions about reimbursement for new medicines are typically made by CMS, an agency within the HHS. CMS decides whether and to what extent products will be covered and reimbursed under Medicare and private payors tend to follow CMS to a substantial degree. Most significantly, in August 2022, the Inflation Reduction Act of 2022, or IRA, was signed into law. Among other things, the IRA requires manufacturers of certain drugs to engage in price negotiations with Medicare, with prices that can be negotiated subject to a cap (with resulting prices for the initial ten drugs first effective in 2026); imposes rebates under Medicare Part B and Medicare Part D to penalize price increases that outpace inflation (first due in 2023); redesigns the Medicare Part D benefit (beginning in 2024); and replaces the Part D coverage gap discount program with a new discounting program (beginning in 2025). The IRA permits the Secretary of the HHS to implement many of these provisions through guidance, as opposed to regulation, for the initial years. On August 29, 2023, HHS announced the list of the first ten drugs that will be subject to price negotiations. HHS has issued and will continue to issue and update guidance implementing the IRA, although the Medicare drug price negotiation program is currently subject to legal challenges. While the impact of the IRA on the pharmaceutical industry cannot yet be fully determined, it is likely to be significant. Only high-expenditure single-source drugs that have been approved for at least 7 years (11 years for single-source biologics) can qualify for negotiation, with the negotiated price taking effect two years after the selection year. For 2026, the first year in which negotiated prices become effective, CMS selected 10 high-cost Medicare Part D drugs in 2023, negotiations began in 2024, and the negotiated maximum fair price for each drug has been announced. CMS has selected 15 additional Medicare Part D drugs for negotiated maximum fair pricing in 2027. For 2028, up to an additional 15 drugs, which may be covered under either Medicare Part B or Part D, will be selected, and for 2029 and subsequent years, up to 20 additional Part B or Part D drugs will be selected. Further, the current administration has issued executive orders focused on decreasing prescription drug prices, including directing the Secretary of HHS to establish a mechanism through which American patients can buy drugs directly from manufacturers who sell at a most-favored-nation price and directing the U.S. Trade Representative and Secretary of Commerce to take action to ensure foreign countries are not engaged in practices that purposefully and unfairly undercut market prices and drive price hikes in the United States. Government agreements with pharmaceutical companies and other government measures that use most-favored-nation pricing targets for prescription drugs, including the use of international pricing reference to set drug prices in the United States, or that increase generic and biosimilar drug entry sooner than expected, can have a material adverse effect on our industry, ability to set adequate pricing for new drugs to recover research and development costs, ability to attract potential investors and potential buyers in the future. We cannot predict the full impact of the executive orders focused on reducing prescription drug prices or increasing domestic drug manufacturing capacity, or other measures that may be implemented by the current administration related to drug pricing, drug supply chain and manufacturing in the United States. Such cost containment policies and executive orders could substantially and negatively impact the prices we may charge for any approved products, which could harm our valuation, ability to generate revenue and achieve and sustain profitability. In addition, the One Big Beautiful Bill Act, or OBBBA, which was signed into law in July 2025, includes provisions that will impact the United States healthcare system in various ways, including by cuts to Medicaid and introducing new participant work and eligibility requirements for Medicaid coverage, which are expected to significantly change the administration and applicability of Medicaid coverage. Although the full impact of the OBBBA on the healthcare system and our business is uncertain, the resulting changes may increase the cost and complexity of completing clinical development of and launching any product candidates for which we may receive regulatory approval, which could adversely affect our business and prospects.

Third-party payors determine which products and procedures they will cover and establish reimbursement levels. Even if a third-party payor covers a particular product or procedure, the resulting reimbursement payment rates may not be adequate. Patients who are treated in-office for a medical condition generally rely on third-party payors to reimburse all or part of the costs associated with the procedure, including costs associated with products used during the procedure, and may be unwilling to undergo such procedures in the absence of such coverage and adequate reimbursement. Physicians may be unlikely to offer procedures for such treatment if they are not covered by insurance and may be unlikely to purchase and use our product candidates, if approved, for our stated diseases unless coverage is provided and reimbursement is adequate. In addition, for products administered under the supervision of a physician, obtaining coverage and adequate reimbursement may be particularly difficult because of the higher prices often associated with such drugs. Further, coverage policies and third-party reimbursement rates may change at any time. Therefore, even if favorable coverage and reimbursement status is attained, less favorable coverage policies and reimbursement rates may be implemented in the future. In addition, companion diagnostic tests require coverage and reimbursement separate and apart from the coverage and reimbursement for their companion pharmaceutical or biological products. Similar challenges to obtaining coverage and reimbursement, applicable to pharmaceutical or biological products, will apply to companion diagnostics.

Reimbursement by a third-party payor may depend upon a number of factors, including the third-party payor's determination that a procedure is a covered benefit under its health plan; safe, effective and medically necessary; appropriate for the specific patient; cost-effective; supported by peer-reviewed medical journals; included in clinical practice guidelines; and neither cosmetic, experimental, nor investigational. Further, increasing efforts by third-party payors in the United States and abroad to cap or reduce healthcare costs may cause such organizations to limit both coverage and the level of reimbursement for newly approved products and, as a result, they may not cover or provide adequate payment for our product candidates. In order to secure coverage and reimbursement for any product that might be approved for sale, we may need to conduct expensive pharmacoeconomic studies in order to demonstrate the medical necessity and cost-effectiveness of our products, in addition to the costs required to obtain FDA or comparable regulatory approvals. Additionally, we may also need to provide discounts to purchasers, private health plans or government healthcare programs. Our product candidates may nonetheless not be considered medically necessary or cost-effective. If third-party payors do not consider a product to be cost-effective compared to other available therapies, they may not cover the product after approval as a benefit under their plans or, if they do, the level of payment may not be sufficient to allow a company to sell its products at a profit. We expect to experience pricing pressures from third-party payors in connection with the potential sale of any of our product candidates.

Foreign governments also have their own healthcare reimbursement systems, which vary significantly by country and region, and we cannot be sure that coverage and adequate reimbursement will be made available with respect to the treatments in which our products are used under any foreign reimbursement system.

There can be no assurance that LX2006, LX2020 or any other product candidates, if approved for sale in the United States or in other countries, will be considered medically reasonable and necessary, that it will be considered cost-effective by third-party payors, that coverage or an adequate level of reimbursement will be available or that reimbursement policies and practices in the United States and in foreign countries where our products are sold will not adversely affect our ability to sell our product candidates profitably, if they are approved for sale.

***Product liability lawsuits against us could cause us to incur substantial liabilities and to limit commercialization of any products that we may develop.***

We face an inherent risk of product liability exposure related to the testing of our product candidates in human clinical trials and will face an even greater risk if we commercially sell any products that we may develop. If we cannot successfully defend ourselves against claims that our product candidates or drugs caused injuries, we will incur substantial liabilities. Regardless of merit or eventual outcome, liability claims may result in:

- decreased demand for any product candidates or drugs that we may develop;
- injury to our reputation and significant negative media attention;
- withdrawal of clinical trial participants;
- significant costs to defend the related litigation;
- substantial monetary awards paid to trial participants or patients;
- loss of revenue;
- reduced resources of our management to pursue our business strategy; and
- the inability to commercialize any products that we may develop.

Although we maintain product liability insurance coverage, such insurance may not be adequate to cover all liabilities that we may incur. We may need to increase our insurance coverage as we expand our clinical trials or if we commence commercialization of our product candidates. Insurance coverage is increasingly expensive. We may not be able to maintain insurance coverage at a reasonable cost or in an amount adequate to satisfy any liability that may arise.

## Risks related to our dependence on third parties

***Currently, we rely on our collaborations with Cornell University and The Regents of UCSD to conduct research and development for many of our pipeline programs, including conducting preclinical and IND-enabling studies for portions of our near-term future pipeline. Failure or delay of Cornell University or The Regents of UCSD to fulfill all or part of their respective obligations to us under our agreements, a breakdown in collaboration between the parties or a complete or partial loss of either of these relationships could materially harm our business.***

Our collaboration with Cornell University is critical to our business and in May 2020, we entered into two separate license agreements with Cornell University pursuant to which we obtained rights under certain patents, know-how and data to exploit products and technologies covered by such intellectual property. As part of our first license agreement, as amended, we assumed oversight for the conduct of the Phase 1/2 clinical trial of LX1001 that was initiated by Cornell University at the end of 2019. As part of our second license agreement with Cornell University, as amended, we obtained certain rights to portfolios for infantile neuronal ceroid lipofuscinosis type 2 (also called CLN2 Batten disease) and FA cardiomyopathy, as well as an assignment of Cornell University's IND to support the development of our LX1004 program. We entered into a third license agreement with Cornell University, or Third Cornell License Agreement, in April 2024 pursuant to which we obtained certain rights for FA cardiomyopathy, including rights to current and future clinical data from an ongoing Cornell University investigator-initiated Phase 1A trial of a gene therapy candidate AAVrh10.hFXN, known as LX2006 at Lexeo. Pursuant to our license agreements with Cornell University, we are obligated to diligently proceed with the development, manufacture, and sale of licensed products. If Cornell University delays or fails to perform its obligations under the license agreements, terminates any of the license agreements in accordance with its terms, or a dispute otherwise arises between the parties concerning the terms of the agreements or our respective rights to licensed technology, our pipeline of product candidates would be significantly adversely affected and our prospects may be materially harmed.

Our collaboration with UCSD is also highly important to our business, as we have licensed from UCSD intellectual property rights related to our LX2020, LX2021 and LX2022 programs under three separate license agreements, and we have entered into sponsored research agreements with UCSD for preclinical research and development for these programs. If The Regents of UCSD delays or fails to perform its obligations under either of the sponsored research agreements or any of the license agreements, disagrees with our interpretation of the terms of the sponsored research agreements, license agreements or our discovery plans, or terminates any of our existing license agreements, our pipeline of product candidates would be significantly adversely affected and our prospects may be materially harmed.

***We intend to continue to rely on third parties to conduct a significant portion of our existing clinical trials and potential future clinical trials for product candidates, and those third parties may not perform satisfactorily, including failing to meet deadlines for the completion of such trials.***

We engage CROs to help conduct our ongoing clinical trials. We expect to continue to rely on third parties, including clinical data management organizations, medical institutions and clinical investigators, to conduct those clinical trials and any future clinical trials. Any of these third parties may terminate their engagements with us, some in the event of an uncured material breach and some at any time for convenience. If any of our relationships with these third parties terminate, we may not be able to timely enter into arrangements with alternative third parties or to do so on commercially reasonable terms, if at all. Switching or adding a CRO involves substantial cost and requires management's time and focus. In addition, there is a natural transition period when a new CRO commences work. As a result, delays occur, which can materially impact our ability to meet our desired clinical development timelines. Though we intend to carefully manage our relationships with our CROs, there can be no assurance that we will not encounter challenges or delays in the future or that these delays or challenges will not have a material adverse impact on our business, financial condition and prospects.

In addition, any third parties conducting our clinical trials will not be our employees, and except for remedies available to us under our agreements with such third parties, we cannot control whether or not they devote sufficient time and resources to our clinical programs. If these third parties do not successfully carry out their contractual duties or obligations or meet expected deadlines, if they need to be replaced or if the quality or accuracy of the clinical data they obtain is compromised due to the failure to adhere to our clinical protocols, regulatory requirements or for other reasons, our clinical trials may be extended, delayed or terminated and we may not be able to obtain regulatory approval for or successfully commercialize our product candidates. Consequently, our results of operations and the commercial prospects for our product candidates would be harmed, our costs could increase substantially and our ability to generate revenue could be delayed significantly.

We rely on these parties for execution of our preclinical studies and clinical trials, and generally do not control their activities. Our reliance on these third parties for research and development activities will reduce our control over these activities but will not relieve us of our responsibilities. For example, we will remain responsible for ensuring that each of our clinical trials is conducted in accordance with the general investigational plan and protocols for the trial. Moreover, the FDA requires us to comply with cGCP regulations, for conducting, recording and reporting the results of clinical trials to assure that data and reported results are credible and accurate and that the rights, integrity and confidentiality of trial participants are protected. We also are required to register ongoing clinical trials and post the results of completed clinical trials on a government-sponsored database, [www.clinicaltrials.gov](http://www.clinicaltrials.gov), within specified time frames. Failure to do so can result in fines, adverse publicity and civil and criminal sanctions. If we or any of our CROs or other third parties, including trial sites, fail to comply with applicable cGCPs, or experience material protocol deviations, the clinical data generated in our clinical trials may be deemed unreliable and the FDA, EMA or comparable foreign regulatory authorities may require us to perform additional clinical trials before approving our marketing applications. If the FDA or any regulatory authority determines that our clinical data are not reliable for any reason, or if we encounter any data integrity issues, the FDA or other regulatory authorities may require us to exclude such data, which may cause the trial to be underpowered and fail to meet the trial endpoints. We cannot assure you that upon inspection by a given regulatory authority, such regulatory authority will determine that any of our clinical trials complies with cGCP regulations. In addition, our clinical trials must be conducted with product produced under cGMP conditions. Our failure to comply with these regulations may require us to repeat clinical trials, which would delay the regulatory approval process.

In addition, principal investigators for our clinical trials may serve as scientific advisors or consultants to us from time to time and receive compensation in connection with such services. Under certain circumstances, we may be required to report some of these relationships to the FDA. The FDA may conclude that a financial relationship between us and a principal investigator has created a conflict of interest or otherwise affected interpretation of the trial. The FDA may therefore question the integrity of the data generated at the applicable clinical trial site and the utility of the clinical trial itself may be jeopardized. This could result in a delay in approval, or rejection, of our marketing applications by the FDA and may ultimately lead to the denial of marketing approval of LX2006, LX2020 or any other product candidates.

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

***We may seek collaborations with non-academic third parties for the development or commercialization of our product candidates. If those collaborations are not successful, we may not be able to capitalize on the market potential of these product candidates.***

We may seek third-party collaborators for the development and commercialization of our product candidates, including for the commercialization of any of our product candidates that are approved for marketing outside the United States. Our likely collaborators for any such arrangements include regional and national pharmaceutical companies and biotechnology companies. If we enter into any additional such arrangements with any third parties, we will likely have limited control over the amount and timing of resources that our collaborators dedicate to the development or commercialization of our product candidates. Our ability to generate revenue from these arrangements will depend on our collaborators' abilities to successfully perform the functions assigned to them in these arrangements. Collaborations involving our product candidates pose the following risks to us:

- collaborators have significant discretion in determining the efforts and resources that they will apply to these collaborations;
- collaborators may not perform their obligations as expected;
- collaborators may not pursue development and commercialization of any product candidates that achieve regulatory approval or may elect not to continue or renew development or commercialization programs based on clinical trial results, changes in the collaborators' strategic focus or available funding, or external factors, such as an acquisition, that divert resources or create competing priorities;
- collaborators may delay clinical trials, provide insufficient funding for a clinical trial program, stop a clinical trial or abandon a product candidate, repeat or conduct new clinical trials or require a new formulation of a product candidate for clinical testing;
- collaborators could independently develop, or develop with third parties, products that compete directly or indirectly with our product candidates if the collaborators believe that competitive products are more likely to be successfully developed or can be commercialized under terms that are more economically attractive than ours;
- we could grant exclusive rights to our collaborators that would prevent us from collaborating with others;

- product candidates discovered in collaboration with us may be viewed by our collaborators as competitive with their own product candidates or drugs, which may cause collaborators to cease to devote resources to the commercialization of our product candidates;
- a collaborator with marketing and distribution rights to one or more of our product candidates that achieve regulatory approval may not commit sufficient resources to the marketing and distribution of such products;
- disagreements with collaborators, including disagreements over proprietary rights, contract interpretation or the preferred course of development, might cause delays or termination of the research, development or commercialization of product candidates, might lead to additional responsibilities for us with respect to product candidates, or might result in litigation or arbitration, any of which would be time-consuming and expensive;
- collaborators may not properly maintain or defend our or their intellectual property rights or may use our or their proprietary information in such a way as to invite litigation that could jeopardize or invalidate such intellectual property or proprietary information or expose us to potential litigation;
- collaborators may infringe the intellectual property rights of third parties, which may expose us to litigation and potential liability; and
- collaborations may be terminated for the convenience of the collaborator and, if terminated, we could be required to raise additional capital to pursue further development or commercialization of the applicable product candidates.

Collaboration agreements may not lead to development or commercialization of product candidates in the most efficient manner or at all. If any future collaborator of ours were to be involved in a business combination, the continued pursuit and emphasis on our product development or commercialization program could be delayed, diminished or terminated.

We face significant competition in seeking appropriate collaborators. Whether we reach a definitive agreement for any collaboration will depend, among other things, upon our assessment of the collaborator's resources and expertise, the terms and conditions of the proposed collaboration and the proposed collaborator's evaluation of a number of factors. Those factors may include the design or results of clinical trials, the likelihood of approval by the FDA or similar regulatory authorities outside the United States, the potential market for the subject product candidate, the costs and complexities of manufacturing and delivering such product candidate to patients, the potential of competing products, the existence of uncertainty with respect to our ownership of technology, which can exist if there is a challenge to such ownership without regard to the merits of the challenge and industry and market conditions generally. The collaborator may also consider alternative product candidates or technologies for similar diseases that may be available to collaborate on and whether such a collaboration could be more attractive than the one with us for our product candidate. Collaborations are complex and time-consuming to negotiate and document. In addition, there have been a significant number of recent business combinations among large pharmaceutical companies that have resulted in a reduced number of potential future collaborators.

We may not be able to negotiate additional collaborations on a timely basis, on acceptable terms, or at all. If we are unable to do so, we may have to curtail the development of such product candidate, reduce or delay its development program or one or more of our other development programs, delay its potential commercialization or reduce the scope of any sales or marketing activities, or increase our expenditures and undertake development or commercialization activities at our own expense. If we elect to increase our expenditures to fund development or commercialization activities on our own, we may need to obtain additional capital, which may not be available to us on acceptable terms or at all. If we do not have sufficient funds, we may not be able to further develop our product candidates or bring them to market and generate revenue.

#### **Risks related to intellectual property**

***If we are unable to obtain or protect intellectual property rights related to any of our product candidates, we may not be able to compete effectively in our market.***

We rely upon a combination of patents, trade secret protection and confidentiality agreements to protect the intellectual property related to our product candidates, including LX2006, LX2020, LX2021, LX2022, LX1001, LX1020, LX1021, and other programs, their respective components, formulations, therapies, methods used to manufacture them and methods of treatment.

Our success depends in large part on our ability to obtain and maintain patent and other intellectual property protection in the United States and in other countries with respect to our proprietary technology and product candidates.

We cannot offer any assurances about which of our patent applications will issue, the breadth of any resulting patent or whether any of the issued patents will be found invalid and unenforceable or will be threatened by third parties. We cannot offer any assurances that the breadth of our granted patents will be sufficient to stop a competitor from developing and commercializing a product, including a biosimilar product that would be competitive with one or more of our product candidates. Furthermore, any successful challenge to these patents or any other patents owned by or licensed to us after patent issuance could deprive us of rights necessary for the successful commercialization of any of our product candidates. Further, if we encounter delays in regulatory approvals, the period of time during which we could market a product candidate under patent protection could be reduced.

The patent prosecution process is expensive and time-consuming. We may not be able to prepare, file and prosecute all necessary or desirable patent applications at a commercially reasonable cost or in a timely manner or in all jurisdictions. It is also possible that we may fail to identify patentable aspects of inventions made in the course of development and commercialization activities before it is too late to obtain patent protection on them. We may not be able to obtain or maintain patent applications and patents due to the subject matter claimed in such patent applications and patents being in the public domain. In some cases, the work of certain academic researchers in the gene therapy field has entered the public domain, which may preclude our ability to obtain patent protection for certain inventions relating to such work. Although we enter into nondisclosure and confidentiality agreements with parties who have access to confidential or patentable aspects of our research and development output, such as our employees, corporate collaborators, outside scientific collaborators, contract manufacturers, consultants, advisors and other third parties, any of these parties may breach these agreements and disclose such output before a patent application is filed, thereby jeopardizing our ability to seek patent protection. Consequently, we would not be able to prevent any third party from using any technology that is in the public domain to compete with our product candidates. In addition, as the licensee of patents from Cornell University and UCSD, we have less control over the prosecution and enforcement of those patents than we would if we owned the patents. While we do have typical rights with respect to those patents under university license agreements, our ability to enforce those patents to maintain exclusivity in our markets may be limited by the terms of our agreements with Cornell University and UCSD. In addition, to the extent the federal government provided research funding to the licensor university for any technology licensed under the license agreements, then if the federal government elects to exercise any of its overriding rights which may apply as a result of provision of such funding, we may be subject to changes in market exclusivity or other material business requirements or constraints. Moreover, depending on the terms of any future in-licenses to which we may become a party, we may not have the right to control the preparation, filing and prosecution of patent applications, or to maintain the patents, covering technology in-licensed from third parties. Therefore, these patents and patent applications may not be prosecuted and enforced in a manner consistent with the best interests of our business.

The patent position of biotechnology and pharmaceutical companies generally is highly uncertain, involves complex legal and factual questions and has, in recent years, been the subject of much litigation. As a result, the issuance, scope, validity, enforceability and commercial value of any patent rights are highly uncertain. Our owned and licensed pending and future patent applications may not result in issued patents which protect our technology or product candidates, effectively prevent others from commercializing competitive technologies and product candidates or otherwise provide any competitive advantage. In fact, patent applications may not issue as patents at all. Even if patent applications we license or own currently or in the future issue as patents, they may not issue in a form that will provide us with any meaningful protection, prevent competitors or other third parties from competing with us, or otherwise provide us with any competitive advantage. Any patents that we hold or in-license may be challenged, narrowed, circumvented, or invalidated by third parties. Consequently, we do not know whether any of our technologies and product candidates will be protectable or remain protected by valid and enforceable patents. In addition, our existing patents and any future patents we obtain may not be sufficiently broad to prevent others from using our technology or from developing competing technologies and products, and the coverage claimed in a patent application can be significantly reduced before the patent is issued, and its scope can be reinterpreted after issuance. Any failure to obtain, maintain or defend our patents and other intellectual property could have a material adverse effect on our business, financial condition, results of operations and prospects.

We cannot be certain that we are the first to invent the inventions covered by pending patent applications and, if we are not, we may be subject to priority or entitlement disputes. We may be required to disclaim part or all of the term of certain patents or all of the term of certain patent applications. There may be prior art of which we are not aware that may affect the validity or enforceability of a patent claim. There also may be prior art of which we are aware, but which we do not believe affects the validity or enforceability of a claim, which may, nonetheless, ultimately be found to affect the validity or enforceability of a claim. Since patent applications in the United States and other countries are confidential for a period of time after filing, at any moment in time, we cannot be certain that we were in the past or will be in the future the first to file any patent application related to our product candidates. For example, some patent applications in the United States may be maintained in secrecy until the patents are issued. Further, publications in the scientific literature often lag behind actual discoveries. Consequently, we cannot be certain that others have not filed patent applications for technology covered by our owned and in-licensed issued patents or our pending applications, or that we or, if applicable, a licensor, were the first to invent or first to file an application for the technology.

It is possible that defects of form in the preparation or filing of our patents or patent applications may exist, or may arise in the future, for example, with respect to proper priority claims, inventorship, claim scope, or requests for patent term adjustments. If there are material defects in the form, preparation, prosecution, or enforcement of our patents or patent applications, such patents may be invalid and/or unenforceable, and such applications may never result in valid, enforceable patents. Any of these outcomes could impair our ability to prevent competition from third parties, which may have an adverse impact on our business.

In addition to the protection provided by our patent estate, we rely on trade secret protection and confidentiality agreements to protect proprietary know-how that is not amenable to patent protection. Although we generally require all of our employees to assign their inventions to us, and all of our employees, consultants, advisors and any third parties who have access to our proprietary know-how, information, or technology to enter into confidentiality agreements, we cannot provide any assurances that all such agreements have been duly executed, or that our trade secrets and other confidential proprietary information will not be disclosed. In addition, while we have undertaken reasonable efforts to ensure such agreements are enforceable and that employees and third parties comply with their obligations thereunder, these agreements may be found insufficient by a court of law or may be breached, or we may not enter into sufficient agreements with such individuals in the first instance, in either case potentially resulting in the unauthorized use or disclosure of our trade secrets and other intellectual property, including to our competitors, which could cause us to lose any competitive advantage resulting from this intellectual property. Individuals not subject to invention assignment agreements may make adverse ownership claims to our current and future intellectual property. Moreover, our competitors may independently develop knowledge, methods and know-how equivalent to our trade secrets. Competitors could purchase our products, if approved, and replicate some or all of the competitive advantages we derive from our development efforts for technologies on which we do not have patent protection. If any of our trade secrets were to be lawfully obtained or independently developed by a competitor, we would have no right to prevent them, or those to whom they communicate it, from using that technology or information to compete with us. If any of our trade secrets were to be disclosed to or independently developed by a competitor, our competitive position would be harmed.

Enforcing a claim that a third-party entity illegally obtained and is using any of our trade secrets is expensive and time-consuming, and the outcome is unpredictable, and we may not be able to obtain adequate remedies for such breaches.

We also seek to preserve the integrity and confidentiality of our data and trade secrets by working to maintain the physical security of our premises and physical and electronic security of our information technology systems. Our confidentiality agreements may, however, be breached, and we may not have adequate remedies for any breach. Our and other applicable security measures may also not be able to prevent security breaches and other compromises of the security of our trade secrets or other data. Also, if the steps taken to maintain our trade secrets are deemed inadequate, we may have insufficient recourse against third parties for misappropriating the trade secret. In addition, others may independently discover our trade secrets and proprietary information. For example, the FDA is considering whether to make additional information publicly available on a routine basis, including information that we may consider to be trade secrets or other proprietary information, and it is not clear at the present time how the FDA's disclosure policies may change in the future. If we are unable to prevent material disclosure of the non-patented intellectual property related to our technologies to third parties, and there is no guarantee that we will have any such enforceable trade secret protection, we may not be able to establish or maintain a competitive advantage in our market, which could materially adversely affect our business, results of operations and financial condition.

***Patent terms may be inadequate to protect our competitive position on our products for an adequate amount of time, and if we do not obtain protection under the Hatch-Waxman Amendments and similar non-United States legislation for extending the term of patents covering each of our product candidates, our business may be materially harmed.***

Given the amount of time required for the development, testing and regulatory review of new product candidates, patents protecting such candidates might expire before or shortly after such product candidates are commercialized. Depending upon the timing, duration and conditions of FDA marketing approval of our product candidates, one or more of our United States patents may be eligible for limited patent term extension under the Drug Price Competition and Patent Term Restoration Act of 1984, referred to as the Hatch-Waxman Amendments, and similar legislation in the European Union. The Hatch-Waxman Amendments permit a patent term extension of up to five years for a patent covering an approved product as compensation for effective patent term lost during product development and the FDA regulatory review process. A patent term extension cannot extend the remaining term of a patent beyond a total of 14 years from the date of product approval. Only one patent may be extended, and only those claims covering the approved drug, a method for using it, or a method for manufacturing it may be extended. However, we may not receive an extension if we fail to apply within applicable deadlines, fail to apply prior to expiration of relevant patents or otherwise fail to satisfy applicable requirements. Moreover, the length of the extension could be less than we request. If we are unable to obtain patent term extension or the term of any such extension is less than we request, the period during which we can enforce our patent rights for that product will be shortened and our competitors may obtain approval to market competing products sooner. As a result, our revenue from applicable products could be reduced and could have a material adverse effect on our business.

***We in-license key intellectual property necessary for the development of each of our current product candidates. If we fail to comply with our obligations in our current and future intellectual property licenses with third parties, resulting in the termination of such licenses, we could lose rights that are important to our business.***

We are heavily reliant upon licenses to certain patent rights and proprietary technology for the development of each of our current product candidates. In particular, we in-license key patents and patent applications from Adverum related to LX2006, we in-license patent applications and know-how from Cornell University related to our LX2006, LX1001, LX1020 and LX1021 product candidates, and we in-license patent applications and know-how from UCSD related to our LX2020, LX2021 and LX2022 product candidates. Our license agreements impose diligence and milestone and royalty payment obligations on us, and also contain certain development requirements. If we fail to comply with our obligations, our licensors may have the right to terminate our licenses, in which event we will not be able to develop, manufacture or market any product using the intellectual property under any such terminated agreement and may face other penalties. Such an occurrence would materially adversely affect our business prospects.

Certain of our licenses may not provide us with exclusive rights to use the licensed intellectual property and technology, or may not provide us with exclusive rights to use such intellectual property and technology in all relevant fields of use and in all territories in which we may wish to develop or commercialize our technology and product candidates in the future. In addition, the intellectual property rights licensed to us by our licensors, including certain intellectual property licensed by Cornell University UCSD, and Adverum, at least in some respects, may be used by such licensors or licensed to third parties, and such third parties may have certain enforcement rights with respect to such intellectual property. Thus, patents licensed to us could be put at risk of being invalidated or interpreted narrowly in litigation filed by or against our licensors or another licensee or in administrative proceedings brought by or against our licensors or another licensee in response to such litigation or for other reasons. As a result, we may not be able to prevent competitors or other third parties from developing and commercializing competitive products, including in territories covered by our licenses.

Licenses to additional third-party technology and materials that may be required for our development programs may not be available in the future or may not be available on commercially reasonable terms, or at all, which could have a material adverse effect on our business and financial condition. In such events, we may be required to expend significant time and resources to redesign our technology, product candidates, or the methods for manufacturing them or to develop or license replacement technology, all of which may not be feasible on a technical or commercial basis. If we are unable to do so, we may be unable to develop or commercialize the affected technology or product candidates. Even if we are able to obtain such additional licenses, they may be non-exclusive thereby giving our competitors and other third parties access to the same technology licensed to us.

If we or our licensors fail to adequately protect our licensed intellectual property, our ability to commercialize our product candidates and technology could suffer. Although we have oversight rights, Cornell University and UCSD generally control the prosecution, maintenance and enforcement of our in-licensed patents and patent applications. Therefore, we cannot be certain that the prosecution, maintenance and enforcement of these patent rights will be in a manner consistent with the best interests of our business, or in compliance with applicable laws and regulations, or will result in valid and enforceable patents and other intellectual property rights. It is possible that our licensors' infringement proceedings or defense activities may be less vigorous than had we conducted them ourselves or may not be conducted in accordance with our best interests. If we or our licensors fail to maintain such patents or patent applications, or if we or our licensor lose rights to those patents or patent applications, the rights we have licensed may be reduced or eliminated and our right to develop and commercialize any of our product candidates that are the subject of such licensed rights could be adversely affected. In addition to the foregoing, the risks associated with patent rights that we license from third parties will also apply to patent rights we may own in the future.

Further, if we fail to comply with our development obligations under our license agreements, we may lose our patent rights with respect to such agreement on a territory-by-territory basis, which would affect our patent rights worldwide. In spite of our efforts, our current and future licensors might conclude that we have materially breached our obligations under our license agreements and might therefore terminate such license agreements, thereby removing or limiting our ability to develop and commercialize products and technology covered by these license agreements. Disputes may also arise between us and our licensors regarding intellectual property subject to a license agreement, including:

- the scope of rights granted under the license agreement and other interpretation-related issues;
- our financial and other obligations under the license agreement;
- whether and the extent to which our technology and processes infringe on intellectual property of the licensor that is not subject to the licensing agreement;
- our right to sublicense patent and other rights to third parties under collaborative development relationships;

- our diligence obligations with respect to the use of the licensed technology in relation to our development and commercialization of our product candidates and what activities satisfy those diligence obligations;
- the inventorship or ownership of inventions and know-how resulting from the joint creation or use of intellectual property by our licensors and us and our partners; and
- the priority of invention of patented technology.

If disputes over intellectual property that we have licensed prevent or impair our ability to maintain our current licensing arrangements on acceptable terms, we may be unable to successfully develop and commercialize the affected technology or product candidates. In addition, if any such disputes result in the termination of our intellectual property licenses, this could result in the loss of our ability to develop and commercialize our lead product candidates, or we could lose other significant rights, experience significant delays in the development and commercialization of our other product candidates, or incur liability for damages, any of which could have a material adverse effect on our business, financial condition, results of operations and prospects. In addition, we may seek to obtain additional licenses from our licensors and, in connection with obtaining such licenses, we may agree to amend our existing licenses in a manner that may be more favorable to the licensors, including by agreeing to terms that could enable third parties, including our competitors, to receive licenses to a portion of the intellectual property that is subject to our existing licenses and to compete with our product candidates.

Some of our future agreements with certain of our third-party research partners may provide that improvements developed in the course of our relationship may be owned solely by either us or our third-party research partner. If we determine that rights to such improvements owned solely by a third-party research partner or other third party with whom we collaborate are necessary to commercialize our therapeutic product candidates or maintain our competitive advantage, we may need to obtain a license from such third party in order to use the improvements and continue developing, manufacturing or marketing our drug candidates. We may not be able to obtain such a license on an exclusive basis, on commercially reasonable terms, or at all, which could prevent us from commercializing our product candidates or allow our competitors or others the chance to access technology that is important to our business.

Termination of our current or any future license agreements would reduce or eliminate our rights under these agreements and may result in our having to negotiate new or reinstated agreements with less favorable terms or cause us to lose our rights under these agreements, including our rights to important intellectual property or technology. Any of the foregoing could prevent us from commercializing our other product candidates, which could have a material adverse effect on our operating results and overall financial condition.

In addition, intellectual property rights that we in-license in the future may be sublicenses under intellectual property owned by third parties, in some cases through multiple tiers. The actions of our licensors may therefore affect our rights to use our sublicensed intellectual property, even if we are in compliance with all of the obligations under our license agreements. Should our licensors or any of the upstream licensors fail to comply with their obligations under the agreements pursuant to which they obtain the rights that are sublicensed to us, or should such agreements be terminated or amended, our ability to develop and commercialize our product candidates may be materially harmed.

In addition, a third party may in the future bring claims that our performance under our license agreements, including our sponsoring of clinical trials, interferes with such third party's rights under its agreement with one of our licensors. If any such claim were successful, it may adversely affect our rights and ability to advance our product candidates as clinical candidates or subject us to liability for monetary damages, any of which would have an adverse effect on our business, financial condition, results of operations and prospects.

***We are generally also subject to all of the same risks with respect to protection of intellectual property that we license as we are for intellectual property that we own, which are described above and below. If we or our licensors fail to adequately protect this intellectual property, our ability to commercialize products could suffer. We may not be successful in obtaining or maintaining necessary rights to product components and processes for our development pipeline through acquisitions and in-licenses.***

Presently, we have obtained rights to certain intellectual property rights through licenses from third parties to develop, manufacture and commercialize our lead product candidates and other potential product candidates in our pipeline. Because the commercialization of our product candidates may require the use of additional intellectual property rights held by third parties, the growth of our business likely will depend, in part, on our ability to acquire or license these intellectual property rights. Our product candidates also require specific formulations and manufacturing processes to work effectively and efficiently, and some of these rights are held by others.

We may be unable to acquire or in-license any compositions, methods of use, processes or other third-party intellectual property rights from third parties that we identify as necessary, important or more expedient to further our business operations. In addition, even if we are able to obtain such licenses, we may fail to obtain any of these licenses at a reasonable cost or on reasonable terms, which would harm our business. Were that to happen, we may need to cease use of the product candidates and technologies covered by those third-party intellectual property rights and may need to seek to develop alternative approaches that do not infringe, misappropriate or violate those intellectual property rights, which may entail additional costs and development delays if we are able to develop such alternatives, or which may not be feasible. Even if we are able to obtain a license, it may be non-exclusive, which means that our competitors may also receive access to the same technologies licensed to us. The licensing and acquisition of third-party intellectual property rights is a competitive practice, and companies that may be more established, or have greater resources than we do, may also be pursuing strategies to license or acquire third-party intellectual property rights that we may consider necessary or attractive in order to commercialize our product candidates. More established companies may have a competitive advantage over us due to their larger size and cash resources or greater clinical development and commercialization capabilities. In addition, companies that perceive us to be a competitor may be unwilling to assign or license rights to us. There can be no assurance that we will be able to successfully complete such negotiations and ultimately acquire the rights to the intellectual property surrounding the additional product candidates that we may seek to acquire. We also may be unable to license or acquire third-party intellectual property rights on terms that would allow us to make an appropriate return on our investment.

If we are unable to successfully obtain rights to required third-party intellectual property or maintain the existing intellectual property rights we have licensed, we may be required to expend significant time and resources to redesign our product candidates, or to develop or license replacement technology, all of which may not be feasible on a technical or commercial basis, and we may have to abandon development of our product candidates, any of which could have a material adverse effect on our business, financial condition, results of operations and prospects.

***Our intellectual property licenses with third parties may be subject to disagreements over contract interpretation, which could narrow the scope of our rights to the relevant intellectual property or technology or increase our financial or other obligations to our licensors.***

We currently depend, and will continue to depend, on our license agreements, including the license agreements with Cornell University and Adverum related to LX2006, with Cornell University related to our LX1001, LX1020 and LX1021 product candidates, and with UCSD related to LX2020, LX2021 and LX2022 product candidates. The agreements under which we currently license intellectual property or technology from third parties are complex, and certain provisions in such agreements may be susceptible to multiple interpretations. The resolution of any contract interpretation disagreement that may arise could narrow what we believe to be the scope of our rights to the relevant intellectual property or technology, or increase what we believe to be our financial or other obligations under the relevant agreement, either of which could have a material adverse effect on our business, financial condition, results of operations and prospects.

If any of our licenses or material relationships or any in-licenses upon which our licenses are based are terminated or breached, we may:

- lose our rights to develop and market our products;
- lose patent protection for our products;
- experience significant delays in the development or commercialization of our products;
- not be able to obtain any other licenses on acceptable terms, if at all; or
- incur liability for damages.

These risks apply to any agreements that we may enter into in the future for our products or for any future product candidates. If we experience any of the foregoing, it could have a material adverse effect on our business, financial condition, results of operations and prospects.

We cannot be certain that any of our or licensed pending patent applications or our future owned or licensed patent applications will result in issued patent claims covering such aspects of our product candidates.

Composition-of-matter patents on the active pharmaceutical ingredient, or API, in prescription drug products are generally considered to be the strongest form of intellectual property protection for drug products because those types of patents provide protection without regard to any particular method of use or manufacture or formulation of the API used. Although we intend to file patent applications in the future that cover these product candidates, we cannot be certain that our future owned or licensed patent applications will cover our current or future product candidates.

Method-of-use patents protect the use of a product for the specified method and formulation patents cover formulations of the API. These types of patents do not prevent a competitor or other third party from developing or marketing an identical product for an indication that is outside the scope of the patented method or from developing a different formulation that is outside the scope of the patented formulation. Moreover, with respect to method-of-use patents, even if competitors or other third parties do not actively promote their product for our targeted indications or uses for which we may obtain patents, physicians may recommend that patients use these products off-label, or patients may do so themselves. Although off-label use may infringe or contribute to the infringement of method-of-use patents, the practice is common, and this type of infringement is difficult to prevent or prosecute. In addition, there are numerous publications and other prior art that may be relevant to our owned or in-licensed method-of-use patents and patent applications and may be used to challenge the validity of these owned or in-licensed patents and patent applications in litigation or other intellectual property-related proceedings. If these types of challenges are successful, our owned or in-licensed patents and patent applications may be narrowed or found to be invalid, and we may lose valuable intellectual property rights. Any of the foregoing could have a material adverse effect on our business, financial condition, prospects and results of operations.

The strength of patents in the biotechnology and pharmaceutical field involves complex legal and scientific questions and can be uncertain. The patent applications that we own or in-license may fail to result in issued patents with claims that cover our product candidates or uses thereof in the United States or in other countries. Even if patents do successfully issue, the issuance of a patent is not conclusive as to its inventorship, scope, validity or enforceability and third parties may challenge the validity, enforceability or scope of our owned and licensed patents in courts or patent offices in the United States and abroad, which may result in those patents being narrowed, invalidated or held unenforceable. Furthermore, even if they are unchallenged, our owned and licensed patents and pending patent applications, if issued, may not adequately protect our intellectual property or prevent competitors or others from designing around our patent claims to circumvent our owned or licensed patents by developing similar or alternative technologies or therapeutics in a non-infringing manner. If the breadth or strength of protection provided by the patents and patent applications we own or license with respect to our product candidates is not sufficient to impede such competition or is otherwise threatened, it could dissuade companies from collaborating with us to develop, and threaten our ability to commercialize, our product candidates. Any of the foregoing could have a material adverse effect on our business, financial condition, results of operations and prospects.

***We may be involved in lawsuits to protect or enforce our patents, which could be expensive, time-consuming and unsuccessful.***

Competitors may infringe the patents for which we have applied. To counter infringement or unauthorized use, we may be required to file infringement claims, which can be expensive and time-consuming. If we initiate legal proceedings against a third party to enforce a patent covering one of our product candidates, the defendant could counterclaim that the patent covering our product or product candidate is invalid and/or unenforceable. In patent litigation in the United States, counterclaims alleging invalidity and/or unenforceability are common, and there are numerous grounds upon which a third party can assert invalidity or unenforceability of a patent. In an infringement proceeding, a court may decide that the patent claims we are asserting are invalid and/or unenforceable, or may refuse to stop the other party from using the technology at issue on the grounds that our patent claims do not cover the technology in question. Third parties may also raise similar claims before administrative bodies in the United States or abroad, even outside the context of litigation. Such mechanisms include re-examination, post-grant review, inter partes review and equivalent proceedings in foreign jurisdictions (for example, opposition proceedings). Such proceedings could result in revocation of or amendment to our patents in such a way that they no longer cover our product candidates. The outcome following legal assertions of invalidity and unenforceability is unpredictable. With respect to the validity question, for example, we cannot be certain that there is no invalidating prior art, of which we, our patent counsel and the patent examiner were unaware during prosecution. If a defendant were to prevail on a legal assertion of invalidity and/or unenforceability, we would lose at least part, and perhaps all, of the patent protection on our product candidates. An adverse result in any litigation or defense proceedings could put one or more of our patents at risk of being invalidated or interpreted narrowly, could put our patent applications at risk of not issuing and could have a material adverse impact on our business.

Interference proceedings provoked by third parties or brought by us may be necessary to determine the priority of inventions with respect to our patent applications. An unfavorable outcome could require us to cease using the related technology or force us to take a license under the patent rights of the prevailing party, if available. Furthermore, our business could be harmed if the prevailing party does not offer us a license on commercially reasonable terms. Our defense of litigation or interference proceedings may fail and, even if successful, may result in substantial costs and distract our management and other employees. We may not be able to prevent misappropriation of our intellectual property rights, particularly in countries where the laws may not protect those rights as fully as in the United States.

Even if we establish infringement of any of our patents by a competitive product, a court may decide not to grant an injunction against further infringing activity, thus allowing the competitive product to continue to be marketed by the competitor. It is difficult to obtain an injunction in U.S. litigation and a court could decide that the competitor should instead pay us a “reasonable royalty” as determined by the court, and/or other monetary damages. A reasonable royalty or other monetary damages may or may not be an adequate remedy. Loss of exclusivity and/or competition from a related product would have a material adverse impact on our business.

For certain of our in-licensed patent rights, such as patent rights in-licensed from Cornell University and Adverum, we may not have the right to file a lawsuit for infringement and may have to rely on a licensor to enforce these rights for us. If we are not able to directly assert our licensed patent rights against infringers or if a licensor does not vigorously prosecute any infringement claims on our behalf, we may have difficulty competing in certain markets where such potential infringers conduct their business, and our commercialization efforts may suffer as a result.

In addition, we or our licensors, as the case may be, may not be able to detect infringement against our owned or in-licensed patents, which may be especially difficult for manufacturing processes or formulation patents. Even if we or our licensors detect infringement by a third party of our owned or in-licensed patents, we or our licensors, as the case may be, may choose not to pursue litigation against or settlement with the third party. If we or our licensors later sue such third party for patent infringement, the third party may have certain legal defenses available to it that otherwise would not be available but for the delay between when the infringement was first detected and when the suit was brought. These legal defenses may make it impossible for us or our licensors to enforce our owned or in-licensed patents, as the case may be, against that third party.

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

***Third parties may initiate legal proceedings alleging that we are infringing their intellectual property rights, the outcome of which would be uncertain.***

As our current and future product candidates progress toward commercialization, the possibility of a patent infringement claim against us increases. We cannot provide any assurance that our current and future product candidates do not infringe other parties’ patents or other proprietary rights, and competitors or other parties may assert that we infringe their proprietary rights in any event. We may become party to, or threatened with, adversarial proceedings or litigation regarding intellectual property rights with respect to our current and future product candidates, including interference or derivation proceedings before the USPTO, or oppositions and other proceedings in foreign jurisdictions. We may be exposed to, or threatened with, future litigation by third parties having patent or other intellectual property rights alleging that our product candidates, manufacturing methods, formulations, administration methods and/or proprietary technologies infringe, misappropriate or otherwise violate their intellectual property rights.

Numerous issued patents and pending patent applications that are owned by third parties exist in the fields in which we are developing our product candidates. As the biotechnology and pharmaceutical industries expand and more patents are issued, the risk increases that our product candidates may give rise to claims of infringement of the patent rights of others. Moreover, it is not always clear to industry participants, including us, the claim scope that may issue from pending patent applications owned by third parties or which patents cover various types of drugs, products or their methods of use or manufacture. Thus, because of the large number of patents issued and patent applications filed in our fields, there may be a risk that third parties, including our competitors, may allege they have patent rights encompassing our product candidates, technologies or methods and that we are employing their proprietary technology without authorization.

If we were sued for patent infringement, we would need to demonstrate that the relevant product or methods of using the product either do not infringe the patent claims of the relevant patent or that the patent claims are invalid or unenforceable, and we may not be able to do this. In order to successfully challenge the validity of any such United States patent in federal court, we would need to overcome a presumption of validity. As this burden is high and requires us to present clear and convincing evidence as to the invalidity of any such United States patent claim, there is no assurance that a court of competent jurisdiction would agree with us and invalidate the claims of any such United States patent. Moreover, given the vast number of patents in our field of technology, we cannot be certain that we do not infringe existing patents or that we will not infringe patents that may be granted in the future.

While we may decide to initiate proceedings to challenge the validity of these or other patents in the future, we may be unsuccessful, and courts or patent offices in the United States and abroad could uphold the validity of any such patent. Furthermore, because patent applications can take many years to issue and may be confidential for 18 months or more after filing, and because pending patent claims can be revised before issuance, there may be applications now pending which may later result in issued patents that may be infringed by the manufacture, use or sale of our product candidates. Regardless of when filed, we may fail to identify relevant third-party patents or patent applications, or we may incorrectly conclude that a third-party patent is invalid or not infringed by our product candidates or activities. If a patent holder believes that one of our product candidates infringes its patent, the patent holder may sue us even if we have received patent protection for our technology. Moreover, we may face patent infringement claims from non-practicing entities that have no relevant drug revenue and against whom our own patent portfolio may thus have no deterrent effect. If a patent infringement suit were threatened or brought against us, we could be forced to stop or delay research, development, manufacturing or sales of the drug or product candidate that is the subject of the actual or threatened suit.

If any third-party patents are held by a court of competent jurisdiction to be valid and enforceable and to cover any of our technology or product candidates, including the manufacturing process of our product candidates, constructs or molecules used in or formed during the manufacturing process, or any final product itself, we could be forced, including by court order, to cease developing, manufacturing or commercializing the infringing product. Alternatively, we may be required to obtain a license from such third party in order to use the infringing technology and continue developing, manufacturing or marketing the infringing product. If we were required to obtain a license to continue to manufacture or market the affected product, we may be required to pay substantial royalties or grant cross-licenses to our patents. We cannot, however, assure that any such license will be available on acceptable terms, if at all. Ultimately, we could be prevented from commercializing a product, or be forced to cease some aspect of our business operations as a result of claims of patent infringement or violation of other intellectual property rights. Further, the outcome of intellectual property litigation is subject to uncertainties that cannot be adequately quantified in advance, including the demeanor and credibility of witnesses and the identity of any adverse party. This is especially true in intellectual property cases that may turn on the testimony of experts as to technical facts upon which experts may reasonably disagree. Furthermore, we may not be able to obtain any required license on commercially reasonable terms or at all. Even if we were able to obtain a license, it could be non-exclusive, thereby giving our competitors access to the same technologies licensed to us; alternatively, or additionally, it could include terms that impede or destroy our ability to compete successfully in the commercial marketplace. In addition, we could be found liable for significant monetary damages, including treble damages and attorneys' fees if we are found to have willfully infringed a patent. A finding of infringement could prevent us from commercializing a product or force us to cease some of our business operations, which could harm our business. Claims that we have misappropriated the confidential information or trade secrets of third parties could have a similar negative impact on our business. Furthermore, because of the substantial amount of discovery required in connection with intellectual property litigation or administrative proceedings, there is a risk that some of our confidential information could be compromised by disclosure. In addition, any uncertainties resulting from the initiation and continuation of any litigation could have material adverse effect on our ability to raise additional funds or otherwise have a material adverse effect on our business, results of operations, financial condition and prospects.

The cost to us in defending or initiating any litigation or other proceeding relating to patent or other proprietary rights, even if resolved in our favor, could be substantial, and litigation would divert our management's attention. Some of our competitors may be able to sustain the costs of complex patent litigation more effectively than we can because they have substantially greater resources. Uncertainties resulting from the initiation and continuation of patent litigation or other proceedings could delay our research and development efforts and limit our ability to continue our operations.

***We may become subject to claims that we and our employees, consultants, or independent contractors have wrongfully used or disclosed confidential information or trade secrets of third parties.***

We employ individuals who were previously employed at other biotechnology or biopharmaceutical companies. Although we require, evidenced by written agreements, that all of our employees, consultants and advisors not use the proprietary information or know-how of others in their work for us, we may be subject to claims that we or our employees, consultants, or independent contractors have inadvertently or otherwise used or disclosed confidential information of our employees' former employers or other third parties. We may also be subject to claims that former employers or other third parties have an ownership interest in our future patents. Litigation may be necessary to defend against these claims, and there is no guarantee of success in defending these claims.

Even if we are successful in defending against these types of claims, litigation or other legal proceedings relating to intellectual property claims may cause us to incur significant expenses and could distract our technical and management personnel from their normal responsibilities. In addition, there could be public announcements of the results of hearings, motions or other interim proceedings or developments, and, if securities analysts or investors perceive these results to be negative, that perception could have a substantial adverse effect on the price of our common stock. This type of litigation or proceeding could substantially increase our operating losses and reduce our resources available for development activities. Some of our competitors may be able to sustain the costs of this type of litigation or proceedings more effectively than we can because of their substantially greater financial resources. Uncertainties resulting from the initiation and continuation of intellectual property litigation or other intellectual property related proceedings could adversely affect our ability to compete in the marketplace.

For additional information regarding our legal proceedings, see Note 11 to the financial statements included in Part II, Item 8 of this Annual Report on Form 10-K.

***We may be subject to claims challenging the inventorship or ownership of our future patents and other intellectual property.***

We may also be subject to claims that former employees, collaborators, or other third parties have an ownership interest in our patent applications, our future patents, or other intellectual property. We may be subject to ownership disputes in the future arising, for example, from conflicting obligations of consultants or others who are involved in developing our product candidates and platform discovery. Although it is our policy to require our employees and contractors who may be involved in the conception or development of intellectual property to execute agreements assigning such intellectual property to us, we may be unsuccessful in executing such an agreement with each party who, in fact, conceives or develops intellectual property that we regard as our own, and we cannot be certain that our agreements with such parties will be upheld in the face of a potential challenge, or that they will not be breached, for which we may not have an adequate remedy. The assignment of intellectual property rights may not be self-executing, or the assignment agreements may be breached, and litigation may be necessary to defend against these and other claims challenging inventorship or ownership. If we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights, such as exclusive ownership of, or right to use, valuable intellectual property. Such an outcome could have a material adverse effect on our business. Even if we are successful in defending against such claims, litigation could result in substantial costs and be a distraction to management and other employees.

***Reliance on third parties requires us to share our trade secrets, which increases the possibility that a competitor will discover them or that our trade secrets will be misappropriated or disclosed.***

If we rely on third parties to manufacture or commercialize our product candidates, or if we collaborate with additional third parties for the development of such product candidates, we must, at times, share trade secrets with them. We may also conduct joint research and development programs that may require us to share trade secrets under the terms of our research and development partnerships or similar agreements. We seek to protect our proprietary technology in part by entering into confidentiality agreements and, if applicable, material transfer agreements, consulting agreements or other similar agreements with our advisors, employees, third-party contractors and consultants prior to beginning research or disclosing proprietary information. These agreements typically limit the rights of the third parties to use or disclose our confidential information, including our trade secrets. Despite the contractual provisions employed when working with third parties, the need to share trade secrets and other confidential information increases the risk that such trade secrets become known by our competitors, are inadvertently incorporated into the technology of others, or are disclosed or used in violation of these agreements. Given that our proprietary position is based, in part, on our know-how and trade secrets, a competitor's discovery of our trade secrets or other unauthorized use or disclosure could have an adverse effect on our business and results of operations.

In addition, these agreements typically restrict the ability of our advisors, employees, third-party contractors and consultants to publish data potentially relating to our trade secrets. Despite our efforts to protect our trade secrets, we may not be able to prevent the unauthorized disclosure or use of our technical know-how or other trade secrets by the parties to these agreements. Moreover, we cannot guarantee that we have entered into such agreements with each party that may have or have had access to our confidential information or proprietary technology and processes. Monitoring unauthorized uses and disclosures is difficult, and we do not know whether the steps we have taken to protect our proprietary technologies will be effective. If any of the collaborators, scientific advisors, employees, contractors and consultants who are parties to these agreements breaches or violates the terms of any of these agreements, we may not have adequate remedies for any such breach or violation, and we could lose our trade secrets as a result. Moreover, if confidential information that is licensed or disclosed to us by our partners, collaborators, or others is inadvertently disclosed or subject to a breach or violation, we may be exposed to liability to the owner of that confidential information. Enforcing a claim that a third party illegally obtained and is using our trade secrets, like patent litigation, is expensive and time consuming, and the outcome is unpredictable. In addition, courts outside the United States are sometimes less willing to protect trade secrets.

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

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

- others may be able to make or use capsids, nucleic acids and vectors that are similar to the biological compositions of our products that are the same as or similar to our product candidates but that are not covered by the claims of owned or in-licensed patents;
- others may independently develop similar or alternative technologies or duplicate any of our technologies;
- it is possible that others may circumvent our owned or in-licensed patents;
- others, including inventors or developers of our owned or in-licensed patented technologies who may become involved with competitors, may independently develop similar technologies that function as alternatives or replacements for any of our technologies without infringing our intellectual property rights;
- it is possible that our owned or in-licensed patents or patent applications omit individual(s) who should be listed as inventor(s) or include individual(s) who should not be listed as inventor(s), which may cause these patents or patents issuing from these patent applications to be held invalid or unenforceable;
- we or our licensors or our other collaboration partners might not have been the first to conceive and reduce to practice the inventions covered by the patents or patent applications that we own, license or will own or license;
- we or our licensors or our other collaboration partners might not have been the first to file patent applications covering certain of the patents or patent applications that we or they own or have obtained a license to, or will own or will have obtained a license;
- we or our licensors may fail to meet obligations to the U.S. government with respect to in-licensed patents and patent applications funded by U.S. government grants, leading to the loss of patent rights;
- it is possible that our pending patent applications will not result in issued patents;
- we may not be able to generate sufficient data to support full patent applications that protect the entire breadth of developments in one or more of our programs;
- no patent protection may be available with regard to formulation or method of use;
- the claims of our owned or in-licensed issued patents or patent applications, if and when issued, may not cover our product candidates;
- it is possible that there are prior public disclosures that could invalidate our or our licensors' patents;
- it is possible that there are unpublished applications or patent applications maintained in secrecy that may later issue with claims covering our products or technology similar to ours;
- issued patents that we own or exclusively license may not provide us with any competitive advantage, or may be held invalid or unenforceable, as a result of legal challenges by our competitors;
- we may not exclusively license our patents and, therefore, may not have a competitive advantage if such patents are licensed to others;
- our competitors might conduct research and development activities in countries where we do not have patent rights, or in countries where research and development safe harbor laws exist, and then use the information learned from such activities to develop competitive products for sale in our major commercial markets;

- the laws of other countries may not protect our or our licensors', as the case may be, proprietary rights to the same extent as the laws of the United States;
- there may be significant pressure on the U.S. government and international governmental bodies to limit the scope of patent protection both inside and outside the United States for disease treatments that prove successful, as a matter of public policy regarding worldwide health concerns;
- countries other than the United States may, under certain circumstances, force us or our licensors to grant a license under our patents to a competitor, thus allowing the competitor to compete with us in that jurisdiction or forcing us to lower the price of our drug in that jurisdiction;
- we have engaged in scientific collaborations in the past and will continue to do so in the future and our collaborators may develop adjacent or competing products that are outside the scope of our patents;
- we may not successfully commercialize the product candidates, if approved, before our relevant patents expire;
- we may not develop additional proprietary technologies for which we can obtain patent protection;
- it is possible that product candidates or technologies we develop may be covered by third parties' patents or other exclusive rights;
- ownership, validity or enforceability of our or our licensors' patents or patent applications may be challenged by third parties; and
- the patents of third parties or pending or future applications of third parties, if issued, may have an adverse effect on our business.

***We may have limited geographical protection with respect to certain patents and we may not be able to protect our intellectual property rights throughout the world.***

Filing and prosecuting patent applications and defending patents covering our product candidates in all countries throughout the world would be prohibitively expensive. Competitors may use our technologies in jurisdictions where we have not obtained patent protection to develop their own products and, further, may export otherwise infringing products to territories where we have patent protection, but enforcement rights are not as strong as those in the United States or Europe. These products may compete with our product candidates, and our future patents or other intellectual property rights may not be effective or sufficient to prevent them from competing.

In addition, we may decide to abandon national and regional patent applications before they are granted. The examination of each national or regional patent application is an independent proceeding. As a result, patent applications in the same family may issue as patents in some jurisdictions, such as in the United States, but may issue as patents with claims of different scope or may even be refused in other jurisdictions. It is also quite common that depending on the country, the scope of patent protection may vary for the same product candidate or technology.

While we intend to protect our intellectual property rights in our expected significant markets, we cannot ensure that we will be able to initiate or maintain similar efforts in all jurisdictions in which we may wish to market our product candidates. Accordingly, our efforts to protect our intellectual property rights in such countries may be inadequate, which may have an adverse effect on our ability to successfully commercialize our product candidates in all of our expected significant foreign markets. If we encounter difficulties in protecting, or are otherwise precluded from effectively protecting, the intellectual property rights important for our business in such jurisdictions, the value of these rights may be diminished, and we may face additional competition from others in those jurisdictions.

The laws of some jurisdictions do not protect intellectual property rights to the same extent as the laws or rules and regulations in the United States and Europe and many companies have encountered significant difficulties in protecting and defending such rights in such jurisdictions. The legal systems of certain countries, particularly certain developing countries, do not favor the enforcement of patents, trade secrets and other intellectual property rights, which could make it difficult for us to stop the infringement of our future patents or marketing of competing products in violation of our proprietary rights generally. Proceedings to enforce our patent rights in other jurisdictions, whether or not successful, could result in substantial costs and divert our efforts and attention from other aspects of our business, could put our future patents at risk of being invalidated or interpreted narrowly and our patent applications at risk of not issuing as patents, and could provoke third parties to assert claims against us. We may not prevail in any lawsuits that we initiate, and the damages or other remedies awarded, if any, may not be commercially meaningful. Accordingly, our efforts to enforce our intellectual property rights around the world may be inadequate to obtain a significant commercial advantage from the intellectual property that we develop or license.

Some countries also have compulsory licensing laws under which a patent owner may be compelled to grant licenses to third parties. In addition, some countries limit the enforceability of patents against government agencies or government contractors. In those countries, the patent owner may have limited remedies, which could materially diminish the value of such patents. If we are forced to grant a license to third parties with respect to any patents relevant to our business, our competitive position may be impaired.

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

Periodic maintenance fees, renewal fees, annuity fees and various other government fees on patents and/or applications will be due to be paid to the USPTO and various government patent agencies outside of the United States over the lifetime of our patents and/or applications and any patent rights we may obtain in the future. Furthermore, the USPTO and various non-United States government patent agencies require compliance with several procedural, documentary, fee payment and other similar provisions during the patent application process. In many cases, an inadvertent lapse of a patent or patent application can be cured by payment of a late fee or by other means in accordance with the applicable rules. There are situations, however, in which non-compliance can result in abandonment or lapse of the patents or patent applications, resulting in partial or complete loss of patent rights in the relevant jurisdiction. In such an event, potential competitors might be able to enter the market, which could have a material adverse effect on our business.

***Changes in patent laws or patent jurisprudence could diminish the value of patents in general, thereby impairing our ability to protect our product candidates.***

As is the case with other biotechnology companies, our success is heavily dependent on intellectual property, particularly patents. Obtaining and enforcing patents in the biotechnology and genetic medicine industries involve both technological and legal complexity. Therefore, obtaining and enforcing biotechnology and genetic medicine patents is costly, time-consuming and inherently uncertain. In addition, the Leahy-Smith America Invents Act, or the AIA, which was passed in September 2011, resulted in significant changes to the U.S. patent system.

An important change introduced by the AIA is that, as of March 16, 2013, the United States transitioned from a “first-to-invent” to a “first-to-file” system for deciding which party should be granted a patent when two or more patent applications are filed by different parties claiming the same invention. Under a “first-to-file” system, assuming the other requirements for patentability are met, the first inventor to file a patent application generally will be entitled to a patent on the invention regardless of whether another inventor had made the invention earlier. A third party that files a patent application in the USPTO after that date but before us could therefore be awarded a patent covering an invention of ours even if we made the invention before it was made by the third party. This will require us to be cognizant going forward of the time from invention to filing of a patent application and be diligent in filing patent applications, but circumstances could prevent us from promptly filing patent applications on our inventions.

Among some of the other changes introduced by the AIA are changes that limit where a patentee may file a patent infringement suit and provide opportunities for third parties to challenge any issued patent in the USPTO. This applies to all of our U.S. patents, even those issued before March 16, 2013. Because of a lower evidentiary standard in USPTO proceedings compared to the evidentiary standard in U.S. federal courts necessary to invalidate a patent claim, a third party could potentially provide evidence in a USPTO proceeding sufficient for the USPTO to hold a claim invalid even though the same evidence would be insufficient to invalidate the claim if first presented in a district court action.

Accordingly, a third party may attempt to use the USPTO procedures to invalidate our patent claims that would not have been invalidated if first challenged by the third party as a defendant in a district court action. It is not clear what, if any, impact the AIA will have on the operation of our business. However, the AIA and its implementation could increase the uncertainties and costs surrounding the prosecution of our or our licensors’ patent applications and the enforcement or defense of our or our licensors’ issued patents.

We may become involved in opposition, interference, derivation, inter partes review or other proceedings challenging our or our licensors’ patent rights, and the outcome of any proceedings are highly uncertain. An adverse determination in any such proceeding could reduce the scope of, or invalidate, our owned or in-licensed patent rights, allow third parties to commercialize our technology or products and compete directly with us, without payment to us, or result in our inability to manufacture or commercialize products without infringing third-party patent rights.

In addition, the United States federal government retains certain rights in inventions produced with its financial assistance under the Bayh-Dole Act. The federal government retains a “nonexclusive, nontransferable, irrevocable, paid-up license” for its own benefit. The Bayh-Dole Act also provides federal agencies with “march-in rights.” March-in rights allow the government, in specified circumstances, to require the contractor or successors in title to the patent to grant a “nonexclusive, partially exclusive, or exclusive license” to a “responsible applicant or applicants.” If the patent owner refuses to do so, the government may grant the license itself. Some of our licensed patents are subject to the provisions of the Bayh-Dole Act. If our licensors fail to comply with the regulations of the Bayh-Dole Act, they could lose title to any patents subject to such regulations, which could affect our license rights under the patents and our ability to stop others from using or commercializing similar or identical technology and products, or limit patent protection for our technology and products.

Additionally, the U.S. Supreme Court has ruled on several patent cases in recent years either narrowing the scope of patent protection available in certain circumstances or weakening the rights of patent owners in certain situations, and there are other open questions under patent law that courts have yet to decisively address. In addition to increasing uncertainty with regard to our ability to obtain patents in the future, this combination of events has created uncertainty with respect to the value of patents once obtained. Depending on decisions by Congress, the federal courts and the USPTO, the laws and regulations governing patents could change in unpredictable ways and could weaken our ability to obtain new patents or to enforce our existing patents and patents that we might obtain in the future. In addition, the European patent system is relatively stringent in the type of amendments that are allowed during prosecution, but the complexity and uncertainty of European patent laws has also increased in recent years. Complying with these laws and regulations could limit our ability to obtain new patents in the future that may be important for our business.

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

Our current or future trademarks or trade names may be challenged, infringed, circumvented or declared generic or descriptive or determined to be infringing on other marks. We may not be able to protect our rights to these trademarks and trade names or may be forced to stop using these names, which we need for name recognition by potential partners or customers in our markets of interest. During trademark registration proceedings, we may receive rejections of our applications by the USPTO or in other foreign jurisdictions. Although we would be given an opportunity to respond to those rejections, we may be unable to overcome such rejections. In addition, in the USPTO and in comparable agencies in many foreign jurisdictions, third parties are given an opportunity to oppose pending trademark applications and to seek to cancel registered trademarks. Opposition or cancellation proceedings may be filed against our trademarks, and our trademarks may not survive such proceedings. If we are unable to establish name recognition based on our trademarks and trade names, we may not be able to compete effectively and our business may be adversely affected. We may license our trademarks and trade names to third parties, such as distributors. Although these license agreements may provide guidelines for how our trademarks and trade names may be used, a breach of these agreements or misuse of our trademarks and tradenames by our licensees may jeopardize our rights in or diminish the goodwill associated with our trademarks and trade names.

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

## Risks related to legal and regulatory compliance matters

***Our current and future relationships with customers, healthcare providers, including physicians, and third-party payors may be subject, directly or indirectly, to federal and state healthcare fraud and abuse laws, false claims laws, health information privacy and security laws and other healthcare laws and regulations. If we are unable to comply, or have not fully complied, with such laws, we could face substantial penalties.***

We are currently or will in the future be subject to healthcare regulation and enforcement by the U.S. federal government and the states in which we will conduct our business once our product candidates are approved by the FDA and commercialized in the United States. In addition to the FDA's restrictions on marketing of pharmaceutical products, the U.S. healthcare laws and regulations that may affect our ability to operate include: the federal fraud and abuse laws, including the federal anti-kickback and false claims laws; federal data privacy and security laws; and federal transparency laws related to payments and/or other transfers of value made to physicians and other healthcare professionals and teaching hospitals. Many states have similar laws and regulations that may differ from each other and federal law in significant ways, thus complicating compliance efforts. For example, states have anti-kickback and false claims laws that may be broader in scope than analogous federal laws and may apply regardless of payor. In addition, state laws regarding the privacy and security of health information may differ from each other and may not be preempted by federal law. Moreover, several states have enacted legislation requiring pharmaceutical manufacturers to, among other things, establish marketing compliance programs, file periodic reports with the state, make periodic public disclosures on sales and marketing activities, report information related to drug pricing, require the registration of sales representatives, and prohibit certain other sales and marketing practices. These laws may adversely affect our sales, marketing and other activities with respect to any product candidate for which we receive approval to market in the United States by imposing administrative and compliance burdens on us. It is possible that governmental authorities will conclude that our current or future business practices may not comply with current or future statutes, regulations or case law involving applicable fraud and abuse or other healthcare laws and regulations. If our operations are found to be in violation of any of these laws or any other governmental regulations that may apply to us, we may be subject to significant penalties, including, without limitation, civil, criminal and administrative penalties, damages, fines, disgorgement, individual imprisonment, exclusion from participating in federal and state funded healthcare programs, such as Medicare and Medicaid, additional reporting requirements and oversight if we become subject to a corporate integrity agreement or similar agreement to resolve allegations of non-compliance with these laws, contractual damages, diminished profits and future earnings, reputational harm and the curtailment or restructuring of our operations, any of which could harm our business.

The risk of being found in violation of these laws is increased by the fact that many of them have not been fully interpreted by the regulatory authorities or the courts, and their provisions are open to a variety of interpretations. Efforts to ensure that our business arrangements with third parties will comply with applicable healthcare laws and regulations will involve substantial costs. Any action against us for violation of these laws, even if we successfully defend against it, could cause us to incur significant legal expenses and divert our management's attention from the operation of our business. The shifting compliance environment and the need to build and maintain robust and expandable systems to comply with multiple jurisdictions with different compliance and/or reporting requirements increases the possibility that a healthcare company may run afoul of one or more of the requirements.

***Even if we obtain FDA or EMA approval for any of our product candidates in the United States or European Union, we may never obtain approval for or commercialize any of them in any other jurisdiction, which would limit our ability to realize their full market potential.***

In order to market any products in any particular jurisdiction, we must establish and comply with numerous and varying regulatory requirements on a country-by-country basis regarding safety and efficacy.

Approval by the FDA in the United States or the EMA in the European Union does not ensure approval by regulatory authorities in other countries or jurisdictions. However, the failure to obtain approval in one jurisdiction may negatively impact our ability to obtain approval elsewhere. In addition, clinical trials conducted in one country may not be accepted by regulatory authorities in other countries, and regulatory approval in one country does not guarantee regulatory approval in any other country.

Approval processes vary among countries and can involve additional product testing and validation and additional administrative review periods. Seeking foreign regulatory approval could result in difficulties and increased costs for us and require additional preclinical studies or clinical trials which could be costly and time consuming.

Regulatory requirements can vary widely from country to country and could delay or prevent the introduction of our products in those countries. We do not have any product candidates approved for sale in any jurisdiction, including in international markets. If we fail to comply with regulatory requirements in international markets or to obtain and maintain required approvals, or if regulatory approvals in international markets are delayed, our target market will be reduced and our ability to realize the full market potential of any product we develop will be unrealized.

***Even if we receive regulatory approval of our product candidates, we will be subject to ongoing regulatory obligations and continued regulatory review, which may result in significant additional expense, and we may be subject to penalties if we fail to comply with regulatory requirements or experience unanticipated problems with our product candidates.***

Any product candidate for which we obtain marketing approval will be subject to ongoing regulatory requirements for, among other things, manufacturing processes, submission of post-approval clinical data and safety information, labeling, packaging, distribution, adverse event reporting, storage, recordkeeping, export, import, advertising, promotional activities and product tracking and tracing. These requirements include submissions of safety and other post-marketing information and reports, establishment registration and drug listing requirements, applicable tracking and tracing requirements, continued compliance with cGMP requirements relating to manufacturing, quality control, quality assurance and corresponding maintenance of records and documents, requirements regarding the distribution of samples to physicians and recordkeeping and cGCP requirements for any clinical trials that we conduct post-approval.

Any regulatory approvals that we receive for our product candidates or any future product candidates may also be subject to a REMS, limitations on the approved indicated uses for which the product may be marketed or to the conditions of approval, or requirements that we conduct potentially costly post-marketing testing, including Phase 4 trials and surveillance to monitor the quality, safety and efficacy of the product. An unsuccessful post-marketing study or failure to complete such a study could result in the withdrawal of marketing approval. We will further be required to immediately report any serious and unexpected adverse events and certain quality or production problems with our products to regulatory authorities along with other periodic reports.

The FDA and EMA closely regulate the post-approval marketing and promotion of genetic therapy medicines to ensure they are marketed only for the approved indications and in accordance with the provisions of the approved labeling. However, companies may share truthful and not misleading information that is otherwise consistent with a product's FDA approved labeling. The FDA imposes stringent restrictions on manufacturers' communications regarding off-label use and if we market our products for uses beyond their approved diseases, we may be subject to enforcement action for off-label marketing. Violations of the FDCA, relating to the promotion of prescription drugs for unapproved uses may lead to enforcement actions and investigations alleging violations of federal and state health care fraud and abuse laws, as well as state consumer protection laws. The holder of an approved BLA must submit new or supplemental applications and obtain prior approval for certain changes to the approved product, product labeling, or manufacturing process. A company that is found to have improperly promoted off-label uses of their products may be subject to significant civil, criminal and administrative penalties.

In addition, later discovery of previously unknown adverse events or other problems with our products, manufacturers or manufacturing processes, including adverse events of unanticipated severity or frequency, or with our third-party manufacturers or manufacturing processes, or failure to comply with regulatory requirements, may yield various results, including:

- restrictions on manufacturing such products;
- restrictions on the labeling or marketing of a product;
- restrictions on product distribution or use;
- refusal to allow entry into supply contracts, including government contracts;
- requirements to conduct post-marketing studies or clinical trials;
- warning or untitled letters, or holds on clinical trials;
- withdrawal of the products from the market;
- refusal to approve pending applications or supplements to approved applications that we submit;
- recall of products;
- fines, restitution or disgorgement of profits or revenues;
- suspension or withdrawal of marketing approvals;
- refusal to permit the import or export of our products;

- product seizure or detention; or
- injunctions or the imposition of administrative, civil or criminal penalties or monetary fines.

The FDA's policies, and the policies of foreign regulatory agencies, may change and additional government regulations may be enacted that could prevent, limit or delay regulatory approval of our product candidates.

We also cannot predict the likelihood, nature or extent of government regulation that may arise from future legislation or administrative or executive action, either in the United States or abroad. For example, executive orders or other actions could impose significant burdens on, or otherwise materially delay, the FDA's ability to engage in routine oversight activities such as implementing statutes through rulemaking, issuance of guidance, and review and approval of marketing applications. If such executive actions were to impose restrictions on the FDA's ability to engage in oversight and implementation activities in the normal course, our business could be negatively impacted. If we are slow or unable to adapt to changes in existing requirements or the adoption of new requirements or policies, or if we are not able to maintain regulatory compliance, we may lose any marketing approval that we may have obtained which would adversely affect our business, prospects and ability to achieve or sustain profitability.

Any government investigation of alleged violations of law could require us to expend significant time and resources in response and could generate negative publicity. The occurrence of any event or penalty described above may inhibit our ability to commercialize our current product candidates or any future product candidates and harm our business, financial condition, results of operations and prospects.

***Enacted and future healthcare legislation may increase the difficulty and cost for us to progress our clinical programs and obtain marketing approval of and commercialize our product candidates and may affect the prices we may set.***

In the United States, the European Union and other jurisdictions, there have been, and we expect there will continue to be, a number of legislative and regulatory changes and proposed changes to the healthcare system that could affect our future results of operations. In particular, there have been and continue to be a number of initiatives at the U.S. federal and state levels that seek to reduce healthcare costs and improve the quality of healthcare.

The continuing efforts of the government, insurance companies, managed care organizations and other payers of healthcare services to contain or reduce costs of healthcare may adversely affect:

- the demand for any of our product candidates, if approved;
- the ability to set a price that we believe is fair for any of our product candidates, if approved;
- our ability to generate revenues and achieve or maintain profitability;
- the level of taxes that we are required to pay; and
- the availability of capital.

Legislative and regulatory proposals have been made to expand post-approval requirements and restrict sales and promotional activities for pharmaceutical and biologic products. We cannot be sure whether additional legislative changes will be enacted, or whether FDA regulations, guidance or interpretations will be changed, or what the impact of such changes on the marketing approvals of our product candidates, if any, may be. In addition, increased scrutiny by Congress of the FDA's approval process may significantly delay or prevent marketing approval, as well as subject us to more stringent product labeling and post-marketing testing and other requirements.

Moreover, payment methodologies may be subject to changes in healthcare legislation and regulatory initiatives. For example, CMS may develop new payment and delivery models, such as bundled payment models. In addition, recently there has been heightened governmental scrutiny over the manner in which manufacturers set prices for their marketed products, which has resulted in several U.S. presidential executive orders, Congressional inquiries and proposed and enacted federal legislation designed to, among other things, bring more transparency to drug pricing, reduce the cost of prescription drugs under Medicare, and review the relationship between pricing and manufacturer patient programs. In April 2025, the President issued an executive order and an accompanying fact sheet focused on lowering drug prices, but it is unclear what actions will be implemented by the Department of Health and Human Services, CMS, and FDA under the new leadership, and how those actions will impact our industry and our business. We expect that additional U.S. federal healthcare reform measures will be adopted in the future, any of which could limit the amounts that the U.S. federal government will pay for healthcare products and services, which could result in reduced demand for our product candidates or additional pricing pressures and could negatively affect our customers and accordingly, our financial operations.

Individual states in the United States have also increasingly passed legislation and implemented regulations designed to control pharmaceutical and biological product pricing, including price or patient reimbursement constraints, discounts, restrictions on certain product access and marketing cost disclosure and transparency measures, and, in some cases, designed to encourage importation from other countries and bulk purchasing. Legally mandated price controls on payment amounts by third-party payors or other restrictions could harm our business, results of operations, financial condition and prospects. In addition, regional healthcare authorities and individual hospitals are increasingly using bidding procedures to determine what pharmaceutical products and which suppliers will be included in their prescription drug and other healthcare programs. This could reduce the ultimate demand for our product candidates or put pressure on our product pricing.

In the European Union, similar political, economic and regulatory developments may affect our ability to profitably commercialize our product candidates, if approved. In addition to continuing pressure on prices and cost containment measures, legislative developments at the European Union or member state level may result in significant additional requirements or obstacles that may increase our operating costs. The delivery of healthcare in the European Union, including the establishment and operation of health services and the pricing and reimbursement of medicines, is almost exclusively a matter for national, rather than European Union, law and policy. National governments and health service providers have different priorities and approaches to the delivery of health care and the pricing and reimbursement of products in that context. In general, however, the healthcare budgetary constraints in most European Union member states have resulted in restrictions on the pricing and reimbursement of medicines by relevant health service providers. Coupled with ever-increasing European Union and national regulatory burdens on those wishing to develop and market products, this could prevent or delay marketing approval of our product candidates, restrict or regulate post-approval activities and affect our ability to commercialize our product candidates, if approved.

In markets outside of the United States and the European Union, reimbursement and healthcare payment systems vary significantly by country, and many countries have instituted price ceilings on specific products and therapies.

We cannot predict the likelihood, nature or extent of government regulation that may arise from future legislation or administrative action in the United States, the European Union or any other jurisdiction. If we or any third parties we may engage are slow or unable to adapt to changes in existing requirements or the adoption of new requirements or policies, or if we or such third parties are not able to maintain regulatory compliance, our product candidates may lose any regulatory approval that may have been obtained and we may not achieve or sustain profitability.

***Changes in federal funding, layoffs, government shutdown, staff departures, lapse in government appropriations, and other policies and executive order impacting the normal operations of the FDA and other government agencies could hinder their ability to hire and retain key leadership and other personnel, or otherwise prevent new products and services from being developed or commercialized in a timely manner, any of which could negatively impact our business.***

The ability of the FDA to review and approve new products can be affected by a variety of factors, including government budget and funding levels, the current government shutdown, ability to hire and retain key personnel, accept the payment of user fees, and statutory, regulatory and policy changes. Average review times at the agency have fluctuated in recent years as a result. In addition, government funding of other government agencies that fund research and development activities is subject to the political process, which is inherently fluid and unpredictable. Changes in the leadership of the FDA and other federal agencies under the current administration, including return-to-office policy, hiring freeze, layoffs, and other policies implemented by the Department of Government Efficiency, may lead to changes in the operations of the FDA, which may have a material impact on the industry and our clinical development plans.

Disruptions at the FDA and other agencies may also slow the time necessary for new drugs to be reviewed and/or approved by necessary government agencies, which would adversely affect our business. Additionally, changes in the FDA leadership, regulatory actions and other actions under the current administration, may result in delays in regulatory approval. Our business depends upon the ability of the FDA to accept and review our potential regulatory filings. If a prolonged government shutdown occurs or if a significant number of federal employees are laid off or leave federal agencies, it could significantly impact the ability of the FDA to timely review and process our regulatory submissions, which could have a material adverse effect on our ability to advance clinical development of our product candidates, delay our clinical development timelines, and increase the costs of clinical development process.

***If we are unable to establish sales, marketing and distribution capabilities either on our own or in collaboration with third parties, we may not be successful in commercializing our product candidates or realizing the synergies in the target diseases of our programs, even if they are approved.***

We do not have any infrastructure for the sales, marketing or distribution of our products, and the cost of establishing and maintaining such an organization may exceed the cost-effectiveness of doing so. We expect to build a focused sales, distribution and marketing infrastructure to market our product candidates in the United States and European Union, if approved. There are significant expenses and risks involved with establishing our own sales, marketing and distribution capabilities, including our ability to hire, retain and appropriately incentivize qualified individuals, generate sufficient sales leads, provide adequate training to sales and marketing personnel, and effectively manage a geographically dispersed sales and marketing team. Any failure or delay in the development of our internal sales, marketing and distribution capabilities could delay any product launch, which would adversely impact the commercialization of our product candidates. Additionally, if the commercial launch of our product candidates for which we recruit a sales force and establish marketing capabilities is delayed or does not occur for any reason, we would have prematurely or unnecessarily incurred these commercialization expenses. This may be costly, and our investment would be lost if we cannot retain or reposition our sales and marketing personnel.

We may not have the resources in the foreseeable future to allocate to the sales and marketing of our product candidates in certain international markets. Therefore, our future sales in these markets will largely depend on our ability to enter into and maintain collaborative relationships for such capabilities, the collaborator's strategic interest in the product and such collaborator's ability to successfully market and sell the product. We may pursue collaborative arrangements regarding the sale and marketing of LX2006 or LX2020, if approved, for certain markets overseas; however, we cannot assure that we will be able to establish or maintain such collaborative arrangements, or if able to do so, that they will have effective sales forces.

If we are unable to build our own sales force or negotiate a collaborative relationship for the commercialization of LX2006, LX2020 or LX1001, or any of our other product candidates, if approved, we may be forced to delay the potential commercialization of LX2006, or LX2020 or any of our other product candidates or reduce the scope of our sales or marketing activities for LX2006 or LX2020 or any of our other product candidates. If we elect to increase our expenditures to fund commercialization activities internationally, we will need to obtain additional capital, which may not be available to us on acceptable terms, or at all. We could enter into arrangements with collaborative partners at an earlier stage than otherwise would be ideal and we may be required to relinquish rights to LX2006 or LX2020 or any of our other product candidates or otherwise agree to terms unfavorable to us, any of which may have an adverse effect on our business, operating results and prospects.

If we are unable to establish adequate sales, marketing and distribution capabilities, either on our own or in collaboration with third parties, we will not be successful in commercializing LX2006, or LX2020 or any of our other product candidates, if approved, and may not become profitable and may incur significant additional losses. We will be competing with many companies that currently have extensive and well-funded marketing and sales operations. Without an internal team or the support of a third party to perform marketing and sales functions, we may be unable to compete successfully against these more established companies.

***If we obtain approval to commercialize any products outside of the United States or the European Union, a variety of risks associated with international operations could adversely affect our business.***

If LX2006, LX2020 or any of our other product candidates are approved for commercialization, we may seek to enter into agreements with third parties to market them in certain jurisdictions outside the United States and the European Union. We expect that we would be subject to additional risks related to international pharmaceutical operations, including:

- different regulatory requirements for drug and biologic approvals and rules governing drug and biologic commercialization in foreign countries;
- reduced protection for intellectual property rights;
- foreign reimbursement, pricing and insurance regimes;

- unexpected changes in tariffs, trade barriers and regulatory requirements;
- economic weakness, including inflation, or political instability in particular foreign economies and markets;
- foreign currency fluctuations, which could result in increased operating expenses and reduced revenues, and other obligations incident to doing business in another country;
- business interruptions resulting from geopolitical actions, including war and terrorism or natural disasters including earthquakes, typhoons, floods and fires, or from economic or political instability;
- greater difficulty with enforcing our contracts;
- potential noncompliance with the U.S. Foreign Corrupt Practices Act, or the FCPA, the U.K. Bribery Act 2010 and similar anti-bribery and anticorruption laws in other jurisdictions; and
- production shortages resulting from any events affecting raw material supply or manufacturing capabilities abroad.

In addition, there are complex regulatory, tax, labor and other legal requirements imposed by individual countries in Europe with which we will need to comply. If we are unable to successfully manage the challenges of international expansion and operations, our business and operating results could be harmed.

***If we fail to comply with environmental, health and safety laws and regulations, we could become subject to fines or penalties or incur costs that could harm our business.***

We are subject to numerous environmental, health and safety laws and regulations, including those governing laboratory procedures and the handling, use, storage, treatment and disposal of hazardous materials and wastes. From time to time and in the future, our operations may involve the use of hazardous and flammable materials, including chemicals and biological materials, and may also produce hazardous waste products. Even if we contract with third parties for the disposal of these materials and waste products, we cannot completely eliminate the risk of contamination or injury resulting from these materials. In the event of contamination or injury resulting from the use or disposal of our hazardous materials, we could be held liable for any resulting damages, and any liability could exceed our resources. We also could incur significant costs associated with civil or criminal fines and penalties for failure to comply with such laws and regulations.

We maintain workers' compensation insurance to cover us for costs and expenses we may incur due to injuries to our employees, but this insurance may not provide adequate coverage against potential liabilities. However, we do not maintain insurance for environmental liability or toxic tort claims that may be asserted against us.

In addition, we may incur substantial costs in order to comply with current or future environmental, health and safety laws and regulations. Environmental laws and regulations may impair our research, development or production efforts. In addition, failure to comply with these laws and regulations may result in substantial fines, penalties or other sanctions.

***We are subject to a variety of privacy, data protection, and cybersecurity laws, rules, regulations, policies, industry standards and contractual obligations, and our failure to comply with them could harm our business.***

We maintain and otherwise process a large quantity of sensitive information, including confidential business and personal information in connection with the conduct of our clinical trials and related to our employees and others, and we are subject to laws and regulations governing the privacy and security of such information. In the United States, numerous federal and state privacy and cybersecurity laws and regulations govern the collection, use, disclosure and protection of personal information, including federal and state health information privacy laws, federal and state security breach notification laws and federal and state consumer protection laws. The legislative and regulatory landscape for privacy, data protection and cybersecurity continues to evolve, and there has been an increasing focus on privacy, data protection and cybersecurity issues, which may affect our business and is expected to increase our compliance costs and exposure to liability. In the United States, numerous federal and state laws and regulations could apply to our operations or the operations of our partners, including state and federal data breach notification laws, federal regulations prohibiting or restricting certain transfers of sensitive personal information, and state health information privacy and cybersecurity laws and federal and state consumer protection laws and regulations that govern the collection, use, disclosure and protection of health-related and other personal information. Among these regulations are: Section 5 of the Federal Trade Commission Act, which prohibits unfair or deceptive commercial practices; rules adopted by the SEC in July 2023, which require public companies to disclose material cybersecurity incidents they experience and to disclose on an annual basis material information regarding their cybersecurity risk management, strategy, and governance; and the HIPAA, as amended by HITECH, and the regulations promulgated thereunder.

We may obtain health and other data from third parties, including research institutions from which we obtain clinical trial data, that are subject to privacy and security requirements under HIPAA, and depending on the facts and circumstances, we could be subject to significant penalties if we obtain, use or disclose individually identifiable health information in a manner that is not authorized or permitted by HIPAA.

In the European Economic Area, or the EEA, and the United Kingdom, or the UK, the collection, use, disclosure, transfer or other processing of personal data, including clinical trial data, of individuals is governed by the General Data Protection Regulation, or European Union GDPR (with regard to the EEA), and UK GDPR (with regard to the UK), as well as applicable national data protection legislation and requirements. In this document, “GDPR” refers to both the European Union GDPR and the UK GDPR, unless specified otherwise. The GDPR is wide ranging in scope and imposes numerous requirements on companies that process personal data, including requirements relating to processing health and other sensitive data, obtaining consent of the individuals to whom the personal data relates, providing information to individuals regarding data processing activities, implementing safeguards to protect the security and confidentiality of personal data, providing notification of data breaches, and taking certain measures when engaging third-party processors. The GDPR imposes substantial fines for breaches and violations (up to the greater of €20 million (£17.5 million for the UK) or 4% of our consolidated annual worldwide gross revenue), and confers a private right of action on data subjects and consumer associations to lodge complaints with supervisory authorities, seek judicial remedies and obtain compensation for damages resulting from violations of the GDPR.

The GDPR also includes restrictions on cross-border data transfers of personal data to countries outside the EEA and the UK that are not considered by the European Commission or UK government as providing “adequate” protection to personal data, or third countries, including the United States, unless a valid GDPR transfer mechanism (for example, the European Commission approved Standard Contractual Clauses, or SCCs, and the UK International Data Transfer Agreement/Addendum, or UK IDTA) has been put in place. Where relying on the SCCs or UK IDTA for data transfers, we may also be required to carry out transfer impact assessments to assess whether the recipient is subject to local laws which allow public authority access to personal data. The international transfer obligations under the EEA and UK data protection regimes require significant effort and cost, and may result in us needing to make strategic considerations around where EEA and UK personal data is transferred and which service providers we can utilize for processing EEA and UK personal data.

The UK government made targeted amendments to its data protection regime in the Data (Use and Access) Act 2025, which entered into force in August 2025. The European Commission renewed its adequacy determination for the UK in 2025, extending to December 2031, but the UK’s adequacy determination remains subject to modification or revocation. The UK’s proposal to modify its data protection regime may impact this adequacy determination. Any loss by the UK of its adequacy determination by the European Commission may lead to additional compliance costs and could increase our overall risk. The respective provisions and enforcement of the European Union GDPR and UK GDPR may further diverge in the future and create additional regulatory challenges and uncertainties.

Compliance with these and any other applicable privacy, data protection and cybersecurity laws and regulations is a rigorous and time-intensive process, and we may be required to put in place additional mechanisms ensuring compliance with new and evolving laws and regulations. Further, many privacy, data protection, and cybersecurity regimes are evolving and are inconsistent across jurisdictions. If we fail, or are alleged to fail, to comply with any such laws or regulations, we may face regulatory inquiries, investigations, and other proceedings, private claims and litigation, and significant fines and penalties that could adversely affect our business, financial condition and results of operations. In addition, states are constantly adopting new laws or amending existing laws, requiring attention to frequently changing regulatory requirements. For example, California enacted the California Consumer Privacy Act, or CCPA, which took effect on January 1, 2020, became enforceable on July 1, 2020 and has been dubbed the first “GDPR-like” law in the United States. The CCPA gives California residents expanded rights to access and delete their personal information, opt out of certain personal information sharing and receive detailed information about how their personal information is used by requiring covered companies to provide new disclosures to California consumers (as that term is broadly defined) and provide such consumers new ways to opt out of certain sales of personal information. The CCPA provides for civil penalties for violations, as well as a private right of action for data breaches that is expected to increase data breach litigation. Further, the California Privacy Rights Act, or CPRA, which became effective as of January 1, 2023, amended the CCPA and imposes additional privacy obligations on companies doing business in California, including additional consumer rights processes, limitations on data uses, new audit requirements for higher risk data and opt outs for certain uses of sensitive data. The amendments introduced by the CPRA also created a new California Privacy Protection Agency to implement and enforce this legislation, and it is anticipated that this development could result in increased privacy and information security enforcement. Although the CCPA, as amended by the CPRA, currently exempts certain health-related information, including clinical trial data, the CCPA may increase our compliance costs and potential liability if we expand our operations into California. Similar broad consumer privacy laws have been enacted in Colorado, Connecticut, Delaware, Florida, Indiana, Iowa, Kentucky, Maryland, Minnesota, Montana, Nebraska, New Hampshire, New Jersey, Oregon, Rhode Island, Tennessee, Texas, Utah and Virginia and have been proposed in numerous other states and at the federal level. If passed, these bills may provide for various requirements that would make compliance challenging, particularly in light of certain conflicting and otherwise various requirements in the state privacy laws that have been enacted.

In addition to these consumer privacy laws, certain states have enacted health-focused privacy legislation. For example, Washington has enacted the My Health, My Data Act, which became effective in March 2024. This law imposes strict requirements on the collection, use and processing of certain health-related information that is not subject to HIPAA. This law also provides for a private right of action. Other states are considering, and in certain cases have enacted, bills with similar requirements. The Washington law and other state bills that have been, or may be, enacted, have added and, if enacted, would add additional complexity to our compliance obligations.

With the GDPR, CCPA, and other laws, regulations and other obligations relating to privacy and data protection imposing new and relatively burdensome obligations, and with substantial uncertainty over the interpretation and application of these and other obligations, we may face challenges in addressing their requirements and making necessary changes to our policies and practices and may incur significant costs and expenses in an effort to do so. We do not currently have formal data privacy policies and procedures in place and have not completed formal assessments of whether we are in compliance with all applicable data privacy laws and regulations. It is possible that both existing and new laws, regulations, and other actual or asserted obligations to which we are or may be subject may be interpreted and applied in manners inconsistent with our existing or future privacy, data protection and cybersecurity practices. Any failure or perceived failure by us to comply with our current or future obligations relating to privacy, data protection or cybersecurity, or any actual or perceived failure by any third party with which we work, such as third-party CMOs, CROs, manufacturers, contractors, consultants, collaborators, vendors, or service providers, to comply with our applicable policies or other applicable obligations, may result in governmental investigations, enforcement actions, or other proceedings, litigation, claims, or public statements against us and could result in significant liability, cause harm to our brand and reputation, and otherwise materially and adversely affect our reputation and business.

***We may be subject to various governmental export control and trade sanctions laws and regulations that could impair our ability to compete in international markets or subject us to liability if we violate these controls.***

Our product candidates may be subject to export control and import laws and regulations, including the U.S. Export Administration Regulations administered by the U.S. Department of Commerce and U.S. Customs laws and regulations and our product candidates and activities may be subject to various economic and trade sanctions regulations administered by the U.S. Treasury Department's Office of Foreign Assets Controls, or collectively, Trade Controls. As such, a license may be required to export or re-export our product candidate, or provide related services, to certain countries and end-users, and for certain end-uses. The process for obtaining necessary licenses may be time-consuming or unsuccessful, potentially causing delays in sales or losses of sales opportunities and these licenses may not be issued. Compliance with applicable regulatory requirements regarding the export or import of our product candidates may create delays in the introduction of our product candidates in international markets or, in some cases, prevent the export of our product candidates to some countries altogether. Furthermore, U.S. export control laws and economic sanctions prohibit the shipment of certain product candidates and services to countries, territories, governments and persons targeted by U.S. sanctions.

Trade Controls are complex and dynamic regimes and monitoring and ensuring compliance can be challenging. Although we have procedures in place designed to ensure our compliance with Trade Controls, any failure to comply could subject us to both civil and criminal penalties, including substantial fines, possible incarceration of responsible individuals for willful violations, possible loss of our export or import privileges, and reputational harm. Although we have no knowledge that our activities have resulted in violations of Trade Controls, there is no certainty that all of our employees, agents, suppliers, manufacturers, contractors or collaborators, or those of our affiliates, will comply with all applicable export and import control and sanctions laws and regulations, particularly given the high level of complexity of these laws. Any failure by us or our partners to comply with applicable laws and regulations could have negative consequences for us, including reputational harm, government investigations, penalties, fines, settlements, investigations, criminal sanctions against us, our officers, or our employees, the closing down of facilities, including those of our suppliers and manufacturers, requirements to obtain export licenses, cessation of business activities in sanctioned countries, implementation of additional compliance programs, and prohibitions on the conduct of our business.

***We are subject to U.S. and certain foreign anti-corruption laws and regulations. We could face liability and other serious consequences for violations.***

We are subject to anti-corruption laws and regulations, including the FCPA, the U.S. domestic bribery statute contained in 18 U.S.C. § 201, the U.S. Travel Act and will be subject to local and national anti-bribery laws in the countries in which we may conduct activities in the future. Anti-corruption laws are interpreted broadly and generally prohibit companies and their employees, agents, representatives, contractors and other third-party intermediaries from offering, promising, giving, or authorizing others to give anything of value, either directly or indirectly through third parties, to any person in the public or private sector to obtain or retain business. The FCPA also requires public companies to make and keep books and records that accurately and fairly reflect the transactions of the corporation and to devise and maintain an adequate system of internal accounting controls.

Our business is heavily regulated and therefore involves significant interaction with public officials, including officials of non-United States governments. Additionally, in many other countries, the healthcare providers who prescribe pharmaceuticals are employed by their government, and therefore will be considered foreign officials for purposes of the FCPA. We also expect to rely on third parties for research, preclinical studies and clinical trials and/or to obtain necessary permits, licenses, patent registrations and other marketing approvals on our behalf outside of the United States. We can be held liable for the corrupt or other illegal activities of our employees, agents, representatives, CROs, contractors and other third-party intermediaries, even if we do not explicitly authorize or have actual knowledge of such activities.

There is no certainty that all of our employees, agents, representatives, contractors or third-party intermediaries will comply with all applicable anti-corruption laws. Allegations concerning or violations of these laws and regulations could result in whistleblower complaints, fines, settlements, investigations, criminal or civil sanctions, adverse media coverage or suspension or debarment from government contracts, all of which could damage our reputation, our brand, our international expansion efforts, our ability to attract and retain employees, and our business, prospects, operating results and financial condition. Responding to any investigation or action will likely result in a materially significant diversion of management's attention and resources and significant defense costs and other professional fees.

### **Risks related to employee matters and managing our growth**

***Our future success depends on our ability to attract and retain key executives and advisors and to attract, retain and motivate qualified personnel.***

We are highly dependent on the management, development, clinical, financial and business development expertise of our executive officers, particularly R. Nolan Townsend, our Chief Executive Officer and a member of our board of directors, Narinder Bhalla, M.D., our Chief Medical Officer, Jose Manuel Otero, our Chief Operating Officer, Louis Tamayo, our Chief Financial Officer, as well as on the scientific expertise of our founder, Ronald G. Crystal, M.D., Professor and Chairman of Weill Cornell Medicine's Department of Genetic Medicine. Each of our executive officers may currently terminate their employment with us at any time and we do not have an employment contract with Dr. Crystal. We do not maintain "key person" insurance for any of our executives or employees.

Recruiting and retaining qualified executives, scientists and clinical personnel and, if we progress the development of our product pipeline toward scaling up for commercialization, manufacturing and sales and marketing personnel, will also be critical to our success. The loss of the services of our executive officers or other key employees, or our inability to recruit certain executives, could impede the achievement of our development and commercialization objectives and seriously harm our ability to successfully implement our business strategy. Furthermore, recruiting executive officers, or replacing executive officers and key employees may be difficult and may take an extended period of time because of the limited number of individuals in our industry with the breadth of skills and experience required to successfully develop, gain regulatory approval of and commercialize gene therapy products.

Competition to hire from this limited pool is intense, and we have experienced and may continue to experience challenges filling certain executive roles. We may be unable to hire, train, retain or motivate key personnel on acceptable terms given the competition among numerous pharmaceutical and biotechnology companies for similar personnel. We also experience competition for the hiring of scientific and clinical personnel from universities and research institutions. In addition, we rely on consultants and advisors, including scientific and clinical advisors, to assist us in formulating our development and commercialization strategy. Our consultants and advisors may be employed by employers other than us and may have commitments under consulting or advisory contracts with other entities that may limit their availability to us. If we are unable to continue to attract and retain high quality personnel, our ability to pursue our growth strategy will be limited.

***We expect to expand our clinical development, manufacturing and regulatory capabilities and potentially implement sales, marketing and distribution capabilities, and as a result, we may encounter difficulties in managing our growth, which could disrupt our operations.***

As of December 31, 2025, we had 59 full-time employees. As our development progresses, we expect to experience significant growth in the number of our employees and the scope of our operations, particularly in the areas of clinical product development, regulatory affairs and, if any of our product candidates receives marketing approval, sales, marketing and distribution. To manage our anticipated future growth, we must continue to implement and improve our managerial, operational and financial systems, expand our facilities and continue to recruit and train additional qualified personnel. Due to our limited financial resources and the limited experience of our management team in managing a company with such anticipated growth, we may not be able to effectively manage the expansion of our operations or recruit and train additional qualified personnel. Our choice to focus on multiple therapeutic areas may negatively affect our ability to develop adequately the specialized capability and expertise necessary for operations. The expansion of our operations may lead to significant costs and may divert our management and business development resources. Any inability to manage growth could delay the execution of our business plans or disrupt our operations.

***Our employees, independent contractors, consultants, collaborators, principal investigators, CROs, suppliers and vendors may be improperly classified and may engage in misconduct or other improper activities, including non-compliance with regulatory standards and requirements.***

We endeavor to properly classify our employees as exempt or non-exempt with respect to wage and hour laws (including, but not limited to, for purposes of minimum wage, overtime and applicable meal and rest periods), and we monitor and evaluate such classifications. Although there are no current, pending, or threatened claims or investigations against us asserting that any employees have been incorrectly classified as exempt, the possibility nevertheless exists that certain job roles could be deemed to have been incorrectly classified as exempt. In addition, we endeavor to classify our workforce properly, and we monitor and evaluate such classifications. Although there are no current, pending, or threatened claims or investigations against us asserting that any independent contractors have been incorrectly classified, the possibility nevertheless exists that certain contractors could be deemed to be employees.

We are exposed to the risk that our employees, independent contractors, consultants, collaborators, principal investigators, CROs, suppliers and vendors may engage in fraudulent conduct or other illegal activity. Misconduct by these parties could include intentional, reckless and/or negligent conduct that violates FDA regulations, including those laws requiring the reporting of true, complete and accurate information to the FDA, manufacturing standards, federal and state healthcare laws and regulations, and laws that require the true, complete and accurate reporting of financial information or data. In particular, sales, marketing and business arrangements in the healthcare industry are subject to extensive laws and regulations intended to prevent fraud, kickbacks, self-dealing and other abusive practices. These laws and regulations may restrict or prohibit a wide range of pricing, discounting, marketing and promotion, sales commission, customer incentive programs and other business arrangements. Misconduct by these parties could also involve the improper use of individually identifiable information, including, without limitation, information obtained in the course of clinical trials, which could result in regulatory sanctions and serious harm to our reputation. Although we have adopted a code of business conduct and ethics, it is not always possible to identify and deter misconduct, and the precautions we take to detect and prevent this activity may not be effective in controlling unknown or unmanaged risks or losses or in protecting us from governmental investigations or other actions or lawsuits stemming from a failure to be in compliance with such laws or regulations. If any such actions are instituted against us, and we are not successful in defending ourselves or asserting our rights, those actions could have a significant impact on our business, including the imposition of significant civil, criminal and administrative penalties, including, without limitation, damages, fines, disgorgement, imprisonment, exclusion from participation in government healthcare programs, such as Medicare and Medicaid, additional reporting requirements and oversight if we become subject to a corporate integrity agreement or similar agreement to resolve allegations of non-compliance with these laws, and the curtailment or restructuring of our operations.

***The administrator of the 2023 Plan is authorized to exercise its discretion to repricing stock options and stock appreciation rights, and if a repricing occurs, there may be adverse consequences to our business.***

The administrator of the 2023 Plan, which is our compensation committee, is authorized, subject to the consent of any award holder whose award is materially impaired by such action, to reduce the exercise price of a stock option or stock appreciation right; to cancel a stock option or stock appreciation right in exchange for a different award, cash or other consideration; or to take any other action that is treated as a repricing under generally accepted accounting principles, or each such action, a repricing.

We have no current expectation that a repricing will occur. However, if the administrator were to implement a repricing without seeking prior stockholder approval, certain proxy advisory firms and/or institutional investors may express a lack of support for the repricing, and proxy advisory firms may recommend an “against” or “withhold” vote for members of our compensation committee or our board of directors. In addition, if we are required to hold an advisory vote on named executive officer compensation (known as a “say on pay” vote) at the time of, or subsequent to, any such repricing, it is likely, based on their current policies, that proxy advisory firms would issue an “against” recommendation on our say on pay proposal. Defending against negative recommendations with respect to our directors and/or say on pay proposal would require management attention, and could be costly and time-consuming.

If our stockholders agree with proxy advisory firms’ recommendations, we may need to make changes to our compensation and corporate governance practices, and perhaps the composition of our board of directors and its committees, potentially leading to business disruptions and a negative impact on our stock price. Even absent negative reactions from proxy advisory firms and institutional investors, we may be required to recognize a compensation expense and the repricing will require management’s time and attention and the payment of administrative costs and attorney and accounting firm fees. As such, a repricing could cause a negative impact on our stock price, and adverse consequences to our business.

## Risks related to ownership of our common stock and our status as a public company

*The trading price of the shares of our common stock may be volatile, and purchasers of our common stock could incur substantial losses.*

The trading price of our common stock is likely to be highly volatile and could be subject to wide fluctuations in response to various factors, some of which are beyond our control, including limited trading volume. The stock market in general and the market for biopharmaceutical companies in particular have experienced extreme volatility that has often been unrelated to the operating performance of particular companies. As a result of this volatility, investors may not be able to sell their common stock at or above the price paid for the shares. The trading price for our common stock may be influenced by many factors, including those discussed in this “*Risk Factors*” section and elsewhere in this Annual Report on Form 10-K and:

- the reporting of unfavorable preclinical and clinical results;
- the commencement, enrollment or results of our clinical trials of LX2006, LX2020 or any future clinical trials we may conduct, or changes in the development status of our product candidates;
- any delay in our regulatory filings for LX2006, LX2020 or any other product candidate we may develop, and any adverse development or perceived adverse development with respect to the applicable regulatory authority’s review of such filings, including without limitation the FDA’s issuance of a “refusal to file” letter or a request for additional information;
- adverse results from, delays in or termination of clinical trials;
- adverse regulatory decisions, including failure to receive regulatory approval of our product candidates;
- unanticipated serious safety concerns related to the use of LX2006, LX2020 or any other product candidates;
- changes in financial estimates by us or by any equity research analysts who might cover our stock;
- conditions or trends in our industry;
- changes in the market valuations of similar companies;
- regulatory or legal developments in the U.S. and other countries;
- stock trading price and volume fluctuations of comparable companies and, in particular, those that operate in the biopharmaceutical industry;
- publication of research reports about us or our industry or positive or negative recommendations or withdrawal of research coverage by securities analysts;
- announcements by us or our competitors of significant acquisitions, strategic partnerships or divestitures;
- our relationships with our collaborators;
- announcements of investigations or regulatory scrutiny of our operations or lawsuits filed against us;
- investors’ general perception of our company and our business;
- recruitment or departure of key personnel;
- overall performance of the equity markets;
- trading volume of our common stock;
- disputes or other developments relating to proprietary rights, including patents, litigation matters and our ability to obtain patent protection for our technologies;

- significant lawsuits, including patent or stockholder litigation or employee or independent contractor litigation;
- changes in the structure of healthcare payment systems;
- unfavorable geopolitical and economic conditions; and
- other events or factors, many of which are beyond our control.

The global economy, including credit and financial markets and the banking sector, has experienced extreme volatility and disruptions, including, among other things, severely diminished liquidity and credit availability, declines in consumer confidence, declines in economic growth, increases in unemployment rates, supply chain shortages, increases in inflation rates, bank failures, higher interest rates, changing foreign trade policies and uncertainty about economic stability. For example, the ongoing wars in Ukraine and the Middle East have created volatility in the global capital markets and may have further global economic consequences, including disruptions of the global supply chain and energy markets. Any such volatility and disruptions may have adverse consequences on us or the third parties on whom we rely. In addition, investor concerns regarding the U.S. or international financial systems and governmental stability, including as a result of recent tariffs announcements and increased volatility in the credit and equity markets, may impair our ability to raise additional capital or enter into opportunistic strategic partnerships or acquisitions in a timely manner or on favorable terms, or at all. The deterioration of the equity and credit markets may also make any necessary debt or equity financings more costly or more dilutive. Increased inflation rates can adversely affect us by increasing our costs, including labor and employee benefit costs. In addition, higher inflation and macro turmoil and uncertainty could also adversely affect our buyers and sellers, which could reduce demand for our products. These factors may negatively affect the trading price of our common stock, regardless of our actual operating performance.

In addition, in the past, stockholders have initiated class action lawsuits against pharmaceutical and biotechnology companies following periods of volatility in the trading prices of these companies' stock. This risk is especially relevant for us because biopharmaceutical companies have experienced significant stock price volatility in recent years. Such litigation, if instituted against us, could cause us to incur substantial costs and divert management's attention and resources, which could harm our business.

***If equity research analysts do not publish research or reports, or publish unfavorable research or reports, about us, our business or our market, our stock price and trading volume could decline.***

The trading market for our common stock is influenced by the research and reports that equity research analysts publish about us and our business. As a newly public company, we have only limited research coverage by equity research analysts. Equity research analysts may elect not to provide research coverage of our common stock, and such lack of research coverage may adversely affect the trading price of our common stock. While we currently have equity research analyst coverage, we will not have any control over the analysts or the content and opinions included in their reports. The price of our stock could decline if one or more equity research analysts downgrade our stock or issue other unfavorable commentary or research. If one or more equity research analysts cease coverage of our company or fail to publish reports on us regularly, demand for our stock could decrease, which in turn could cause our stock price or trading volume to decline.

***We are an "emerging growth company" and a "smaller reporting company" and, as a result of the reduced disclosure and governance requirements applicable to emerging growth companies and smaller reporting companies, our common stock may be less attractive to investors.***

We are an "emerging growth company" as defined in the JOBS Act, and we intend to take advantage of some of the exemptions from reporting requirements that are applicable to other public companies that are not emerging growth companies, including:

- not being required to comply with the auditor attestation requirements in the assessment of our internal control over financial reporting;
- not being required to comply with any requirement that may be adopted by the Public Company Accounting Oversight Board regarding mandatory audit firm rotation or a supplement to the auditor's report providing additional information about the audit and the financial statements;
- reduced disclosure obligations regarding executive compensation in our periodic reports, proxy statements and registration statements; and
- not being required to hold a nonbinding advisory vote on executive compensation and stockholder approval of any golden parachute payments not previously approved.

We cannot predict if investors will find our common stock less attractive because we will rely on these exemptions. If some investors find our common stock less attractive as a result, there may be a less active trading market for our common stock and our stock price may be more volatile. We may take advantage of these reporting exemptions until we are no longer an emerging growth company. We will remain an emerging growth company until the last day of the fiscal year ending after the fifth anniversary of our IPO, or, if earlier, (i) the last day of the fiscal year in which we have total annual gross revenue of at least \$1.235 billion, (ii) the date on which we are deemed to be a large accelerated filer, which means the market value of our common stock that is held by non-affiliates exceeds \$700 million as of the prior June 30, or (iii) the date on which we have issued more than \$1.0 billion in non-convertible debt during the prior three-year period.

In addition, we have elected to take advantage of the extended transition period to comply with new or revised accounting standards and to adopt certain of the reduced disclosure requirements available to emerging growth companies. As a result of the accounting standards election, we will not be subject to the same implementation timing for new or revised accounting standards as other public companies that are not emerging growth companies, which may make comparison of our financials to those of other public companies more difficult. As a result of these elections, the information that we provide in this Annual Report may be different than the information investors may receive from other public companies in which they hold equity interests. In addition, it is possible that some investors will find our common stock less attractive as a result of these elections, which may result in a less active trading market for our common stock and higher volatility in our trading price.

Even after we no longer qualify as an emerging growth company, we may, under certain circumstances, still qualify as a “smaller reporting company,” which would allow us to take advantage of many of the same exemptions from disclosure requirements, including reduced disclosure obligations regarding executive compensation in our periodic reports and proxy statements.

***Because we do not anticipate paying any cash dividends on our common stock in the foreseeable future, capital appreciation, if any, will be your sole source of gains and you may never receive a return on your investment.***

You should not rely on an investment in our common stock to provide dividend income. We have not declared or paid cash dividends on our common stock to date. We currently intend to retain our future earnings, if any, to fund the development and growth of our business. As a result, capital appreciation, if any, of our common stock will be your sole source of gain for the foreseeable future. Investors seeking cash dividends should not purchase our common stock.

***Our amended and restated certificate of incorporation provides that the Court of Chancery of the State of Delaware and the federal district courts of the United States of America will be the exclusive forums for substantially all disputes between us and our stockholders, which could limit our stockholders’ ability to obtain a favorable judicial forum for disputes with us or our directors, officers or employees.***

Our amended and restated certificate of incorporation provides that the Court of Chancery of the State of Delaware is the exclusive forum for the following types of actions or proceedings under Delaware statutory or common law:

- any derivative action or proceeding brought on our behalf;
- any action asserting a breach of fiduciary duty;
- any action asserting a claim against us arising under the Delaware General Corporation Law, or DGCL, our amended and restated certificate of incorporation, or our amended and restated bylaws;
- any claim or cause of action seeking to interpret, apply, enforce or determine the validity of our restated certificate or our amended and restated bylaws;
- any claim or cause of action as to which the DGCL confers jurisdiction on the Court of Chancery of the State of Delaware; and
- any action asserting a claim against us that is governed by the internal-affairs doctrine.

This provision would not apply to suits brought to enforce a duty or liability created by the Exchange Act. Furthermore, Section 22 of the Securities Act creates concurrent jurisdiction for federal and state courts over all such Securities Act actions. Accordingly, both state and federal courts have jurisdiction to entertain such claims. To prevent having to litigate claims in multiple jurisdictions and the threat of inconsistent or contrary rulings by different courts, among other considerations, our amended and restated certificate of incorporation further provides that the federal district courts of the United States will be the exclusive forum for resolving any complaint asserting a cause of action arising under the Securities Act. While the Delaware courts have determined that such choice of forum provisions are facially valid, a stockholder may nevertheless seek to bring a claim in a venue other than those designated in the exclusive forum provisions. In such instance, we would expect to vigorously assert the validity and enforceability of the exclusive forum provisions of our amended and restated certificate of incorporation. This may require significant additional costs associated with resolving such action in other jurisdictions and there can be no assurance that the provisions will be enforced by a court in those other jurisdictions.

These exclusive forum provisions may result in increased costs for investors to bring a claim. Further, these exclusive forum provisions may limit a stockholder's ability to bring a claim in a judicial forum that it finds favorable for disputes with us or our directors, officers, or other employees, which may discourage lawsuits against us and our directors, officers and other employees. If a court were to find either exclusive forum provision in our amended and restated certificate of incorporation to be inapplicable or unenforceable in an action, we may incur further significant additional costs associated with resolving the dispute in other jurisdictions, all of which could seriously harm our business.

***Provisions in our corporate charter documents and under Delaware law could make an acquisition of us, which may be beneficial to our stockholders, more difficult and may prevent attempts by our stockholders to replace or remove our current management.***

Provisions in our certificate of incorporation and our bylaws may discourage, delay, or prevent a merger, acquisition, or other change in control of our company that stockholders may consider favorable, including transactions in which you might otherwise receive a premium for your shares. These provisions could also limit the price that investors might be willing to pay in the future for shares of our common stock, thereby depressing the trading price of our common stock. In addition, because our board of directors is responsible for appointing the members of our management team, these provisions may frustrate or prevent any attempts by our stockholders to replace or remove our current management by making it more difficult for stockholders to replace members of our board of directors. Among other things, these provisions:

- establish a classified board of directors such that only a portion of our directors stand for election at any given annual stockholder meeting;
- allow the authorized number of our directors to be changed from time to time by our stockholders or our board of directors;
- limit the manner in which stockholders can remove directors from our board of directors;
- establish requirements for stockholder proposals that can be acted on at stockholder meetings;
- require that stockholder actions must be effected at a duly called stockholder meeting and allow actions by our stockholders by written consent, with certain requirements;
- limit who may call stockholder meetings; and
- authorize our board of directors to issue preferred stock without stockholder approval, which could be used to institute a "poison pill" that would work to dilute the stock ownership of a potential hostile acquirer, effectively preventing acquisitions that have not been approved by our board of directors.

Moreover, because we are incorporated in Delaware, we are governed by the provisions of Section 203 of the DGCL, which prohibits a person who owns in excess of 15% of our outstanding voting stock from merging or combining with us for a period of three years after the date of the transaction in which the person acquired in excess of 15% of our outstanding voting stock, unless the merger or combination is approved in a prescribed manner.

## General risks

### ***Unstable market and economic conditions may have serious adverse consequences on our business and financial condition.***

The global economy, including credit and financial markets, has experienced extreme volatility and disruptions recently, including, among other things, recent tariff announcements, diminished liquidity and credit availability, declines in consumer confidence, declines in economic growth, supply chain shortages, increases in inflation rates, higher interest rates, and uncertainty about economic stability. The Federal Reserve has raised interest rates multiple times in response to concerns about inflation and it may raise them again. Higher interest rates, coupled with reduced government spending and volatility in financial markets, may increase economic uncertainty and affect consumer spending. Similarly, the ongoing military conflicts in Ukraine and the Middle East and increasing tensions between China and Taiwan have created extreme volatility in the global capital markets and may have further global economic consequences, including disruptions of the global supply chain. Any such volatility and disruptions may adversely affect our business or the third parties on whom we rely. If the equity and credit markets deteriorate, including as a result of new tariffs, political unrest or war, it may make any necessary debt or equity financing more difficult to complete, more costly, and more dilutive. Failure to secure any necessary financing in a timely manner and on favorable terms could have a material adverse effect on our growth strategy, financial performance and share price and could require us to delay or abandon development or commercialization plans. In addition, there is a risk that one or more of our service providers, manufacturers or other partners would not survive or be able to meet their commitments to us under such circumstances, which could directly affect our ability to attain our operating goals on schedule and on budget. We have experienced and may in the future experience disruptions as a result of such macroeconomic conditions, including delays or difficulties in initiating or expanding clinical trials and manufacturing sufficient quantities of materials. Any one or a combination of these events could have a material and adverse effect on our results of operations and financial condition.

### ***If we fail to maintain proper and effective internal controls, our ability to produce accurate financial statements on a timely basis could be impaired.***

We are subject to the reporting requirements of the Exchange Act, the Sarbanes-Oxley Act of 2002, or the Sarbanes-Oxley Act, and the rules and regulations of Nasdaq Stock Market. The Sarbanes-Oxley Act requires, among other things, that we maintain effective disclosure controls and procedures and internal control over financial reporting. In addition, Section 404 of the Sarbanes-Oxley Act requires our management and independent registered public accounting firm to report on the effectiveness of our internal control over financial reporting. We are also required to disclose changes made in our internal controls and procedures on a quarterly basis. However, for so long as we remain an emerging growth company as defined in the JOBS Act, we intend to take advantage of certain exemptions from various reporting requirements that are applicable companies that are not emerging growth companies, including, but not limited to, not being required to comply with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act. Once we are no longer an emerging growth company or, if prior to such date we opt to no longer take advantage of the applicable exemption, we will be required to include an opinion from our independent registered public accounting firm on the effectiveness of our internal control over financial reporting. An independent assessment of the effectiveness of our internal controls over financial reporting could detect problems that our management's assessment might not.

The requirements of these rules and regulations are difficult, time-consuming and costly, and place significant strain on our personnel, systems and resources. Effective internal control over financial reporting are necessary for us to provide reliable financial reports and, together with adequate disclosure controls and procedures, are designed to prevent fraud. In order to maintain and improve the effectiveness of our disclosure controls and procedures and internal control over financial reporting, we have expended and anticipate we will continue to expend significant resources, including accounting-related costs, and provide significant management oversight. Any failure to implement required new or improved controls, or difficulties encountered in their implementation could cause us to fail to meet our reporting obligations. In addition, if we are unable to continue to meet these requirements, we may not be able to remain listed on the Nasdaq Global Market.

We may identify weaknesses in our system of internal financial and accounting controls and procedures that could result in a material misstatement of our financial statements. Our internal control over financial reporting will not prevent or detect all errors and all fraud. A control system, no matter how well designed and operated, can provide only reasonable, not absolute, assurance that the control system's objectives will be met. Because of the inherent limitations in all control systems, no evaluation of controls can provide absolute assurance that misstatements due to error or fraud will not occur or that all control issues and instances of fraud will be detected. If we fail to identify material weaknesses in our internal control over financial reporting, if we are not able to comply with the requirements of Section 404 of the Sarbanes-Oxley Act in a timely manner, or if we are unable to maintain proper and effective internal controls, we may not be able to produce timely and accurate financial statements. If that were to happen, the trading price of our stock could decline and we could be subject to sanctions or investigations by the stock exchange on which our common stock is listed, the Securities and Exchange Commission, or the SEC, or other regulatory authorities.

***Our ability to utilize our net operating loss carryforwards and research tax credits to offset future taxable income may be subject to additional limitations.***

As of December 31, 2025, we had approximately \$182.0 million of U.S. federal net operating loss carryforwards, or NOLs, \$362.9 million of U.S. state and local NOLs, and \$7.2 million of federal tax credits. U.S. federal NOLs generated in taxable years beginning after December 31, 2017, do not expire and may be carried forward indefinitely, but the deductibility of such NOLs is limited to no more than 80% of current year taxable income. Our U.S. state and local NOLs begin to expire in 2040 and our federal research tax credits begin to expire in 2040.

In addition, under Sections 382 and 383 of the Internal Revenue Code of 1986, as amended, or the Code, if a corporation undergoes an “ownership change,” which is generally defined as a greater than 50% change, by value, in its equity ownership by certain stockholders over a rolling three-year period, our ability to use our pre-change NOLs and certain other pre-change tax attributes (such as research tax credits) to offset our post-change taxable income or taxes may be limited. We have determined that we experienced ownership changes on November 7, 2023 and May 28, 2025, each of which has limited our ability to utilize our federal NOLs and federal tax credits. As a result of these ownership changes, we are subject to an annual limitation of approximately \$2.4 million on our ability to utilize our federal NOLs and federal tax credits recognized before May 28, 2025, inclusive of the ownership change that occurred on November 7, 2023. Due to the limitations, no federal NOLs are expected to expire unutilized and \$10.6 million of federal tax credits that had been available to offset future tax liabilities, prior to the date of the ownership changes, are expected to expire unutilized. If we undergo additional ownership changes, and our ability to use our pre-change NOLs and other pre-change tax attributes (such as tax credits) to offset our post-change income or taxes is further limited, it may harm our future results of operations by effectively increasing our future tax obligations. U.S. state and local NOLs may be similarly limited. In addition, at the U.S. state and local level, there may be periods during which the use of NOLs is suspended or otherwise limited, which could accelerate or permanently increase U.S. state and local taxes owed.

Irrespective of the above, our ability to utilize our NOLs and research tax credits to offset future taxable income or taxes is conditioned on our attaining profitability and generating taxable income. We do not know if and when we will generate sufficient taxable income to utilize our NOLs and research tax credits.

***Changes in tax laws or regulations that are applied adversely to us or our customers may materially harm our business.***

New tax laws, statutes, rules, regulations, or ordinances could be enacted at any time. Further, existing tax laws, statutes, rules, regulations, or ordinances could be interpreted differently, changed, repealed, or modified at any time. Any such enactment, interpretation, change, repeal, or modification could adversely affect us, possibly with retroactive effect. For example, the IRA enacted a 15% minimum tax on the adjusted financial statement income of certain large U.S. corporations for taxable years beginning after December 31, 2022, as well as a 1% excise tax on stock repurchases made by public corporations after December 31, 2022. Further, the Tax Cuts and Jobs Act of 2017, or the Tax Act, enacted many significant changes in U.S. federal tax laws, some of which were further modified by the Coronavirus Aid, Relief, and Economic Security Act, or the CARES Act, and may be modified in the future by the current or a future presidential administration. Among other changes, the Tax Act amended the Code to require that certain research and experimental (“R&E”) expenditures be capitalized and amortized over five years if incurred in the United States or fifteen years if incurred in foreign jurisdictions for taxable years beginning after December 31, 2021. For tax years beginning after December 31, 2024, OBBBA was enacted that includes provisions with effective dates in 2025 and beyond, and for some small business taxpayers, certain provisions may have retroactive effects. Among other changes, the OBBBA amended the Code under which domestic R&E expenditures may be immediately deducted. The remaining unamortized domestic R&E expenditures may be deducted in full in the first taxable year, ratably over two taxable years, or can continue being amortized over the remaining life. However, foreign R&E expenditures must continue to be capitalized and amortized over fifteen years. In addition, it is uncertain if and to what extent various states will conform to the IRA, the Tax Act, the CARES Act, OBBBA, or any future U.S. federal tax laws. Changes in corporate tax rates, the realization of net deferred tax assets relating to our operations, the taxation of foreign earnings, and the deductibility of expenses could have a material impact on the value of our deferred tax assets, result in significant one-time charges, and increase our future U.S. tax expenses.

***Our business and operations would suffer in the event of system failures, cyberattacks or a deficiency in our or our CMOs’, CROs’, manufacturers’, contractors’, consultants’ or collaborators’ cybersecurity.***

In the ordinary course of our business, we and third parties with whom we conduct business, including third-party CMOs, CROs, manufacturers, contractors (including sites performing our clinical trials), consultants, and collaborators, collect and store sensitive data, including intellectual property, clinical trial data, proprietary and confidential business information, and personal data and personal information of our clinical trial subjects, employees, and others. The secure maintenance, transmission, and other processing of this information is critical to our operations.

Despite the implementation of security measures, our internal systems, as well as those of third parties on which we rely, are vulnerable to damage from, among other things, computer viruses, ransomware and other forms of malware, unauthorized access, natural disasters, terrorism, war, telecommunication and electrical failures, system malfunctions, cyberattacks or cyber-intrusions, email attachment compromise, denial of service attacks, phishing attacks, and unauthorized or otherwise improper acts or omissions by persons inside our organization, or persons with access to systems inside our organization. Any such event could lead to the prevention of access to, loss, destruction, alteration, disclosure, or dissemination of, or damage or unauthorized access to or other processing of, our data (including trade secrets or other confidential information, intellectual property, proprietary business information and personal data) or other data that is processed or maintained on our behalf, and cause disruptions to our operations, which could lead to a material disruption of our product candidate development programs. For example, the loss, corruption or unavailability of preclinical study or clinical trial data from completed, ongoing or planned trials could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data.

More generally, despite the implementation of security measures in an effort to protect our systems and data, we cannot ensure that our information technology and infrastructure or any of our relevant policies or measures will prevent breakdowns or disruptions of, or breaches or incidents impacting, our systems or those of third parties on which we rely, or other cybersecurity incidents that lead to loss, destruction, unavailability, or unauthorized alteration, dissemination or other processing of, or damage or unauthorized access to, our data, including personal data, our other assets, or other data processed or maintained on our behalf, that could delay clinical development of our product candidates and have a material adverse effect upon our reputation, business, operations or financial condition. To our knowledge, we have not experienced any security breach to date that has had a material impact on our business or operations, but we and the third parties with whom we conduct business have faced, and we anticipate continuing to face, cybersecurity risks, including risks of security breaches and incidents. Risks of security breaches and incidents and other types of system disruptions, particularly through cyberattacks or cyber intrusions, including by hackers, foreign governments, cyber terrorists, and associated actors, have increased generally as the number, intensity, and sophistication of attempted attacks and intrusions from around the world have increased. Geopolitical conflicts and tensions may also increase such risks.

Any security breach or incident resulting in any loss, destruction, unavailability, alteration, disclosure, dissemination, or other processing of, or damage or unauthorized access to, our data, systems or applications, or inappropriate disclosure or other processing of confidential or proprietary information or personal data, or any other event resulting in unauthorized access to, or disclosure or processing of, such information, or for it to be believed or reported that any of the foregoing has occurred, could lead to us facing regulatory inquiries and proceedings, incurring material legal claims and proceedings, and incurring material damage to our reputation. Any such event could also disrupt our operations, lead to delays in the further development of our product candidates, cause a loss of confidence in us and our ability to conduct clinical trials, compel us to comply with federal and state breach notification laws and foreign law equivalents, subject us to mandatory corrective action, and subject us to material liability under applicable laws, rules, regulations and standards, which could result in significant legal and financial exposure and reputational damage that could have a material adverse effect on our business, operations, and financial condition.

Notifications and follow-up actions related to a security breach or other security incident could impact our reputation and cause us to incur significant costs, including significant legal expenses and remediation costs. We expect to incur significant costs in an effort to detect and prevent security incidents, and we may face increased costs and requirements to expend substantial resources in the event of an actual or perceived security incident. However, we cannot guarantee that we will be able to detect or prevent any such incidents, or that we can remediate any such incidents in an effective or timely manner. Our efforts to improve security and protect data from compromise may also identify previously undiscovered instances of data breaches or other cybersecurity incidents. Any security breach or incident resulting in any loss, destruction, or alteration of, damage or unauthorized access to, or unauthorized or otherwise inappropriate disclosure, dissemination, or other processing of, our data, including personal data, or other information that is processed or maintained on our behalf, any significant damage to or disruption of our systems, or any perception that any of these has occurred, could expose us to litigation and governmental investigations and inquiries, lead to delays in further development and commercialization of our product candidates, and subject us to significant fines, penalties and other liabilities.

Our insurance policies may not be adequate to compensate us for the potential losses arising from any failure or other disruption of, security breach of, or incident impacting, our systems or third-party systems where information important to our business operations is stored or otherwise processed, or any other unauthorized disclosure of information. In addition, such insurance may not be available to us in the future on economically reasonable terms, or at all. Further, our insurance may not cover all claims made against us and could have high deductibles in any event, and defending a suit, regardless of its merit, could be costly and divert management attention.

***We incur increased costs and demands upon management as a result of being a public company.***

As a public company listed in the United States, we incur significant additional legal, accounting and other expenses that we did not incur as a private company, including the cost of director and officer liability insurance. These additional costs could negatively affect our financial results. In addition, changing laws, regulations and standards relating to corporate governance and public disclosure, including regulations implemented by the SEC and the Nasdaq Stock Market, may increase legal and financial compliance costs and make some activities more time-consuming. These laws, regulations and standards are subject to varying interpretations and, as a result, their application in practice may evolve over time as new guidance is provided by regulatory and governing bodies. We intend to invest resources to comply with evolving laws, regulations and standards, and this investment may result in increased general and administrative expenses and a diversion of management's time and attention from revenue-generating activities to compliance activities. If notwithstanding our efforts to comply with new laws, regulations and standards, we fail to comply, regulatory authorities may initiate legal proceedings against us and our business may be harmed.

Failure to comply with these rules might also make it more difficult for us to obtain some types of insurance, including director and officer liability insurance, and we might be forced to accept reduced policy limits and coverage or incur substantially higher costs to obtain the same or similar coverage. The impact of these events could also make it more difficult for us to attract and retain qualified persons to serve on our board of directors, on committees of our board of directors, or as members of senior management.

***Our disclosure controls and procedures may not prevent or detect all errors or acts of fraud.***

We are subject to certain reporting requirements of the Exchange Act. Our disclosure controls and procedures are designed to reasonably assure that information required to be disclosed by us in reports we file or submit under the Exchange Act is accumulated and communicated to management, recorded, processed, summarized, and reported within the time periods specified in the rules and forms of the SEC. We believe that any disclosure controls and procedures or internal controls and procedures, no matter how well conceived and operated, can provide only reasonable, not absolute, assurance that the objectives of the control system are met.

These inherent limitations include the realities that judgments in decision-making can be faulty, and that breakdowns can occur because of simple error or mistake. Additionally, controls can be circumvented by the individual acts of some persons, by collusion of two or more people, or by an unauthorized override of the controls. Accordingly, because of the inherent limitations in our control system, misstatements or insufficient disclosures due to error or fraud may occur and not be detected.

**Item 1B. Unresolved Staff Comments.**

None.

**Item 1C. Cybersecurity**

**Risk Management and Strategy**

We have established policies and processes for assessing, identifying, and managing risk from cybersecurity threats. We periodically assess risks from cybersecurity threats, including any potential unauthorized occurrence on or conducted through our information systems that may result in adverse effects on the confidentiality, integrity, or availability of our information systems or any information residing therein. Following these risk assessments, we evaluate how to reasonably address any identified gaps in existing safeguards and monitor the effectiveness of our safeguards. We devote resources and designate high-level personnel, including our Vice President, Associate General Counsel, who reports to our Chief Financial Officer, to manage the risk assessment and mitigation process in a manner that is consistent and integrated with our overall risk management processes.

As part of our overall risk management system, we monitor and test our safeguards and train our employees on these safeguards, including our cybersecurity safeguards, in collaboration with human resources, IT, and management. Personnel at all levels and departments receive periodic trainings on cybersecurity best practices and threats. For example, we conduct e-mail phishing campaigns with our employees and contractors which send phishing-style e-mails, monitor user responses, and automatically assign further training, as needed, and also provide convenient solutions for our employees to report suspicious messages.

We engage specialized third parties in connection with our risk assessment processes. These third parties assist us in designing and implementing our cybersecurity policies and procedures, as well as in monitoring and testing our safeguards.

We also conduct IT security assessments on our key third-party service providers and require them to implement and maintain reasonable security measures in connection with their work with us, and to promptly report any suspected breach of their security measures that may affect our company.

We have not previously experienced any cybersecurity risk or cybersecurity incident which has been determined to be material. For additional information regarding whether any risks from cybersecurity threats are reasonably likely to materially affect our company, including our business strategy, results of operations, or financial condition, please refer to Item 1A, “Risk Factors” in this Annual Report on Form 10-K, including the risk factor entitled “Our business and operations would suffer in the event of system failures, cyberattacks or a deficiency in our or our CMOs’, CROs’, manufacturers’, contractors’, consultants’ or collaborators’ cybersecurity”.

## **Governance**

One of the key functions of our board of directors is informed oversight of our risk management process, including risks from cybersecurity threats. Our board of directors is responsible for monitoring and assessing strategic risk exposure, and our executive officers are responsible for the day-to-day management of the material risks we face. Our board of directors administers its cybersecurity risk oversight function directly as a whole, as well as through our audit committee.

Our security governance steering committee, which includes senior representatives from key functions including Clinical Operations, Legal, Finance and Human Resources, has primary responsibility for assessing and managing our risks from cybersecurity threats. Our security governance steering committee oversees our cybersecurity policies and processes, including those described in “Risk Management and Strategy” above. The day-to-day implementation of our cybersecurity policies and processes is primarily overseen by our Vice President, Associate General Counsel in consultation with our Chief Financial Officer and external advisors including our IT and Cybersecurity consultant. Our Vice President, Associate General Counsel has more than a decade of advising on risk management and compliance in the highly regulated biopharmaceutical industry. Our IT and Cybersecurity consultant has over 20 years of experience with IT, with an emphasis on cybersecurity over the past four years. Our management is informed about the prevention, detection, mitigation, and remediation of cybersecurity incidents through support by our security governance steering committee which, in consultation with external advisors, oversees, works with, and delegates daily operations to the IT/IS and other teams. Our information security policies are developed and implemented, and are reviewed at least annually with updates authorized and approved, by the security governance steering committee.

Representatives from our security governance steering committee provide periodic briefings to the audit committee regarding our company’s cybersecurity risks and activities, including any recent cybersecurity incidents and related responses, cybersecurity systems testing, activities of third parties, and the like. Our audit committee provides updates to the board of directors on such reports.

## **Item 2. Properties.**

Our corporate headquarters is located at 345 Park Avenue South, 6<sup>th</sup> Floor, New York, NY 10010, where we lease a total of approximately 15,839 square feet of office and laboratory space, excluding the additional approximate 11,588 square feet that we began to lease in the first quarter of 2026, used for our research and development activities, general and administrative activities and other activities. This lease agreement began in April 2022 and expires in July 2029 with an additional five-year option to extend at the then-prevailing effective market rental rate. In March 2026, the original lease agreement was amended to include an additional approximate 11,588 square feet of leased office and laboratory space at the same address as the original headquarters lease and to be used for the same purposes. The amended lease agreement began in March 2026 with an initial term of three months, which will automatically renew for an additional three-month period(s) unless either party provides written notice to terminate the agreement.

We believe that our current facilities are sufficient to meet our current and near term needs and that suitable additional or substitute space will be available at commercially reasonable terms as and when needed.

## **Item 3. Legal Proceedings.**

From time to time, we may become involved in legal proceedings arising in the ordinary course of our business. On October 12, 2023, Rocket filed a lawsuit in the U.S. District Court for the Southern District of New York against Lexeo and two former employees claiming, among other things, misappropriation of confidential information and trade secrets. The complaint alleges that while employees of Rocket, the individual defendants downloaded confidential Rocket company documents and other proprietary materials and that Lexeo used this information to advance its programs after the individuals became employed by Lexeo. The complaint seeks unspecified damages and injunctive relief. In August 2024, we asserted counterclaims against Rocket and Spacecraft Seven LLC, a wholly owned subsidiary of Rocket, for misappropriation of trade secrets, correction of inventorship of certain PKP2-ACM patents, breach of contract, and tortious interference with contract in connection with their diligence of a PKP2-ACM program at UCSD that was later licensed by Lexeo. Our counterclaims seek equitable relief, damages, attorneys' fees and costs from Rocket and Spacecraft Seven LLC. In June 2025, the litigation was resolved amicably and without admission of liability by any party, and all claims have been dismissed with prejudice.

## **Item 4. Reserved.**

## PART II

### **Item 5. Market for Registrant’s Common Equity, Related Stockholder Matters and Issuer Purchases of Equity Securities.**

#### **Market Information**

Our common stock has been listed on the Nasdaq Global Market under the symbol “LXEO” since November 3, 2023. Prior to that date there was no public trading market for our common stock.

#### **Holders**

As of March 27, 2026, there were approximately 19 registered holders of record of our common stock. We believe a greater number of holders of our common stock are “street name” or beneficial holders, whose shares of record are held by banks, brokers and other financial institutions.

#### **Dividend Policy**

We have never declared or paid, and do not anticipate declaring or paying, in the foreseeable future, any cash dividends on our capital stock. We currently intend to retain all available funds and any future earnings to support our operations and finance the growth and development of our business. Any future determination related to our dividend policy will be made at the discretion of our board of directors and will depend upon, among other factors, our results of operations, financial condition, capital requirements, contractual restrictions, business prospects and other factors our board of directors may deem relevant.

#### **Recent Sales of Unregistered Securities; Use of Proceeds**

There have been no unregistered sales of securities other than previously disclosed by us in our Current Report on Form 8-K, as filed with the SEC on October 17, 2025.

#### **Item 6. Reserved**

## Item 7. Management's Discussion and Analysis of Financial Condition and Results of Operations.

*You should read the following discussion and analysis of our financial condition and results of operations together with the financial statements and related notes included elsewhere in this Annual Report on Form 10-K. This discussion contains forward-looking statements based upon current expectations that involve risks and uncertainties. Our actual results may differ materially from those anticipated in these forward-looking statements as a result of various factors, including those discussed in "Item 1A. Risk Factors" and in other parts of this Annual Report on Form 10-K.*

### Overview

We are a clinical stage genetic medicine company dedicated to reshaping heart health by applying pioneering science to fundamentally change how cardiovascular diseases are treated. We are advancing a portfolio of therapeutic candidates that take aim at the underlying genetic causes of conditions, including FA cardiomyopathy, plakophilin-2, or PKP2, arrhythmogenic cardiomyopathy, or ACM, and other devastating diseases with high unmet need.

### *LX2006 for the treatment of FA cardiomyopathy*

Our most advanced cardiovascular product candidate, LX2006 for the treatment of FA cardiomyopathy, is currently being evaluated in SUNRISE-FA, our ongoing Phase 1/2 clinical trial and in a Cornell investigator-initiated trial.

### *Interim Clinical Data Updates*

In October 2025, we provided an interim clinical update, which included baseline characteristics and safety data from the 17 treated participants across the two studies, and clinical efficacy data from the 16 participants who had reached at least six months follow-up as of that time. A summary of key observations from the October 2025 interim update is set forth below.

- Improvements in LVMI among participants with abnormal baseline LVMI (n=6). Five of six participants achieved >10% by 12-month visit or sooner and six of six participants achieved LVMI measurements within the normal range as of latest visit. A 18% mean improvement in LVMI was observed at 6-month visit and a 23% mean improvement in LVMI was observed at 12-month visit, exceeding 10% FDA-aligned threshold for pivotal study. In the mid- and high-dose cohorts (n=3), a 28% mean improvement in LVMI was observed at 6-month visit and a 33% mean improvement in LVMI was observed at 12-month visit, suggesting dose-dependent improvement. Among participants with normal baseline LVMI (n=10), the majority demonstrated LVMI improvement or stabilization over time.
- Improvements in secondary cardiac biomarkers. Fourteen of sixteen participants achieved >25% reduction in high-sensitivity troponin I from baseline at latest visit. Fourteen of sixteen participants achieved reduction or stabilization in LWT from baseline at latest visit.
- Improvements in mFARS as demonstrated by a 2.0-point mean improvement from baseline at latest visit across all participants. Eleven of sixteen participants achieved reduction or stabilization in mFARS from baseline at latest visit.
- Previously reported data in April 2025 from ongoing SUNRISE-FA trial (n=8) showed that all study participants achieved increases in frataxin protein expression from baseline at 3-month visit, with dose-dependent increases observed across cohorts.

Based on the data observed as of the October 2025 update, LX2006 remained generally well tolerated with no Grade 3+ SAEs and no signs of complement activation or other immunogenicity.

### *Regulatory Alignment*

In February 2025, we announced further alignment on elements of the accelerated development pathway following a Type B RMAT meeting with the FDA including frataxin expression co-primary endpoint to be evaluated for any increase from baseline rather than numerical threshold.

In July 2025, we announced Breakthrough Therapy designation based on interim clinical data from Phase 1/2 trials showing clinically meaningful improvements in cardiac biomarkers and functional measures. We also announced that LX2006 was selected for the CDRP Program, created to facilitate CMC registrational readiness and support faster patient access.

In October 2025, in response to our questions regarding the possibility of a faster path to BLA, the FDA indicated openness to a BLA submission for accelerated approval that includes clinical data from the ongoing Phase 1/2 studies of LX2006 pooled with new clinical data to be generated in the planned pivotal study. The FDA also previously agreed to evaluate the co-primary endpoint of LVMI at a time point earlier than 12 months. Collectively, we believe this regulatory feedback has the potential to reduce the size and length of the planned pivotal study, possibly accelerating the overall timeline to BLA submission. We plan to initiate the LX2006 pivotal study in the first half of 2026, pending finalization of the trial protocol.

#### ***LX2020 for the treatment of arrhythmogenic cardiomyopathy***

Our second most advanced cardiovascular product candidate, LX2020 for the treatment of arrhythmogenic cardiomyopathy, or ACM, caused by mutations in the PKP2 gene, referred to as PKP2-ACM, is currently being evaluated in HEROIC-PKP2, an ongoing Phase 1/2 clinical trial.

#### ***Interim Clinical Data Updates***

In October 2025, we provided an interim clinical data update for the three participants dosed in cohort 1 (n=2 at 12-month visit; n=1 at 9-month visit), assessing multiple cardiac parameters at latest visit relative to baseline. A summary of key observations from the October 2025 interim update is set forth below.

- Premature ventricular contractions, or PVCs, were reduced in one of three participants and non-sustained ventricular tachycardia, or NSVT, was reduced or stable in two of three participants.
- QRS duration was normalized or stable in two of three participants and T-wave inversions were reduced in two of three participants.
- Left ventricular ejection fraction, or LVEF, and right ventricular ejection fraction, or RVEF, were stable in three of three participants.

In January 2026, we provided an interim clinical data update, which included baseline characteristics and safety data from the ten participants dosed, including three participants in cohort 1 at the low dose and seven participants in cohorts 2 and 3 at the high dose. Clinical efficacy data were inclusive of those participants with at least 6 months of follow-up (n=8). A summary of key observations from the January 2026 interim update is set forth below.

- Arrhythmia burden stabilized or improved in majority of participants with dose-dependent response in NSVT and PVCs. NSVT reduced or stabilized in the majority of participants, with a 22% mean improvement in high-dose cohorts at latest visit (n=5). PVCs reduced or stabilized in the majority of participants, with a 14% mean improvement in high-dose cohorts at latest visit.
- Four of five participants in high-dose cohorts report improvement relative to baseline on the PGIC scale, a patient-reported outcome measure.
- Participants stable across other clinical measures including QRS duration, T-wave inversion, RVEF and NYHA Class.
- Increases in PKP2 protein expression versus baseline at three months in all participants that had undergone cardiac biopsies (n=7) assessed by western blot. Dose-dependent increases observed, with mean increase of 93% in the low-dose cohort (n=2) and 162% mean increase in the high-dose cohorts (n=5).

Based on the data observed as of the October 2025 and January 2026 readouts, LX2020 remained generally well tolerated with no clinically significant complement activation.

Each of our gene therapy candidates utilizes the vector construct, dose and route of administration that we believe will result in the most favorable biodistribution and safety profile for our product candidate for each disease. Our most advanced programs use the AAVrh10 vector due to its high transduction efficiency in myocardial cells, potential for lower toxicity given the opportunity to utilize lower doses compared to other well-established AAV serotypes, and low pre-existing immunity.

#### **Current and Future Capital Requirements**

To date, we have funded our operations primarily through proceeds from the sale of shares of our convertible preferred stock, common stock, and pre-funded warrants and warrants to purchase our common stock, including from our October 2025 Financing Transactions, May 2025 Private Placement, March 2024 Private Placement, IPO and the subsequent partial exercise of the underwriters' 30-day option to purchase additional shares of common stock. As of December 31, 2025, we had \$246.6 million of cash, cash equivalents, and investments in U.S. Treasury securities. We have incurred significant operating losses since the commencement of our operations. Our ability to generate product revenue sufficient to achieve profitability will depend heavily on the successful development and

eventual commercialization of one or more of our current gene therapy candidates or any future gene therapy candidates. Our net losses for the years ended December 31, 2025 and December 31, 2024 were \$100.0 million and \$98.3 million, respectively, and our accumulated deficit was \$380.1 million at December 31, 2025. We expect to continue to incur significant losses for the foreseeable future as we advance our current and future product candidates through preclinical and clinical development, continue to build our operations and operate as a public company.

We expect to continue to incur net operating losses for at least the next several years, and we expect our research and development expenses, general and administrative expenses, and capital expenditures to continue to increase. We expect our expenses and capital requirements will increase significantly in connection with our ongoing activities as we:

- continue our ongoing and planned clinical trials as well as research and development of our FA cardiomyopathy (LX2006) and arrhythmogenic cardiomyopathy caused by mutations in the PKP2 gene, or PKP2-ACM (LX2020) programs and other product candidates;
- initiate preclinical studies and clinical trials for any additional product candidates that we may pursue in the future;
- seek to discover and develop additional product candidates and further expand our clinical product pipeline;
- seek regulatory approvals for any product candidates that successfully complete clinical trials;
- invest in capital equipment in order to expand our research and development and manufacturing activities;
- attract, hire and retain additional clinical, scientific, quality control, regulatory, and manufacturing management and administrative personnel;
- add clinical, operational, financial and management information systems and personnel, including personnel to support our product development;
- develop, maintain, expand, protect and enforce our intellectual property portfolio, including patents, trade secrets and know-how;
- acquire or in-license other product candidates and technologies;
- expand our operations in the United States and to other geographies;
- incur additional legal, accounting, investor relations and other general and administrative expenses associated with operating as a public company; and
- establish a sales, marketing and distribution infrastructure, either ourselves or in partnership with others, to commercialize any product candidates, if approved, and related additional commercial manufacturing costs.

## **Components of our results of operations**

### ***Revenue***

To date, we have not generated any revenue from product sales. If our development efforts for LX2006 and LX2020, or any future product candidates, are successful and result in regulatory approval, or if we enter into collaboration or license agreements with third parties, we may generate revenue in the future from product sales, royalties or payments from such collaboration or license agreements, or a combination of product sales and payments from such agreements.

### ***Operating expenses***

#### ***Research and development***

Research and development expenses consist of costs incurred for our research activities, including our discovery efforts and the preclinical and clinical development of our programs. These expenses include:

- employee-related expenses, including salaries, benefits, and stock-based compensation expense for employees engaged in research and development functions;

- expenses incurred under agreements with third parties, such as consultants, clinical investigators, contractors and CROs that assist with (i) identification of potential product candidates in discovery platforms and (ii) the preclinical and clinical studies of our product candidates;
- the cost of developing and scaling our manufacturing process and manufacturing product candidates for use in our research, preclinical studies and clinical trials, including under agreements with third parties, such as consultants, contractors and CMOs;
- costs to maintain compliance with FDA and other regulatory requirements;
- laboratory supplies and research materials;
- facilities, depreciation, and other expenses, which include direct and allocated expenses for rent and maintenance of facilities;
- payments made under our licensing agreements with third parties, including milestone payments; and
- other expenses incurred as a result of research and development activities.

We record research and development costs as expenses when incurred. Nonrefundable advance payments for goods and services that will be used in future research and development activities are expensed when the activity has been performed or when the goods have been received rather than when the payment is made. When third-party service providers' billing terms do not coincide with our period-end, we are required to make estimates of our obligations to those third parties incurred in a given accounting period and record accruals at the end of the period. We base these estimates on our knowledge of the research and development programs, services performed for the period, past history for related activities and the expected duration of the third-party service contract, where applicable. If timelines or contracts are modified based upon changes in the scope of work to be performed, we modify our estimates of accrued expenses accordingly on a prospective basis; therefore, actual results could differ from our estimates. Upfront payments under license agreements are expensed upon receipt of the license, and annual maintenance fees under license agreements are expensed in the period in which they are incurred. Milestone payments under license agreements are accrued, with a corresponding expense being recognized, in the period in which the milestone is determined to be probable of achievement and the related amount is reasonably estimable.

Our direct research and development expenses are tracked on a program-by-program basis and consist primarily of external costs, such as fees paid to CROs, CMOs, central laboratories and certain outside consultants in connection with our research and discovery, preclinical development, process development, manufacturing, clinical development, clinical trials, regulatory and quality assurance activities. We do not allocate professional services costs and licensing fees and other similar costs to specific programs because these costs are deployed across multiple programs.

Research and development activities are central to our business model and account for a significant portion of our operating expenses. Product candidates in later stages of clinical development generally have higher development costs than those in earlier stages of clinical development, primarily due to the increased size and duration of later-stage clinical trials. As a result, we expect our research and development expenses to increase for the foreseeable future as we further advance LX2006 and LX2020, and any other future product candidates that we may develop, into and through preclinical studies and clinical trials and pursue regulatory approvals. We cannot determine with certainty the timing of initiation, the duration or the completion costs of current or future preclinical studies and clinical trials of our product candidates due to the inherently unpredictable nature of preclinical and clinical development. Clinical and preclinical development timelines, the probability of success and development costs can differ materially from expectations. We anticipate that we will make determinations as to which product candidates to pursue and how much funding to direct to each product candidate on an ongoing basis in response to the results of ongoing and future research, preclinical studies and clinical trials, regulatory developments and our assessments as to each product candidate's commercial potential. In addition, we cannot forecast whether any of our current or future product candidates will be subject to future collaborations, when such arrangements will be secured, if at all, and to what degree such arrangements would affect our development plans and capital requirements. We are also unable to predict when, if ever, we will generate revenue from our product candidates to offset our expenses.

#### *General and administrative*

General and administrative expenses consist primarily of personnel expenses, including salaries, benefits and stock-based compensation expense for personnel in executive, accounting, business development, legal, human resources and administrative functions. General and administrative expenses also include corporate facility costs not otherwise included in research and development expenses, depreciation, and other expenses, which include direct or allocated expenses for rent and maintenance of facilities and insurance, not otherwise included in research and development expenses, as well as professional fees for legal, consulting, investor and public relations, accounting and audit services.

We expect that in the near to medium term future our general and administrative expenses will decrease relative to those incurred during the year ended December 31, 2025 on an annualized basis, primarily due to lower anticipated third-party legal fees and related costs, while supporting the continued research and development of our programs and the growth of our business.

***Gain on long-term investment***

Gain on long-term investment includes non-operating income related to an unrealized gain on convertible preferred stock received that was recorded at fair value to long-term investment.

***Other income (expense), net***

Other income (expense), net includes foreign exchange gains and (losses).

***Interest expense***

Interest expense is primarily associated with our finance right of use asset equipment leases.

***Interest income***

Interest income is primarily related to interest earned from our investments in U.S. government money market funds and interest earned from our investments in U.S. Treasury securities, as well as interest earned on interest-bearing demand deposit cash accounts and a cash collateral account.

***(Amortization of premium) accretion of discount on investments in U.S. Treasury securities, net***

Amortization of premium and accretion of discount on investments consists of amortization of premium and accretion of discount on our investments in U.S. Treasury securities.

***Income taxes***

Provision for income taxes consists of U.S. federal and state income taxes in the jurisdictions where we conduct business. Since our inception, we have not recorded any income tax benefits for the net losses we have incurred in each year or for our research and development tax credits, as we believe, based upon the weight of available evidence, that it is more likely than not that all of our net operating loss, or NOL, carryforwards and tax credits will not be realized. Accordingly, we have recorded a full valuation allowance against our net deferred tax assets at December 31, 2025 and December 31, 2024. As of December 31, 2025 and December 31, 2024 we had no unrecognized tax benefits.

## Results of operations

### Comparison of the Years Ended December 31, 2025 and 2024

The following table summarizes our results of operations for the years ended December 31, 2025 and 2024 (in thousands):

	2025	Years Ended December 31, 2024	Change
Operating expenses			
Research and development	\$ 63,797	\$ 74,091	\$ (10,294)
General and administrative	45,460	31,675	13,785
Total operating expenses	<u>109,257</u>	<u>105,766</u>	<u>3,491</u>
Operating loss	<u>(109,257)</u>	<u>(105,766)</u>	<u>(3,491)</u>
Other income and expense			
Gain on long-term investment	3,390	-	3,390
Other expense, net	(18)	(9)	(9)
Interest expense	(95)	(137)	42
Interest income	6,130	7,556	(1,426)
(Amortization of premium) accretion of discount on investments in U.S. Treasury securities, net	(111)	23	(134)
Total other income and expense	<u>9,296</u>	<u>7,433</u>	<u>1,863</u>
Loss from operations before income taxes	<u>(99,961)</u>	<u>(98,333)</u>	<u>(1,628)</u>
Income taxes	-	-	-
Net Loss	<u>\$ (99,961)</u>	<u>\$ (98,333)</u>	<u>\$ (1,628)</u>

### Research and development expenses

The following table summarizes our research and development expenses incurred for the years ended December 31, 2025 and 2024 (in thousands):

	2025	Years Ended December 31, 2024	Change
Direct external research and development expenses by program:			
LX2006	\$ 18,163	\$ 14,256	\$ 3,907
LX2020	15,413	23,451	(8,038)
LX1001	1,168	4,501	(3,333)
Other programs	2,075	3,424	(1,349)
Total direct external research and development expenses by program	<u>36,819</u>	<u>45,632</u>	<u>(8,813)</u>
Unallocated research and development expenses:			
Employee and stock-based compensation expenses	20,707	22,011	(1,304)
Lab-related costs and supplies	1,550	1,367	183
Professional fees	1,701	2,030	(329)
Other unallocated costs, including facilities	3,020	3,051	(31)
Total unallocated research and development expenses:	<u>26,978</u>	<u>28,459</u>	<u>(1,481)</u>
Total research and development expenses	<u>\$ 63,797</u>	<u>\$ 74,091</u>	<u>\$ (10,294)</u>

The net decrease of \$10.3 million in total research and development expenses for the year ended December 31, 2025 compared to the year ended December 31, 2024 was primarily due to decreases in (i) milestone expenses of \$6.1 million, consisting primarily of a \$6.0 million development milestone for our LX2020 program that was achieved during the third quarter of 2024 and subsequently paid to the selling shareholders of Stelios, (ii) clinical trial costs of \$3.2 million excluding an adjustment that reduced estimated accrued clinical trial expenses in 2024 by \$2.2 million for our LX1001 program, (iii) employee and stock-based compensation costs of \$1.3 million primarily due to decreased headcount, (iv) CMC expenses of \$1.2 million, and (v) license fees of \$0.6 million related to the Third Cornell License Agreement that were incurred in 2024. These decreases were partially offset by an adjustment that reduced estimated accrued clinical trial expenses in 2024 by \$2.2 million for our LX1001 program.

### *General and administrative expenses*

The net increase of \$13.8 million in general and administrative expenses for the year ended December 31, 2025 compared to the year ended December 31, 2024 was primarily due to increases in third-party legal fees and associated costs of \$11.7 million, as well as employee compensation costs of \$1.9 million primarily due to increased headcount.

### *Gain on long-term investment*

We recorded non-operating income of approximately \$3.4 million during the year ended December 31, 2025 related to an unrealized gain on convertible preferred stock received that was recorded at fair value to long-term investment.

### *Interest income*

We recognized interest income of \$6.1 million and \$7.6 million for the years ended December 31, 2025 and December 31, 2024, respectively, primarily related to interest earned on our investments in U.S. government money market funds and our investments in U.S Treasury securities, with the decrease primarily due to lower interest rates.

## **Liquidity and capital resources**

### ***Sources of liquidity***

Since our inception, we have not generated any revenue from product sales and have incurred significant operating losses and negative cash flows from our operations. We expect to incur significant expenses and operating losses for the foreseeable future as we advance the clinical development of our product candidates. Since our inception through December 31, 2025, we funded our operations primarily through proceeds from the sale of shares of our convertible preferred stock, common stock, and pre-funded warrants and warrants to purchase our common stock, including from our October 2025 Financing Transactions, May 2025 Private Placement, March 2024 Private Placement, IPO and the subsequent partial exercise of the underwriters' 30-day option to purchase additional shares of common stock, with estimated net proceeds since our inception totaling \$594.9 million. As of December 31, 2025 and December 31, 2024, we had cash, cash equivalents, and investments in U.S. Treasury Securities of \$246.6 million and \$128.5 million, respectively.

On March 24, 2025 we filed a prospectus supplement to the prospectus which forms a part of our effective shelf registration statement on Form S-3, dated December 13, 2024, which registers the offering, issuance, and sale of up to \$75.0 million of common stock pursuant to our ATM Program. As of December 31, 2025, we have not sold any shares of common stock pursuant to the ATM Program. In October 2025, we raised \$153.8 million in equity capital from the October 2025 Financing Transactions, receiving net proceeds of \$143.9 million after transaction costs.

We believe that our cash, cash equivalents, and investments in U.S. Treasury securities balances will be sufficient to fund our planned operating expenses and capital expenditure requirements into 2028. Our total future capital requirements will depend on many factors and are subject to the risks and uncertainties set forth in "*Item 1A Risk Factors.*"

### ***Cash flows***

The following table summarizes our sources and uses of cash for the years ended December 31, 2025 and December 31, 2024 (in thousands):

	Years Ended December 31,	
	2025	2024
Net cash used in operating activities	\$ (98,561)	\$ (81,151)
Net cash used in investing activities	(90,252)	(94,077)
Net cash provided by financing activities	216,795	88,776
Net increase (decrease) in cash	\$ 27,982	\$ (86,452)

### *Operating activities*

During the year ended December 31, 2025, net cash used in operating activities consisted primarily of our net loss of \$100.0 million, which included an unrealized gain of \$3.4 million recorded to other non-operating income upon receipt of our long-term investment in convertible preferred stock, in addition to \$9.5 million of net cash used in changes in operating assets and liabilities, which were partially offset by \$12.1 million of stock-based compensation expense and \$2.1 million of depreciation expense and amortization of operating and finance right-of-use assets.

During the year ended December 31, 2024, net cash used in operating activities consisted primarily of our net loss of \$98.3 million, which was partially offset by (i) \$12.5 million of stock-based compensation expense, (ii) \$2.7 million of net cash provided by changes in operating assets and liabilities, and (iii) \$2.0 million of depreciation expense and amortization of operating and finance right-of-use assets.

#### *Investing activities*

During year ended December 31, 2025, net cash used in investing activities was \$90.3 million and consisted primarily of purchases of investments in U.S. Treasury securities of \$195.4 million, which was partially offset by proceeds from sales or maturities of investments in U.S. Treasury securities of \$105.5 million and purchases of lab equipment of \$0.4 million.

During the year ended December 31, 2024, net cash used in investing activities was \$94.1 million and consisted primarily of the purchase of investments in U.S. Treasury securities and lab equipment.

#### *Financing activities*

During the year ended December 31, 2025, net cash provided by financing activities consisted primarily of the net proceeds received from the October 2025 Financing Transactions of \$143.9 million, the May 2025 Private Placement of \$73.1 million, as well as proceeds received from common stock issuances under the 2023 ESPP (as defined below) of \$0.3 million and proceeds received from the exercise of stock options of \$0.1 million, which were partially offset by \$0.5 million of principal payments made on equipment finance leases.

During the year ended December 31, 2024, net cash provided by financing activities consisted primarily of the net proceeds received from the March 2024 Private Placement of \$88.7 million, as well as \$0.5 million of proceeds received from the exercise of stock options, which were partially offset by \$0.4 million of principal payments made on equipment finance leases.

We expect our expenses and capital requirements to increase significantly in connection with our ongoing activities, particularly as we advance our lead product candidates and other development programs. Accordingly, we will continue to require substantial additional funding to support our continuing operations.

The timing and amount of our future operating and capital requirements will largely depend on many factors, including:

- the initiation, scope, progress, timing, results and costs of product discovery, preclinical studies and clinical trials for our product candidates or any future candidates we may develop;
- our ability to maintain our relationships with Cornell University, Adverum, The Regents of UCSD, and any other key licensors or collaborators;
- the scope, prioritization and number of our research and development programs;
- the costs, timing and outcome of seeking and obtaining regulatory approvals from the FDA and comparable foreign regulatory authorities, including the potential for such authorities to require that we perform more preclinical studies or clinical trials than those that we currently expect or change their requirements on studies that had previously been agreed to;
- our ability to establish and maintain collaborations on favorable terms, if at all;
- the achievement of milestones or occurrence of other developments that trigger payments under any collaboration agreements we have or may enter into;
- the extent to which we are obligated to reimburse, or entitled to reimbursement of, clinical trial costs under collaboration agreements, if any;
- the costs to establish, maintain, expand, enforce and defend the scope of our intellectual property portfolio, including preparing, filing and prosecuting patent applications, maintaining and enforcing our intellectual property rights and defending intellectual property-related claims;
- the extent to which we acquire or in-license other product candidates and technologies;
- the costs of securing manufacturing arrangements for commercial production;

- the costs of establishing or contracting for sales and marketing capabilities if we obtain regulatory approvals to market our product candidates; and
- our need to implement additional internal systems and infrastructure.

We may be unable to raise additional funds or enter into potential collaborations, strategic partnerships or marketing, distribution, licensing or other similar agreements or arrangements on favorable terms, or at all. To the extent that we raise additional capital through the sale of equity or convertible debt securities, your stockholder ownership interest will be diluted. If we fail to raise capital or enter into such agreements or arrangements as, and when, needed, we may have to significantly delay, scale back or discontinue the development or commercialization of our product candidates or grant rights to develop and market product candidates that we would otherwise prefer to develop and market ourselves.

### **Critical accounting policies and significant judgments and estimates**

Our management's discussion and analysis of our financial condition and results of operations are based on our financial statements, which have been prepared in accordance with U.S. generally accepted accounting principles, or U.S. GAAP. The preparation of our financial statements and related disclosures requires us to make estimates and judgments that affect the reported amounts of assets, liabilities, expenses, and the disclosure of our contingent liabilities in our financial statements. We base our estimates on historical experience, known trends and events and various other factors that we believe are 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. We evaluate our estimates and assumptions on an ongoing basis. Our actual results may differ from these estimates under different assumptions or conditions.

While our significant accounting policies are described in more detail in Note 2 to our audited financial statements included elsewhere in this Annual Report, we believe that the following accounting policies are those most critical to the judgments and estimates used in the preparation of our audited financial statements.

#### ***Research and development***

As part of the process of preparing our financial statements, we are required to estimate our accrued research and development expenses. This process involves estimating the level of service performed and the associated cost incurred for the service when we have not yet been invoiced or otherwise notified of actual costs. The majority of our service providers invoice us in arrears for services performed on a pre-determined schedule or when contractual milestones are met; however, some require advance payments. We make estimates of our accrued expenses as of each balance sheet date in the financial statements based on facts and circumstances known to us at that time. At each period end, we corroborate the accuracy of these estimates with the service providers and make adjustments, if necessary. Examples of estimated accrued research and development expenses include those related to fees paid to:

- vendors in connection with discovery and preclinical development activities;
- CROs in connection with preclinical studies and testing; and
- CMOs in connection with the process development and scale up activities and the production of materials.

We record the expense and accrual related to contract research and manufacturing based on our estimates of the services received and efforts expended considering a number of factors, including our knowledge of the progress towards completion of the research, development, and manufacturing activities; invoicing to date under contracts; communication from the CROs, CMOs, and other companies of any actual costs incurred during the period that have not yet been invoiced; and the costs included in the contracts and purchase orders. The financial terms of these agreements are subject to negotiation, vary from contract to contract, and may result in uneven payment flows. There may be instances in which payments made to our vendors will exceed the level of services provided and result in a prepayment of the expense. In accruing service fees, we estimate the time period over which services will be performed and the level of effort to be expended in each period. If the actual timing of the performance of services or the level of effort varies from the estimate, we adjust the accrual or the amount of prepaid expense 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 may result in reporting amounts that are too high or too low in any particular period. To date, there have not been any material adjustments to our prior estimates of accrued research and development expenses; however, we cannot guarantee that such adjustments will not be made in the future.

### ***Stock option awards***

We recognize share-based compensation expense related to stock option awards granted based on the estimated fair value of the awards on the date of grant using a Black-Scholes option pricing model. The grant date fair value of the share-based awards is recognized on an accelerated basis under the graded vesting method over the requisite service period of the award, which is the vesting period of the respective awards. Share-based payments to non-employees issued in exchange for services are based upon the fair value of the equity instruments issued. Compensation expense for stock options issued to non-employees is calculated using the Black-Scholes option pricing model and is recorded over the requisite service performance period.

The Black-Scholes option-pricing model requires the use of highly subjective assumptions, which are used to determine the fair value stock option awards granted. These assumptions include:

*Expected Term.* The expected term represents the period that our stock option awards granted are expected to be outstanding and is determined using the simplified method (based on the mid-point between the vesting date and the end of the contractual term).

*Expected Volatility.* The expected stock price volatility is primarily based on the historical volatility of a publicly traded set of peer companies for stock options granted since the IPO, each for a period equal to the expected life of the stock options granted, and we expect to continue to do so until such time as we have adequate historical data regarding the volatility of our own traded stock price. Our peer group of publicly traded companies was chosen based on their similar size, stage in the life cycle or area of specialty.

*Risk-Free Interest Rate.* The risk-free interest rate is based on the interest rates paid on securities issued by the U.S. Treasury with a term approximating the expected life of stock options granted.

*Expected Dividend.* We have never paid, and do not anticipate paying, cash dividends on our shares of common stock. Therefore, the expected dividend yield was assumed to be zero.

*Fair Value of Common Stock.* The fair value of shares of our common stock is measured by the stock price on the date of grant.

### **Emerging growth company and smaller reporting company status**

The JOBS Act permits an “emerging growth company” such as us to take advantage of an extended transition period to comply with new or revised accounting standards applicable to public companies until those standards would otherwise apply to private companies. We have elected not to “opt out” of such extended transition period, which means that when a standard is issued or revised and it has different application dates for public or private companies, we will adopt the new or revised standard at the time private companies adopt the new or revised standard and will do so until such time that we either (i) irrevocably elect to “opt out” of such extended transition period or (ii) no longer qualify as an emerging growth company. There are other exemptions and reduced reporting requirements provided by the JOBS Act that we are currently evaluating. For example, as an “emerging growth company,” we are exempt from Sections 14A(a) and (b) of the Exchange Act, which would otherwise require us to (1) submit certain executive compensation matters to shareholder advisory votes, such as “say-on-pay,” “say-on-frequency,” and “golden parachutes;” and (2) disclose certain executive compensation related items such as the correlation between executive compensation and performance and comparisons of our chief executive officer’s compensation to our median employee compensation. We also rely on an exemption from the rule requiring us to provide an auditor’s attestation report on our internal controls over financial reporting pursuant to Section 404(b) of the Sarbanes-Oxley Act. We will continue to remain an “emerging growth company” until the earliest of the following: (1) the last day of the fiscal year following the fifth anniversary of the date of the completion of our IPO; (2) the last day of the fiscal year in which our total annual gross revenue is equal to or more than \$1.235 billion; (3) the date on which we have issued more than \$1.0 billion in nonconvertible debt during the previous three years; or (4) the date on which we are deemed to be a large accelerated filer under the rules of the SEC.

We are also a “smaller reporting company,” meaning that the market value of our stock held by non-affiliates was less than \$700.0 million as of June 30, 2025 and our annual revenue was less than \$100.0 million during the most recently completed fiscal year. We may continue to be a smaller reporting company if either (1) the market value of our stock held by non-affiliates is less than \$250.0 million or (2) our annual revenue is less than \$100.0 million during the most recently completed fiscal year and the market value of our stock held by non-affiliates is less than \$700.0 million as of June 30 of such year. If we are a smaller reporting company at the time we cease to be an emerging growth company, we may continue to rely on exemptions from certain disclosure requirements that are available to smaller reporting companies. Specifically, as a smaller reporting company we may choose to present only the two most recent fiscal years of audited consolidated financial statements in our Annual Report on Form 10-K and, similar to emerging growth companies, smaller reporting companies have reduced disclosure obligations regarding executive compensation.

**Recently issued accounting pronouncements**

A description of recently issued accounting pronouncements that may potentially impact our financial position, results of operations and cash flows is disclosed in Note 2 to our audited financial statements included elsewhere in this Annual Report.

**Qualitative and quantitative disclosures about market risk***Interest rate risk*

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

**Item 7A. Reserved**

**Item 8. Financial Statements and Supplementary Data.**

**LEXEO THERAPEUTICS, INC.  
INDEX TO FINANCIAL STATEMENTS**

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KPMG LLP  
345 Park Avenue  
New York, NY 10154-0102

Report of Independent Registered Public Accounting Firm

To the Stockholders and Board of Directors  
Lexeo Therapeutics, Inc.:

Opinion on the Financial Statements

We have audited the accompanying balance sheets of Lexeo Therapeutics, Inc. (the Company) as of December 31, 2025 and 2024, the related statements of operations and comprehensive loss, convertible preferred stock and stockholders' equity, and cash flows for the years then ended, and the related notes (collectively, the financial statements). In our opinion, the financial statements present fairly, in all material respects, the financial position of the Company as of December 31, 2025 and 2024, and the results of its operations and its cash flows for the years then ended, in conformity with U.S. generally accepted accounting principles.

Basis for Opinion

These financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on these financial statements based on our audits. We are a public accounting firm registered with the 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 financial statements are free of material misstatement, whether due to error or fraud. Our audits included performing procedures to assess the risks of material misstatement of the 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 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 financial statements. We believe that our audits provide a reasonable basis for our opinion.

*KPMG LLP*

We have served as the Company's auditor since 2021.

New York, New York  
March 30, 2026

KPMG LLP, a Delaware limited liability partnership and a member firm of the KPMG global organization of independent member firms affiliated with KPMG International Limited, a private English company limited by guarantee.

# Lexeo Therapeutics, Inc.

## Balance Sheets

(In thousands, except share and per share amounts)

	December 31, 2025	December 31, 2024
<b>Assets</b>		
Current assets:		
Cash and cash equivalents	\$ 63,000	\$ 35,014
Current portion of investments in U.S. Treasury securities	118,767	86,504
Prepaid expenses and other current assets	6,625	4,603
Total current assets	188,392	126,121
Non-current portion of investments in U.S. Treasury securities	64,801	7,012
Long-term investment	3,390	-
Restricted cash	3,248	3,252
Property and equipment, net	1,139	1,028
Lease right-of-use assets - finance	1,140	1,460
Lease right-of-use assets - operating	6,578	8,069
Total assets	<u>\$ 268,688</u>	<u>\$ 146,942</u>
<b>Liabilities and stockholders' equity</b>		
Current liabilities:		
Accounts payable	\$ 4,856	\$ 6,417
Accrued expenses and other current liabilities	9,277	13,759
Current portion of lease liabilities - finance	518	570
Current portion of lease liabilities - operating	2,157	2,100
Total current liabilities	16,808	22,846
Non-current liabilities		
Non-current portion of lease liabilities - finance	327	817
Non-current portion of lease liabilities - operating	4,884	6,437
Total liabilities	<u>22,019</u>	<u>30,100</u>
Commitments and contingencies (Note 11)		
Stockholders' equity:		
Common stock, \$0.0001 par value, 500,000,000 shares authorized as of December 31, 2025; 73,001,766 shares issued and 73,001,350 shares outstanding as of December 31, 2025; 500,000,000 shares authorized as of December 31, 2024; 33,079,209 shares issued and 33,071,261 shares outstanding as of December 31, 2024	7	3
Treasury stock, at cost, 3,827 common shares as of December 31, 2025 and as of December 31, 2024	(17)	(17)
Additional paid-in capital	626,625	397,132
Accumulated other comprehensive gain (loss)	188	(103)
Accumulated deficit	(380,134)	(280,173)
Total stockholders' equity	246,669	116,842
Total liabilities and stockholders' equity	<u>\$ 268,688</u>	<u>\$ 146,942</u>

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

# Lexeo Therapeutics, Inc.

## Statements of Operations and Comprehensive Loss

(In thousands, except share and per share amounts)

	Year Ended December 31,	
	2025	2024
Operating expenses		
Research and development	\$ 63,797	\$ 74,091
General and administrative	45,460	31,675
Total operating expenses	109,257	105,766
Operating loss	(109,257)	(105,766)
Other income and expense		
Gain on long-term investment	3,390	-
Other expense, net	(18)	(9)
Interest expense	(95)	(137)
Interest income	6,130	7,556
(Amortization of premium) accretion of discount on investments in U.S. Treasury securities, net	(111)	23
Total other income and expense	9,296	7,433
Loss from operations before income taxes	(99,961)	(98,333)
Income taxes	-	-
Net loss	\$ (99,961)	\$ (98,333)
Other comprehensive gain (loss)		
Net unrealized gain (loss) on investments	291	(103)
Total other comprehensive gain (loss)	291	(103)
Comprehensive loss	\$ (99,670)	\$ (98,436)
Net loss per common share, basic and diluted	\$ (1.86)	\$ (3.09)
Weighted-average number of shares outstanding used in computation of net loss per common share, basic and diluted	53,645,323	31,787,491

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

# Lexeo Therapeutics, Inc.

## Statements of Stockholders' Equity

(In thousands, except share amounts)

	Common Stock		Treasury Stock	Additional Paid-in Capital	Accumulated Other Comprehensive Income (Loss)	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount					
Balances at December 31, 2024	33,071,261	\$ 3	\$ (17)	\$ 397,132	\$ (103)	\$ (280,173)	\$ 116,842
Exercise of stock options	15,300	-	-	59	-	-	59
Issuance of common stock upon vesting and settlement of restricted stock units	101,448	-	-	-	-	-	-
Issuance of common stock upon vesting and settlement of performance restricted stock units	76,800	-	-	-	-	-	-
Issuance of common stock under 2023 ESPP	102,837	-	-	270	-	-	270
Amounts reclassified from deposit liabilities upon the vesting of early-exercised stock options previously subject to repurchase	7,532	-	-	37	-	-	37
Issuance of common stock, pre-funded warrants and warrants to purchase common stock upon May 2025 Private Placement, net of commissions and offering costs of \$6,917	20,790,120	2	-	73,080	-	-	73,082
Issuance of common stock and pre-funded warrants to purchase common stock upon October 2025 Financing Transactions, net of commissions and offering costs of \$9,833	17,968,750	2	-	143,915	-	-	143,917
Issuance of common stock upon exercise of pre-funded warrants	867,302	-	-	-	-	-	-
Unrealized comprehensive gain on investments in U.S. Treasury securities	-	-	-	-	291	-	291
Stock-based compensation expense	-	-	-	12,132	-	-	12,132
Net loss	-	-	-	-	-	(99,961)	(99,961)
Balances at December 31, 2025	73,001,350	7	(17)	626,625	188	(380,134)	246,669

	Common Stock		Treasury Stock	Additional Paid-in Capital	Accumulated Other Comprehensive Income (Loss)	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount					
Balances at December 31, 2023	26,646,378	\$ 3	\$ -	\$ 295,372	\$ -	\$ (181,840)	\$ 113,535
Exercise of stock options	135,646	-	-	491	-	-	491
Amounts reclassified from deposit liabilities upon the vesting of early-exercised stock options previously subject to repurchase	14,159	-	-	59	-	-	59
Issuance of common stock upon March 2024 Private Placement, net of commissions and offering costs of \$6,287	6,278,905	-	-	88,712	-	-	88,712
Additional issuance costs incurred related to issuance of convertible SAFE Note	(3,827)	-	-	24	-	-	24
Treasury stock repurchase	-	-	(17)	-	-	-	(17)
Unrealized comprehensive loss on investments in U.S. Treasury securities	-	-	-	-	(103)	-	(103)
Stock-based compensation expense	-	-	-	12,474	-	-	12,474
Net loss	-	-	-	-	-	(98,333)	(98,333)
Balances at December 31, 2024	33,071,261	\$ 3	\$ (17)	\$ 397,132	\$ (103)	\$ (280,173)	\$ 116,842

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

# Lexeo Therapeutics, Inc.

## Statements of Cash Flows

(In thousands)

	Years Ended December 31,	
	2025	2024
<b>Cash flows from operating activities:</b>		
Net loss	\$ (99,961)	\$ (98,333)
Adjustments to reconcile net loss to net cash used in operating activities:		
Reduction in the carrying amount of ROU assets, operating	1,491	1,373
Reduction in the carrying amount of ROU assets, finance	320	325
Stock-based compensation expense	12,132	12,474
Depreciation and amortization expense	268	304
Non-cash gain on long-term investment	(3,390)	-
Net realized gain on investments in U.S. Treasury securities	(17)	-
Amortization of premium (accretion of discount) on investments in U.S. Treasury securities	111	(23)
Changes in operating assets and liabilities:		
Prepaid expenses and other current assets	(2,004)	(1,690)
Accounts payable	(1,561)	2,378
Accrued expenses and other current liabilities	(4,445)	3,367
Lease liabilities, operating	(1,496)	(1,336)
Lease liabilities, finance	(9)	10
Net cash used in operating activities	(98,561)	(81,151)
<b>Cash flows from investing activities:</b>		
Purchase of property and equipment	(397)	(481)
Purchases of investments	(195,382)	(93,596)
Proceeds from sales or maturities of investments	105,527	-
Net cash used in investing activities	(90,252)	(94,077)
<b>Cash flows from financing activities:</b>		
Proceeds from exercise of stock options	59	491
Payments on finance leases	(533)	(410)
Proceeds from common stock issuances under 2023 ESPP	270	-
Treasury stock repurchase	-	(17)
Proceeds from issuance of common stock upon March 2024 Private Placement	-	94,999
Third-party commissions and offering costs incurred upon March 2024 Private Placement	-	(6,287)
Proceeds from issuance of common stock, pre-funded warrants and warrants to purchase common stock upon May 2025 Private Placement	79,999	-
Third-party commissions and offering costs incurred upon May 2025 Private Placement	(6,917)	-
Proceeds from issuance of common stock and pre-funded warrants to purchase common stock upon October 2025 Financing Transactions	153,750	-
Third-party commissions and offering costs incurred upon October 2025 Financing Transactions	(9,833)	-
Net cash provided by financing activities	216,795	88,776
Net change in cash, cash equivalents and restricted cash	27,982	(86,452)
Cash, cash equivalents and restricted cash at beginning of period	38,266	124,718
Cash, cash equivalents and restricted cash at end of period	\$ 66,248	\$ 38,266
<b>Supplemental disclosures of cash flow information</b>		
Interest paid	\$ 104	\$ 127
<b>Supplemental disclosure of non-cash activities</b>		
Unrealized gain (loss) on investments	\$ 291	\$ (103)
Amounts reclassified from deposit liabilities upon the vesting of early-exercised stock options previously subject to repurchase, net of proceeds received from early exercise of unvested stock options subject to repurchase and recorded as deposit liabilities	\$ 37	\$ 59
Unpaid offering costs included in accounts payable and accrued expenses	\$ 25	\$ -
Reclassifications from property and equipment to prepaid expenses and other current assets	\$ 18	\$ 98
Finance lease right-of use assets and finance lease liabilities recognized	\$ -	\$ 22
Property and equipment purchased in the prior period and paid in the current period, net of property and equipment purchased in the current period included in accounts payable	\$ -	\$ (120)

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

# Lexeo Therapeutics, Inc.

## Notes to Financial Statements

(Table amounts in thousands, except share and per share amounts)

### 1. Description of Business and Basis of Presentation

**Description of Business**—Lexeo Therapeutics, Inc. (the “Company”) is a clinical stage genetic medicine company dedicated to pioneering novel treatments for cardiovascular disease. The Company utilizes adeno-associated viruses (“AAV”) that have been engineered to transfer genes to patients. The Company’s therapeutic investigational treatments include AAV-based gene therapies primarily in the clinical and late pre-clinical stages of research and development.

The Company is located in New York, NY and was first formed on February 17, 2017, as an LLC under the laws of the State of Delaware under the legal name Lexeo Therapeutics, LLC. The Company filed and executed a certificate of conversion to a corporation on November 20, 2020, to convert the LLC to Lexeo Therapeutics, Inc, a Delaware corporation. All of the Company’s tangible assets are held in the United States (“U.S.”).

**Basis of Presentation and Principles of Consolidation**—The accompanying financial statements and these notes reflect the operations of the Company that have been prepared in conformity with generally accepted accounting principles in the United States of America (“GAAP”). Any reference in these notes to applicable guidance is meant to refer to the authoritative GAAP as found in the Accounting Standards Codification (“ASC”) and Accounting Standards Updates (“ASU”) of the Financial Accounting Standards Board (“FASB”). The Company has no unconsolidated subsidiaries. Certain prior period balances have been reclassified to conform to the current period presentation.

**Need for Additional Capital**—Since inception, the Company has incurred net losses and negative cash flows from operations, including net losses of \$100.0 million and \$98.3 million during the years ended December 31, 2025 and December 31, 2024, respectively. As of December 31, 2025, the Company had cash, cash equivalents, and investments in U.S. Treasury Securities of \$246.6 million and an accumulated deficit of \$380.1 million and expects to incur substantial operating losses and negative cash flows from operations for the foreseeable future. During the year ended December 31, 2024, the Company received total net proceeds of \$88.7 million after deducting approximately \$6.3 million of commissions and offering expenses in the March 2024 Private Placement (as defined below) (see Note 7). During the year ended December 31, 2025, the Company received total net proceeds of approximately \$73.1 million after deducting approximately \$6.9 million of commissions and offering expenses in the May 2025 Private Placement (as defined below) (see Note 7), as well as net proceeds of approximately \$143.9 million after deducting approximately \$9.8 million of underwriter commissions, placement agent fees and estimated offering expenses in the October 2025 Financing Transactions (as defined below) (see Note 7). Management estimates that the Company’s current cash, cash equivalents, and investments in U.S. Treasury securities balances are sufficient to fund its operations for at least 12 months from the issuance date of these financial statements.

If the Company is unable to obtain additional funding before achieving sufficient profitability and positive cash flows from operations, if ever, the Company will be forced to delay, reduce or eliminate some or all of its research and development programs, which could adversely affect its business prospects, or the Company may be unable to continue operations. Although management continues to pursue plans to obtain additional funding before achieving sufficient profitability and positive cash flows from operations, there is no assurance that the Company will be successful in obtaining sufficient funding on terms acceptable to the Company to fund continuing operations, if at all.

**Risks and Uncertainties**—The Company is subject to risks and uncertainties common to early-stage companies in the biopharmaceutical industry, including, but not limited to, successful discovery and development of its product candidates, development by competitors of new technological innovations, dependence on key personnel, the ability to attract and retain qualified employees, protection of proprietary technology, compliance with governmental regulations, the ability to secure additional capital to fund operations, and commercial success of its product candidates. Any of the Company’s current product candidates and future product candidates that it may develop will require extensive non-clinical and clinical testing and regulatory approval prior to commercialization. These efforts require significant amounts of additional capital, adequate personnel, infrastructure, and extensive compliance- reporting capabilities. Even if the Company’s product development efforts are successful, it is uncertain when, if ever, the Company will realize significant revenue from product sales.

## 2. Summary of Significant Accounting Policies

**Use of Estimates**—The preparation of the financial statements in accordance with U.S. GAAP requires management to make estimates and assumptions that affect the reported amounts of assets and liabilities and disclosure of contingent assets and liabilities at the date of the financial statements, and the reported amounts of income and expense during the reporting period. The most significant estimates relate to the accruals of research and development costs, including accruals of research contract costs, and assumptions used to estimate the fair value of the Company's stock option awards. Management evaluates its estimates and assumptions on an ongoing basis using historical experience and other factors, including the current economic environment, and makes adjustments when facts and circumstances dictate. These estimates are based on information available as of the date of the financial statements; therefore, actual results could differ from those estimates.

**Cash and Cash Equivalents and Restricted Cash**—The Company considers all highly liquid investments with a remaining maturity when purchased of three months or less to be cash equivalents. Cash equivalents are reported at carrying values, which approximated their fair values and are based on quoted prices in active markets for identical securities. At December 31, 2025 and December 31, 2024, the Company's cash equivalents were held in U.S. government money market funds. As of December 31, 2025 and 2024, cash consists of cash on deposit with U.S. banks denominated in U.S. dollars, and restricted cash consists of cash on deposit with U.S. banks denominated in U.S. dollars for which its use is restricted and is classified as non-current in the Company's balance sheet according to the timing of maturity, and is associated with collateral for letters of credit issued in connection with its operating lease right-of-use and finance lease right-of-use assets and corresponding lease liabilities. Cash is stated at its historical carrying amount, which approximates fair value due to its short-term nature. Restricted cash is stated at its historical carrying amount, which approximates fair value. The Company regularly maintains cash and cash equivalents and restricted cash balances with financial institutions that exceed Federal Deposit Insurance Corporation insurance limits.

**Investments**—Investments consist primarily of U.S. Treasury securities. Management determines the appropriate classification of these securities at the time they are acquired and evaluates the appropriateness of such classifications at each balance sheet date, including the determination of current and non-current classifications based on the contractual maturity dates of the underlying investments. Investments may be sold by the Company prior to contractual maturity. The Company classifies its investments as available-for-sale pursuant to ASC Topic 320, *Investments—Debt and Equity Securities*. Investments are recorded at fair value, with unrealized gains and losses included as a component of accumulated other comprehensive income (loss) in stockholders' equity and a component of total comprehensive loss in the statements of comprehensive loss, until realized. The Company's current and non-current investments are presented net of premiums of approximately \$149,000 and \$39,000, respectively, at December 31, 2025.

**Net Loss per Share**—The Company follows the two-class method when computing net income (loss) as the Company has issued shares that meet the definition of participating securities. The two-class method determines net income (loss) per common share for each class of common and participating securities according to dividends declared or accumulated and participation rights in undistributed earnings. The two-class method requires income (loss) available to common stockholders for the period to be allocated between common and participating securities based upon their respective rights to receive dividends as if all income for the period had been distributed. The Company considers the shares issued upon the early exercise of stock options that are subject to repurchase to be participating securities because holders of such shares have non-forfeitable dividend rights in the event a dividend is paid on common stock. There is no allocation required under the two-class method during periods of loss since the participating securities do not have a contractual obligation to share in the losses of the Company.

Basic net income (loss) per common share is computed by dividing the net income (loss) per common share by the weighted-average number of common shares and pre-funded warrants to purchase shares of common stock outstanding for the period. In accordance with ASC Topic 260, *Earnings per Share*, the pre-funded warrants are included in the computation of basic net loss per share because the exercise price is negligible and they are fully vested and exercisable at any time after the original issuance date. Diluted net income (loss) per common share is computed by adjusting net income (loss) to reallocate undistributed earnings based on the potential impact of dilutive securities. Diluted net loss per common share is computed by dividing the diluted net loss by the weighted-average number of common shares outstanding for the period, including potential dilutive common shares.

In periods in which the Company reported a net loss, diluted net loss per common share was the same as basic net loss per common share since dilutive common shares were not assumed to have been issued if their effect was anti-dilutive. During the years ended December 31, 2025 and December 31, 2024, 19,117,976 and 3,724,218 total potential common shares, respectively, related to the exercise of outstanding stock options, settlement of outstanding restricted stock units ("RSUs"), settlement of outstanding RSUs that vest upon the achievement of certain performance conditions ("PRSUs"), and exercise of outstanding warrants were excluded from the computation of diluted net loss per common share because including them would have had an anti-dilutive effect as the Company reported net losses for those periods.

**Deferred Offering Costs**—The Company capitalizes certain legal, accounting and other third-party fees that are directly associated with equity financings as deferred offering costs until such financings are consummated. After consummation of the equity financing, these costs are recorded as a reduction of the proceeds from the offering in stockholders' equity as a reduction of additional capital generated as a result of the offering. Should the equity financing be abandoned, the deferred offering costs would be expensed immediately as a charge to operating expenses in the statement of operations and comprehensive loss.

**Research and Development**—Research and development costs are expensed as incurred. Research and development expenses consist of costs incurred to discover, research, and develop drug candidates, including personnel expenses, allocated facility-related and depreciation expenses, and third-party license fees. Costs incurred to obtain technology licenses are charged immediately to research and development expense if the technology licensed has not reached technological feasibility and has no alternative future use.

**General and Administrative**—General and administrative expenses consist primarily of the cost of employees to engage in corporate functions, such as finance and accounting, information technology, human resources, and legal and executive management. General and administrative expenses also include rent and occupancy costs, office expenses, depreciation and amortization, other general overhead costs, insurance premiums, professional service fees, and costs related to regulatory and litigation matters.

**Stock Based Compensation Expense**—The Company accounts for stock-based payment awards granted to employees and non-employees, as well as shares issued to its employees under the Company's 2023 Employee Stock Purchase Plan (the "2023 ESPP"), as stock-based compensation expense at fair value. The Company issues stock-based awards to employees and non-employees in the form of stock options and also issues stock-based awards to employees in the form of RSUs and PRSUs. The measurement date for employee stock option, RSU, and PRSU awards is the date of grant, and stock-based compensation costs for employee non-performance awards are recognized as expense over the employees' requisite service period, which is the vesting period, on a graded basis. Compensation expense for equity awards with performance conditions, including PRSUs, is recognized if and when the Company concludes that it is probable that the performance conditions will be achieved. The Company reassesses the probability of vesting at each reporting period for equity awards with such performance conditions and adjusts compensation expense based on its probability assessment. The measurement date for non-employee awards is the date of grant without changes in the fair value of the award, and stock-based compensation costs for non-employees are recognized as expense over the vesting period on a graded basis. The measurement date for shares issued under the 2023 ESPP is the first date of each employee stock purchase plan offering period, and stock-based compensation costs are recognized as expense over the offering period on a straight-line basis. Stock-based compensation expense for all equity awards is classified in the accompanying statement of operations based on the function to which the related services are provided. As the Company is permitted to repurchase shares legally issued for unvested stock options exercised at their exercise price under the Company's 2021 Equity Incentive Plan (the "2021 Plan"), (i) cash received for unvested stock options exercised under the 2021 Plan is recorded as a deposit liability as a reclassification from additional paid-in capital in the Company's balance sheet, which is relieved to additional paid-in capital as such awards vest, (ii) stock based compensation expense is recognized over the requisite service period for each such award, and (iii) the amount and number of shares of common stock outstanding in the Company's balance sheet and statement stockholders' equity are reduced until such awards vest. Forfeitures are recorded as they occur.

The fair value of each stock option grant under the 2021 Plan and the Company's 2023 Equity Incentive Plan (the "2023 Plan", and together with the 2021 Plan, the "Plans"), and shares issued under the 2023 ESPP, are estimated on the date of stock option grant or on the first date of each employee stock purchase plan offering period, respectively, using the Black-Scholes option-pricing model, while the fair value of each RSU and PRSU granted under the 2023 Plan is equal to the closing price of the Company's common stock on the date of the grant. Prior to its initial public offering ("IPO") on November 3, 2023, the Company was a private company and lacked company-specific historical and implied volatility information for stock options granted. Therefore, the Company estimated its expected stock volatility entirely based on the historical volatility of a publicly traded set of peer companies for stock options granted prior to its IPO, and estimates its expected stock price volatility primarily based on the historical volatility of a publicly traded set of peer companies for stock options granted since its IPO, each for a period equal to the expected life of the stock options granted, and expects to continue to do so until such time as it has adequate historical data regarding the volatility of its own traded stock price. The expected term of the Company's stock options granted has been determined utilizing the "simplified" method for awards that qualify as "plain-vanilla" options and expects to continue to do so until such time as it has adequate historical data regarding the expected term of its own stock options. The risk-free interest rate is determined by reference to the U.S. Treasury yield curve in effect at the time of grant of the award for time periods approximately equal to the expected term of the award. The expected dividend yield is zero as the Company has never paid cash dividends on its common stock and does not expect to pay any cash dividends in the foreseeable future.

**Income Taxes**—Prior to November 20, 2020, the Company was an LLC entity and elected to be treated under the Partnership provisions of the Internal Revenue Code. Accordingly, the LLC entity was not viewed as a tax-paying entity in any jurisdiction and all income and deductions of the LLC entity flowed through to the individual members and therefore no income taxes were recorded by the Company. On November 20, 2020, the Company converted to a C Corporation.

The Company accounts for income taxes under the asset and liability method pursuant to ASC 740, *Income Taxes*. Under this method, the Company recognizes deferred tax assets and liabilities for the future tax consequences attributable to differences between the financial statement carrying amounts of existing assets and liabilities and their respective tax bases and operating loss and tax credit carryforwards. Deferred tax assets and liabilities are measured using enacted tax rates expected to apply to taxable income in the years in which those temporary differences are expected to be recovered or settled. The effect on deferred tax assets and liabilities of a change in tax rates is recognized in income in the period that includes the enactment date. A valuation allowance is recorded for deferred tax assets if it is more likely than not that some portion or all of the deferred tax assets will not be realized based on all available positive and negative evidence. As of December 31, 2025 and December 31, 2024, the Company continues to maintain a full valuation allowance against its deferred tax assets (see Note 10).

The Company recognizes a tax benefit only if it is more likely than not the tax position will be sustained on examination by the local taxing authorities, based on the technical merits of the position. The tax benefits recognized in the financial statements from such positions are then measured based on the largest benefit greater than 50% likelihood of being realized upon settlement with the related tax authority. The changes in recognition or measurement are reflected in the period in which the change in judgment occurs. As of December 31, 2025 and December 31, 2024, the Company has not identified any uncertain tax positions.

The Company's policy, to the extent uncertain tax positions exist, is to record interest and penalties related to uncertain tax positions in the provision for income taxes.

**Fair Value Measurements**—Fair value is defined as the price that would be received to sell an asset or paid to transfer a liability in an orderly transaction between market participants at the measurement date. The three levels of inputs that may be used to measure fair value are as follows:

*Level 1*—Inputs are quoted prices (unadjusted) in active markets for identical assets or liabilities that the Company has the ability to access at the measurement date.

*Level 2*—Inputs are observable, unadjusted quoted prices in active markets for similar assets or liabilities, unadjusted quoted prices for identical or similar assets or liabilities in markets that are not active, or other inputs that are observable or can be corroborated by observable market data for substantially the full term of the related assets or liabilities.

*Level 3*—Inputs are unobservable inputs for the asset or liability.

**Long-term Investment**—The Company accounts for its long-term investment in nonredeemable convertible preferred stock in accordance with the provisions of ASC Topic 321, *Investments - Equity Securities*, and elected to use the measurement alternative therein. The fair value of the nonredeemable convertible preferred stock received was based upon an observable price of cash paid for an identical security in an orderly transaction with a third-party and was capitalized as a long-term investment in the Company's balance sheet and recorded as an unrealized gain to other non-operating income in the Company's statement of operations and comprehensive loss. The long-term investment will be remeasured upon future observable price change(s) in orderly transaction(s) or upon impairment, if any.

**Concentrations of Credit Risk**—Financial instruments that potentially subject the Company to concentrations of credit risk consist primarily of cash, cash equivalents, restricted cash, and investments. The Company's cash and restricted cash balances exceed Federal Deposit Insurance Corporation insurance limits. The Company's cash equivalents consist of investments in U.S. government money market funds, and the Company's cash investments consist of U.S. Treasury securities. The Company's cash, cash equivalents, and restricted cash are held with large financial institutions that management believes to be of high credit quality. To date, the Company has not recognized any losses caused by uninsured balances.

**Segment Information**—The Company manages its operations as a single segment for the purposes of assessing performance and allocating resources. The Company is focused on preclinical and clinical stage gene therapies, and specifically on hereditary and acquired diseases of high unmet need. The Company's Chief Operating Decision Maker ("CODM") is its Chief Executive Officer ("CEO") and its senior leadership team. The CODM reviews the Company's financial information on an aggregated basis for purposes of assessing performance and allocating resources. All assets are held in the United States. The Company has not earned any product revenue through December 31, 2025.

**Property and Equipment**—Property and equipment are stated at cost less accumulated depreciation and amortization. Depreciation and amortization are recognized using the straight-line method over the estimated useful life of each asset. Such costs are periodically reviewed for recoverability when impairment indicators are present. Such indicators include, among other factors, unused capacity, market value declines, and obsolescence of technology. Recorded values of asset groups of equipment that are not expected to be recovered through undiscounted future net cash flows are written down to current fair value, which generally is determined from estimated discounted future net cash flows (assets held for use) or net realizable value (assets held for sale).

Costs for capital assets not yet placed into service are capitalized as construction-in-progress and depreciated once placed into service. Upon retirement or sale, the cost of assets disposed of and the related accumulated depreciation and amortization are removed from the accounts and any resulting gain or loss is included in loss from operations. Expenditures for repairs and maintenance that do not improve or extend the life of the respective assets are charged to expense in the period incurred. See Note 6 for information regarding the Company's capitalized operating and finance lease right-of-use assets.

The following is the summary of property and equipment and related accumulated depreciation as of December 31, 2025, and December 31, 2024:

	<u>Useful Life</u>	<u>December 31, 2025</u>	<u>December 31, 2024</u>
Furniture and fixtures	5 years	\$ 386	\$ 386
Lab equipment	7 years	1,222	844
Leasehold improvements	7 years	272	272
Total property and equipment		<u>1,880</u>	<u>1,502</u>
Less: accumulated depreciation		<u>(741)</u>	<u>(474)</u>
Total property and equipment, net		<u>\$ 1,139</u>	<u>\$ 1,028</u>

**Leases**—In accordance with ASC Topic 842, *Leases* ("ASC 842"), the Company determines if an arrangement is or contains a lease at inception. A contract is or contains a lease if the contract conveys the right to control the use of an identified asset for a period of time in exchange for consideration. The Company classifies leases at the lease commencement date as operating or finance leases and records a right-of-use asset and a lease liability on the balance sheet for all leases with an initial lease term of greater than 12 months. Leases with an initial term of 12 months or less are not recorded in the balance sheet, but payments are recognized as expense on a straight-line basis over the lease term. The Company has elected not to recognize leases with terms of 12 months or less.

A lease qualifies as a finance lease if any of the following criteria are met at the inception of the lease: (i) there is a transfer of ownership of the leased asset to the Company by the end of the lease term, (ii) the Company holds an option to purchase the leased asset that it is reasonably certain to exercise, (iii) the lease term is for a major part of the remaining economic life of the leased asset, (iv) the present value of the sum of lease payments equals or exceeds substantially all of the fair value of the leased asset, or (v) the nature of the leased asset is specialized to the point that it is expected to provide the lessor no alternative use at the end of the lease term. All other leases are recorded as operating leases.

The Company enters into contracts that contain both lease and non-lease components. Non-lease components may include maintenance, utilities, and other operating costs. The Company combines the lease and non-lease components of fixed costs in its lease arrangements as a single lease component. Variable costs, such as utilities or maintenance costs, are not included in the measurement of right-of-use assets and lease liabilities but rather are expensed when the event determining the amount of variable consideration to be paid occurs.

Operating lease right-of-use assets and lease liabilities are recognized at the lease commencement date based on the present value of the lease payments over the lease term using the discount rate implicit in the lease. If the implicit rate is not readily determinable, the Company utilizes an estimate of its incremental borrowing rate based upon the available information at the operating lease commencement date. Operating lease right-of-use assets are further adjusted for prepaid or accrued lease payments. Operating lease payments are expensed using the straight-line method as an operating expense over the lease term. The Company's lease terms may include options to extend or terminate the lease when it is reasonably certain that the Company will exercise that option. Finance lease right-of-use assets and lease liabilities are recognized upon receipt of the assets based on their fair values and are amortized to depreciation expense using the straight-line method over the estimated useful lives of the related assets. Finance lease liability payments are bifurcated into (i) a portion that is recorded as interest expense and (ii) a portion that reduces the finance liability associated with the lease, according to the interest rates implicit in the leases.

Certain of the Company's leases include options to extend or terminate the lease. The amounts determined for the Company's right-of-use assets and lease liabilities generally do not assume that renewal options or early-termination provisions, if any, are exercised, unless it is reasonably certain that the Company will exercise such options.

**Recent Accounting Pronouncements Adopted**—In December 2023, the FASB issued ASU No. 2023-09, *Income Taxes—Improvements to Income Tax Disclosures*, which focuses on the rate reconciliation and income taxes paid (Topic 740) ("ASU No. 2023-09"). ASU No. 2023-09 requires a public business entity (a "PBE") to disclose, on an annual basis, a tabular rate reconciliation using both percentages and currency amounts, broken out into specified categories with certain reconciling items further broken out by nature and jurisdiction to the extent those items exceed a specified threshold. In addition, all entities are required to disclose income taxes paid, net of refunds received disaggregated by federal, state/local, and foreign and by jurisdiction if the amount is at least 5% of total income tax payments, net of refunds received. For PBEs, ASU No. 2023-09 is effective for annual periods beginning after December 15, 2024, with early adoption permitted. An entity may apply the amendments in ASU No. 2023-09 prospectively by providing the revised disclosures for the period ending December 31, 2025 and continuing to provide the pre-ASU disclosures for all periods presented, or may apply the amendments retrospectively by providing the revised disclosures for all period presented. As of December 31, 2025, the Company adopted ASU No. 2023-09 retrospectively, which only impacted the Company's income tax disclosures with no impact to its balance sheet or its statements of operations and comprehensive loss, stockholders' equity, or cash flows (see Note 10).

**Recent Accounting Pronouncements Not Adopted**—In November 2024, the FASB issued ASU No. 2024-03, *Income Statement—Reporting Comprehensive Income—Expense Disaggregation Disclosures* (Subtopic 220-40) ("ASU No. 2024-03"). ASU No. 2024-03 requires disclosure of specified information about certain costs and expenses in the notes to financial statements. ASU No. 2024-03 will be effective for the Company starting in annual periods after December 15, 2026 and interim reporting periods beginning after December 15, 2027, with early adoption permitted. The Company is currently assessing the impact of adopting this guidance on its financial statements.

In December 2025, the FASB issued ASU No. 2025-11, *Interim Reporting* (Topic 270) ("ASU No. 2025-11"). ASU No. 2025-11 clarifies interim disclosure requirements and the applicability of Topic 270. ASU No. 2025-11 will be effective for the Company starting in interim reporting periods within annual reporting periods beginning after December 15, 2027, with early adoption permitted. The Company is currently assessing the impact of adopting this guidance on its financial statements.

### 3. Fair Value Measurements

The Company's cash equivalents consist of investments in U.S. government money market funds stated at carrying value, which approximates fair value and is based on quoted prices in active markets for identical securities. Cash is stated at carrying value, which approximates fair value due to its short-term nature. The Company classifies its investments in U.S. Treasury securities as Level 2 assets as these assets are not traded in an active market and have been valued through a third-party pricing service based on quoted prices for similar assets. The carrying values of the Company's prepaid expenses, other current assets, accounts payable and accrued expenses approximate their fair values due to their short-term nature.

The following table presents information about the Company's financial assets and investments measured at fair value on a recurring basis and indicates the level of the fair value hierarchy utilized to determine such fair values:

	As of December 31, 2025:			
	Level 1	Level 2	Level 3	Total
<b>Assets:</b>				
Cash equivalents (money market)	\$ 40,178	\$ -	\$ -	\$ 40,178
Current portion of investments in U.S. Treasury securities	-	118,767	-	118,767
<b>Investments:</b>				
Non-current portion of investments in U.S.				
Treasury securities	-	64,801	-	64,801
	<u>\$ 40,178</u>	<u>\$ 183,568</u>	<u>\$ -</u>	<u>\$ 223,746</u>
	As of December 31, 2024:			
	Level 1	Level 2	Level 3	Total
<b>Assets:</b>				
Cash equivalents (money market)	\$ 17,636	\$ -	\$ -	\$ 17,636
Current portion of investments in U.S. Treasury securities	-	86,504	-	86,504
<b>Investments:</b>				
Non-current portion of investments in U.S.				
Treasury securities	-	7,012	-	7,012
	<u>\$ 17,636</u>	<u>\$ 93,516</u>	<u>\$ -</u>	<u>\$ 111,152</u>

Investments in U.S. Treasury securities, which are classified as available-for-sale securities, consisted of the following as of December 31, 2025 and December 31, 2024:

	As of December 31, 2025:			
	Amortized Cost Basis	Gross Unrealized Gains	Gross Unrealized Losses	Fair Value
Available-for-sale securities:				
U.S. Treasury securities	\$ 183,380	\$ 188	\$ -	\$ 183,568
	<u>\$ 183,380</u>	<u>\$ 188</u>	<u>\$ -</u>	<u>\$ 183,568</u>

	As of December 31, 2024:			
	Amortized Cost Basis	Gross Unrealized Gains	Gross Unrealized Losses	Fair Value
Available-for-sale securities:				
U.S. Treasury securities	\$ 93,619	\$ -	\$ (103)	\$ 93,516
	<u>\$ 93,619</u>	<u>\$ -</u>	<u>\$ (103)</u>	<u>\$ 93,516</u>

All investments in U.S. Treasury securities classified as available-for-sale securities held as of December 31, 2025 and December 31, 2024 had contractual maturities of less than one year for the current portion of investments in U.S. Treasury securities and between one to two years for the non-current portion of investments in U.S. Treasury securities. There have been no material realized gains or losses on investments classified as available-for-sale securities for the periods presented.

#### 4. Long-term Investment

In June 2025, the Company entered into a collaboration and license agreement (the "June 2025 CLA") and a preferred stock purchase agreement (the "June 2025 Preferred Stock Purchase Agreement") with Tilia Therapeutics, Inc., now known as Myoventive, Inc., a privately-held Delaware corporation (the "Investee"), to develop therapies for genetic cardiac diseases utilizing a novel non-viral RNA platform. In exchange for access to certain of the Company's expertise in developing cardiac genetic medicines, certain existing preclinical intellectual property, and technology contributed under the terms of the June 2025 CLA, the Company received 10,000,000 shares of the Investee's nonredeemable convertible preferred stock issued under the June 2025 Preferred Stock Purchase Agreement, initially representing approximately 12% of the Investee's issued and outstanding voting stock as of June 30, 2025. Under the terms of the June 2025 CLA, the Company is entitled to certain future milestone payments, royalties, and opt-in rights to certain program(s) (see Note 12).

The Company accounts for its investment in the Investee in accordance with the provisions of ASC Topic 321, *Investments - Equity Securities*, and elected to use the measurement alternative therein. The fair value of the nonredeemable convertible preferred stock received of approximately \$3.4 million, or approximately \$0.338983 per share, was based upon an observable price of cash paid for an identical security in an orderly transaction between the Investee and a third-party and was capitalized as a long-term investment in the Company's balance sheet and recorded as an unrealized gain to other non-operating income in the Company's statement of operations and comprehensive loss. The long-term investment will be remeasured upon future observable price change(s) in orderly transaction(s) or upon impairment, if any.

#### 5. Accrued Expenses and Other Current Liabilities

Accrued expenses and other current liabilities consisted of the following:

	December 31, 2025	December 31, 2024
Accrued research and development expenses	\$ 4,299	\$ 6,696
Accrued compensation and other personnel costs	4,066	4,112
Accrued general and administrative expenses and other professional fees	839	2,406
Other current liabilities	73	545
Total accrued expenses and other current liabilities	<u>\$ 9,277</u>	<u>\$ 13,759</u>

## 6. Leases

### *Operating Lease Right-of-Use Asset*

In January 2022, the Company entered into a lease agreement for an office facility and laboratory space in New York, New York that commenced in April 2022 and ends in July 2029 with an additional five-year option to extend the lease beyond July 2029 at the then-prevailing effective market rental rate. Upon commencement of this lease, the Company recorded operating lease right-of-use assets and operating lease liabilities of \$11.6 million based on the present value of payments over the lease term using an estimated incremental borrowing rate of 8.53% in accordance with the provisions of ASC 842. In connection with the Company's lease of office space and laboratory space, the Company provided a security deposit to the landlord in the form of a letter of credit totaling \$1.2 million. The cash collateralizing the letter of credit was included in long-term restricted cash in the Company's balance sheets as of December 31, 2025, and December 31, 2024. This lease was classified as an operating lease in accordance with the provisions of ASC 842. The Company did not recognize any right-of-use assets and lease liabilities associated with the potential option to renew or extend. The Company's operating lease agreement does not contain any significant residual value guarantees or restrictive covenants.

The remaining lease terms and payment terms as of December 31, 2025 and December 31, 2024 were 3.6 years and 4.6 years, respectively. The components of this operating lease were as follows:

	Year Ended December 31,	
	2025	2024
Operating lease expense	\$ 2,147	\$ 2,147
Variable lease expense	503	459
Total operating lease expense	\$ 2,650	\$ 2,606
Cash paid for amounts included in the measurement of lease liabilities, included in operating cash flows	\$ 2,152	\$ 2,110

The following table provides a reconciliation of the Company's remaining undiscounted contractual rent obligations due within each year ended December 31 to the operating lease liabilities recognized as of December 31, 2025:

Year ended December 31	Operating Leases
2026	2,206
2027	2,261
2028	2,318
2029	1,372
Thereafter	-
Total lease payments	8,157
Less: present value adjustment	(1,116)
Total operating lease liabilities	\$ 7,041
Included in the balance sheet:	
Current portion of lease liabilities - operating	\$ 2,157
Non-current portion of lease liabilities - operating	4,884
Total operating lease liabilities	\$ 7,041

### Equipment Finance Leases

Commencing in April 2022, the Company leases certain laboratory equipment under financing arrangements accounted for as finance leases in accordance with the provisions of ASC 842 that are classified in the Company's balance sheet as finance lease liabilities with related right-of-use assets recorded and depreciated on a straight-line basis over the estimated useful life of 7 years. In connection with the Company's leases of laboratory equipment, the Company provided a security deposit to the lessor in the form of a letter of credit totaling \$1.9 million and assigned all rights and interests in the equipment to the lessor. The cash collateralizing the letter of credit is included in long-term restricted cash in the Company's balance sheet as of December 31, 2025. The total gross, accumulated amortization, and net book values of equipment finance lease right-of-use assets capitalized under such finance lease arrangements at December 31, 2025 were \$2.2 million, \$1.1 million and \$1.1 million, respectively. Under the terms of the equipment finance lease agreements executed through the issuance date of these financial statements, the principal balances plus interest for the equipment are to be repaid in full after 60 monthly installments following lease commencement, with lease commencement dates ranging from April 1, 2022 to April 1, 2023, annual imputed interest rates ranging from 7.90% to 9.30%, and monthly installment payment amounts ranging from \$4,000 to \$18,000. The total aggregate monthly installment payment amount is \$49,000 for the Company's equipment finance lease agreements.

The weighted-average remaining lease payment term, weighted-average remaining amortization term, and weighted-average effective interest rate for the Company's equipment finance lease agreements as of December 31, 2025 were 1.7 years, 3.8 years, and 8.61%, respectively. The weighted-average remaining lease payment term, weighted-average remaining amortization term, and weighted-average effective interest rate for the Company's equipment finance lease agreements as of December 31, 2024 were 2.7 years, 4.8 years, and 8.59%, respectively. The components of the equipment finance leases were as follows:

	Year Ended December 31,	
	2025	2024
Reduction in the carrying amount of ROU assets, finance	\$ 320	\$ 325
Interest on finance lease liabilities	104	127
Total finance lease expense	\$ 424	\$ 452
Cash paid for amounts included in the measurement of lease liabilities, included in financing cash flows	\$ 533	\$ 410

The following table provides a reconciliation of the Company's remaining equipment finance lease obligations due within each year ending December 31 to the equipment finance lease liabilities recognized at December 31, 2025:

Year ended December 31	Equipment Finance Leases
2026	537
2027	359
2028	11
2029	-
Thereafter	-
Total lease payments	907
Less: imputed interest	(62)
Total finance lease liabilities	\$ 845
Included in the balance sheet:	
Current portion of lease liabilities - finance	\$ 518
Non-current portion of lease liabilities - finance	327
Total finance lease liabilities	\$ 845

## 7. Capital Stock

As of December 31, 2025 and December 31, 2024, the Company's amended and restated certificate of incorporation provided that the authorized capital stock of the Company was 510,000,000 shares consisting of 500,000,000 shares of common stock and 10,000,000 shares of undesignated preferred stock, both with a par value of \$0.0001 per share. As of December 31, 2025 and December 31, 2024, 73,001,766 shares and 33,079,209 shares, respectively, of the Company's common stock authorized were issued, including 416 shares and 7,948 shares, respectively, that were legally issued upon the early exercise of unvested stock options and that are excluded from the number of shares outstanding until the right to repurchase subsequently lapses upon vesting. The Company did not repurchase any shares of its common stock during the year ended December 31, 2025, and repurchased a total of 3,827 shares of its common stock issued pursuant to the early exercise of stock options granted under the 2021 Plan for a total of approximately \$17,000 during the year ended December 31, 2024, which was recorded to treasury stock in the Company's balance sheet (see Note 8). Each common share entitles the holder to one vote on all matters submitted to a vote of the Company's stockholders. Common stockholders are entitled to receive dividends, if any, as may be declared by the Company's Board of Directors. No cash dividends have been declared or paid by the Company.

On March 11, 2024, the Company entered into a common stock purchase agreement to issue and sell an aggregate of 6,278,905 shares of its common stock at a price of \$15.13 per share, in a private placement that closed on March 13, 2024 (the "March 2024 Private Placement"). The gross and net proceeds received from the March 2024 Private Placement were approximately \$95.0 million and \$88.7 million, respectively, after deducting approximately \$6.3 million of commissions and other offering costs.

On March 24, 2025, the Company entered into a sales agreement (the "Sales Agreement") with Leerink Partners LLC ("Leerink") to sell shares of the Company's common stock having an aggregate offering price of up to \$75.0 million from time to time through an at-the-market equity offering program (the "ATM Program") under which Leerink is acting as the Company's agent. Leerink is entitled to compensation for its services in an amount equal to 3% of the gross proceeds of any of the shares of the Company's common stock sold under the Sales Agreement. No shares of the Company's common stock have been sold under the Sales Agreement through December 31, 2025.

On May 28, 2025, pursuant to a securities purchase agreement, the Company issued and sold an aggregate of 20,790,120 shares of its common stock, as well as pre-funded warrants to purchase 6,963,556 shares of its common stock to certain purchasers, together with accompanying warrants to purchase 13,876,838 shares of its common stock, at a purchase price of \$2.8825 per share of common stock and accompanying common warrant, or \$2.8824 per pre-funded warrant and accompanying warrant (the "May 2025 Private Placement"). The gross and net proceeds received from the May 2025 Private Placement were approximately \$80.0 million and \$73.1 million, respectively, after deducting approximately \$6.9 million of commissions and other offering costs. The warrants and pre-funded warrants issued in the May 2025 Private Placement are immediately exercisable at exercise prices of \$2.82 per share and \$0.0001 per share, respectively, except that the warrants and pre-funded warrants cannot be exercised by the holder if, after giving effect thereto, the holder would beneficially own more than 9.99% of the Company's common stock (the "Maximum Percentage"). The Maximum Percentage can be changed at the holder's election to any other percentage not in excess of 19.99%, and any increase in the Maximum Percentage will not be effective until the 61st day after the holder provides notice to the Company of the desired increase. The warrants are exercisable until May 28, 2029, and the pre-funded warrants do not expire. The warrants and pre-funded warrants are classified as equity in accordance with ASC Topic 815-40, *Derivatives and Hedging - Contracts in an Entity's Own Equity*. Upon issuance, the total pre-allocated fair value of the warrants of approximately \$26.8 million, or approximately \$1.93 per warrant share, was estimated using a Black-Scholes option pricing model with the following assumptions:

Stock Price	\$	2.74
Exercise Price	\$	2.82
Risk-free interest rate		4.00%
Expected term (in years)		4.00
Expected volatility		100.35%
Expected dividend yield		0.00%

Approximately \$44.3 million, \$20.8 million and \$14.9 million of the total gross proceeds received in the May 2025 Private Placement were allocated to the shares of common stock, warrants and pre-funded warrants, respectively, on a relative fair value basis, and were recorded as credits to additional paid-in capital, and are not subject to remeasurement. As of December 31, 2025, none of the warrants and 867,302 pre-funded warrants issued in the May 2025 Private Placement had been exercised. In January 2026, a total of 867,302 warrants issued in the May 2025 Private Placement were exercised (see Note 14).

On October 16, 2025, the Company entered into an underwriting agreement to issue and sell an aggregate of 15,625,000 shares of its common stock at a price of \$8.00 per share, less underwriting discounts and commissions, in a public offering that closed on October 20, 2025 (the "October 2025 Public Offering"). In addition, the Company granted the underwriters of the October 2025 Public Offering a 30-day option to purchase up to an additional 2,343,750 shares of the Company's common stock a price of \$8.00 per share, less underwriting discounts and commissions (the "30-day Underwriter Option"), which was exercised in full. The gross and net proceeds received from the October 2025 Public Offering including the full exercise of the 30-day Underwriter Option were approximately \$143.8 million and \$134.6 million, respectively, after deducting approximately \$9.2 million of underwriter commissions and other estimated offering costs.

On October 16, 2025, the Company entered into a securities purchase agreement to sell and issue pre-funded warrants to purchase 1,250,015 shares of the Company's common stock at a purchase price of \$7.9999 per pre-funded warrant, in a private placement that closed on October 20, 2025 (the "October 2025 Private Placement", and together with the October 2025 Public Offering and the 30-day Underwriter Option, the "October 2025 Financing Transactions"). The gross and net proceeds received from the October 2025 Private Placement were approximately \$10.0 million and \$9.3 million, respectively, after deducting approximately \$0.7 million of placement agent fees and other estimated offering costs. The pre-funded warrants issued in the October 2025 Private Placement do not expire and are immediately exercisable at an exercise price of \$0.0001 per pre-funded warrant share, except that the pre-funded warrants cannot be exercised by the holder if, after giving effect thereto, the holder would beneficially own more than 9.99% of the Company's common stock (the "October 2025 Private Placement Maximum Percentage"). The October 2025 Private Placement Maximum Percentage can be changed at the holder's election to any other percentage not in excess of 19.99%, and any increase in the October 2025 Private Placement Maximum Percentage will not be effective until the 61<sup>st</sup> day after the holder provides notice to the Company of the desired increase.

Approximately \$143.8 million and \$10.0 million of the total gross proceeds received in the October 2025 Financing Transactions were allocated to the shares of common stock and pre-funded warrants, respectively, on a relative fair value basis, and were recorded as credits to additional paid-in capital, and are not subject to remeasurement. As of December 31, 2025, none of the pre-funded warrants issued in the October 2025 Financing Transactions had been exercised.

The Company had reserved the following number of shares of common stock for the exercise of outstanding warrants, exercise of outstanding pre-funded warrants, exercise of outstanding stock options, settlement of outstanding RSUs and PRSUs, and future issuances of stock-based awards:

	December 31, 2025	December 31, 2024
Warrants to purchase shares of common stock	13,876,838	-
Pre-funded warrants to purchase shares of common stock	6,096,254	-
Options to purchase shares of common stock under the 2021 Plan and 2023 Plan	4,396,860	3,457,918
RSUs and PRSUs subject to settlement in shares of common stock under the 2023 Plan	844,278	266,300
Shares available for issuance under the 2023 Plan	2,132,156	2,185,328
Shares available for issuance under the 2023 ESPP	733,239	505,284
Total shares of common stock reserved for future issuance	<u>28,079,625</u>	<u>6,414,830</u>

## 8. Stock-based Compensation

In February 2021, the Company adopted the 2021 Plan for the issuance of stock options granted to the Company's key directors, officers, employees and consultants, as a means to secure the benefits arising from capital stock ownership. In connection with the Company's IPO in November 2023, the Company adopted the 2023 Plan for the issuance of equity awards granted to the Company's directors, officers, employees, and consultants, and for the issuance of shares purchased under the 2023 ESPP to the Company's employees, as a means to secure the benefits arising from capital stock ownership. The purposes of the Plans and the 2023 ESPP are also to promote the alignment of the interests of directors, officers, employees, and consultants with the success of the Company and to provide compensation opportunities to attract, retain and motivate directors, officers, employees, and consultants of the Company. Upon adoption of the 2023 Plan, no further grants may be made under the 2021 Plan.

The number of shares of common stock reserved for issuance under the 2023 Plan will automatically increase on January 1 of each year, beginning on January 1, 2024, and continuing through and including January 1, 2033, by 5% of the total number of shares of common stock outstanding on December 31 of the immediately preceding calendar year, or a lesser number of shares determined by the Company's Board of Directors prior to the applicable January 1. The number of shares of common stock reserved for issuance under the 2023 ESPP will automatically increase on January 1 of each calendar year, beginning on January 1, 2024 and continuing through and including January 1, 2033, by the lesser of (i) 1% of the total number of shares of capital stock outstanding on December 31 of the preceding calendar year, (ii) 477,200 shares, and (iii) a number of shares determined by the Company's Board of Directors. Shares subject to purchase rights granted under the 2023 ESPP that terminate without having been exercised in full do not reduce the number of shares available for issuance under the 2023 ESPP.

On January 1, 2026, the number of shares of common stock reserved for issuance under the 2023 Plan and the 2023 ESPP automatically increased by 3,650,088 shares and 477,200 shares, respectively, to totals of 6,804,770 shares and 1,210,439 shares, respectively. As of December 31, 2025, 2,132,156 shares and 733,239 shares were available for future issuance under the 2023 Plan and 2023 ESPP, respectively. A total of 102,837 shares have been issued under the 2023 ESPP through December 31, 2025.

On November 4, 2025, the Company's board of directors adopted the Lexeo Therapeutics, Inc. 2025 Inducement Equity Incentive Plan (the "2025 Inducement Equity Plan") and, subject to the adjustment provisions of the 2025 Inducement Equity Plan, reserved 2,000,000 shares of the Company's common stock for issuance pursuant to equity awards granted under the 2025 Inducement Equity Plan.

#### *Stock option activity*

Stock options granted under the 2021 Plan are issued from new shares upon exercise, have a contractual term of 10 years from grant date, and generally (i) are subject to requisite service requirements, (ii) vest over a four-year period with 25% of the options granted vesting after one year and the remainder vesting in equal monthly installments over the following 36 months, and (iii) allow for early exercise subject to repurchase. Stock options granted under the 2021 Plan to certain of the Company's non-employees vest in equal monthly installments over a four-year period or vested upon the achievement of a certain milestone event. The Company did not repurchase any shares of its common stock during the year ended December 31, 2025, and repurchased a total of 3,827 shares of its common stock issued pursuant to the early exercise of stock options granted under the 2021 Plan for a total of approximately \$17,000 during the year ended December 31, 2024, which was recorded to treasury stock in the Company's balance sheet (see Note 7).

Stock options granted under the 2023 Plan are issued from new shares upon exercise, have a contractual term of 10 years from grant date, and generally vest over a four-year period with 25% of the options granted vesting after approximately one year and the remainder vesting in equal monthly installments over the following 36 months, subject to requisite service requirements. Stock options granted under the 2023 Plan to certain of the Company's non-employee directors vest in equal annual installments over a three-year period for initial grants or over a one-year period for subsequent annual grants, subject to requisite service requirements.

The following table summarizes the stock option activity under the 2021 Plan and the 2023 Plan for the year ended December 31, 2025:

	Number of Shares	Weighted- Average Exercise Price (per share)	Weighted- Average Remaining Contractual Term (in years)	Aggregate Intrinsic Value
Outstanding as of December 31, 2024	3,457,918	\$ 11.37	8.18	\$ 2,851
Granted	1,450,904	5.57		
Exercised	(15,300)	3.83		62
Forfeited	(415,707)	8.96		
Expired	(80,955)	7.66		
Outstanding as of December 31, 2025	<u>4,396,860</u>	<u>\$ 9.75</u>	<u>7.60</u>	<u>\$ 11,304</u>
Options exercisable at December 31, 2025	<u>2,556,582</u>	<u>\$ 9.94</u>	<u>6.69</u>	<u>\$ 6,529</u>

The weighted-average grant date fair value of stock options granted during the years ended December 31, 2025 and December 31, 2024 was \$4.22 per share and \$11.58 per share, respectively.

The aggregate intrinsic value of stock options is calculated as the difference between the exercise price of the stock options and the fair value of the Company's common stock for those stock options that had exercise prices lower than the fair value of the Company's common stock. The total aggregate intrinsic value of stock options exercised during the year ended December 31, 2024 was \$1.3 million.

The total grant date fair values of stock options vested during the years ended December 31, 2025 and December 31, 2024 were \$10.3 million and \$7.1 million, respectively.

The Company estimated the fair value of options granted using a Black-Scholes option pricing model with the following assumptions during the years ended December 31, 2025 and December 31, 2024:

	Year Ended December 31,	
	2025	2024
Risk-free interest rates	3.78 - 4.57%	3.48 - 4.57%
Expected term (in years), weighted-average	5.79	6.02
Expected volatility, range	88.12 - 98.90%	84.61 - 88.19%
Expected volatility, weighted-average	92.25%	85.85%
Expected dividend yield	0.00%	0.00%

The expected dividend yields are 0.00% as the Company has never paid cash dividends and does not expect to pay any cash dividends in the foreseeable future.

As of December 31, 2025 there was \$6.3 million of unrecognized stock-based compensation expense related to stock options estimated to be recognized over a weighted-average period of 2.20 years.

#### *RSU and PRSU activity*

RSUs granted under the 2023 Plan generally vest over a four-year period with 25% of the RSUs vesting after approximately one year and the remainder vesting in equal quarterly installments over the following 12 quarters, subject to requisite service requirements. During the year ended December 31, 2025, a total of 217,500 PRSUs were granted to certain employees under the 2023 Plan that vest upon the achievement of certain performance conditions generally over an approximate 18-month period, subject to requisite service requirements. Additionally, during the year ended December 31, 2025, a total of 77,650 RSUs were granted to employees under the 2023 Plan that vest over a two-year period with 50% of the RSUs vesting after approximately one year and the remainder vesting in equal quarterly installments over the following four quarters, subject to requisite service requirements.

The following table summarizes the RSU activity under the 2023 Plan for the year ended December 31, 2025:

	Number of Shares	Weighted- Average Grant Date Fair Value (per share)
Unvested as of December 31, 2024	266,300	\$ 15.59
Granted	664,684	5.45
Vested	(101,448)	15.51
Forfeited	(125,958)	7.49
Unvested as of December 31, 2025	<u>703,578</u>	<u>\$ 7.47</u>

As of December 31, 2025 there was \$3.0 million of unrecognized stock-based compensation expense related to RSUs estimated to be recognized over a weighted-average period of 2.66 years.

The following table summarizes the PRSU activity under the 2023 Plan for the nine months ended December 31, 2025:

	Number of Shares	Weighted- Average Grant Date Fair Value (per share)
Unvested as of December 31, 2024	-	\$ -
Granted	217,500	7.25
Vested	(76,800)	7.11
Forfeited	-	-
Unvested as of December 31, 2025	<u>140,700</u>	<u>\$ 7.33</u>

### Employee Stock Purchase Plan

Under the 2023 ESPP, eligible employees may purchase shares of the Company's common stock through payroll deductions at a price equal to 85% of the lower of the fair market values of the stock as of the beginning of the offering period or the end of each six-month purchase period. Each offering period of two years will consist of four six-month purchase periods, subject to reset on the first day of each purchase period depending on the fair market values of the Company's stock. Employees may not purchase more than 6,000 shares of the Company's common stock during any purchase period. The first purchase period within the first offering period under the 2023 ESPP commenced during the three months ended December 31, 2024 and concluded during the three months ended March 31, 2025; following reset, the first purchase period within the second offering period commenced during the three months ended March 31, 2025 and concluded during the three months ended September 30, 2025; and the second purchase period within the second offering period commenced during the three months ended September 30, 2025. As of and during the year ended December 31, 2025, stock-based compensation expense recorded, as well as the estimated fair value of unearned and unrecognized stock-based expense expected to be recorded, related to the 2023 ESPP were not material. During the year ended December 31, 2025, a total of 102,837 shares were issued under the 2023 ESPP at a weighted average purchase price of \$2.62 per share with total proceeds of approximately \$0.3 million received by the Company.

### Stock-based compensation expense

Stock-based compensation expense was classified as follows in the Company's statements of operations and comprehensive loss:

	Year Ended December 31,	
	2025	2024
Research and development expense	\$ 5,524	\$ 6,001
General and administrative expense	6,608	6,473
Total stock-based compensation expense	\$ 12,132	\$ 12,474

## 9. Net Loss per Share

Basic and diluted net loss per common share attributable to common stockholders was calculated as follows:

	Year Ended December 31,	
	2025	2024
Numerator:		
Net loss attributable to common stockholders	\$ (99,961)	\$ (98,333)
Denominator:		
Weighted-average common shares outstanding, basic and diluted	53,645,323	31,787,491
Net loss per share attributable to common stockholders, basic and diluted	\$ (1.86)	\$ (3.09)

## 10. Income Taxes

The components of net income (loss) before income tax expense are as follows:

	Year Ended December 31,	
	2025	2024
Domestic	\$ (99,670)	\$ (98,436)
Total	\$ (99,670)	\$ (98,436)

A reconciliation of the Company's statutory income tax rate to the Company's effective income tax rate is as follows for the years ended December 31, 2025, and December 31, 2024:

	Year Ended December 31,			
	2025		2024	
	Amount	Percent	Amount	Percent
Pretax Income (Loss)	\$ (99,670)		\$ (98,436)	
US Federal Statutory Tax Rate	(20,931)	21.00%	(20,671)	21.00%
Tax Credits:				
Tax Credit - Federal R&D Credit	5,523	(5.54)	(678)	0.69
Tax Credit - Orphan Drug Credit	(1,970)	1.98	(2,995)	3.04
Change in valuation allowance	16,430	(16.48)	22,903	(23.27)
Nontaxable or Nondeductible Items:				
Other	948	(0.95)	1,441	(1.46)
<b>Total</b>	<b>\$ -</b>	<b>0.00%</b>	<b>\$ -</b>	<b>0.00%</b>

The Company's effective tax rate includes the effects of state and local income taxes, net of the federal income tax benefit, which are primarily attributable to New York and New York City, where the Company has significant business activities. This state has higher effective tax rates compared to other jurisdictions where the Company operates, and together, it accounts for more than half of the Company's total state tax expense.

The principal components of the Company's deferred tax assets and liabilities at December 31, 2025 and December 31, 2024 are as follows:

	Year Ended December 31,			
	2025		2024	
	Amount	Percent	Amount	Percent
Deferred tax assets:				
Net operating loss carryforwards	\$ 56,868		\$ 33,295	
Tax credits	7,190		10,743	
Intangibles	5,167		5,918	
Capitalized R&D expenses	43,720		39,338	
Lease liabilities	2,204		2,818	
Other deferred tax assets	6,225		6,292	
Total deferred tax assets	121,374		98,404	
Valuation allowance	(118,935)		(95,426)	
Net deferred tax assets	\$ 2,439		\$ 2,978	
Deferred tax liabilities:				
Right of use asset	\$ (2,059)		\$ (2,663)	
Other deferred tax liabilities	(380)		(315)	
Total deferred tax liabilities	(2,439)		(2,978)	
Net deferred tax assets (liabilities)	\$ -		\$ -	

As of December 31, 2025 and 2024, the Company had federal net operating loss ("NOL") carryforwards of approximately \$182.0 million and \$106.4 million, respectively, which can be carried forward indefinitely. As of December 31, 2025 and 2024, the Company had state and local net operating loss carryforwards of approximately \$362.9 million and \$212.7 million, respectively, which begin to expire in 2040.

As of December 31, 2025 and 2024, the Company had federal tax credits of \$7.2 million and \$10.7 million, respectively, which begin to expire in 2040.

The Tax Cuts and Jobs Act (the "TCJA") resulted in significant changes to the treatment of research and developmental ("R&D") expenditures under Section 174 of the Internal Revenue Code of 1986, as amended (the "IRC"). For tax years beginning after December 31, 2021, taxpayers are required to capitalize and amortize all R&D expenditures that are paid or incurred in connection with their trade or business. Specifically, costs for U.S.-based R&D activities must be amortized over five years and costs for foreign R&D activities must be amortized over 15 years, both using a midyear convention.

For tax years beginning after December 31, 2024, the One Big Beautiful Bill Act enacted a new rule under Section 174A of the IRC allowing companies to immediately expense any domestic R&D expenditures. For domestic R&D, companies may either immediately expense or elect to capitalize and amortize over at least 60 months under Section 174A of the IRC. However, foreign R&D continues to require capitalization subject to the mandatory 15-year amortization period under Section 174 of the IRC.

For domestic R&D expenditures that were previously capitalized in tax years 2022-2024 under the prior TCJA, companies may elect to recover the remaining unamortized balance by either (1) expensing fully in the first year beginning after December 31, 2024, (2) expensing ratably over two years beginning after December 31, 2024, or (3) continue amortizing over the remaining life.

The Company has elected to continue amortizing the previously capitalized costs over their remaining life. Beginning in tax year 2025, instead of immediately expensing domestic R&D expenditures under Section 174A of the IRC, the Company elected to capitalize and amortize its domestic R&D expenditures under Section 59(e) of the IRC over a 10-year period. Any foreign R&D costs will continue to be capitalized and amortized over 15 years in accordance with the requirements of Section 174 of the IRC.

Future realization of the tax benefits of existing temporary differences and net operating loss carryforwards ultimately depends on the existence of sufficient taxable income within the carryforward period. As of December 31, 2025 and December 31, 2024, the Company performed an evaluation to determine whether a valuation allowance was needed. The Company considered all available evidence, both positive and negative, which included the results of operations for the current and preceding years. The Company determined that it was not possible to reasonably quantify future taxable income and determined that it is more likely than not that all of the deferred tax assets will not be realized. As a result, the Company increased its valuation allowance by \$23.5 million as of December 31, 2025.

The utilization of NOLs and tax credit carryforwards to offset future taxable income may be subject to an annual limitation as a result of ownership changes that have occurred previously or may occur in the future. Under Sections 382 and 383 of the IRC, a corporation that undergoes an ownership change may be subject to limitations on its ability to utilize its pre-change NOLs and other tax attributes otherwise available to offset future taxable income and/or tax liability. An ownership change is defined as a cumulative change of 50% or more in the ownership positions of certain stockholders during a rolling three-year period.

The Company conducted ownership analyses under IRC Section 382 as of September 30, 2024 and December 31, 2025 and determined the Company experienced ownership changes on November 7, 2023 and May 28, 2025, respectively, that limit the Company's ability to utilize its federal NOLs and tax credits. As a result of the ownership changes, the Company is subject to an annual limitation of approximately \$2.4 million on its ability to utilize its federal NOLs and tax credits recognized before May 28, 2025, inclusive of the ownership change that occurred on November 7, 2023. Due to the limitations, no federal NOLs are expected to expire unutilized and \$10.6 million of federal tax credits that had been available to offset future tax liabilities, prior to the date of the ownership changes, are expected to expire unutilized. As a result, during the year ended December 31, 2025, the Company reduced its deferred tax assets related to the federal tax credits, which was offset by a corresponding decrease in the valuation allowance.

The calculation of the Company's tax liabilities involves dealing with uncertainties in the application of complex tax laws and regulations for both federal taxes and the many states in which it operates or does business in. ASC 740 states that a tax benefit from an uncertain tax position may be recognized when it is more likely than not that the position will be sustained upon examination, including resolutions of any related appeals or litigation processes, on the basis of the technical merits.

The Company records uncertain tax positions as liabilities in accordance with ASC 740 and adjusts these liabilities when its judgment changes as a result of the evaluation of new information not previously available. Because of the complexity of some of these uncertainties, the ultimate resolution may result in a payment that is materially different from the Company's current estimate of the unrecognized tax benefit liabilities. These differences will be reflected as increases or decreases to income tax expense in the period in which new information is available. As of December 31, 2025 and December 31, 2024, the Company has not recorded any uncertain tax positions in its financial statements.

The Company recognizes interest and penalties related to uncertain tax positions on the income tax expense line in the accompanying statements of operations. As of December 31, 2025 and December 31, 2024, no accrued interest or penalties are included on the related tax liability line in the balance sheet.

The Company files tax returns as prescribed by the tax laws of the jurisdictions in which it operates. In the normal course of business, the Company is subject to examination by federal and state jurisdictions, where applicable. The resolution of tax matters is not expected to have a material effect on the Company's financial statements. The Company's tax years remain open under statute from December 31, 2022 to the present.

The following summarizes the Company's capital base and franchise taxes paid (net of refunds received) for the years presented below:

	Year Ended December 31,	
	2025	2024
Federal	\$ -	\$ -
State	551	133
<b>Total</b>	<b>\$ 551</b>	<b>\$ 133</b>

The following summarizes the jurisdictions that exceeded 5% of the Company's total capital base and franchise taxes paid (net of refunds) for the years presented below:

	Year Ended December 31,	
	2025	2024
New York	\$ 352	\$ 98
New York City	\$ 192	\$ 35

## 11. Commitments and Contingencies

**Leases**—As of December 31, 2025, the Company had entered into commitments under lease agreements to rent laboratory and office space and finance equipment (see Note 6).

**Commitments**—As of December 31, 2025, the Company had entered into commitments under license, acquisition, research collaboration and sponsored research agreements with third parties (see Note 12). In addition, the Company has entered into services agreements with third parties for pharmaceutical manufacturing and research activities in the normal course of business, which can generally be terminated by the Company with 30- to 60-days' written notice, unless otherwise indicated. Further, certain of the Company's manufacturing agreements could require early termination and wind-down payments due from the Company upon either the termination of its clinical trials or if the Company terminates such agreements for convenience.

**Contingencies**—From time to time, the Company may have certain contingent liabilities that arise in the ordinary course of business. The Company recognizes any associated legal fees as incurred and accrues a liability for such contingent liability matters when it is probable that future expenditures will be made, and such expenditures can be reasonably estimated. For all periods presented, the Company was not a party to any pending material litigation or other material legal proceedings, except that on October 12, 2023, Rocket Pharmaceuticals, Inc. ("Rocket") filed a lawsuit in the U.S. District Court for the Southern District of New York against the Company and two former employees that claimed, among other things, misappropriation of confidential information and trade secrets. The complaint alleged the individual defendants downloaded confidential Rocket company documents and other proprietary materials prior to leaving Rocket in 2021 and that the Company used this information to advance its programs after they became employed at the Company. The complaint sought unspecified damages and asked the court to enjoin the Company from competing and working in the market for gene therapy treatments targeting cardiac diseases. In August 2024, the Company asserted counterclaims against Rocket and Spacecraft Seven LLC, a wholly owned subsidiary of Rocket, for misappropriation of trade secrets, correction of inventorship of certain patents, breach of contract, and tortious interference with contract. The Company's counterclaims sought equitable relief, damages, attorneys' fees and costs from Rocket and Spacecraft Seven LLC. In June 2025, the litigation was resolved amicably and without admission of liability by any party, and all claims have been dismissed with prejudice.

**Indemnification Agreements**—In the ordinary course of business, the Company may provide indemnification of varying scope and terms to vendors, lessors, business partners and other parties with respect to certain matters including, but not limited to, losses arising out of breach of such agreements or from intellectual property infringement claims made by third parties. In addition, the Company has entered into indemnification agreements with members of its Board of Directors and executive officers that will require the Company, among other things, to indemnify them against certain liabilities that may arise by reason of their status or service as directors or officers. The maximum potential amount of future payments the Company could be required to make under these indemnification agreements is, in many cases, unlimited. For all periods presented, the Company has not incurred any material costs as a result of such indemnifications.

## 12. License, Acquisition, Research and Collaboration and Sponsored Research Agreements

**Adverum Biotechnologies**—On January 25, 2021, the Company entered into an exclusive license agreement with Adverum Biotechnologies Inc. (“Adverum”) to in-license materials and technology related to the treatment of cardiomyopathy due to Friedreich's ataxia (“FA”) (as amended in February 2022, the “Adverum Agreement”). In connection with the Adverum Agreement, the Company gained access to a portfolio of inventions, patent rights, technology, and licensed methods that the Company continues to develop, and the Company will assume all development and commercialization activities worldwide. Pursuant to the Adverum Agreement, the Company paid a one-time up-front non-refundable fee of \$7.5 million, and is obligated to pay aggregate development and regulatory milestones of up to \$17.5 million including a \$3.5 million development milestone that was achieved and paid in the first quarter of 2023, and aggregate sales event and commercialization milestones of up to \$49.0 million. The Company is obligated to pay Adverum tiered royalties ranging from high single-digits to sub teens based on annual aggregate worldwide net sales of Products (as defined in the Adverum Agreement). As of December 31, 2025, there were no research and development expenses recorded by the Company or payments made to Adverum under the terms of the Adverum Agreement other than the one-time up-front non-refundable fee of \$7.5 million and the \$3.5 million development milestone that was achieved and paid in the first quarter of 2023.

The Adverum Agreement remains in effect until termination at the date of the last royalty term to expire. The Company can terminate the Adverum Agreement with 120 days’ written notice. The Adverum Agreement can also be terminated as a result of a patent challenge, material breach of contractual terms, or insolvency by either party.

**Cornell University**—On May 28, 2020, the Company entered into two exclusive license agreements with Cornell University (“Cornell”) (each, as amended, the “First Cornell License Agreement” and the “Second Cornell License Agreement,” respectively; collectively the “May 2020 Cornell License Agreements”). The First Cornell License Agreement is for the in-license of technology related to portfolios for APOE-associated Alzheimer’s disease and Anti-Tau, although the Company’s license is not restricted by such indications and it includes assignment to the Company of Cornell’s IND for the use of AAVrh10.hAPOE2 vector to treat APOE4 homozygous patients who are at risk for or have Alzheimer’s disease to support development of the Company’s LX1001 program. The Second Cornell License Agreement is for the in-license of technology related to a portfolio for FA although the Company’s license is not restricted by such indications, and it includes assignment to the Company of Cornell’s IND for the use of AAVrh.10cUhCLN2 to treat children with CLN2 Batten disease to support development of the Company’s LX1004 program. Through the May 2020 Cornell License Agreements, the Company gains access to a portfolio of inventions, patent rights, technology, and licensed methods that the Company continues to develop. Under the terms of the May 2020 Cornell License Agreements, the Company has assumed all development and commercialization activities worldwide with respect to the licensed technology.

As initial consideration for the May 2020 Cornell License Agreements, the Company paid Cornell an upfront payment in cash of \$0.3 million and issued \$1.3 million of notes (the “Cornell Notes”). In November 2020, the Cornell Notes with outstanding principal of \$1.3 million were cancelled in exchange for 1,337,610 shares of series A convertible preferred stock, which converted into 126,258 shares of common stock upon the closing of the Company’s IPO on November 7, 2023. As additional consideration, the Company is required to pay Cornell up to \$8.4 million upon the achievement of specific clinical and regulatory milestones under the First Cornell License Agreement and up to \$4.3 million in two portfolios and up to \$0.6 million for a third portfolio upon the achievement of specific clinical and regulatory milestones under the Second Cornell License Agreement. In the second quarter of 2022, a clinical and regulatory milestone of \$0.1 million was recognized and paid to Cornell in connection with the Second Cornell License Agreement. The Company is also required to pay Cornell a flat royalty in the mid-single-digits based on net sales of the products covered by the licenses, subject to certain adjustments.

Upon expiration of the royalty term of a given licensed product in a country, the respective license becomes non-exclusive and royalty-free. In addition, each of the May 2020 Cornell License Agreements may be terminated by the Company for any reason upon ninety (90)-days’ advance notice to Cornell and by Cornell upon the Company’s material uncured breach, and all licenses and rights granted by either party under such agreement will concurrently terminate.

On April 21, 2024, the Company entered into the Third License Agreement (as amended, the “Third Cornell License Agreement,” together with the May 2020 Cornell License Agreements, the “Cornell License Agreements”) with Cornell. Pursuant to the Third Cornell License Agreement, Cornell has granted the Company an exclusive license to practice under certain patent rights generated in animal studies conducted by Cornell on behalf of the Company and a non-exclusive license to know-how concerning a gene therapy for FA cardiomyopathy and current and future data generated in an ongoing investigator-initiated Phase 1A trial of AAVrh.10hFXN to treat FA cardiomyopathy. Both licenses are worldwide and cover products with human and non-human prophylactic and therapeutic uses. Cornell has also granted the Company a right of reference to Cornell’s Investigational New Drug application for a gene therapy for FA cardiomyopathy.

Under the Third Cornell License Agreement, the Company paid a license issue fee and an initial data transfer fee to Cornell totaling \$0.6 million. Additionally, the Company will be paying an annual data transfer fee of \$50,000 until data is no longer being gathered. The Company has agreed to pay annual license maintenance fees ranging from \$2,500 to \$25,000 until such time the Company commercializes a licensed product. In addition, the Company will pay Cornell up to an aggregate of \$2.1 million in regulatory milestones and up to an aggregate of \$100 million in commercial milestones, plus low single digit royalties on net sales.

The Third Cornell License Agreement contains other customary license terms including terms related to sublicensing, development, commercialization, milestones, royalties, intellectual property, and termination. Upon expiration of the applicable royalty term for a product in a given country, the Company shall retain a non-exclusive, royalty free license to the data and know-how, including to continue selling such product in that country.

Cornell may terminate the Third Cornell License Agreement if the Company (i) breaches the Third Cornell License Agreement (subject to a cure period), (ii) participates in any proceeding challenging the validity of the licensed patents, (iii) publishes the licensed data without Cornell's prior written consent, or (iv) does not reach certain milestones. Cornell may also terminate the Third Cornell License Agreement in part on a product-by-product basis if the Company does not diligently develop and sell a product. The Company may terminate the Third Cornell License Agreement, in whole or in part with respect to the right of reference, or the licensed data, know-how, or patent rights, with 90 days' prior written notice to Cornell.

On December 17, 2025, the Company executed a Second Amendment to the Third Cornell License Agreement (the "Second Amendment to the Third Cornell License Agreement"), which added a one-time fee of \$0.1 million for additional data transfer and processing.

During the year ended December 31, 2025, the Company incurred \$0.2 million and paid \$0.1 million in connection with the Amended Cornell License Agreements. During the year ended December 31, 2024, the Company incurred and paid \$0.7 million in connection with the Amended Cornell License Agreements.

**Stelios Therapeutics, Inc.**—Stelios Therapeutics, Inc. ("Stelios") was an early-stage company developing novel adeno-associated AAV-based gene therapies for rare cardiac conditions including arrhythmogenic cardiomyopathy and TNNI3-associated hypertrophic cardiomyopathy. On July 16, 2021, the Company acquired 100% of the outstanding stock of Stelios that was accounted for as an asset acquisition pursuant to FASB ASC 805, *Business Combinations*. The Company is required to pay up to a remaining aggregate of \$12.5 million to the selling shareholders of Stelios upon the achievement of certain development milestones, excluding a \$6.0 million development milestone that was achieved in the third quarter of 2024 and paid in the fourth quarter of 2024. Dr. Eric Adler, the Company's former Chief Medical Officer and Head of Research, was a co-founder of Stelios and a selling shareholder. Of the \$6.0 million development milestone payment, Dr. Adler received approximately \$1.3 million. Stelios was merged into the Company on December 15, 2022 and ceased to exist.

**The Regents of the University of California, San Diego**—Stelios entered into an exclusive worldwide license agreement on April 23, 2020 (as amended, the "First UCSD Agreement") with The Regents of UCSD to in-license materials and intellectual property related to a gene therapy for arrhythmogenic right ventricular cardiomyopathy. The First UCSD Agreement relates to the Company's development efforts for its LX2021 program. In connection with the First UCSD Agreement, the Company gained access to inventions, patent rights, technology, and licensed methods that it continues to develop, and it has assumed all worldwide development and commercialization activities with respect to the licensed technology. The First UCSD Agreement required Stelios to pay one-time up-front non-refundable cash fees of \$20,000 for each agreement and requires the Company to pay aggregate development and commercialization milestones of up to \$4.8 million, and low- to mid-single digit royalties and low-single digit royalties based on aggregate net sales. The only research and development expense incurred by Stelios or the Company, and payment made to The Regents of UCSD through December 31, 2025 under the terms of the First UCSD Agreement, was the one-time up-front non-refundable cash fee of \$20,000. The Company has the right to terminate the First UCSD Agreement at any time upon sixty (60)-days' written notice to The Regents of UCSD.

Stelios entered into an exclusive worldwide license agreement on August 6, 2020 (as amended, the "Second UCSD Agreement") with The Regents of UCSD to in-license materials and intellectual property related to a gene therapy for hypertrophic cardiomyopathy. On November 14, 2025, the Company entered into an amended and restated license agreement with UCSD, which amended and restated in its entirety, the Second UCSD Agreement (the "Amended and Restated Second UCSD Agreement"). The Amended and Restated Second UCSD Agreement maintained the Company's prior exclusive worldwide license to materials and intellectual property related to a gene therapy for hypertrophic cardiomyopathy, replaced Stelios with the Company as licensee, and exclusively in-licensed additional materials and intellectual property related to the LX2022 program. Stelios previously paid a one-time up-front non-refundable cash fee of \$20,000 under the Second UCSD Agreement. The Amended and Restated Second UCSD Agreement requires the Company to pay aggregate development and commercialization milestones of up to \$2.4 million, and low-single digit royalties based on aggregate net sales. The only research and development expense incurred by Stelios or the Company, and payment made to The Regents of UCSD through December 31, 2025 under the terms of the Second UCSD Agreement and the Amended and Restated Second UCSD Agreement, was the one-time up-front non-refundable cash fee of \$20,000. The Company has the right to terminate the Amended and Restated Second UCSD Agreement at any time upon sixty (60)-days' written notice to The Regents of UCSD.

On October 4, 2021, the Company entered into an exclusive worldwide license agreement (as amended, the "Third UCSD Agreement" and collectively with the First UCSD Agreement and the Amended and Restated Second UCSD Agreement, the "UCSD Agreements") with The Regents of UCSD to in-license materials and intellectual property related to LX2020, a gene therapy for arrhythmogenic right ventricular cardiomyopathy. The Third UCSD Agreement relates to the Company's development efforts for its LX2020 program. In connection with the Third UCSD Agreement, the Company gained access to inventions, patent rights, technology, and licensed methods that it continues to develop, and it has assumed all worldwide development and commercialization activities with respect to the licensed technology. The Third UCSD Agreement required the Company to pay a one-time up-front non-refundable cash fee of \$20,000 and requires the Company to pay aggregate development and commercialization milestones of up to \$4.0 million, and low-to mid-single digit royalties based on aggregate net sales. Research and development expenses incurred by the Company and payments made to The Regents of UCSD under the terms of the Third UCSD Agreement include the one-time up-front non-refundable cash fee of \$20,000 as well as a \$0.1 million development milestone that was achieved during the third quarter of 2024. The Company has the right to terminate the Third UCSD Agreement at any time upon sixty (60)-days' written notice to The Regents of UCSD.

On December 3, 2021, the Company entered into two sponsored research agreements with The Regents of UCSD (the "First UCSD SRA", the "Second UCSD SRA", and collectively, the "Initial UCSD SRAs") for the Company's LX2020, LX2021 and LX2022 programs in connection with the UCSD Agreements. Under the terms of the Initial UCSD SRAs, the Company has the first rights to obtain non-exclusive or exclusive, sublicensable, royalty-bearing, perpetual and transferable worldwide licenses in any resulting inventions owned by The Regents of UCSD or resulting inventions jointly owned between the Company and The Regents of UCSD, and the Company retains the rights to any resulting inventions owned by the Company. The Initial UCSD SRAs each have a two-year term and may be terminated early by the Company at any time upon the giving of thirty (30)-days' written notice to The Regents of UCSD. The total costs to be invoiced to the Company over the terms of the Initial UCSD SRAs are \$5.6 million.

On April 13, 2024, the Company entered into a third sponsored research agreement with The Regents of UCSD (as amended, the "Third UCSD SRA") for the Company's LX2022 program in connection with the Second UCSD Agreement. Under the terms of the Third UCSD SRA, the Company has the first rights to obtain non-exclusive or exclusive, sublicensable, royalty-bearing, perpetual and transferable worldwide licenses in any resulting inventions owned by The Regents of UCSD or resulting inventions jointly owned between the Company and The Regents of UCSD, and the Company retains the rights to any resulting inventions owned by the Company. The Third UCSD SRA has a two-year term and may be terminated early by the Company at any time upon the giving of thirty (30)-days' written notice to The Regents of UCSD. The costs to be invoiced to the Company over the term of the Third UCSD SRA are \$0.7 million, and the Company may incur additional costs of \$0.6 million under the Third UCSD SRA if certain study objectives are met.

On April 13, 2024, the Company also entered into an amendment to the Second UCSD SRA (as amended, the "Amended Second UCSD SRA") for the Company's LX2022 program that extended the term of the Second UCSD SRA to December 2024.

On each of April 19, 2024 and September 27, 2024, the Company also entered into an amendment to the First UCSD SRA (as amended, the "Amended First UCSD SRA", and together with the Amended Second UCSD SRA and the Third UCSD SRA, the "UCSD SRAs") for the Company's LX2021 program in connection with the First UCSD Agreement. The Amended First UCSD SRA extends the term of the First UCSD SRA to December 2026 and provides for additional research and development studies and expenses. The total costs to be invoiced to the Company under the Amended First UCSD SRA are \$0.7 million.

During the year ended December 31, 2025, the Company incurred \$0.5 million and paid \$0.1 million to The Regents of UCSD in connection with the Amended UCSD SRAs. During the year ended December 31, 2024, the Company incurred \$1.2 million and paid \$1.6 million to The Regents of UCSD in connection with the Amended UCSD SRAs. The Company has incurred and paid cumulative totals of \$5.7 million and \$4.9 million, respectively, to The Regents of UCSD as of December 31, 2025, in connection with the Amended UCSD SRAs.

**Myoventive, Inc.**—In June 2025, the Company entered into a Collaboration and License Agreement with Tilia Therapeutics, Inc., now known as Myoventive, Inc. ("Myoventive" and the "Myoventive Agreement"), to develop therapies for genetic cardiac diseases utilizing a novel non-viral RNA platform.

Under the terms of the Myoventive Agreement, the Company contributed expertise in developing cardiac genetic medicines, certain existing preclinical intellectual property, and technology, which will be combined with a novel non-viral delivery platform. As consideration for its contributions, the Company received a double-digit percentage equity position in Myoventive and will be entitled to future milestone payments, royalties, and opt-in rights to certain program(s).

**Abiomed, Inc. (Johnson & Johnson subsidiary)**—In December 2025, the Company entered into a Collaboration Agreement with Abiomed, Inc. ("Abiomed" and the "Abiomed Agreement"), to investigate localized cardiac delivery of gene therapy. Under the Abiomed Agreement, the parties intend to advance the potential efficacy and safety profile of gene therapy for genetically mediated cardiovascular diseases by concentrating AAV-vector delivery to the heart by investigating cutting-edge routes of administration using the Impella™ heart pump technology. The Company did not incur or pay any research and development costs in connection with the Abiomed Agreement for the year ended December 31, 2025.

### 13. Segment Information

The Company's CODM is its CEO and senior leadership team. All of the Company's operating results are reviewed by the Company's CODM within the same statement of operations and comprehensive loss whether research and development (by program) or general and administrative in nature. Accordingly, the Company has determined that it has a single reportable segment and operating segment structure. The Company's CODM regularly reviews total expenses and expenses by significant areas such as departments and programs to make decisions when evaluating the Company's financial performance. The Company does not evaluate performance or allocate resources based on segment asset data and therefore such information is not presented. All long-lived assets are located in the United States.

The following table contains additional information on the Company's net loss, including significant segment expenses for the years ended December 31, 2025 and December 31, 2024:

	Year Ended December 31,	
	2025	2024
Research and development expense		
LX2006	\$ 18,163	\$ 14,256
LX2020	15,413	23,451
LX1001	1,168	4,501
Other programs	2,075	3,424
Employee compensation expenses	15,183	16,010
Lab-related costs and supplies	1,550	1,367
All other research and development expense	10,245	11,082
General and administrative expense		
Employee compensation expenses	8,461	6,685
All other general and administrative expense	36,999	24,990
Other income and expense, net	(9,296)	(7,433)
Net loss	\$ (99,961)	\$ (98,333)

### 14. Subsequent Events

Subsequent events have been evaluated through March 30, 2026, which is the date that these financial statements were issued and were available to be issued.

In January 2026, warrants to purchase 867,302 shares of the Company's common stock at an exercise price of \$2.82 per share that were issued in the May 2025 Private Placement were exercised with total proceeds of approximately \$2.4 million received by the Company (see Note 7).

**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**

Our management, with the participation of our principal executive officer and principal financial officer, has evaluated the effectiveness of our disclosure controls and procedures (as defined in Rules 13a-15(e) and 15d-15(e) under the Exchange Act), as of December 31, 2025. Based on such evaluation, our principal executive officer and principal financial officer have concluded that, as of such date, the design and operation of our disclosure controls and procedures were effective at a reasonable assurance level.

**Management's Report on Internal Control Over Financial Reporting**

Our management is responsible for establishing and maintaining adequate internal control over financial reporting, as such term is defined in Rule 13a-15(f) promulgated under the Exchange Act. Under the supervision and with the participation of our management, including our Chief Executive Officer and Chief Financial Officer, our management assessed and conducted an evaluation of the effectiveness of our internal control over financial reporting based on the Internal Control—Integrated Framework (2013 framework) issued by the Committee of Sponsoring Organizations of the Treadway Commission. Based on this assessment, our management concluded that our internal control over financial reporting was effective as of December 31, 2025.

**Attestation Report of the Registered Public Accounting Firm**

This Annual Report on Form 10-K does not include an attestation report of our registered public accounting firm regarding the effectiveness of internal control over financial reporting as required by Section 404(b) of the Sarbanes-Oxley Act of 2002. Management's report was not subject to attestation by our registered public accounting firm pursuant to rules of the SEC related to emerging growth and smaller reporting companies.

**Changes in Internal Control Over Financial Reporting**

There were no changes in our internal control over financial reporting identified in management's evaluation pursuant to Rules 13a-15(d) or 15d-15(d) of the Exchange Act during the fourth quarter of the year ended December 31, 2025, that materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

**Limitations on Effectiveness of Controls and Procedures**

Our management, including our principal executive officer and principal financial officer, do not expect that our disclosure controls and procedures or our internal control over financial reporting will prevent all errors and all fraud. A control system, no matter how well designed and operated, can provide only reasonable, not absolute, assurance that the objectives of the control system are met. Further, the design of a control system must reflect the fact that there are resource constraints, and the benefits of controls must be considered relative to their costs. Because of the inherent limitations in all control systems, no evaluation of controls can provide absolute assurance that all control issues and instances of fraud, if any, have been detected. These inherent limitations include the realities that judgments in decision-making can be faulty, and that breakdowns can occur because of a simple error or mistake. Additionally, controls can be circumvented by the individual acts of some persons, by collusion of two or more people or by management override of the controls. The design of any system of controls is also based in part upon certain assumptions about the likelihood of future events, and there can be no assurance that any design will succeed in achieving its stated goals under all potential future conditions; over time, controls may become inadequate because of changes in conditions, or the degree of compliance with policies or procedures may deteriorate. Due to inherent limitations in a cost-effective control system, misstatements due to error or fraud may occur and not be detected.

**Item 9B. Other Information.**

*Securities Trading Plans of Directors and Executive Officers*

On November 13, 2025, R. Nolan Townsend, our Chief Executive Officer and a member of our board of directors, adopted a Rule 10b5-1 trading arrangement providing for the sale from time to time of an aggregate of up to 116,500 shares of common stock, commencing on April 3, 2026. The trading arrangement is intended to satisfy the affirmative defense in Rule 10b5-1(c). The duration of the trading arrangement is until January 11, 2027, or earlier if all transactions under the trading arrangement are completed.

No other officers, as defined in Rule 16a-1(f), or directors adopted or terminated a “Rule 10b5-1 trading arrangement” or a “non-Rule 10b5-1 trading arrangement,” as defined in Regulation S-K Item 408, during the last fiscal quarter.

**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 definitive proxy statement for the annual meeting of stockholders to be held in June 2026 to be filed with the SEC on or before April 30, 2026, or the Proxy Statement, and is incorporated herein by reference.

### **Item 11. Executive Compensation.**

The information required by this item will be included in the Proxy Statement and is incorporated herein by reference.

### **Item 12. Security Ownership of Certain Beneficial Owners and Management and Related Stockholder Matters.**

The information required by this item will be included in the Proxy Statement and is incorporated herein by reference.

### **Item 13. Certain Relationships and Related Transactions, and Director Independence.**

The information required by this item will be included in the Proxy Statement and is incorporated herein by reference.

### **Item 14. Principal Accounting Fees and Services.**

The information required by this item will be included in the Proxy Statement and is incorporated herein by reference.

**PART IV**

**Item 15. Exhibits and Financial Statement Schedules.**

**EXHIBIT INDEX**

Exhibit Number	Description	Form	File No.	Number	Filing Date
3.1	<u>Amended and Restated Certificate of Incorporation of the Registrant (as amended and currently in effect)</u>	8-K	001-41855	3.1	November 7, 2023
3.2	<u>Amended and Restated Bylaws of the Registrant (as amended and currently in effect)</u>	8-K	001-41855	3.2	November 7, 2023
4.1 <del>¶</del>	<u>Amended and Restated Investors' Rights Agreement, dated August 10, 2021, by and among the Registrant and certain of its stockholders</u>	S-1	333-274777	4.1	September 29, 2023
4.2	<u>Form of Pre-Funded Warrant</u>	8-K	001-41855	4.1	May 27, 2025
4.3	<u>Form of Pre-Funded Warrant</u>	8-K	001-41855	4.1	October 17, 2025
4.4	<u>Form of Common Warrant</u>	8-K	001-41855	4.2	May 27, 2025
4.5	<u>Form of Common Stock Certificate of the Registrant</u>	S-1/A	333-274777	4.2	October 30, 2023
4.6	<u>Description of Securities of the Registrant</u>	10-K	001-41855	4.3	March 11, 2024
10.1+	<u>2021 Equity Incentive Plan, as amended from time to time and Form of Stock Option Agreement, Early Exercise Notice and Restricted Stock Purchase Agreement, and Exercise Notice</u>	10-Q	001-41855	10.1	August 12, 2024
10.2	<u>Form of Registration Rights Agreement dated, May 27, 2025, by and among the Company and the Purchasers</u>	8-K	001-41855	10.2	May 27, 2025
10.3	<u>Form of Registration Rights Agreement dated, October 16, 2025, by and among the Company and the Purchaser</u>	8-K	001-41855	10.2	October 17, 2025
10.4+	<u>2023 Equity Incentive Plan and Forms of Option Grant Notice and Agreement, Exercise Notice and Restricted Stock Unit Award Notice</u>	S-1/A	333-274777	10.2	October 30, 2023
10.5+	<u>2023 Employee Stock Purchase Plan</u>	S-1/A	333-274777	10.3	October 30, 2023
10.6 <del>¶</del> †	<u>Third License Agreement, dated April 21, 2024, by and between Cornell University and the Company</u>	10-Q	001-41855	10.3	August 12, 2024
10.7†	<u>Amendment No. 1, dated May 21, 2024, to the Third License Agreement, dated April 21, 2024, by and between Cornell University and the Company</u>	10-Q	001-41855	10.3	August 14, 2025
10.8+	<u>Employment Agreement, as amended on September 14, 2025, by and between the Company and R. Nolan Townsend</u>	10-Q	001-41855	10.3	November 5, 2025
10.9+	<u>Employment Agreement, as amended on September 14, 2025, by and between the Company and Jose Manuel Otero</u>	10-Q	001-41855	10.4	November 5, 2025
10.10+	<u>Form of Indemnification Agreement with Executive Officers and Directors</u>	S-1	333-274777	10.4	September 29, 2023
10.11+	<u>2025 Inducement Equity Incentive Plan and related form documents</u>	10-Q	001-41855	10.5	November 5, 2025
10.12+	<u>Employment Agreement, as amended on September 14, 2025, by and between the Company and Louis Tamayo</u>	10-Q	001-41855	10.6	November 5, 2025

10.13+†	<u>Consulting Agreement, dated October 9, 2020, between LEXEO Therapeutics, LLC and Ronald G. Crystal, M.D.</u>	S-1	333-274777	10.8	September 29, 2023
10.14*+†	<u>Transition and Consulting Agreement, effective February 1, 2026, by and between LEXEO Therapeutics, Inc. and Eric Adler, M.D.</u>				
10.15†	<u>Amendment No. 4, effective March 5, 2025, to the First License Agreement, dated May 28, 2020, by and between LEXEO Therapeutics, LLC and Cornell University.</u>	10-Q	001-41855	10.8	November 5, 2025
10.16†	<u>Amendment No. 3, effective April 1, 2024, to the Second License Agreement, dated May 28, 2020, by and between LEXEO Therapeutics, LLC and Cornell University.</u>	10-Q	001-41855	10.9	November 5, 2025
10.17¥†	<u>Stock Purchase Agreement, dated July 16, 2021, by and among LEXEO Therapeutics, Inc., Stelios Therapeutics, Inc., The Cystinosis Research Foundation, Eric Adler, M.D., Farah Sheikh, Ph.D., Jeffrey M. Ostrove, Ph.D., and Stephanie Cherqui, Ph.D., and Jeffery M. Ostrove, Ph.D., as Stockholders' Representative</u>	S-1	333-274777	10.10	September 29, 2023
10.18¥†	<u>License Agreement, dated October 4, 2021, by and between LEXEO Therapeutics, Inc. and The Regents of the University of California</u>	S-1	333-274777	10.11	September 29, 2023
10.19†	<u>Amendment No. 1, effective August 8, 2024 to the License Agreement, dated October 4, 2021, by and between LEXEO Therapeutics, Inc. and The Regents of the University of California</u>	10-Q	001-41855	10.10	November 5, 2025
10.20*†	<u>Amendment No. 2, effective May 28, 2025 to the License Agreement, dated October 4, 2021, by and between LEXEO Therapeutics, Inc. and The Regents of the University of California</u>				
10.21¥†	<u>License Agreement, dated April 23, 2020, by and between Stelios Therapeutics, Inc. (as successor-in-interest to ARVC Therapeutics, Inc.) and The Regents of the University of California</u>	S-1	333-274777	10.12	September 29, 2023
10.22†	<u>Amendment No. 1, effective June 4, 2025, to the License Agreement, dated August 6, 2020, by and between Stelios Therapeutics, Inc. and The Regents of the University of California</u>	10-Q	001-41855	10.12	November 5, 2025
10.23*†	<u>Amended and Restated License Agreement, effective November 14, 2025, by and between Lexeo Therapeutics, Inc. and The Regents of the University of California</u>				
10.24¥†	<u>License Agreement, dated August 6, 2020, by and between Stelios Therapeutics, Inc. and The Regents of the University of California</u>	S-1	333-274777	10.13	September 29, 2023
10.25†	<u>Amendment No. 1, effective May 30, 2025, to the License Agreement, dated April 23, 2020, by and between Stelios Therapeutics, Inc. (as successor-in-interest to ARVC Therapeutics, Inc.) and The Regents of the University of California</u>	10-Q	001-41855	10.13	November 5, 2025
10.26¥†	<u>ARVC Research Agreement, dated December 3, 2021, between LEXEO Therapeutics, Inc. and The Regents of the University of California, as amended by Amendment No. 1, dated April 5, 2023</u>	S-1	333-274777	10.14	September 29, 2023

10.27†	<u>TNNI3 Research Agreement, dated December 3, 2021, between LEXEO Therapeutics, Inc. and The Regents of the University of California, as amended by Amendment No. 1, dated April 19, 2023</u>	S-1	333-274777	10.15	September 29, 2023
10.28†	<u>First License Agreement, dated May 28, 2020, between LEXEO Therapeutics, LLC and Cornell University.</u>	S-1	333-274777	10.16	September 29, 2023
10.29†	<u>Second License Agreement, dated May 28, 2020, between LEXEO Therapeutics, LLC and Cornell University.</u>	S-1	333-274777	10.17	September 29, 2023
10.30†	<u>Amendment No. 1, dated January 13, 2022, to the Second License Agreement, dated May 28, 2020, by and between LEXEO Therapeutics, Inc. and Cornell University.</u>	S-1	333-274777	10.18	September 29, 2023
10.31†	<u>Amendment No. 1, dated July 4, 2022, to the First License Agreement, dated May 28, 2020, by and between LEXEO Therapeutics, Inc. and Cornell University and Amendment No. 2, dated July 1, 2022, to the Second License Agreement, dated May 28, 2020 (as amended by Amendment No. 1 to the Second License Agreement, dated January 13, 2022)</u>	S-1	333-274777	10.19	September 29, 2023
10.32†	<u>Amendment No. 2, dated September 28, 2022, to the First License Agreement, dated May 28, 2020, by and between LEXEO Therapeutics, Inc. and Cornell University (as amended by Amendment No. 1 to the First License Agreement, dated July 4, 2022)</u>	S-1	333-274777	10.20	September 29, 2023
10.33†	<u>License Agreement, dated January 19, 2021, between Adverum Biotechnologies, Inc. and LEXEO Therapeutics, Inc., as amended by the First Amendment, dated February 28, 2022.</u>	S-1	333-274777	10.22	September 29, 2023
10.34	<u>Amendment No. 3, dated February 11, 2024, to the First License Agreement, dated May 28, 2020, by and between Lexeo Therapeutics, Inc. and Cornell University (as amended by Amendment No. 1 to the First License Agreement, dated July 4, 2022 and Amendment No. 2 to the First License Agreement, dated September 28, 2022)</u>	S-1	333-278566	10.21	April 9, 2024
10.35†	<u>Third Sponsored Research Agreement, dated April 13, 2024, by and between the Company and The Regents of University of California, San Diego</u>	10-K	001-41855	10.27	March 24, 2025
10.36†	<u>Amendment No. 2, dated April 13, 2024, to the TNNI3 Research Agreement, dated December 3, 2021, between LEXEO Therapeutics, Inc. and The Regents of the University of California, as amended</u>	10-K	001-41855	10.28	March 24, 2025
10.37†	<u>Amendment No. 2, dated August 31, 2023, to the ARVC Research Agreement, dated December 3, 2021, between LEXEO Therapeutics, Inc. and The Regents of the University of California, as amended</u>	10-K	001-41855	10.29	March 24, 2025
10.38†	<u>Amendment No. 3, dated April 19, 2024, to the ARVC Research Agreement, dated December 3, 2021, between LEXEO Therapeutics, Inc. and The Regents of the University of California, as amended</u>	10-K	001-41855	10.30	March 24, 2025
10.39†	<u>Amendment No. 4, dated September 27, 2024 to the ARVC Research Agreement, dated December 3, 2021, between LEXEO Therapeutics, Inc. and The Regents of the University of California, as amended</u>	10-K	001-41855	10.31	March 24, 2025

10.40	<u>Sales Agreement, dated as of March 24, 2025, by and between Lexeo Therapeutics, Inc. and Leerink Partners LLC</u>	10-K	001-41855	10.32	March 24, 2025
10.41*†	<u>Amendment No. 2, effective December 22, 2025, to the Third License Agreement, dated April 21, 2024, by and between Lexeo Therapeutics, Inc. and Cornell University, as amended</u>				
10.42**†	<u>Transition and Consulting Agreement, effective December 1, 2025, by and between LEXEO Therapeutics, Inc. and Jenny Robertson</u>				
14.1	<u>Code of Ethics</u>	10-K	001-41855	14.1	March 11, 2024
19.1	<u>Insider Trading and Window Period Policy</u>	10-K	001-41855	19.1	March 24, 2025
23.1*	<u>Consent of KPMG LLP, independent registered public accounting firm.</u>				
24.1*	<u>Power of Attorney (contained in the signature page to this Annual Report on Form 10-K).</u>				
31.1*	<u>Certification of Principal Executive Officer Pursuant to Rules 13a-14(a) and 15d-14(a) under the Securities Exchange Act of 1934, as Adopted Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.</u>				
31.2*	<u>Certification of Principal Financial Officer Pursuant to Rules 13a-14(a) and 15d-14(a) under the Securities Exchange Act of 1934, as Adopted Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.</u>				
32.1**	<u>Certification of Principal Executive Officer Pursuant to 18 U.S.C. Section 1350, as Adopted Pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.</u>				
32.2**	<u>Certification of Principal Financial Officer Pursuant to 18 U.S.C. Section 1350, as Adopted Pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.</u>				
97.1	<u>Policy Relating to Recovery of Erroneously Awarded Compensation</u>	10-K	001-41855	97.1	March 11, 2024
101.INS	Inline XBRL Instance Document-the Instance Document does not appear in the Interactive Data File because XBRL tags are embedded within the Inline XBRL document.				
101.SCH	Inline XBRL Taxonomy Extension Schema Document				
101.CAL	Inline XBRL Taxonomy Extension Calculation Linkbase Document				
101.DEF	Inline XBRL Taxonomy Extension Definition Linkbase Document				
101.LAB	Inline XBRL Taxonomy Extension Label Linkbase Document				
101.PRE	Inline XBRL Taxonomy Extension Presentation Linkbase Document				
104	Cover Page Interactive Data File (formatted as inline XBRL with applicable taxonomy extension information contained in Exhibits 101)				

\* Filed herewith.

+ Indicates management contract or compensatory plan.

¥ Schedules have been omitted pursuant to Item 601(a)(5) of Regulation S-K. The Registrant undertakes to furnish supplemental copies of any of the omitted schedules upon request by the SEC.

† Portions of this exhibit (indicated by [\*\*]) have been omitted because the registrant has determined that the information is both not material and is the type that the Registrant treats as private or confidential.

# The certification attached as Exhibit 32.1 that accompanies this Annual Report on Form 10-K is not deemed filed with the Securities and Exchange Commission and is not to be incorporated by reference into any filing of the Registrant under the Securities Act of 1933, as amended, or the Securities Exchange Act of 1934, as amended, whether made before or after the date of this Annual Report on Form 10-K, irrespective of any general incorporation language contained in such filing.

**Item 16. Form 10-K Summary**

The Company has elected not to include summary information.

## 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 Annual Report to be signed on its behalf by the undersigned, thereunto duly authorized.

### LEXEO THERAPEUTICS, INC.

By: /s/ R. Nolan Townsend  
R. Nolan Townsend  
*Chief Executive Officer*

## POWER OF ATTORNEY

KNOW ALL PERSONS BY THESE PRESENTS, that each person whose signature appears below constitutes and appoints R. Nolan Townsend, Louis Tamayo and Youjin Choi as his or her 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 attorney-in-fact and agent full power and authority to do and perform each and every act and thing requisite and necessary to be done in and about the premises, as fully to all intents and purposes as he or she might or could do in person, hereby ratifying and confirming that the attorney-in-fact and agent, or his or her substitute or substitutes, may lawfully do or cause to be done by virtue hereof.

Pursuant to the requirements of the Securities Exchange Act of 1934, this Annual Report on Form 10-K has been signed below by the following persons on behalf of the Registrant and in the capacities and on the dates indicated:

<u>Signature</u>	<u>Title</u>	<u>Date</u>
<u>/s/ R. Nolan Townsend</u> R. Nolan Townsend	Chief Executive Officer and Director <i>(Principal Executive Officer)</i>	March 30, 2026
<u>/s/ Louis Tamayo</u> Louis Tamayo	Chief Financial Officer <i>(Principal Financial and Accounting Officer)</i>	March 30, 2026
<u>/s/ Mette Kirstine Agger</u> Mette Kirstine Agger	Director	March 30, 2026
<u>/s/ Steven Altschuler</u> Steven Altschuler, M.D.	Director	March 30, 2026
<u>/s/ Paula HJ Cholmondeley</u> Paula HJ Cholmondeley	Director	March 30, 2026
<u>/s/ Brenda Cooperstone</u> Brenda Cooperstone, M.D.	Director	March 30, 2026
<u>/s/ Reinaldo Diaz</u> Reinaldo Diaz	Director	March 30, 2026
<u>/s/ Tolga Tanguler</u> Tolga Tanguler	Director	March 30, 2026
<u>/s/ Tim Van Hauwermeiren</u> Tim Van Hauwermeiren	Director	March 30, 2026

[\*\*\*] = CERTAIN CONFIDENTIAL INFORMATION CONTAINED IN THIS DOCUMENT, MARKED BY BRACKETS, HAS BEEN OMITTED BECAUSE IT IS BOTH (I) NOT MATERIAL AND (II) IS THE TYPE THAT THE REGISTRANT TREATS AS PRIVATE OR CONFIDENTIAL.

**TRANSITION AND CONSULTING AGREEMENT**

January 30, 2026

Eric Adler, M.D.  
[\*\*\*]  
[\*\*\*]

Dear Dr. Adler:

This letter sets forth the agreement (the “**Agreement**”) between you and Lexeo Therapeutics, Inc. (the “**Company**”) in connection with your separation of employment from the Company, and your transition into a temporary consulting role.

**1. Separation Date.** Your last day of employment with the Company will be January 31, 2026 (the “**Separation Date**”). Regardless of whether you sign this Agreement, you will be paid at your current rate of pay, through the Separation Date, less applicable federal, state and local withholding taxes and authorized deductions (the “**Accrued Compensation**”). As of the Separation Date, you will cease holding any position or title with the Company as an employee (although you may be authorized to speak, and make representations, on behalf of the Company in your capacity as a consultant as set forth in Paragraph 2 below), and you will no longer be eligible or entitled to receive any compensation other than as expressly set forth herein.

**2. Transition Consulting Services.**

(a) Beginning on February 1, 2026 and continuing through July 31, 2026, or on an earlier date if terminated by either party as provided for below (the “**Consulting Period**” and the last day of the Consulting Period, the “**Service End Date**”), you will provide certain advisory services (the “**Services**”) for the Company as a consultant, and specifically in the role of Senior Scientific Advisor, including the following:

- Provide high-level scientific advisory input and guidance on the Company’s preclinical pipeline around major milestones or at key decision points.
- Offer ad hoc support for the Company’s research collaboration with Johnson & Johnson.
- Represent the Company at predetermined conferences and investor interactions.

(b) You and the Company agree that the maximum number of hours per month that you will provide Services to the Company during the Consulting Period is twenty (20) hours.

(c) As compensation for the Services and as consideration for the promises and obligations in this Agreement, the Company will provide the following (collectively, the “**Consulting Compensation**”): cash compensation in the total amount of \$62,500, paid monthly (i.e., six payments of \$10,416.67) no later than the last day of each month of the Consulting Period.

(d) Your relationship with Company during the Consulting Period will be that of an independent contractor, and nothing in this Agreement is intended to, or should be construed to, create a partnership, agency, joint venture or employment relationship. Except as provided herein, you will not be entitled to any of the benefits that the Company may make available to its employees, including, but not limited to, group health, life insurance, profit-sharing or retirement benefits, paid vacation, holidays or sick leave. You will not be authorized to make any representation, contract or commitment on behalf of the Company unless specifically requested or authorized in writing to do so by the Chief Executive Officer of the Company. You will be solely responsible for obtaining any business or similar licenses required by any federal, state or local authority. You acknowledge and agree that you will indemnify the Company for any tax liability associated with the Services provided during the Consulting Period under this Agreement.

(e) In accordance with the Company's objectives, you will determine the method, details and means of performing the services required by this Agreement. The Company shall have no right to, and shall not, control the manner or determine the method of performing your services. You shall provide the services for which you are engaged to the reasonable satisfaction of the Company. The Company may suggest to you, from time to time, methods or strategies the Company believes may assist you in the performance of your services under this Agreement. Consistent with your independent contractor status, however, you shall exercise your independent business discretion in determining whether or not to follow the Company's suggestions. You may perform the services required by this Agreement at any place or location and at such times as you shall determine. You agree to provide all tools and instrumentalities, if any, required to perform the services under this Agreement.

(f) During the Consulting Period, you will be in a "continuous service relationship as a Service Provider" with the Company under the Company's 2021 Equity Incentive Plan (the "**2021 Plan**") and in "Continuous Service" with the Company under the Company's 2023 Equity Incentive Plan (the "**2023 Plan**") and, together with the 2021 Plan, the "**Plans**"), such that any equity awards (the "**Equity Awards**") issued pursuant to the Plans will continue to vest during the Consulting Period. Notwithstanding the foregoing, none of the Performance Share Units awarded pursuant to your February 14, 2025 compensation letter (the "**PSUs**") remaining unvested as of the date of this letter shall vest following the Separation Date. Instead, the PSUs will forfeit as of the Separation Date. The period in which to exercise any options issued pursuant to the Plans shall be extended to six (6) months following the Service End Date.

(g) You and the Company agree that the Company's offer for you to continue providing services as a consultant following the Separation Date, constitutes consideration for the promises and obligations set forth in this Agreement, including but not limited to the release in paragraph 15 below.

(h) During the Consulting Period, the Company shall provide you with indemnification coverage under the Company's D&O liability insurance policies to the same extent as directors and executive officers.

**3. Term and Termination; Supplemental Release.** This Agreement is effective as of the Effective Date (as defined herein) and will continue during the Consulting Period, unless earlier terminated as provided for herein.

(a) The Company may terminate this Agreement immediately upon written notice to you, in the event of your (i) material breach of this Agreement or the Employee Confidential Information and Inventions Assignment Agreement between you and the Company; (ii) misappropriation or attempted misappropriation of any Company funds or material Company property, or any appropriation or attempted appropriation by you of a business or corporate opportunity of the Company; (iii) engagement in misconduct that adversely affects the business or reputation of the Company; (iv) commission of a willful or intentional act which results in material injury or is reasonably likely to result in material injury to the business or reputation of the Company; (v) use of alcohol or drugs in a manner which affects your job performance; or (vi) arrest, conviction, or plea of guilty or "no contest" to any felony or misdemeanor involving dishonestly, theft, fraud, misrepresentation, or moral turpitude. If this Agreement is terminated by the Company pursuant to this Paragraph 3(a), you will not be required to pay any of the Consulting Compensation to the Company.

(b) You may terminate this Agreement upon seven (7) days written notice to the Company. If this Agreement is terminated by you, you will have no obligation to repay any portion of the Consulting Compensation that you have already received and will receive the full monthly payment for the month during which you terminate the Agreement, but you will not be entitled to receive any portion of the Consulting Compensation for any future months remaining in the Consulting Period.

(c) You agree that on or within a reasonable time after the end of the Service End Date, you will execute and return to the Company a Supplemental Release (in the form attached as **Exhibit A** hereto). As consideration for the Supplemental Release, you will receive \$1,000 as a lump sum payment within thirty (30) days of your execution of the Supplemental Release.

**4. Compensation or Benefits.** You acknowledge that, except as expressly provided in this Agreement, you have not earned and will not receive from the Company any additional compensation (including base salary, bonus, incentive compensation, or equity), severance, or benefits before or after the Separation Date, with the exception of any vested right you may have under the express terms of a written ERISA-qualified benefit plan (e.g., 401(k) account) or any vested stock options.

**5. Return of Company Property.** Except to the extent any retention of Company property is authorized in writing by the Chief Executive Officer of the Company (in connection with your provision of the Services), you agree that, by the Service End Date, you will return to the Company all Company documents (and all copies thereof) and other Company property in your possession or control, including, but not limited to, Company files, notes, drawings, records, plans, forecasts, reports, studies, analyses, proposals, agreements, drafts, financial and operational information, research and development information, sales and marketing information, customer lists, prospect information, pipeline reports, sales reports, personnel information, specifications, code, software, databases, computer-recorded information, tangible property and equipment (including, but not limited to, computing and electronic devices, mobile telephones, servers), credit cards, entry cards, identification badges and keys; and any materials of any kind which contain or embody any proprietary or confidential information of the Company (and all reproductions or embodiments thereof in whole or in part). You agree that you will make a diligent search to locate any such documents, property and information by the close of business on the Separation Date or as soon as possible thereafter. If you have used any personally-owned computer or other electronic device, server, or e-mail system to receive, store, review, prepare or transmit any Company confidential or proprietary data, materials or information, by the Service End Date, you shall provide the Company with a computer-useable copy of such information and then permanently delete and expunge such Company confidential or proprietary information from those systems; and you agree to provide the Company access to your system as requested to verify that the necessary copying and/or deletion is completed.

**6. Proprietary Information Obligations.** You acknowledge and reaffirm your continuing obligations under your Employee Confidential Information and Inventions Assignment Agreement, a copy of which is attached hereto as **Exhibit B**.

**7. Confidentiality.** The provisions of this Agreement will be held in strictest confidence by you and will not be publicized or disclosed by you in any manner whatsoever; *provided, however*, that: (a) you may disclose this Agreement in confidence to your immediate family and to your attorneys, accountants, tax preparers and financial advisors; and (b) you may disclose this Agreement insofar as such disclosure may be necessary to enforce its terms or as otherwise required by law. In particular, and without limitation, you agree not to disclose the terms of this Agreement to any current or former Company employee or independent contractor, except to the extent permitted by Paragraph 9.

**8. Non-disparagement.** You agree not to disparage the Company, its officers, directors, employees, shareholders, parents, subsidiaries, affiliates, and agents, in any manner likely to be harmful to its or their business, business reputation, or personal reputation; provided that you may respond accurately and fully to any request for information if required by legal process or in connection with a government investigation. The Company agrees that it will direct its senior executives not to disparage you in any manner that may be harmful to your business or personal reputation. Nothing in this provision or this Agreement is intended to prohibit or restrain you in any manner from making disclosures protected under the whistleblower provisions of federal or state law or regulation or other applicable law or regulation. You agree to revise and update publicly available information, including professional and social networking websites such as LinkedIn and Facebook, within one (1) week of the Service End Date to remove any indication that you are employed by or providing services to the Company.

**9. Transition Communications.** The Company and you shall mutually agree on the substance and timing of any internal or external communications regarding your transition, including but not limited to announcements to employees, investors, partners, or the public. Neither party shall make or authorize any such communications without the prior written consent of the other party, which shall not be unreasonably withheld or delayed. The parties shall use good faith efforts to finalize such communications promptly following the date of signature.

**10. References.** In response to any reference request from a prospective employer, the Company will only confirm your dates of employment and position.

**11. No Voluntary Adverse Action.** You agree that you will not voluntarily (except in response to legal compulsion or as permitted under the Protected Rights section below) assist any person in bringing or pursuing any proposed or pending litigation, arbitration, administrative claim or other formal proceeding against the Company, its parent or subsidiary entities, affiliates, officers, directors, employees or agents.

**12. Cooperation.** During the one (1) year period following the Service End Date, you agree to cooperate fully with the Company in connection with its actual or contemplated defense, prosecution, or investigation of any claims or demands by or against third parties, or other matters arising from events, acts, or failures to act that occurred during the period of your employment by the Company. Such cooperation includes, without limitation, making yourself available to the Company upon reasonable notice, without subpoena, to provide complete, truthful and accurate information in witness interviews, depositions, and trial testimony. The Company will reimburse you for reasonable out-of-pocket expenses you incur in connection with any such cooperation (excluding foregone wages and attorneys' fees) and will make reasonable efforts to accommodate your scheduling needs and in no event will you be required to provide such cooperation for more than one (1) full business day in any one (1) week period.

**13. No Conflict of Interest.** During the term of this Agreement, you will not accept work, enter into a contract, or accept an obligation, inconsistent or incompatible with your obligations, or the scope of services rendered for the Company, under this Agreement. You warrant that, to the best of your knowledge, there is no other contract or duty on your part that conflicts with or is inconsistent with this Agreement. This paragraph does not prevent you from performing the same or similar services for clients other than the Company so long as such services do not directly or indirectly conflict with your obligations under this Agreement.

**14. No Admissions.** You understand and agree that the promises and payments in consideration of this Agreement shall not be construed to be an admission of any liability or obligation by the Company to you or any other person, and the Company makes no such admission.

**15. Release of Claims.** In exchange for the consideration provided to you under this Agreement to which you would not otherwise be entitled, you hereby generally and completely release the Company, and its affiliated, related, parent and subsidiary entities, and its and their current and former directors, officers, employees, shareholders, partners, agents, attorneys, predecessors, successors, insurers, affiliates, and assigns from any and all claims, liabilities, demands, causes of action, and obligations, both known and unknown, arising from or in any way related to events, acts, conduct, or omissions occurring at any time prior to and including the date you sign this Agreement. This general release includes, but is not limited to: (a) all claims arising from or in any way related to your employment with the Company or the termination of that employment; (b) all claims related to your compensation or benefits from the Company, including salary, bonuses, commissions, vacation pay, expense reimbursements, severance pay, fringe benefits, stock, stock options, or any other ownership, equity, or profits interests in the Company; (c) all claims for breach of contract, wrongful termination, and breach of the implied covenant of good faith and fair dealing; (d) all tort claims, including claims for fraud, defamation, emotional distress, and discharge in violation of public policy; and (e) all federal, state, and local statutory claims, including claims for discrimination, harassment, retaliation, attorneys' fees, or other claims arising under the federal Civil Rights Act of 1964 (as amended), the federal Americans with Disabilities Act of 1990, the Age Discrimination in Employment Act ("ADEA"), the New York State Human Rights Law, the New York Labor Law, the New York Wage Theft Prevention Act, Section 125 of the New York Workers' Compensation Law, the New York City Human Rights Law, the New York City Administrative Code, the Massachusetts Fair Employment Practices Act, the Massachusetts Equal Pay Act, the Massachusetts Civil Rights Act, the Massachusetts Labor and Industries Act, the Massachusetts Right to Privacy law, and the Massachusetts Wage Act, all as amended, and any other federal, state or local statutes, regulations, ordinances prohibiting discrimination or regulating employment or the payment of wages. Notwithstanding the foregoing, you are not releasing the Company hereby from: (i) any obligation to indemnify you pursuant to the Articles and Bylaws of the Company, the Indemnification Agreement, applicable law, or applicable directors and officers liability insurance; (ii) any claims that cannot be waived by law; or (iii) any claims for breach of this Agreement.

**16. ADEA Release.** You acknowledge that you are knowingly and voluntarily waiving and releasing any rights you have under the ADEA, and that the consideration given for the waiver and releases you have given in this Agreement is in addition to anything of value to which you were already entitled. You further acknowledge that you have been advised, as required by the ADEA, that: (a) your waiver and release does not apply to any rights or claims arising after the date you sign this Agreement; (b) you should consult with an attorney prior to signing this Agreement (although you may choose voluntarily not to do so); (c) you have twenty-one (21) days to consider this Agreement (although you may choose voluntarily to sign it sooner); (d) you have seven (7) days following the date you sign this Agreement to revoke this Agreement (in a written revocation sent to the Company, specifically to Jennifer Speer via email at [jcspeer@lexeotx.com](mailto:jcspeer@lexeotx.com)); and (e) this Agreement will not be effective until the date upon which the revocation period has expired, which will be the eighth day after you sign this Agreement provided that you do not revoke it (the "**Effective Date**").

**17. Protected Rights.** You understand that nothing in this Agreement limits your ability to file a charge or complaint with the Equal Employment Opportunity Commission, the Department of Labor, the National Labor Relations Board, the Occupational Safety and Health Administration, the New York State Department of Labor, the Securities and Exchange Commission or any other federal, state or local governmental agency or commission ("**Government Agencies**"). You further understand this Agreement does not limit your ability to communicate with any Government Agencies or otherwise participate in any investigation or proceeding that may be conducted by any Government Agency, including providing documents or other information, without notice to the Company. While this Agreement does not limit your right to receive an award for information provided to the Securities and Exchange Commission, you understand and agree that, to maximum extent permitted by law, you are otherwise waiving any and all rights you may have to individual relief based on any claims that you have released and any rights you have waived by signing this Agreement.

**18. Breach; Attorneys' Fees.** You acknowledge and agree that any material breach of this Agreement, unless such breach constitutes a legal action by you challenging or seeking a determination in good faith of the validity of the waiver herein under the ADEA, or its exhibits (if any) shall entitle the Company immediately to recover and/or cease providing the consideration provided to you under this Agreement and to obtain damages, except as provided by law, provided, however, that the Company shall not recover Fifty Dollars (\$50.00) of the consideration already paid pursuant to this Agreement and such amount shall serve as full and complete consideration for the promises and obligations assumed by you under this Agreement and its exhibits. In addition, except with regard to a legal action challenging or seeking a determination in good faith of the validity of the waiver herein under the ADEA, in the event that either party brings an action to enforce or effect its rights under this Agreement, the prevailing party shall be entitled to recover its costs and expenses, including the costs of mediation, arbitration, litigation, court fees, and reasonable attorneys' fees incurred in connection with such an action.

**19. Representations.** You hereby represent that you have: been paid all compensation owed and for all hours worked; received all leave and leave benefits and protections for which you are eligible pursuant to the Family and Medical Leave Act and any state leave laws; and have not suffered any on-the-job injury for which you have not already filed a workers' compensation claim.

**20. Dispute Resolution.** You and the Company agree that any and all disputes, claims, or controversies of any nature whatsoever arising from, or relating to, this Agreement or its interpretation, enforcement, breach, performance or execution, your employment or the termination of such employment (including, but not limited to, any statutory claims) (collectively, "**Claims**", each a "**Claim**"), shall be resolved, pursuant to the Federal Arbitration Act, 9 U.S.C. §1-16, and to the fullest extent permitted by law, by final, binding and confidential arbitration in New York, New York (or another mutually acceptable location) conducted before a single neutral arbitrator by JAMS, Inc. ("**JAMS**") or its successor, under the then applicable JAMS Arbitration Rules and Procedures for Employment Disputes (available at <http://www.jamsadr.com/rules-employment-arbitration/>). **By agreeing to this arbitration procedure, both you and the Company waive the right to have any Claim resolved through a trial by jury or judge or an administrative proceeding.** You will have the right to be represented by legal counsel at any arbitration proceeding, at your own expense. This paragraph shall not apply to any action or claim that cannot be subject to mandatory arbitration as a matter of law. The arbitrator shall have sole authority for determining if a Claim is subject to arbitration, and any other procedural questions related to the dispute and bearing on the final disposition. In addition, the arbitrator shall: (a) have the authority to compel adequate discovery for the resolution of the dispute and to award such relief as would otherwise be available under applicable law in a court proceeding; and (b) issue a written statement signed by the arbitrator regarding the disposition of each claim and the relief, if any, awarded as to each claim, the reasons for the award, and the arbitrator's essential findings and conclusions on which the award is based. You and the Company shall equally share all JAMS' arbitration fees. To the extent JAMS does not collect or you otherwise do not pay to JAMS an equal share of all JAMS' arbitration fees for any reason, and the Company pays JAMS your share, you acknowledge and agree that the Company shall be entitled to recover from you half of the JAMS arbitration fees invoiced to the parties (less any amounts you paid to JAMS) in a federal or state court of competent jurisdiction. Nothing in this letter agreement is intended to prevent either you or the Company from obtaining injunctive relief in court to prevent irreparable harm pending the conclusion of any such arbitration. Any awards or orders in such arbitrations may be entered and enforced as judgments in the federal and state courts of any competent jurisdiction.

**21. Complete Agreement.** This Agreement, including Exhibits A and B, constitutes the complete, final and exclusive embodiment of the entire agreement between you and the Company with regard to its subject matter. You agree and acknowledge that this Agreement is entered into without reliance on any promise or representation, written or oral, other than those expressly contained herein, and it supersedes any other such promises, warranties or representations.

**22. Miscellaneous.**

(a) This Agreement may not be modified or amended except in a writing signed by both you and a duly authorized officer of the Company.

(b) This Agreement will bind the heirs, personal representatives, successors and assigns of both you and the Company, and inure to the benefit of both you and the Company, their heirs, successors and assigns.

(c) If any provision of this Agreement is determined to be invalid or unenforceable, in whole or in part, this determination will not affect the validity or enforceability of any other provision of this Agreement and the provision in question will be modified so as to be rendered enforceable to the fullest extent permitted by law, consistent with the intent of the parties. Moreover, if one or more of the provisions contained in this Agreement is held to be excessively broad as to duration, scope, or subject, such provisions shall be construed by limiting or reducing them so as to be enforceable to the maximum extent compatible with applicable law.

(d) This Agreement will be deemed to have been entered into and will be construed and enforced in accordance with the laws of the State of New York without regard to conflict of laws principles.

(e) Any ambiguity in this Agreement shall not be construed against either party as the drafter. Any waiver of any condition or breach of this Agreement shall be in writing and shall not be deemed to be a waiver of any other condition or breach, nor shall the failure or delay by either of the parties in exercising any right, power, or privilege hereunder operate as a waiver thereof to preclude any other or further exercise thereof or the exercise of any other such right, power, or privilege.

(f) This Agreement may be executed in counterparts and electronic or facsimile signatures will suffice as original signatures.

[signature on following page]

If this Agreement is acceptable to you, please sign below and return the original to me. You have twenty-one calendar days to decide whether to accept this Agreement, and the Company's offer contained herein will automatically expire if you do not sign and return it within that timeframe.

Sincerely,

By: /s/ R. Nolan Townsend  
**R. NOLAN TOWNSEND**  
Chief Executive Officer

**I HAVE READ, UNDERSTAND AND AGREE FULLY TO THE FOREGOING AGREEMENT:**

/s/ Eric Adler, M.D.  
**ERIC ADLER, M.D.**  
1/30/2026  
Date

EXHIBIT A

SUPPLEMENTAL RELEASE

[\*\*\*]

**EXHIBIT B  
CONFIDENTIAL INFORMATION  
AND INVENTIONS ASSIGNMENT AGREEMENT**

[\*\*\*]

[\*\*\*] = CERTAIN CONFIDENTIAL INFORMATION CONTAINED IN THIS DOCUMENT, MARKED BY BRACKETS, HAS BEEN OMITTED BECAUSE IT IS BOTH (I) NOT MATERIAL AND (II) IS THE TYPE THAT THE REGISTRANT TREATS AS PRIVATE OR CONFIDENTIAL.

**AMENDMENT No. 2  
TO THE LICENSE AGREEMENT UC CONTROL No. [\*\*\*] BETWEEN LEXEO THERAPEUTICS AND  
THE REGENTS OF THE UNIVERSITY OF CALIFORNIA**

**Lexeo Therapeutics and the Regents of the University of California** (“UNIVERSITY”) represented by its San Diego campus (“UC SAN DIEGO”) entered into a License Agreement, UC SAN DIEGO Control Number [\*\*\*] (“Agreement”) effective October 4, 2021 and amended (Amendment No.1) effective August 8, 2024. The parties now wish to amend such Agreement, to be effective upon the date of last signature of this Amendment No.2 (“Amendment Effective Date”) to include certain modifications. These modifications are to be substituted for the relevant sections and/or paragraphs in the Agreement, and are effective from the Amendment Effective Date.

For these purposes, the parties agree on the following changes as detailed below.

**ARTICLE 5. PATENT MATTERS.**

**Delete Paragraph 5.1 in its entirety and replace it with the following:**

“

- (a) LICENSEE shall be responsible for the prosecution and maintenance of any Patent Rights claiming or covering any Inventions, and UNIVERSITY shall reasonably cooperate with LICENSEE in connection with the same, including, upon the request of LICENSEE, promptly executing any and all patent applications, formal documents, assignments, or other instruments which LICENSEE deems necessary or reasonably useful for the filing, prosecution, and maintenance, of any Patent Rights claiming or covering any such Inventions, which may be filed or prepared at LICENSEE's cost and expense.
- (b) LICENSEE shall keep UNIVERSITY reasonably informed of the prosecution of Patent Rights. LICENSEE shall provide UNIVERSITY with a copy of material communications from any patent authority regarding such Inventions, and shall provide drafts of any material filings or responses to be made to such patent authorities a reasonable amount of time in advance of submitting such filings or responses so that UNIVERSITY may have an opportunity to review and comment.
- (c) If LICENSEE plans to abandon, cease prosecution or not maintain any patent(s) or patent application(s) in Patent Rights, then to avoid abandonment or other loss of rights, LICENSEE will provide UNIVERSITY with [\*\*\*] notice of such plan and UNIVERSITY shall have the right, in UNIVERSITY's sole discretion, to file for or continue prosecution and/or maintenance of such patent or patent application at its own expense.
- (d) For clarity, nothing in this Amendment relieves LICENSEE from its obligation to make disclosures to UNIVERSITY.”

In consideration for this Amendment No. 2, LICENSEE shall pay UNIVERSITY an amendment fee of [\*\*\*] within [\*\*\*] of receipt of an invoice.

All other terms and conditions in the Agreement between LICENSEE and UNIVERSITY effective October 4, 2021 shall remain unchanged and in effect.

The parties agree this Amendment No. 2 may be executed electronically and in two or more counterparts each of which shall be deemed an original and all of which together shall constitute but one and the same document.

*IN WITNESS WHEREOF*, both UNIVERSITY and LICENSEE have executed this Agreement by their respective and duly authorized officers on the day and year written.

**LEXEO THERAPEUTICS, INC.:**

By: /s/ Jenny Robertson  
(Signature)

Jenny Robertson

Chief Business & Legal Officer

Date: 5/28/2025

**THE REGENTS OF THE  
UNIVERSITY OF CALIFORNIA:**

By: /s/ Anthony Reza Razavi, Ph.D.  
(Signature)

Anthony Reza Razavi, Ph.D.

Associate Director - Innovation and  
Commercialization

Date: 2025-05-16

[\*\*\*] = CERTAIN CONFIDENTIAL INFORMATION CONTAINED IN THIS DOCUMENT, MARKED BY BRACKETS, HAS BEEN OMITTED BECAUSE IT IS BOTH (I) NOT MATERIAL AND (II) IS THE TYPE THAT THE REGISTRANT TREATS AS PRIVATE OR CONFIDENTIAL.

**AMENDED AND RESTATED LICENSE AGREEMENT BETWEEN  
LEXEO THERAPEUTICS, INC.**

**AND**

**THE REGENTS OF THE UNIVERSITY OF CALIFORNIA FOR  
CASE NO. [\*\*\*]  
CASE NO. [\*\*\*]  
CASE NO. [\*\*\*]**

“Drug Development for TNNI3 Mutations”

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## LICENSE AGREEMENT

This agreement (“Agreement”) is entered into as of the date of August 6, 2020 (the "Effective Date") by and between LEXEO THERAPEUTICS, INC., a Delaware corporation having an address at 345 Park Avenue South, 6<sup>th</sup> Floor, New York, NY 10010 (“LICENSEE”) and The Regents of the University of California, a California public corporation having its statewide administrative offices at 1111 Franklin Street, Oakland, California 94607-5200 (“UNIVERSITY”), represented by its San Diego campus having an address at University of California, San Diego, Office of Innovation and Commercialization, Mail Code 0910, 9500 Gilman Drive, La Jolla, California 92093-0910 (“UC SAN DIEGO”).

### BACKGROUND

A. The inventions disclosed in UC San Diego Disclosure Docket Nos. below (“Inventions”)

Docket #	Title	Inventors
[***]	[***]	[***]
[***]	[***]	[***]
[***]	[***]	[***]

were made in the course of research at UC San Diego by [\*\*\*] (hereinafter, and collectively the “Inventors”), and are covered by Patent Rights as defined below.

B. Each Inventor is an employee of UC San Diego, and is obligated to assign all of their rights, titles and interests in the Inventions to UNIVERSITY.

C. The research was sponsored in part by the Government of the United States of America and as a consequence this license is subject to overriding obligations to the Federal Government under 35 U.S.C. §§ 200-212 and applicable regulations.

D. UC San Diego and Stelios Therapeutics, Inc. entered into a license agreement effective August 6, 2020 for the license of UC San Diego Disclosure Docket No. [\*\*\*] to Stelios Therapeutics, Inc. (the “First License”).

E. LICENSEE acquired Stelios Therapeutics, Inc. on July 16, 2021, and LICENSEE became the successor in interest to Stelios Therapeutics, Inc. under the First License.

F. LICENSEE and UC San Diego subsequently amended the First License in an amendment dated June 4, 2025.

G. LICENSEE and UC San Diego now desire to enter into this Agreement to amend and restate the First License to the Patent Rights and Technology to LICENSEE for commercial development, use, and sale of the Inventions.

The parties agree as follows:

## **ARTICLE 1. DEFINITIONS**

The terms, as defined herein, have the same meanings in both their singular and plural forms.

1.1 "Affiliate" means any corporation, firm, limited liability company, partnership or other entity that directly or indirectly Controls or is Controlled by or is under common control with LICENSEE. "Control" means (i) having the actual, present capacity to elect a majority of the directors of such entity; (ii) having the power to direct at least fifty percent (50%) of the voting rights entitled to elect directors; or (iii) in any country where the local law will not permit foreign equity participation of a majority, ownership or control, directly or indirectly, of the maximum percentage of such outstanding stock or voting rights permitted by local law.

1.2 "Commercially Reasonable Efforts" means exerting the efforts and employing the resources that would normally and objectively be exerted or employed by a similarly situated company in its industry for a product of similar market potential, profit potential and strategic value at a similar stage of its product life, taking into account the competitiveness of the relevant marketplace, the patent, intellectual property and development positions of third parties, the applicable regulatory situation, the pricing/reimbursement situation, the commercial viability of the product and other relevant development and commercialization factors based upon then-prevailing conditions. Commercially Reasonable Efforts shall in no case involve a shelving of the development, marketing or sale of Licensed Products or a suspension of the diligent pursuit of needed governmental approval for Licensed Products for a period longer than [\*\*\*].

1.3 "Field" means all diagnostic and therapeutic uses.

1.4 "Licensed Product" means any service, composition or product which is composed of or incorporates, or is directly or indirectly discovered, developed and/or identified using, the Technology, or that is covered by a valid claim in the Patent Rights, or the manufacture, use, sale, offer for sale, or importation of which would constitute, but for the license granted to LICENSEE under this Agreement, an infringement, an inducement to infringe or contributory infringement, of any pending or issued claim within the Patent Rights.

1.5 "Net Sales" means the total of the gross invoice prices of Licensed Products sold or leased by LICENSEE, Sublicensee, Affiliate, or any combination thereof, less the sum of the following actual and customary deductions where applicable and separately listed: cash, trade, or quantity discounts, credits, refunds or rebates (as allowed under applicable law); sales tax, use tax, tariff, custom and import/export duties or other excise taxes imposed on sales of Licensed Product (except for value-added and income taxes imposed on the sales of Licensed Product in foreign countries); transportation charges; and credits to customers, including allowances, charge backs or rebates actually granted because of rejections or returns. For purposes of calculating Net Sales, transfers to a Sublicensee or an Affiliate of Licensed Product under this Agreement for (a) end use (but not resale) by the Sublicensee or Affiliate shall be treated as sales by LICENSEE at list price of LICENSEE, or (b) resale by a Sublicensee or an Affiliate shall be treated as sales at the list price of the Sublicensee or Affiliate. Net Sales shall not include reasonable quantities

of Licensed Products provided without charge for charitable, promotional, pre-clinical, clinical, regulatory, or governmental purposes.

1.6 “Patent Costs” means all out-of-pocket expenses for the preparation, filing, prosecution, and maintenance of all United States and foreign patents included in Patent Rights. Patent Costs include out-of-pocket expenses for patentability opinions, inventorship determination, preparation and prosecution of patent application, re-examination, re-issue, interference, post-grant review and other administrative proceedings in patent offices, and opposition activities, and the like, related to patents or applications in Patent Rights.

1.7 “Patent Rights” means UNIVERSITY’s rights in the claims of any of the following: (a) any patent or patent applications containing claims that cover the Inventions, (b) the U.S. patents and patent applications listed in Exhibit A; and (c) continuing applications thereof including divisions, substitutions, and continuations-in-part (but only to the extent the claims thereof are entirely supported in the specification and entitled to the priority date of the parent application); any patents issuing on said applications including reissues, reexaminations and extensions; and any corresponding foreign applications or patents covered by subsections (a) through (c) above.

1.8 “Sublicense” means an agreement into which LICENSEE enters with a third party that is not an Affiliate for the purpose of (a) granting certain rights; (b) granting an option to certain rights; or (c) forbearing the exercise of any rights, in each case granted to LICENSEE under this Agreement. “Sublicensee” means a third party with whom LICENSEE enters into a Sublicense.

1.9 “Sublicense Fees” means all upfront fees, milestone payments and similar license fees received by LICENSEE from its Sublicensees in consideration for the grant of a Sublicense, but excluding: (i) any royalty payments; (ii) payments for [\*\*\*]; (iii)[\*\*\*]; and (iv) payments and reimbursement of [\*\*\*]. For the purpose of the above paragraph, [\*\*\*].

1.10 “Technology” means the written nonpublic technical information, if any, relating to the Invention which the Inventors provided to LICENSEE prior to the Effective Date and which is detailed in Exhibit C.

1.11 “Term” means the period of time beginning on the Effective Date and ending on the expiration date of the longest-lived Patent Rights.

1.12 “Territory” means (a) for Technology, worldwide, to the extent UNIVERSITY may lawfully grant such Technology rights; and (b) for Patent Rights, worldwide, to the extent Patent Rights exist in a respective jurisdiction.

1.13 “Valid Claim” means (a) any claim of an issued and unexpired patent of Patent Rights, which has not been finally rejected, revoked, or declared invalid or unenforceable by a patent office or court of competent jurisdiction by a decision, which is unappealable or unappealed or (b) any claim of a pending patent application of Patent Rights, which claim was filed and is prosecuted in good faith with the goal of ultimately reaching allowable claims and has not been disclaimed, abandoned, or finally disallowed without the possibility of appeal or refiling of such application.

## ARTICLE 2. GRANTS

2.1 **License.** Subject to the limitations set forth in this Agreement and to the extent that it may lawfully do so, UNIVERSITY hereby grants to LICENSEE an exclusive license under Patent

Rights to make, use, sell, offer for sale, and import Licensed Products and a non-exclusive license to use Technology, in the Field within the Territory and during the Term. LICENSEE may extend such license to its AFFILIATES, provided that LICENSEE will be responsible for such AFFILIATES.

## 2.2 Sublicense.

(a) The license granted in Paragraph 2.1 includes the right of LICENSEE to grant Sublicenses to third parties during the Term but only for as long as [\*\*\*].

(b) With respect to Sublicense granted pursuant to Paragraph 2.2(a), LICENSEE shall:

- (i) not [\*\*\*] for the license grant under Sublicense[\*\*\*];
- (ii) to the extent applicable, include all the rights of and obligations due to UNIVERSITY and contained in this Agreement;
- (iii) promptly provide UNIVERSITY with a copy of each Sublicense issued which may be reasonably redacted; and
- (iv) collect and guarantee payment of all payments due, directly or indirectly, to UNIVERSITY from Sublicensees and summarize and deliver all reports due, directly or indirectly, to UNIVERSITY from Sublicensees.

(c) Upon termination of this Agreement for any reason, UNIVERSITY, at its sole discretion, shall determine whether LICENSEE shall cancel or assign to UNIVERSITY any and all Sublicenses; provided, however, that if the Sublicensee requests an assignment, UNIVERSITY will accept such assignment of the Sublicense, provided (i) that the Sublicensee is in good standing upon termination of this Agreement; (ii) [\*\*\*]; and (iii) to the extent UNIVERSITY is legally able to accept such assignment. In no case, however, will UNIVERSITY be bound by duties and obligations contained in any Sublicense that extends beyond the duties and obligations of the UNIVERSITY set forth in this Agreement. For the avoidance of doubt, AFFILIATES' rights extended by LICENSEE also terminate upon termination of this Agreement.

2.1 **Reservation of Rights.** UNIVERSITY reserves the right to:

- (a) use the Invention, Technology, and Patent Rights for educational and research purposes;
- (b) publish or otherwise disseminate any information about the Invention, Technology, and Patent Rights at any time; and
- (c) allow other nonprofit institutions to use, publish, or otherwise disseminate any information about Invention, Technology, and Patent Rights for educational and research purposes.

**ARTICLE 3. CONSIDERATION**

3.1 **Fees and Royalties.** The parties hereto understand that the fees and royalties payable by LICENSEE to UNIVERSITY under this Agreement are partial consideration for the license granted herein to LICENSEE under Technology and Patent Rights. LICENSEE shall pay UNIVERSITY:

- (a) a first license issue fee of twenty thousand dollars (US\$20,000), no later than [\*\*\*] after the Effective Date;

A second license issue fee of twenty thousand dollars (US\$20,000), within [\*\*\*] following the date an itemized invoice sent from UNIVERSITY to LICENSEE.

This Paragraph 3.1(a) will survive the termination, expiration or assignment of this Agreement.

- (b) the following license maintenance fees per year and payable on the [\*\*\*] of the Effective Date and annually thereafter on each anniversary; provided however, that (i) such maintenance fees will be creditable against earned royalties in any given payment period and (ii) LICENSEE’s obligation to pay this fee shall end on the date when LICENSEE is commercially selling a Licensed Product

Maintenance Fee	Anniversary
[***]	[***]
[***]	[***]
[***]	[***]
[***]	[***]
[***]	[***]

- (c) LICENSEE shall pay UNIVERSITY the following milestone payments within [\*\*\*] of achievement for each time such event occurs with regard to [\*\*\*]:

<u>Amount</u>	<u>Event</u>
[***]	[***]
[***]	[***]
[***]	[***]
[***]	[***]

\*\*\*]                    [\*\*\*]  
\*\*\*]                    [\*\*\*]

(d) During the Term, LICENSEE shall pay UNIVERSITY an earned royalty of [\*\*\*] on annual Net Sales of Licensed Products by LICENSEE, Sublicensees, and/or Affiliates; provided, however, that in the event LICENSEE is required to pay royalties to one or more third parties for patent rights necessary to make, use or sell Licensed Products, LICENSEE may deduct [\*\*\*] from the earned royalties payable to UNIVERSITY for every [\*\*\*] LICENSEE actually pays to said third parties; provided, however, in no event shall the amount payable to UNIVERSITY be less than [\*\*\*] of the amount otherwise due; and

(e) [\*\*\*] of all Sublicense Fees until [\*\*\*], at which time the percentage of Sublicense Fees due to UNIVERSITY shall be reduced to [\*\*\*].

All fees and royalty payments specified in Paragraphs 3.1(a) through 3.1(e) above shall be paid by LICENSEE pursuant to Paragraph 4.3 and shall be delivered by LICENSEE to UNIVERSITY as noted in Paragraph 10.1.

**3.2 Patent Costs.** LICENSEE shall reimburse UNIVERSITY for all past (prior to the date of the last signature of this Agreement) Patent Costs incurred in the Territory within [\*\*\*] following the date an itemized invoice sent from UNIVERSITY to LICENSEE.

### **3.3 Due Diligence.**

(a) LICENSEE shall, either directly or through its Affiliate(s) or Sublicensee(s) use Commercially Reasonable Efforts to:

- (i) diligently develop, manufacture, and sell Licensed Products; and
- (ii) achieve the milestones described in Exhibit B.

(b) If LICENSEE fails to perform any of its obligations specified in Paragraphs 3.3(a)(i)-(ii), then UNIVERSITY shall have the right and option to either terminate this Agreement or change LICENSEE's exclusive license under Patent Rights to a nonexclusive license. This right, if exercised by UNIVERSITY, supersedes the rights granted in Article 2. Notwithstanding the foregoing, the deadlines for the diligence milestones set forth in Paragraph 3.3.(a) shall be extended for up to two (2) years by the proportionate length of any delay caused by an applicable regulatory authority where such delay by the applicable regulatory authority was not the result of the LICENSEE's actions or inactions and was not the result of LICENSEE's failure to abide by the applicable regulatory authority's instructions or LICENSEE's failure to provide data to the applicable regulatory authority in the form and manner required by such regulatory authority. For clarity, any extension of a diligence milestone will automatically extend each subsequent diligence milestone by the same amount of time.

## **ARTICLE 4. REPORTS, RECORDS AND PAYMENTS**

### **4.1 Reports.**

(a) **Progress Reports.** Beginning [\*\*\*] after the Effective Date and within [\*\*\*] after the end of each of LICENSEE's fiscal years until the first commercial sale of a Licensed Product, Licensee shall furnish UNIVERSITY with a written report on the progress of its efforts during the immediately preceding fiscal year to develop and commercialize Licensed Products. The report shall provide a discussion, [\*\*\*], of [\*\*\*]. LICENSEE's fiscal year begins on January 1.

(b) **Royalty Reports.** After the first commercial sale of a Licensed Product anywhere in the world, LICENSEE shall submit to UNIVERSITY annual royalty reports on or before [\*\*\*]. Each royalty report shall cover LICENSEE's (and each Affiliate's and Sublicensee's) most recently completed calendar year and shall show:

- (i) the date of first commercial sale of a Licensed Product in each country;
- (ii) the gross sales, deductions as provided in Paragraph 1.4 (Net Sales), and Net Sales during the most recently completed calendar year and the royalties, in US dollars, payable with respect thereto;
- (iii) the number of each type of Licensed Product sold;
- (iv) Sublicense Fees and royalties received during the most recently completed calendar year in US dollars, payable with respect thereto;
- (v) the method used to calculate the royalties; and
- (vi) the exchange rates used.

If no sales of Licensed Products have been made and no Sublicense Fees have been received by LICENSEE during any reporting period, LICENSEE shall so report. The reports referred to in this Paragraph 4.1(b) should be marked with the following title and case number: "License Agreement between UC SAN DIEGO and STELIOS for case [\*\*\*]." Reports shall be submitted as an attachment to UC SAN DIEGO's email address: [\*\*\*].

#### 4.2 Records & Audits.

(a) LICENSEE shall keep, and shall require its Affiliates and Sublicensees to keep, accurate and correct records of all Licensed Products manufactured, used, sold, offered for sale, and imported and Sublicensee Fees received under this Agreement. Such records shall be retained by LICENSEE for at least [\*\*\*] following a given reporting period.

(b) All records shall be available during normal business hours for inspection at the expense of UNIVERSITY by UNIVERSITY's Internal Audit Department or by a Certified Public Accountant selected by UNIVERSITY and in compliance with the other terms of this Agreement for the sole purpose of verifying reports and payments or other compliance issues. Such inspector shall be subject to a confidentiality agreement with LICENSEE and such inspector shall not disclose to UNIVERSITY any information other than information relating to the accuracy of reports and payments made under this Agreement or other compliance issues. In the event that any such inspection shows an under reporting and underpayment in excess of [\*\*\*] for any [\*\*\*], then LICENSEE shall pay the cost of the audit as well as any additional sum that would have been payable to UNIVERSITY had the LICENSEE reported correctly, plus an interest charge at a rate of [\*\*\*] per year. Such interest shall be calculated from the date the correct payment was due to UNIVERSITY up to the date when such payment is actually made by LICENSEE. For underpayment not in excess of [\*\*\*] for [\*\*\*], LICENSEE shall pay the difference within [\*\*\*] without interest charge or inspection cost.

#### 4.3 Payments.

(a) All fees, reimbursements and royalties due UNIVERSITY shall be paid in United States dollars and all checks (should payment by wire not be possible) shall be made payable to [\*\*\*], and sent to UNIVERSITY according to Paragraph 10.1 (Correspondence).

(b) **Royalty Payments.** Royalties shall accrue when [\*\*\*]. LICENSEE shall pay earned royalties annually on or before February 28 of each calendar year. Each such payment shall be for earned royalties accrued within LICENSEE's most recently completed calendar year.

(c) **Late Payments.** In the event royalty, reimbursement and/or fee payments are not received by UNIVERSITY when due, LICENSEE shall pay to UNIVERSITY interest charges at a rate of [\*\*\*]. Such interest shall be calculated from [\*\*\*].

(d) **Taxes.** Taxes imposed by any governmental agency on any payments to be made to UNIVERSITY by LICENSEE hereunder shall be paid by LICENSEE without deduction from any payment due to UNIVERSITY hereunder.

## **ARTICLE 5. PATENT MATTERS**

### **5.1 Patent Prosecution and Maintenance.**

(a) LICENSEE shall be responsible for the prosecution and maintenance of any Patent Rights claiming or covering any Inventions, and UNIVERSITY shall reasonably cooperate with LICENSEE in connection with the same, including, upon the request of LICENSEE, promptly executing any and all patent applications, formal documents, assignments, or other instruments which LICENSEE deems necessary or reasonably useful for the filing, prosecution, and maintenance, of any Patent Rights claiming or covering any such Inventions, which may be filed or prepared at LICENSEE's cost and expense.

(b) LICENSEE shall keep UNIVERSITY reasonably informed of the prosecution of Patent Rights. LICENSEE shall provide UNIVERSITY with a copy of material communications from any patent authority regarding such Inventions, and shall provide drafts of any material filings or responses to be made to such patent authorities a reasonable amount of time in advance of submitting such filings or responses so that UNIVERSITY may have an opportunity to review and comment.

(c) If LICENSEE plans to abandon, cease prosecution or not maintain any patent(s) or patent application(s) in Patent Rights, then to avoid abandonment or other loss of rights, LICENSEE will provide UNIVERSITY with at least thirty (30) days' notice of such plan and UNIVERSITY shall have the right, in UNIVERSITY's sole discretion, to file for or continue prosecution and/or maintenance of such patent or patent application at its own expense.

### **5.2 Patent Infringement.**

(a) If UC SAN DIEGO (based on actual knowledge of the licensing professional responsible for administering this Invention) or LICENSEE learns of potential infringement of commercial significance of any patent licensed under this Agreement, the knowledgeable party promptly will inform the other party of the infringement and provide evidence of infringement available to the knowledgeable party ("Infringement Notice"). In a jurisdiction where LICENSEE has exclusive rights under this Agreement, neither UNIVERSITY nor LICENSEE will notify a third party (including the infringer) of infringement or put such third party on notice of the existence of any Patent Rights without first obtaining consent of the other. UNIVERSITY and LICENSEE agree to discuss and determine how best to proceed. If LICENSEE notifies a third party of infringement or puts such third party on notice of the existence of any Patent Rights regarding such infringement without first obtaining the written consent of UNIVERSITY and UNIVERSITY is sued for declaratory judgment (or its equivalent), UNIVERSITY will have the right to terminate this Agreement immediately, notwithstanding Paragraph 7.1. UNIVERSITY and LICENSEE will use diligent efforts to cooperate with each other to terminate such infringement without litigation. Subject to statutory deadlines, if such infringement has not ended within [\*\*\*] of the effective date of the Infringement Notice, then LICENSEE may initiate suit (or earlier depending on an applicable statutory deadline to file); and, if such infringement has not

ended within [\*\*\*] of the effective date of the Infringement Notice, and LICENSEE has not initiated suit, then UNIVERSITY may initiate suit.

- (b) Notwithstanding the foregoing: (1) UNIVERSITY may not be joined in any suit without its prior written consent; (2) LICENSEE may not admit liability or wrongdoing on behalf of UNIVERSITY without its prior written consent; (3) Each party will cooperate with the other in litigation initiated under Paragraph 5.2, but at the expense of the party who initiated the suit; (4) If UNIVERSITY is joined in any suit under Paragraph 5.2, LICENSEE will pay all of UNIVERSITY's costs; (5) If UNIVERSITY is a party to a suit under Paragraph 5.2, then the recovery to UNIVERSITY will be [\*\*\*] of net recoveries;
- (6) Any agreement made by LICENSEE for purposes of settling litigation or other dispute regarding Patent Rights will comply with the requirements of Paragraph 2.2 (Sublicense); and
- (7) If LICENSEE or UC SAN DIEGO (but not both) sues a third party for infringement of Patent Rights, then the non-suing party may not thereafter sue such infringer for the acts of infringement raised in the suit.

### 5.3 Patent Marking.

LICENSEE shall mark all Licensed Products made, used, sold, offered for sale, or imported under the terms of this Agreement, or their containers, in accordance with the applicable patent marking laws. LICENSEE shall be responsible for all monetary and legal liabilities arising from or caused by (a) failure to abide by applicable patent marking laws and (b) any type of incorrect or improper patent marking.

## ARTICLE 6. EXPORT CONTROL AND REGISTRATION

6.1 **Export Control.** LICENSEE shall observe all applicable United States and foreign laws with respect to the transfer of Licensed Products, Technology, and related technical data to foreign countries, including, without limitation, the International Traffic in Arms Regulations and the Export Administration Regulations.

6.2 **Governmental Approval or Registration.** If this Agreement or any associated transaction is required by the law of any nation to be either approved or registered with any governmental agency, LICENSEE shall assume all legal obligations to do so. LICENSEE shall make all necessary filings and pay all costs including fees, penalties, and all other out-of-pocket costs associated with such reporting or approval process.

**6.3 Preference for United States Industry.** If LICENSEE sells a Licensed Product or Combination Product in the US, LICENSEE shall manufacture said product substantially in the US.

## **ARTICLE 7. TERMINATION OR EXPIRATION OF THE AGREEMENT**

### **7.1 Termination by UNIVERSITY.**

- (a) UNIVERSITY may terminate this Agreement if LICENSEE:
- (i) is delinquent on any report or payment;
  - (ii) is not diligently developing and commercializing Licensed Product in accordance with obligations under Section 3.3;
  - (iii) misses a milestone described in Exhibit B, subject to the provisions of Section 3.3(b);
  - (iv) is in breach of any provision;
  - (v) provides any intentionally false report; or
  - (vi) files a claim including in any way the assertion that any portion of UNIVERSITY's Patent Rights is invalid or unenforceable.
- (b) Termination under this Paragraph 7.1 will take effect [\*\*\*] after written notice by UNIVERSITY unless LICENSEE remedies the problem in that [\*\*\*] period.

### **7.2 Termination by LICENSEE.**

- (a) LICENSEE shall have the right at any time and for any reason to terminate this Agreement in whole or on a Licensed Product-by-Licensed Product, country-by-country basis, upon [\*\*\*] written notice to UNIVERSITY. Said notice shall state LICENSEE's reason for terminating this Agreement.
- (b) Any termination under Paragraph 7.2(a) shall not relieve LICENSEE of any obligation or liability accrued under this Agreement prior to termination or rescind any payment made to UNIVERSITY or action by LICENSEE prior to the time termination becomes effective. Termination shall not affect in any manner any rights of UNIVERSITY arising under this Agreement prior to termination.

**7.3 Survival on Termination or Expiration.** The rights and obligations under Paragraphs and Articles 3.1(a) (license issue fee), 4 (Reports, Records and Payments), 8 (Limited Warranty and Indemnification), 9 (Use of Names and Trademarks), 10.2 (Secrecy), 10.3 (Assignability), and 10.5 (Failure to Perform) shall survive the termination or expiration of this Agreement.

## **ARTICLE 8. LIMITED WARRANTY AND INDEMNIFICATION**

### **8.1 No Warranty.**

- (a) The license granted herein and the associated Technology are provided "AS IS" and without WARRANTY OF MERCHANTABILITY or WARRANTY OF FITNESS FOR A

PARTICULAR

PURPOSE or any other warranty, express or implied. UNIVERSITY makes no representation or warranty that the Licensed Product or the use of Patent Rights or Technology will not infringe any other patent or other proprietary rights.

UNIVERSITY WILL NOT BE LIABLE FOR ANY LOST PROFITS, COSTS OF PROCURING SUBSTITUTE GOODS OR SERVICES, LOST BUSINESS, ENHANCED DAMAGES FOR INTELLECTUAL PROPERTY INFRINGEMENT OR MISAPPROPRIATION OF TECHNOLOGY, OR FOR ANY INDIRECT, INCIDENTAL, CONSEQUENTIAL, PUNITIVE, OR OTHER SPECIAL DAMAGES SUFFERED BY LICENSEE, SUBLICENSEES, JOINT VENTURES, OR AFFILIATES ARISING OUT OF OR RELATED TO THIS AGREEMENT FOR ALL CAUSES OF ACTION OF ANY KIND (INCLUDING TORT, CONTRACT, NEGLIGENCE, STRICT LIABILITY AND BREACH OF WARRANTY) EVEN IF UNIVERSITY HAS BEEN ADVISED OF THE POSSIBILITY OF SUCH DAMAGES. ALSO, UNIVERSITY WILL NOT BE LIABLE FOR ANY DIRECT DAMAGES SUFFERED BY LICENSEE, SUBLICENSEES, JOINT VENTURES, OR AFFILIATES ARISING OUT OF OR RELATED TO PATENT RIGHTS TO THE EXTENT ASSIGNED, OR OTHERWISE LICENSED, BY UNIVERSITY'S INVENTORS TO THIRD PARTIES.

(b) Nothing in this Agreement shall be construed as:

- (i) a warranty or representation by UNIVERSITY as to the validity, enforceability, or scope of any Patent Rights or Technology;
- (ii) a warranty or representation that anything made, used, sold or otherwise disposed of under any license granted in this Agreement is or shall be free from infringement of patents of third parties;
- (iii) an obligation to bring or prosecute actions or suits against third parties for patent infringement or misappropriation of Technology except as provided in Paragraph 5.2 hereof;
- (iv) conferring by implication, estoppel or otherwise any license or rights under any patents of UNIVERSITY other than Patent Rights, or any technology other than Technology, regardless of whether those patents are dominant or subordinate to Patent Rights;
- (v) an obligation to furnish any know-how not provided in Patent Rights and Technology; or
- (vi) an obligation to update or provide assistance regarding Technology.

**8.2 Indemnification.**

(a) LICENSEE will, and will require Sublicensees to, indemnify, hold harmless, and defend UNIVERSITY and its officers, employees, and agents; the sponsors of the research that led to the Invention and Technology; and the creators of Technology and inventors of patents or patent applications under Patent Rights, and their employers (collectively, the "Indemnitees"); against any and all claims, suits, losses, damages, costs, fees, and expenses brought by third parties against such Indemnitees to the extent resulting from, or arising out of Licensee's exercise of this license or any Sublicense. This indemnification will include, but will not be limited to, any product liability. Notwithstanding the foregoing, Licensee shall not be required to indemnify any Indemnitee to the extent such claim is due to the gross negligence or willful misconduct of an Indemnitee in with the exercise of this Agreement.

(b) UNIVERSITY shall notify LICENSEE in writing of any claim or suit brought against UNIVERSITY in respect of which UNIVERSITY intends to invoke the provisions of this Article. LICENSEE shall keep UNIVERSITY informed on a current basis of its defense of any claims under this Article. LICENSEE will not settle any claim against UNIVERSITY without UNIVERSITY's written consent, where (a) such settlement would include any admission of liability or wrongdoing on the part of UNIVERSITY or other indemnified party, (b) such settlement would impose any restriction on UNIVERSITY/indemnified party's conduct of any of its activities, or (c) such settlement would not include an unconditional release of UNIVERSITY/indemnified party from all liability for claims that are the subject matter of the settled claim.

### 8.3 Insurance.

(a) LICENSEE, at its sole cost and expense, shall insure its activities in connection with the work under this Agreement and obtain, keep in force and maintain insurance as follows:

(i) commercial general liability insurance (contractual liability included) with limits of at least: [\*\*\*];

If the above insurance is written on a claims-made form, it shall continue for [\*\*\*] following termination or expiration of this Agreement. The insurance shall have a retroactive date of placement prior to or coinciding with the Effective Date;

(ii) Worker's Compensation as legally required in the jurisdiction in which the LICENSEE is doing business; and

(iii) the coverage and limits referred to above shall not in any way limit the liability of LICENSEE.

(b) LICENSEE shall furnish UNIVERSITY with certificates of insurance showing compliance with all requirements. Such certificates shall: (i) provide for [\*\*\*] advance written notice to UNIVERSITY of any modification; (ii) indicate that UNIVERSITY has been endorsed as an additional insured party under the coverage referred to above; and (iii) include a provision that the coverage shall be primary and shall not participate with nor shall be excess over any valid and collectable insurance or program of self-insurance carried or maintained by UNIVERSITY.

## ARTICLE 9. USE OF NAMES AND TRADEMARKS

Nothing contained in this Agreement confers any right to use in advertising, publicity, or other promotional activities any name, trade name, trademark, or other designation of UNIVERSITY by LICENSEE without prior written approval by UNIVERSITY (including contraction, abbreviation or simulation of any of the foregoing). LICENSEE hereby grants permission for UNIVERSITY (including UC SAN DIEGO) to include LICENSEE's name and a link to LICENSEE's website in UNIVERSITY's and UC SAN DIEGO's annual reports and on UNIVERSITY's (including UC SAN DIEGO's) websites that showcase innovation and commercialization stories.

## ARTICLE 10. MISCELLANEOUS PROVISIONS

**10.1 Correspondence.** Any notice or payment required to be given to either party under this Agreement shall be deemed to have been properly given and effective: (a) on the date of delivery if delivered in person, (b) five (5) days after mailing if mailed by first-class or certified mail, postage paid, to the respective addresses given below, or to such other address as is designated by written notice given to the other party, or (c) upon confirmation by recognized national overnight courier, confirmed facsimile transmission, or confirmed electronic mail, to the following addresses or facsimile numbers of the parties.

If sent to LICENSEE:

Invoices only:

[\*\*\*]

If to LICENSEE (other notices):

[\*\*\*]

If sent to UNIVERSITY by mail:

[\*\*\*]

If sent to UNIVERSITY by overnight delivery:

[\*\*\*]

### 10.2 Secrecy.

(a) "Confidential Information" shall mean information relating to the Invention and disclosed by UNIVERSITY to LICENSEE during the Term, which if disclosed in writing shall be marked "Confidential", or if first disclosed otherwise, shall within [\*\*\*] of such disclosure be reduced to writing by UNIVERSITY and sent to LICENSEE. The Technology will be deemed to be Confidential Information.

(b) LICENSEE shall:

- (i) use the Confidential Information for the sole purpose of performing under the terms of this Agreement;
- (ii) safeguard Confidential Information against disclosure to others with the same degree of care as it exercises with its own data of a similar nature;
- (iii) not disclose Confidential Information to others (except to its employees, agents or consultants who are bound to LICENSEE by a like obligation of confidentiality) without

the express written permission of UNIVERSITY, except that LICENSEE shall not be prevented from using or disclosing any of the Confidential Information that:

- (A) LICENSEE can demonstrate by written records was previously known to it;
- (B) is now, or becomes in the future, public knowledge other than through acts or omissions of LICENSEE;
- (C) is lawfully obtained by LICENSEE from sources independent of UNIVERSITY;
- or
- (D) is required to be disclosed by law or a court of competent jurisdiction.

(c) The secrecy obligations of LICENSEE with respect to Confidential Information shall continue for a period ending [\*\*\*] from the termination date of this Agreement.

(d) Notwithstanding the foregoing, UNIVERSITY may disclose to the Inventors, senior administrators employed by UNIVERSITY, and individual Regents the terms and conditions of this Agreement upon their request. If such disclosure is made, UNIVERSITY shall request the individuals not disclose such terms and conditions to others. UNIVERSITY may acknowledge the existence of this Agreement and the extent of the grant in Article 2 to third parties, but UNIVERSITY shall not disclose the negotiable financial terms of this Agreement to third parties, except where UNIVERSITY is required by law to do so, such as under the California Public Records Act.

**10.3 Assignability.** This Agreement may be assigned by UNIVERSITY, but is personal to LICENSEE and assignable by LICENSEE only with the written consent of UNIVERSITY; provided however that LICENSEE may assign this Agreement without the written consent of University to (i) an Affiliate of LICENSEE or (ii) an acquirer of all or substantially all of the business (whether structured as a merger, asset sale, exclusive license or otherwise) to which this Agreement relates as long as (a) such acquirer agrees to assume all the terms and conditions of this Agreement and (b) solely with respect to the first assignment of this Agreement, within [\*\*\*] of such assignment, LICENSEE or such acquirer shall pay UNIVERSITY an assignment fee equal to the greater of (x) [\*\*\*] or (y) [\*\*\*].

**10.4 No Waiver.** No waiver by either party of any breach or default of any agreement set forth in this Agreement shall be deemed a waiver as to any subsequent and/or similar breach or default.

**10.5 Failure to Perform.** In the event of a failure of performance due under this Agreement and if it becomes necessary for either party to undertake legal action against the other on account thereof, then the prevailing party shall be entitled to reasonable attorneys' fees in addition to costs and necessary disbursements.

**10.6 Governing Laws.** THIS AGREEMENT SHALL BE INTERPRETED AND CONSTRUED IN ACCORDANCE WITH THE LAWS OF THE STATE OF CALIFORNIA, but the scope and validity of any patent or patent application shall be governed by the applicable laws of the country of the patent or patent application.

**10.7 Force Majeure.** A party to this Agreement may be excused from any performance required herein if such performance is rendered impossible or unfeasible due to any catastrophe or other major event beyond its reasonable control, including, without limitation, war, riot, and insurrection; laws, proclamations, edicts, ordinances, or regulations; strikes, lockouts, or other serious labor disputes; and floods, fires, explosions, or other natural disasters. When such events have abated, the non-performing party's obligations herein shall resume.

**10.8 Headings.** The headings of the several sections are inserted for convenience of reference only and are not intended to be a part of or to affect the meaning or interpretation of this Agreement.

**10.9 Entire Agreement.** This Agreement, including Exhibits A and B, embodies the entire understanding of the parties and supersedes all previous communications, representations or understandings, either oral or written, between the parties relating to the subject matter hereof.

**10.10 Amendments.** No amendment or modification of this Agreement shall be valid or binding on the parties unless made in writing and signed on behalf of each party.

**10.11 Severability.** In the event that any of the provisions contained in this Agreement is held to be invalid, illegal, or unenforceable in any respect, such invalidity, illegality or unenforceability shall not affect any other provisions of this Agreement, and this Agreement shall be construed as if the invalid, illegal, or unenforceable provisions had never been contained in it.

**10.12 Counterparts.** This Agreement may be executed in one or more counterparts, each of which together will constitute one and the same Agreement. For purposes of executing this Agreement, a facsimile (including a PDF image delivered via email) copy of this Agreement, including the signature pages, will be deemed an original. The parties agree that neither party will have any rights to challenge the use or authenticity of a counterpart of this Agreement based solely on that its signature, or the signature of the other party, on such counterpart is not an original signature.

*(signature page follows)*

*IN WITNESS WHEREOF*, both UNIVERSITY and LICENSEE have executed this Agreement by their respective and duly authorized officers on the day and year written

**LEXEO THERAPEUTICS, INC.:**

By: /s/ R. Nolan Townsend  
R. Nolan Townsend  
Chief Executive Officer

Date: 2025-11-14

**THE REGENTS OF THE UNIVERSITY OF CALIFORNIA:**

By: /s/ Anthony Reza Razavi, Ph.D.  
Anthony Reza Razavi, Ph.D.  
Associate Director - Innovation and  
Commercialization

Date: 2025-11-14

Exhibit A – Patent Rights

[\*\*\*]

Exhibit B – Due Diligence Milestones

[\*\*\*]

Exhibit C – Technology

\*\*\*

[\*\*\*] = CERTAIN CONFIDENTIAL INFORMATION CONTAINED IN THIS DOCUMENT, MARKED BY BRACKETS, HAS BEEN OMITTED BECAUSE IT IS BOTH (I) NOT MATERIAL AND (II) IS THE TYPE THAT THE REGISTRANT TREATS AS PRIVATE OR CONFIDENTIAL.



Center for Technology Licensing  
1155 York Avenue  
New York, NY 10065  
P: 646.962.7045  
[innovation.weill.cornell.edu](http://innovation.weill.cornell.edu)

December 17, 2025

[\*\*\*]

**RE: SECOND AMENDMENT**

to the THIRD LICENSE AGREEMENT by and between Lexeo Therapeutics, Inc. (hereinafter “Licensee”) and Cornell University (“Cornell”) (each a “Party”) effective April 21, 2024, and amended a first time effective May 24, 2024 (the “Third License Agreement”) (CTL Contract No. [\*\*\*])

Effective the date of the last signature hereto (“Second Amendment Date”), the Parties agree to hereby modify the Third License Agreement as follows:

1) Paragraph 3.1 is hereby deleted and replaced with the following:

“3.1 (a) (i) a license issue fee of [\*\*\*], and (ii) an initial data transfer fee of [\*\*\*], each paid in cash within [\*\*\*] of the Effective Date; and (iii) for the data [\*\*\*] as described in Appendix B, an additional one-time data transfer fee of [\*\*\*], due within [\*\*\*] of the Second Amendment Date, and (iv) an annual data transfer fee of [\*\*\*] paid [\*\*\*] of the Effective Date until [\*\*\*].”

2) The lead paragraph in **Appendix B: Data and Reports** is hereby deleted and replaced with the following:

[\*\*\*]

3) These changes do not otherwise change the terms and conditions of the Third License Agreement.

4) This Second Amendment may be executed by electronic signatures or by facsimile and in two (2) or more counterparts, each of which shall be deemed an original and all of which together shall constitute but one and the same instrument.

*IN WITNESS WHEREOF*, both Cornell and Licensee have executed this Second Amendment by their respective and duly authorized officers on the day and year written.

**CORNELL UNIVERSITY**

**LEXEO THERAPEUTICS, INC.**

By: /s/ Lisa Placanica  
[Signature of Authorized Officer]  
Name: Lisa Placanica  
Title: Senior Managing Director  
  
Date: 12/19/2025

By: /s/ R. Nolan Townsend  
[Signature of Authorized Officer]  
Name: R. Nolan Townsend  
Title: Chief Executive Officer  
  
Date: 12/22/2025

[\*\*\*] = CERTAIN CONFIDENTIAL INFORMATION CONTAINED IN THIS DOCUMENT, MARKED BY BRACKETS, HAS BEEN OMITTED BECAUSE IT IS BOTH (I) NOT MATERIAL AND (II) IS THE TYPE THAT THE REGISTRANT TREATS AS PRIVATE OR CONFIDENTIAL.

**TRANSITION AND CONSULTING AGREEMENT**

December 1, 2025

Jenny R. Robertson

[\*\*\*]

[\*\*\*]

[\*\*\*]

[\*\*\*]

Dear Jenny:

This letter sets forth the agreement (the “**Agreement**”) between you and Lexeo Therapeutics, Inc. (the “**Company**”) in connection with your separation of employment from the Company, and your transition into a temporary consulting role.

**1. Separation Date.** Your last day of employment with the Company will be December 31, 2025 (the “**Separation Date**”). Regardless of whether you sign this Agreement, you will be paid at your current rate of pay, through the Separation Date, less applicable federal, state and local withholding taxes and authorized deductions (the “**Accrued Compensation**”). As of the Separation Date, you will cease holding any position or title with the Company as an employee (although you may be authorized to speak, and make representations, on behalf of the Company in your capacity as a consultant as set forth in Paragraph 2 below), and you will no longer be eligible or entitled to receive any compensation other than as expressly set forth herein. The Company acknowledges that there is a Company holiday starting December 22 through December 31, 2025, and that you will be paid through December 31, 2025.

**2. Transition Consulting Services.**

(a) Beginning on January 1, 2026 and continuing through March 31, 2026, or on an earlier date if terminated by either party as provided for below (the “**Consulting Period**” and the last day of the Consulting Period, the “**Service End Date**”), you will provide advisory and ad hoc legal counseling services (the “**Services**”) for the Company as a consultant.

(b) As compensation for the Services and as consideration for the promises and obligations in this Agreement, the Company will provide the following (collectively, the “**Consulting Compensation**”):

i. A lump sum payment in the amount of \$229,800, to be paid on or before January 5, 2026 (representing six months’ salary);

ii. A lump sum payment of your annual bonus for 2025 (the “**2025 Bonus**”) as determined by Compensation Committee-approved annual company performance, to be paid in accordance with the annual compensation review process for executives; and

iii. If you are currently participating in the Company’s group health insurance plans, subject to your eligibility for and election of COBRA continuation coverage, the Company shall pay on your behalf the full amount of the premiums to continue the health insurance you had prior to the Separation Date for you (and your eligible dependents, if applicable) through June 30, 2026.

(c) Your relationship with Company during the Consulting Period will be that of an independent contractor, and nothing in this Agreement is intended to, or should be construed to, create a partnership, agency, joint venture or employment relationship. Except as provided herein, you will not be entitled to any of the benefits that the Company may make available to its employees, including, but not limited to, group health, life insurance, profit-sharing or retirement benefits, paid vacation, holidays or sick leave. You will not be authorized to make any representation, contract or commitment on behalf of the Company unless specifically requested or authorized in writing to do so by the Chief Executive Officer of the Company. You will be solely responsible for obtaining any business or similar licenses required by any federal, state or local authority. You acknowledge and agree that you will indemnify the Company for any tax liability associated with the Services provided during the Consulting Period under this Agreement.

(d) In accordance with the Company's objectives, you will determine the method, details and means of performing the services required by this Agreement. The Company shall have no right to, and shall not, control the manner or determine the method of performing your services. You shall provide the services for which you are engaged to the reasonable satisfaction of the Company. The Company may suggest to you, from time to time, methods or strategies the Company believes may assist you in the performance of your services under this Agreement. Consistent with your independent contractor status, however, you shall exercise your independent business discretion in determining whether or not to follow the Company's suggestions. You may perform the services required by this Agreement at any place or location and at such times as you shall determine. You agree to provide all tools and instrumentalities, if any, required to perform the services under this Agreement.

(e) During the Consulting Period, you will be in a "continuous service relationship as a Service Provider" with the Company under the Company's 2021 Equity Incentive Plan (the "**2021 Plan**") and in "Continuous Service" with the Company under the Company's 2023 Equity Incentive Plan (the "**2023 Plan**") and, together with the 2021 Plan, the "**Plans**"), such that any equity awards (the "**Equity Awards**") issued pursuant to the Plans will continue to vest during the Consulting Period. The period in which to exercise any options issued pursuant to the Plans shall be extended to twelve (12) months following the Service End Date. So long as you do not terminate the Consulting Period prior to March 31, 2026, an additional portion of the Equity Awards that would have vested as of September 1, 2026 had you remained in a "continuous service relationship as a Service Provider" or in "Continuous Service" through such date shall vest as of the Service End Date.

(f) You and the Company agree that the Company's offer for you to continue providing services as a consultant following the Separation Date, constitutes consideration for the promises and obligations set forth in this Agreement, including but not limited to the release in paragraph 14 below.

(g) If within the ninety (90) day period before the Service End Date the Company enters into a definitive agreement which results in a Change in Control (as that term is defined in the 2023 Plan) and such Change in Control actually occurs, whether the Change in Control occurs before or after the Service End Date, you will also be entitled to the severance benefits set forth in, and according to the terms of, Section 5.5 of the employment agreement between you and the Company dated September 28, 2023 (the "**Employment Agreement**"), namely: (a) a lump sum payment equal to \$459,600 (representing 12 months' of your base salary as of the Separation Date), (b) payment of COBRA premiums for continued health coverage for you (and your eligible dependents, if applicable) for 12 months following the Separation Date, (c) a lump sum payment equal to \$183,840 (representing a bonus for calendar year 2026), and (d) acceleration of any options, shares, or other equity grants outstanding and unvested as of the date of the Change in Control.

(h) During the Consulting Period the Company shall provide you with indemnification coverage under the Company's D&O liability insurance policies to the same extent as directors and executive officers, and you shall be considered an "agent and in "Corporate Status" for purposes of the indemnification agreement between the Company and you dated November 7, 2023 (the "**Indemnification Agreement**").

**3. Term and Termination; Supplemental Release.** This Agreement is effective as of the Effective Date (as defined herein) and will continue during the Consulting Period, unless earlier terminated as provided for herein.

(a)The Company may terminate this Agreement immediately upon written notice to you, in the event of your (i) material breach of this Agreement or the Employee Confidential Information and Inventions Assignment Agreement between you and the Company; (ii) misappropriation or attempted misappropriation of any Company funds or material Company property, or any appropriation or attempted appropriation by you of a business or corporate opportunity of the Company; (iii) engagement in misconduct that adversely affects the business or reputation of the Company; (iv) commission of a willful or intentional act which results in material injury or is reasonably likely to result in material injury to the business or reputation of the Company; (v) use of alcohol or drugs in a manner which affects your job performance; or (vi) arrest, conviction, or plea of guilty or “no contest” to any felony or misdemeanor involving dishonestly, theft, fraud, misrepresentation, or moral turpitude. If this Agreement is terminated by the Company pursuant to this Paragraph 3(a), you will not be required to repay any of the Consulting Compensation to the Company.

(b)You may terminate this Agreement upon seven (7) days written notice to the Company. If this Agreement is terminated by you, you are only entitled to retain a portion of the Consulting Compensation representing the actual portion of the Consulting Period during which you provided Services to the Company and must, within thirty (30) days of the date you provide written notice to the Company, repay the remaining pro-rated portion of the Consulting Compensation to the Company, provided, however, that for purposes of this Paragraph 3(b) Consulting Compensation shall not include the 2025 Bonus.

(c)You agree that on or within a reasonable time after the end of the Service End Date, you will execute and return to the Company a Supplemental Release (in the form attached as **Exhibit A** hereto). As consideration for the Supplemental Release, you will receive \$1,000 as a lump sum payment within thirty (30) days of your execution of the Supplemental Release.

**4. Compensation or Benefits.** You acknowledge that, except as expressly provided in this Agreement, you have not earned and will not receive from the Company any additional compensation (including base salary, bonus, incentive compensation, or equity), severance, or benefits before or after the Separation Date, with the exception of any vested right you may have under the express terms of a written ERISA-qualified benefit plan (e.g., 401(k) account) or any vested equity awards.

**5. Return of Company Property.** Except to the extent any retention of Company property is authorized in writing by the Chief Executive Officer of the Company (in connection with your provision of the Services), you agree that, by the Service End Date, you will return to the Company all Company documents (and all copies thereof) and other Company property in your possession or control, including, but not limited to, Company files, notes, drawings, records, plans, forecasts, reports, studies, analyses, proposals, agreements, drafts, financial and operational information, research and development information, sales and marketing information, customer lists, prospect information, pipeline reports, sales reports, personnel information, specifications, code, software, databases, computer-recorded information, tangible property and equipment (including, but not limited to, computing and electronic devices, mobile telephones, servers), credit cards, entry cards, identification badges and keys; and any materials of any kind which contain or embody any proprietary or confidential information of the Company (and all reproductions or embodiments thereof in whole or in part). You agree that you will make a diligent search to locate any such documents, property and information by the close of business on the Separation Date or as soon as possible thereafter. If you have used any personally-owned computer or other electronic device, server, or e-mail system to receive, store, review, prepare or transmit any Company confidential or proprietary data, materials or information, by the Service End Date, you shall provide the Company with a computer-useable copy of such information and then permanently delete and expunge such Company confidential or proprietary information from those systems; and you agree to provide the Company access to your system as requested to verify that the necessary copying and/or deletion is completed.

**6. Proprietary Information Obligations.** You acknowledge and reaffirm your continuing obligations under your Employee Confidential Information and Inventions Assignment Agreement, a copy of which is attached hereto as **Exhibit B**.

**7. Confidentiality.** The provisions of this Agreement will be held in strictest confidence by you and will not be publicized or disclosed by you in any manner whatsoever; provided, however, that: (a) you may disclose this Agreement in confidence to your immediate family and to your attorneys, accountants, tax preparers and financial advisors; and (b) you may disclose this Agreement insofar as such disclosure may be necessary to enforce its terms or as otherwise required by law. In particular, and without limitation, you agree not to disclose the terms of this Agreement to any current or former Company employee or independent contractor, except to the extent permitted by Paragraph 9.

**8. Non-disparagement.** You agree not to disparage the Company, its officers, directors, employees, shareholders, parents, subsidiaries, affiliates, and agents, in any manner likely to be harmful to its or their business, business reputation, or personal reputation; provided that you may respond accurately and fully to any request for information if required by legal process or in connection with a government investigation. The Company agrees that it will direct its senior executives not to disparage you in any manner that may be harmful to your business or personal reputation. Nothing in this provision or this Agreement is intended to prohibit or restrain you in any manner from making disclosures protected under the whistleblower provisions of federal or state law or regulation or other applicable law or regulation. You agree to revise and update publicly available information, including professional and social networking websites such as LinkedIn and Facebook, within one (1) week of the Service End Date to remove any indication that you are employed by or providing services to the Company.

**9. Transition Communications.** The Company and you shall mutually agree on the substance and timing of any internal or external communications regarding your transition, including but not limited to announcements to employees, investors, partners, or the public. Neither party shall make or authorize any such communications without the prior written consent of the other party, which shall not be unreasonably withheld or delayed. The parties shall use good faith efforts to finalize such communications within five (5) business days following the Effective Date.

**10. References.** In response to any reference request from a prospective employer, the Company will only confirm your dates of employment and position. The Company and you will agree upon the content of a reference letter to be signed by the Chief Executive Officer for use with any prospective employers.

**11. No Voluntary Adverse Action.** You agree that you will not voluntarily (except in response to legal compulsion or as permitted under the Protected Rights section below) assist any person in bringing or pursuing any proposed or pending litigation, arbitration, administrative claim or other formal proceeding against the Company, its parent or subsidiary entities, affiliates, officers, directors, employees or agents.

**12. Cooperation.** During the one (1) year period following the Service End Date, you agree to cooperate fully with the Company in connection with its actual or contemplated defense, prosecution, or investigation of any claims or demands by or against third parties, or other matters arising from events, acts, or failures to act that occurred during the period of your employment by the Company. Such cooperation includes, without limitation, making yourself available to the Company upon reasonable notice, without subpoena, to provide complete, truthful and accurate information in witness interviews, depositions, and trial testimony. The Company will reimburse you for reasonable out-of-pocket expenses you incur in connection with any such cooperation (excluding foregone wages and attorneys' fees) and will make reasonable efforts to accommodate your scheduling needs and in no event will you be required to provide such cooperation for more than one (1) full business day in any one (1) week period.

**13. No Conflict of Interest.** During the term of this Agreement, you will not accept work, enter into a contract, or accept an obligation, inconsistent or incompatible with your obligations, or the scope of services rendered for the Company, under this Agreement. You warrant that, to the best of your knowledge, there is no other contract or duty on your part that conflicts with or is inconsistent with this Agreement. This paragraph does not prevent you from performing the same or similar services for clients other than the Company so long as such services do not directly or indirectly conflict with your obligations under this Agreement.

**14. No Admissions.** You understand and agree that the promises and payments in consideration of this Agreement shall not be construed to be an admission of any liability or obligation by the Company to you or any other person, and the Company makes no such admission.

**15. Release of Claims.** In exchange for the consideration provided to you under this Agreement to which you would not otherwise be entitled, you hereby generally and completely release the Company, and its affiliated, related, parent and subsidiary entities, and its and their current and former directors, officers, employees, shareholders, partners, agents, attorneys, predecessors, successors, insurers, affiliates, and assigns from any and all claims, liabilities, demands, causes of action, and obligations, both known and unknown, arising from or in any way related to events, acts, conduct, or omissions occurring at any time prior to and including the date you sign this Agreement. This general release includes, but is not limited to: (a) all claims arising from or in any way related to your employment with the Company or the termination of that employment; (b) all claims related to your compensation or benefits from the Company, including salary, bonuses, commissions, vacation pay, expense reimbursements, severance pay, fringe benefits, stock, stock options, or any other ownership, equity, or profits interests in the Company; (c) all claims for breach of contract, wrongful termination, and breach of the implied covenant of good faith and fair dealing; (d) all tort claims, including claims for fraud, defamation, emotional distress, and discharge in violation of public policy; and (e) all federal, state, and local statutory claims, including claims for discrimination, harassment, retaliation, attorneys' fees, or other claims arising under the federal Civil Rights Act of 1964 (as amended), the federal Americans with Disabilities Act of 1990, the Age Discrimination in Employment Act ("ADEA"), the New York State Human Rights Law, the New York Labor Law, the New York Wage Theft Prevention Act, Section 125 of the New York Workers' Compensation Law, the New York City Human Rights Law, the New York City Administrative Code, the Massachusetts Fair Employment Practices Act, the Massachusetts Equal Pay Act, the Massachusetts Civil Rights Act, the Massachusetts Labor and Industries Act, the Massachusetts Right to Privacy law, and the Massachusetts Wage Act, all as amended, and any other federal, state or local statutes, regulations, ordinances prohibiting discrimination or regulating employment or the payment of wages. Notwithstanding the foregoing, you are not releasing the Company hereby from: (i) any obligation to indemnify you pursuant to the Articles and Bylaws of the Company, the Indemnification Agreement, applicable law, or applicable directors and officers liability insurance; (ii) any claims that cannot be waived by law; or (iii) any claims for breach of this Agreement.

**16. ADEA Release.** You acknowledge that you are knowingly and voluntarily waiving and releasing any rights you have under the ADEA, and that the consideration given for the waiver and releases you have given in this Agreement is in addition to anything of value to which you were already entitled. You further acknowledge that you have been advised, as required by the ADEA, that: (a) your waiver and release does not apply to any rights or claims arising after the date you sign this Agreement; (b) you should consult with an attorney prior to signing this Agreement (although you may choose voluntarily not to do so); (c) you have twenty-one (21) days to consider this Agreement (although you may choose voluntarily to sign it sooner); (d) you have seven (7) days following the date you sign this Agreement to revoke this Agreement (in a written revocation sent to the Company, specifically to Jennifer Speer via email at [jcspeer@lexeotx.com](mailto:jcspeer@lexeotx.com)); and (e) this Agreement will not be effective until the date upon which the revocation period has expired, which will be the eighth day after you sign this Agreement provided that you do not revoke it (the "**Effective Date**").

**17. Protected Rights.** You understand that nothing in this Agreement limits your ability to file a charge or complaint with the Equal Employment Opportunity Commission, the Department of Labor, the National Labor Relations Board, the Occupational Safety and Health Administration, the New York State Department of Labor, the Securities and Exchange Commission or any other federal, state or local governmental agency or commission ("**Government Agencies**"). You further understand this Agreement does not limit your ability to communicate with any Government Agencies or otherwise participate in any investigation or proceeding that may be conducted by any Government Agency, including providing documents or other information, without notice to the Company. While this Agreement does not limit your right to receive an award for information provided to the Securities and Exchange Commission, you understand and agree that, to maximum extent permitted by law, you are otherwise waiving any and all rights you may have to individual relief based on any claims that you have released and any rights you have waived by signing this Agreement.

**18. Breach; Attorneys' Fees.** You acknowledge and agree that any material breach of this Agreement, unless such breach constitutes a legal action by you challenging or seeking a determination in good faith of the validity of the waiver herein under the ADEA, or its exhibits (if any) shall entitle the Company immediately to recover and/or cease providing the consideration provided to you under this Agreement and to obtain damages, except as provided by law, provided, however, that the Company shall not recover Fifty Dollars (\$50.00) of the consideration already paid pursuant to this Agreement and such amount shall serve as full and complete consideration for the promises and obligations assumed by you under this Agreement and its exhibits. In addition, except with regard to a legal action challenging or seeking a determination in good faith of the validity of the waiver herein under the ADEA, in the event that either party brings an action to enforce or effect its rights under this Agreement, the prevailing party shall be entitled to recover its costs and expenses, including the costs of mediation, arbitration, litigation, court fees, and reasonable attorneys' fees incurred in connection with such an action.

**19. Representations.** You hereby represent that you have: been paid all compensation owed and for all hours worked; received all leave and leave benefits and protections for which you are eligible pursuant to the Family and Medical Leave Act, the New York Paid Family Leave Law, the Massachusetts Parental Leave Law, or otherwise; and have not suffered any on-the-job injury for which you have not already filed a workers' compensation claim.

**20. Dispute Resolution.** You and the Company agree that any and all disputes, claims, or controversies of any nature whatsoever arising from, or relating to, this Agreement or its interpretation, enforcement, breach, performance or execution, your employment or the termination of such employment (including, but not limited to, any statutory claims) (collectively, "**Claims**", each a "**Claim**"), shall be resolved, pursuant to the Federal Arbitration Act, 9 U.S.C. §1-16, and to the fullest extent permitted by law, by final, binding and confidential arbitration in New York, New York (or another mutually acceptable location) conducted before a single neutral arbitrator by JAMS, Inc. ("**JAMS**") or its successor, under the then applicable JAMS Arbitration Rules and Procedures for Employment Disputes (available at <http://www.jamsadr.com/rules-employment-arbitration/>). **By agreeing to this arbitration procedure, both you and the Company waive the right to have any Claim resolved through a trial by jury or judge or an administrative proceeding.** You will have the right to be represented by legal counsel at any arbitration proceeding, at your own expense. This paragraph shall not apply to any action or claim that cannot be subject to mandatory arbitration as a matter of law. The arbitrator shall have sole authority for determining if a Claim is subject to arbitration, and any other procedural questions related to the dispute and bearing on the final disposition. In addition, the arbitrator shall: (a) have the authority to compel adequate discovery for the resolution of the dispute and to award such relief as would otherwise be available under applicable law in a court proceeding; and (b) issue a written statement signed by the arbitrator regarding the disposition of each claim and the relief, if any, awarded as to each claim, the reasons for the award, and the arbitrator's essential findings and conclusions on which the award is based. You and the Company shall equally share all JAMS' arbitration fees. To the extent JAMS does not collect or you otherwise do not pay to JAMS an equal share of all JAMS' arbitration fees for any reason, and the Company pays JAMS your share, you acknowledge and agree that the Company shall be entitled to recover from you half of the JAMS arbitration fees invoiced to the parties (less any amounts you paid to JAMS) in a federal or state court of competent jurisdiction. Nothing in this letter agreement is intended to prevent either you or the Company from obtaining injunctive relief in court to prevent irreparable harm pending the conclusion of any such arbitration. Any awards or orders in such arbitrations may be entered and enforced as judgments in the federal and state courts of any competent jurisdiction.

**21. Complete Agreement.** This Agreement, including Exhibits A and B, constitutes the complete, final and exclusive embodiment of the entire agreement between you and the Company with regard to its subject matter. You agree and acknowledge that this Agreement is entered into without reliance on any promise or representation, written or oral, other than those expressly contained herein, and it supersedes any other such promises, warranties or representations.

**22. Miscellaneous.**

(a) This Agreement may not be modified or amended except in a writing signed by both you and a duly authorized officer of the Company.

(b) This Agreement will bind the heirs, personal representatives, successors and assigns of both you and the Company, and inure to the benefit of both you and the Company, their heirs, successors and assigns.

(c) If any provision of this Agreement is determined to be invalid or unenforceable, in whole or in part, this determination will not affect the validity or enforceability of any other provision of this Agreement and the provision in question will be modified so as to be rendered enforceable to the fullest extent permitted by law, consistent with the intent of the parties. Moreover, if one or more of the provisions contained in this Agreement is held to be excessively broad as to duration, scope, or subject, such provisions shall be construed by limiting or reducing them so as to be enforceable to the maximum extent compatible with applicable law.

(d) This Agreement will be deemed to have been entered into and will be construed and enforced in accordance with the laws of the State of New York without regard to conflict of laws principles.

(e) Any ambiguity in this Agreement shall not be construed against either party as the drafter. Any waiver of any condition or breach of this Agreement shall be in writing and shall not be deemed to be a waiver of any other condition or breach, nor shall the failure of or delay by either of the parties in exercising any right, power, or privilege hereunder operate as a waiver thereof to preclude any other or further exercise thereof or the exercise of any other such right, power, or privilege.

(f) This Agreement may be executed in counterparts and electronic or facsimile signatures will suffice as original signatures.

If this Agreement is acceptable to you, please sign below and return the original to me. You have twenty-one calendar days to decide whether to accept this Agreement, and the Company's offer contained herein will automatically expire if you do not sign and return it within that timeframe.

Sincerely,

By: /s/ R. Nolan Townsend  
**R. NOLAN TOWNSEND**

**I HAVE READ, UNDERSTAND AND AGREE FULLY TO THE FOREGOING AGREEMENT:**

/s/ Jenny R. Robertson

**JENNY R. ROBERTSON**

12/2/2025

Date

EXHIBIT A

SUPPLEMENTAL RELEASE

[\*\*\*]

**EXHIBIT B  
CONFIDENTIAL INFORMATION  
AND INVENTIONS ASSIGNMENT AGREEMENT**

[\*\*\*]



KPMG LLP  
345 Park Avenue  
New York, NY 10154-0102

**Consent of Independent Registered Public Accounting Firm**

We consent to the incorporation by reference in the registration statements (No. 333-275374, 333-277816, 333-286040, and 333-291268) on Form S-8 and (No. 333-283781, 333-288025 and 333-291269) on Form S-3 of our report dated March 30, 2026, with respect to the financial statements of Lexeo Therapeutics, Inc.

/s/ KPMG LLP

New York, New York  
March 30, 2026

KPMG LLP, a Delaware limited liability partnership and a member firm of the KPMG global organization of independent member firms affiliated with KPMG International Limited, a private English company limited by guarantee.

**CERTIFICATION OF THE PRINCIPAL EXECUTIVE OFFICER  
PURSUANT TO  
EXCHANGE ACT RULES 13a-14(a) AND 15d-14(a),  
AS ADOPTED PURSUANT TO  
SECTION 302 OF THE SARBANES-OXLEY ACT OF 2002**

I, R. Nolan Townsend certify that:

1. I have reviewed this Annual Report on Form 10-K of Lexeo Therapeutics, Inc.;
2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;
3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;
4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:
  - (a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;
  - (b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;
  - (c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and
  - (d) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and
5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):
  - (a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and
  - (b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Dated: March 30, 2026

/s/ R. Nolan Townsend

By: R. Nolan Townsend

Chief Executive Officer (Principal Executive Officer)

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**CERTIFICATION OF THE PRINCIPAL FINANCIAL OFFICER  
PURSUANT TO  
EXCHANGE ACT RULES 13a-14(a) AND 15d-14(a),  
AS ADOPTED PURSUANT TO  
SECTION 302 OF THE SARBANES-OXLEY ACT OF 2002**

I, Louis Tamayo certify that:

1. I have reviewed this Annual Report on Form 10-K of Lexeo Therapeutics, Inc.;
2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;
3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;
4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:
  - (a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;
  - (b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;
  - (c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and
  - (d) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and
5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):
  - (a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and
  - (b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Dated: March 30, 2026

/s/ Louis Tamayo

By: Louis Tamayo

Chief Financial Officer (Principal Financial and Accounting Officer)

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**CERTIFICATION OF THE CHIEF EXECUTIVE OFFICER  
PURSUANT TO  
18 U.S.C. SECTION 1350,  
AS ADOPTED PURSUANT TO  
SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002**

In connection with the Annual Report of Lexeo Therapeutics, Inc. (the "Company") on Form 10-K for the period ended December 31, 2025 as filed with the Securities and Exchange Commission on the date hereof (the "Report"), I certify, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that:

1. The Report fully complies with the requirements of Section 13(a) or Section 15(d) of the Exchange Act; and
2. The information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

Dated: March 30, 2026

/s/ R. Nolan Townsend

R. Nolan Townsend

Chief Executive Officer (Principal Executive Officer)

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**CERTIFICATION OF THE CHIEF FINANCIAL OFFICER  
PURSUANT TO  
18 U.S.C. SECTION 1350,  
AS ADOPTED PURSUANT TO  
SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002**

In connection with the Annual Report of Lexeo Therapeutics, Inc. (the "Company") on Form 10-K for the period ended December 31, 2025 as filed with the Securities and Exchange Commission on the date hereof (the "Report"), I certify, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that:

1. The Report fully complies with the requirements of Section 13(a) or Section 15(d) of the Exchange Act; and
2. The information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

Dated: March 30, 2026

/s/ Louis Tamayo

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Louis Tamayo

Chief Financial Officer (Principal Financial and Accounting Officer)

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