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 001-39208

Beam Therapeutics Inc.

(Exact name of Registrant as specified in its Charter)

Delaware	**81-5238376**
(*State or other jurisdiction of incorporation or organization*)	(*I.R.S. Employer Identification No.*)
238 Main Street **Cambridge, MA**	**02142**
(*Address of principal executive offices*)	(*Zip Code*)

Registrant's telephone number, including area code: 857-327-8775

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 $0.01 per share	**BEAM**	**Nasdaq Global Select 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	☒	Accelerated filer	☐
Non-accelerated filer	☐	Smaller reporting company	☐
		Emerging growth company	☐

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

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

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

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

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

The aggregate market value of the voting and non-voting common stock held by non-affiliates of the registrant was $1.70 billion, based on the closing price of the registrant's common stock on Nasdaq on June 30, 2025, the last business day of the registrant's most recently completed second quarter.

The number of shares of registrant's common stock outstanding as of February 17, 2026 was 101,856,245.

DOCUMENTS INCORPORATED BY REFERENCE

Registrant incorporates by reference into Part III (Items 10, 11, 12, 13 and 14) of this Annual Report on Form 10-K portions of the Registrant's definitive Proxy Statement for the 2026 Annual Meeting of Stockholders to be filed with the Securities and Exchange Commission pursuant to Regulation 14A.

Table of Contents

CAUTIONARY NOTE REGARDING FORWARD-LOOKING STATEMENTS

This Annual Report on Form 10-K contains forward-looking statements within the meaning of Section 27A of the Securities Act of 1933, as amended, or the Securities Act, and Section 21E of the Securities Exchange Act of 1934, as amended, or the Exchange Act. Such forward-looking statements reflect, among other things, our current expectations and anticipated results of operations, all of which are subject to known and unknown risks, uncertainties and other factors that may cause our actual results, performance or achievements, market trends, or industry results to differ materially from those expressed or implied by such forward-looking statements. Therefore, any statements contained herein that are not statements of historical fact may be forward-looking statements and should be evaluated as such. Without limiting the foregoing, the words "anticipate," "expect," "suggest," "plan," "believe," "intend," "project," "forecast," "estimates," "targets," "projections," "should," "could," "would," "may," "might," "will," and the negative thereof and similar words and expressions are intended to identify forward-looking statements. These forward-looking statements are subject to a number of important risks, uncertainties and assumptions, including those described in "Risk Factors Summary" and in "Risk Factors" in Part I, Item 1A of this report. Unless legally required, we assume no obligation to update any such forward-looking information to reflect actual results or changes in the factors affecting such forward-looking information. These forward-looking statements reflect, among other things:

- our current expectations and anticipated results of operations;
- our expectations regarding the timing, progress and results of our clinical trials, including our Phase 1/2 clinical trial designed to assess the safety and efficacy of risto-cel for the treatment of sickle cell disease, our Phase 1/2 clinical trial designed to assess the safety and efficacy of BEAM-302 for the treatment of alpha-1 antitrypsin deficiency, our Phase 1/2 clinical trial designed to assess the safety and efficacy of BEAM-301 for the treatment of glycogen storage disease type 1a, and our Phase 1 healthy volunteer clinical trial of BEAM-103;
- our ability to file a biologics license application within a certain time period, and to demonstrate to applicable regulators that our product candidates are safe and effective and that their benefits outweigh known and potential risks for the intended patient population;
- our expectations regarding the initiation, timing, progress and results of our research and development programs and preclinical studies;
- our ability to develop and maintain a sustainable portfolio of product candidates;
- our ability to develop life-long, curative, precision genetic medicines for patients through base editing;
- our ability to create a hub for partnering with other companies;
- our plans for preclinical studies for product candidates in our pipeline;
- our ability to advance any product candidates that we may develop and successfully complete any clinical trials or preclinical studies, including the manufacture of any such product candidates;
- our ability to pursue a broad suite of clinically validated delivery modalities;
- our expectations regarding our ability to generate additional novel lipid nanoparticles that we believe could accelerate novel nonviral delivery of gene editing or other nucleic acid payloads to tissues beyond the liver and our ability to expand the reach of our programs;
- the scope of protection we are able to establish and maintain for intellectual property rights covering our product candidates and technology;
- developments related to our competitors and our industry;
- the expected timing, progress and success of our collaborations with third parties, including any future payments we may receive under our collaboration and license agreements, and our ability to identify and enter into future license agreements and collaborations;
- developments related to base editing technologies;
- our ability to successfully develop our delivery modalities and obtain and maintain approval for our product candidates;
- our ability to successfully maintain a commercial-scale current Good Manufacturing Practice, or cGMP, manufacturing facility;
- regulatory developments in the United States and foreign countries;
- our ability to attract and retain key scientific and management personnel;

- our expectations regarding the strategic and other potential benefits of our acquisition of any additional technologies, as well as the potential of contingent payments in connection with such acquisitions;
- our estimates regarding the period over which we believe that our existing cash, cash equivalents and marketable securities will be sufficient to fund our operating expenses and capital expenditure requirements; and
- the impact on our business of macro-economic conditions, as well as the prevailing level of macro-economic, business, and operational uncertainty, including as a result of geopolitical events, federal government shutdowns, the imposition of new or revised global trade tariffs or other global or regional events.

When we use the terms "Beam," the "Company," "we," "us" or "our" in this Annual Report on Form 10-K, we mean Beam Therapeutics Inc. and its subsidiaries on a consolidated basis, unless the context indicates otherwise.

TRADEMARKS

We use BEAM and other marks as trademarks in the United States and/or in other countries. This Annual Report on Form 10-K contains references to our trademarks and service marks and to those belonging to other entities. Solely for convenience, trademarks and trade names referred to in this report, including logos, artwork and other visual displays, may appear without the ® or TM symbols, but such references are not intended to indicate in any way that we will not assert, to the fullest extent under applicable law, our rights or the rights of the applicable licensor to these trademarks and trade names. We do not intend our use or display of other entities' trade names, trademarks or service marks to imply a relationship with, or endorsement or sponsorship of us by, any other entity.

MARKET AND INDUSTRY DATA

Unless otherwise indicated, information contained in this Annual Report on Form 10-K concerning our industry and the markets in which we operate, including our general expectations, market position and market opportunity, is based on our management's estimates and research, as well as industry and general publications and research, surveys and studies conducted by third parties. We believe that the information from these third-party publications, research, surveys and studies included in this Annual Report on Form 10-K is reliable. Management's estimates are derived from publicly available information, their knowledge of our industry and their assumptions based on such information and knowledge, which we believe to be reasonable. This data involves a number of assumptions and limitations which are necessarily subject to a high degree of uncertainty and risk due to a variety of factors, including those described in "Risk Factors Summary" and "Risk Factors" in Part I, Item 1A of this report. These and other factors could cause our future performance to differ materially from our assumptions and estimates.

RISK FACTORS SUMMARY

An investment in our common stock involves risks. You should consider carefully the following risks, which are discussed more fully in "Risk Factors" in Part I, Item 1A of this report, and all of the other information contained in this Annual Report on Form 10-K before investing in our common stock. These risks include, but are not limited to, the following:

- Base editing is a novel technology that is not yet clinically validated for human therapeutic use. The approaches we are taking to discover and develop novel therapeutics are unproven and may never lead to marketable products.
- We have incurred significant losses since inception. We expect to incur losses for the foreseeable future and may never achieve or maintain profitability.
- We will need substantial additional funding. If we are unable to raise capital when needed, we would be forced to delay, reduce, or eliminate our research and product development programs or future commercialization efforts.
- Our short operating history may make it difficult for you to evaluate the success of our business to date and to assess our future viability.
- We may not be successful in our efforts to identify and develop potential product candidates. If these efforts are unsuccessful, we may never become a commercial stage company or generate any revenues.
- We are early in our development efforts. Our product candidates are still in preclinical or clinical development and we have not, and may never, commercialize a product candidate. If we are unable to advance our product candidates to and through clinical development, obtain regulatory approval and ultimately commercialize our product candidates, or experience significant delays in doing so, our business will be materially harmed.
- If we experience delays or difficulties in the enrollment or treatment of patients in clinical trials, our receipt of necessary regulatory approvals could be delayed or prevented.
- If clinical trials of any product candidates we identify and develop fail to demonstrate safety, purity and potency to the satisfaction of regulatory authorities or do not otherwise produce positive results, we may incur additional costs or experience delays in completing, or ultimately be unable to complete, the development and commercialization of such product candidates.
- If any of the product candidates we may develop or the delivery modalities we rely on cause serious adverse events, undesirable side effects or unexpected characteristics, such events, side effects or characteristics could delay or prevent regulatory approval of the product candidates, limit the commercial potential, or result in significant negative consequences following any potential marketing approval.
- We face significant competition in an environment of rapid technological change, and there is a possibility that our competitors may achieve regulatory approval before us or develop therapies that are safer or more advanced or effective than ours, which may harm our financial condition and our ability to successfully market or commercialize any product candidates we may develop.
- We are conducting clinical trials at sites outside the United States. The U.S. Food and Drug Administration, or the FDA, may not accept data from trials conducted in such locations, and the conduct of trials outside the United States could subject us to additional delays and expense.
- We have not tested many of our proposed delivery modalities and product candidates in clinical trials and any favorable preclinical results are not predictive of results that may be observed in clinical trials.
- Adverse public perception of genetic medicines, and gene editing and base editing in particular, may negatively impact regulatory approval of, and/or demand for, our potential products.
- The gene editing field is relatively new and is evolving rapidly. We are focusing our research and development efforts on gene editing using base editing technology, but other gene editing technologies may be discovered that provide significant advantages over base editing, which could materially harm our business.
- Regulatory requirements governing genetic medicines, and in particular any novel genetic medicines we may develop, have changed frequently and may continue to change in the future.

- Genetic medicines are novel, and any product candidates we develop may be complex and difficult to manufacture. We could experience delays in satisfying regulatory authorities or production problems that result in delays in our development or commercialization programs, limit the supply of our product candidates we may develop, or otherwise harm our business.
- We contract with third parties for the manufacture and supply of materials for our research programs, preclinical studies and clinical trials and expect to continue to do so for at least a portion of our future research programs, preclinical studies, and clinical trials and for commercialization of any product candidates that we may develop. This reliance on third parties increases the risk that we will not have sufficient quantities of such materials, product candidates, or any medicines that we may develop and commercialize, or that such supply will not be available to us at an acceptable cost, which could delay, prevent, or impair our development or commercialization efforts.
- Because we are developing product candidates in the field of genetics medicines, a field that includes gene therapy and gene editing, in which there is little clinical experience, there is increased risk that the FDA, the European Medicines Agency or other regulatory authorities may not consider the endpoints of our clinical trials to provide clinically meaningful results and that these results may be difficult to analyze.
- If we are unable to obtain and maintain patent protection for any product candidates we develop and for our technology, or if the scope of the patent protection obtained is not sufficiently broad, or if we or our licensors are unable to successfully defend our or our licensors' patents against third-party challenges or enforce our or our licensors' patents against third parties, our competitors could develop and commercialize products and technology similar or identical to ours, and our ability to successfully commercialize any product candidates we may develop, and our technology may be adversely affected.
- Our rights to develop and commercialize technology and product candidates are subject, in part, to the terms and conditions of licenses granted to us by others.
- Third parties have asserted and may in the future assert that we, our employees, consultants, or advisors have wrongfully used or disclosed confidential information or misappropriated trade secrets.
- The intellectual property landscape around gene editing technology, including base editing and delivery technology, is highly dynamic, and third parties may initiate legal proceedings alleging that we are infringing, misappropriating, or otherwise violating their intellectual property rights, the outcome of which would be uncertain and may prevent, delay or otherwise interfere with our product discovery and development efforts.
- Our activities rely on information technology in our own systems and those of our business partners. These systems are subject to a wide and growing variety of cybersecurity risks that may adversely impact our business activities or our ability to engage in various transactions to support our business activities.
- Our clinical research activities depend on the use and disclosure of personal data related to individuals participating in our clinical trials. The rules addressing this data are changing across the world, and these rules may adversely impact our ability to identify individuals for clinical trials or conduct our trials.
- Our owned and in-licensed patents and other intellectual property may be subject to priority disputes or inventorship disputes or we may be subject to claims that we have infringed, misappropriated or otherwise violated the intellectual property of a third party and similar proceedings. If we or our licensors are unsuccessful in any of these proceedings, we may be required to obtain licenses from third parties, which may not be available on commercially reasonable terms or at all, or to cease the development, manufacture, and commercialization of one or more of the product candidates we may develop, which could have a material adverse impact on our business.
- The terms of our financing agreement with Sixth Street Lending Partners and our indebtedness could adversely affect our operations and limit our ability to plan for or respond to changes in our business. If we are unable to comply with restrictions in the financing agreement, the repayment of our existing indebtedness could be accelerated.
- Unstable market and economic conditions may have serious adverse consequences on our business, financial condition and stock price.

PART I

Item 1. Business.

Overview

We are a biotechnology company committed to establishing the leading, fully integrated platform for precision genetic medicines. Our vision is to provide life-long cures to patients suffering from serious diseases. To achieve this vision, we have assembled a platform that includes a suite of gene editing and delivery technologies as well as internal manufacturing capabilities.

Our suite of gene editing technologies is anchored by our proprietary base editing technology, which potentially enables a differentiated class of precision genetic medicines that target a single base in the genome without making a double-stranded break in the DNA. This approach uses a chemical reaction designed to create precise, predictable and efficient genetic outcomes at the targeted sequence. Our proprietary base editors have two principal components: (i) a clustered regularly interspaced short palindromic repeats, or CRISPR, protein, bound to a guide RNA, that leverages the established DNA-targeting ability of CRISPR, but is modified to not cause a double-stranded break, and (ii) a base editing enzyme, such as a deaminase, which carries out the desired chemical modification of the target DNA base. We believe this design contributes to a more precise and efficient edit compared to traditional gene editing methods, with the potential to dramatically increase the impact of gene editing. We are also pursuing a suite of delivery modalities, including both *ex vivo* and *in vivo* approaches, depending on tissue type. The elegance of the base editing approach, combined with a tissue specific delivery modality, provides the basis for a targeted, efficient, precise, and highly versatile gene editing system that is designed to be capable of gene correction, gene silencing, gene activation, gene modification, and/or multiplex editing of several genes simultaneously.

Our goal is to advance a broad, diversified portfolio of base editing programs against distinct, genetically validated editing targets, as well as an innovative, platform business model that will expand the reach of our programs to more patients. Overall, we are seeking to build the leading integrated platform for precision genetic medicine, which may have broad therapeutic applicability and the potential to transform the field of precision genetic medicines.

We are currently prioritizing the lead programs in our hematology and genetic disease portfolios, each of which have the potential to provide differentiated therapies for significant patient populations with high unmet medical need:

- Ristoglogene autogetemcel, or risto-cel (formerly known as BEAM-101), is a patient-specific, autologous hematopoietic stem cell, or HSC, investigational therapy designed to offer a potentially best-in-class profile, incorporating base edits that are intended to alleviate the effects of sickle cell disease by increasing fetal hemoglobin, which is expected to increase functional hemoglobin production and, in the case of sickle cell disease, inhibit hemoglobin S polymerization. In December 2025, we announced additional positive data from our Phase 1/2 clinical trial of risto-cel, which we refer to as the BEACON trial. We expect to submit a biologics license application, or BLA, for risto-cel as early as year-end 2026.
- BEAM-302 is a liver-targeting lipid nanoparticle, or LNP, formulation of base editing reagents designed to offer a one-time treatment to correct the E342K point mutation (PiZZ genotype), which is most commonly responsible for severe alpha-1 antitrypsin deficiency, or AATD. We have reached alignment with the U.S. Food and Drug Administration, or FDA, on a potential accelerated approval pathway for BEAM-302 based on alpha-1 antitrypsin biomarkers evaluated over 12 months. To support a future BLA submission, we anticipate enrolling approximately 50 additional patients to be treated with the selected optimal biological dose of BEAM-302 in an expansion of the ongoing Phase 1/2 clinical trial. We expect to report updated data from the trial and next steps for pivotal development by the end of the first quarter of 2026.

We are also continuing to advance our other hematology and genetic diseases programs:

- We continue to make significant investments in developing targeted LNPs for the *in vivo* delivery of gene editing payloads to HSCs. Based on recent advancements in this technology, we are now prioritizing *in vivo* delivery for our next wave approach to treating sickle cell disease. We have identified multiple targeted LNPs that have the potential for HSC delivery and are currently engaged in lead optimization. We are also continuing development of our proprietary ESCAPE platform, a technology to potentially enable non-genotoxic treatment strategies that can be delivered either *ex vivo* or *in vivo*, including as part of any future *in vivo* program for sickle cell disease. The ongoing Phase 1 healthy volunteer clinical trial of BEAM-103, an anti-CD117 monoclonal antibody that enables ESCAPE, is expected to complete dosing in the first half of 2026.
- BEAM-304 is a newly announced liver-targeting LNP formulation of base editing reagents designed to correct disease-causing mutations responsible for phenylketonuria, or PKU. Initial clinical development will focus on base editors addressing the two most prevalent variants found in nearly half of patients with PKU, with ongoing research efforts to address additional pathogenic mutations. In 2026, we plan to submit a regulatory application to initiate a Phase 1/2 dose-escalation clinical trial of BEAM-304 in PKU patients with the R408W mutation.

- BEAM-301 is a liver-targeting LNP formulation of base editing reagents designed to correct the R83C mutation, the most prevalent disease-causing mutation for, and the mutation which results in the most severe form of, glycogen storage disease type 1a, or GSDIa. We are evaluating BEAM-301 in a Phase 1/2 clinical trial, and expect to report initial clinical data in 2026.

Limitations of nuclease editing

Gene editing works by disrupting, inserting, or modifying genes in the natural context of the genome. Most established gene editing methods rely on a class of enzymes, called nucleases, to make a double-stranded break in the DNA at a targeted location. Nuclease editors have several significant limitations.

First, there is a lack of predictability in genetic outcomes when altering gene sequences with nucleases. Nuclease editors can be effective if the desired outcome is to knock out or switch off the whole gene, but do not allow for precise control of the specific genetic outcome at the target site and its effects may vary from individual to individual.

Second, there are potential toxicities associated with double-stranded breaks, such as activating the cell death response and/or genomic instability. In addition, if the double-stranded break occurs in the wrong place, the break can also lead to unwanted gene disruptions. Multiple edits using double-stranded breaks can compound this issue and lead to large-scale genomic translocations and rearrangements, potentially limiting the applicability of nuclease-based approaches in multiplex editing.

Third, while gene disruption with nucleases is efficient, making specific sequence changes to correct or modify genes remains largely inefficient. It also requires the simultaneous delivery of an additional DNA template containing the desired, corrected gene sequence, which needs to be positioned at the precise location where the double-stranded break has occurred. The requirement of an additional DNA template significantly increases the complexity of delivery.

Finally, nuclease editing does not allow for the correction of genes in non-dividing cells, further limiting their applications, given that the majority of cells in the adult body are non-dividing.

Base editors: A potential differentiated class of gene editors

Our base editing technology is a differentiated therapeutic approach to gene editing, potentially capable of altering the human genome at the foundational level of genetic information – a single base – without making a double-stranded break in the DNA. The elegance and simplicity of this approach can be thought of as a "pencil," where the error is erased and the correct letter is written.

We believe our base editing platform offers meaningful advantages over established approaches in gene editing, including:

- Highly precise and predictable gene editing, designed to make only one type of base edit at the desired target location;
- Highly efficient and therapeutically relevant levels of gene correction, which are generally unachievable by nuclease-based editing methods;
- Broad applicability in a wide range of cell types, including both dividing and non-dividing cells;
- Direct chemical modification of DNA with no requirement for delivery of the corrected DNA sequence;
- Avoidance of unwanted DNA modifications associated with double-stranded breaks, including gene disruptions and chromosomal rearrangements, such as translocations or deletions;
- The potential for permanent editing of genes, creating the opportunity for a life-long therapeutic outcome, including the ability to treat infants or young children since the edit will be passed on by dividing cells as the child grows;
- Multiple applications, including gene correction, gene silencing, gene activation, gene modification and/or multiplex editing of several genes simultaneously

- Preservation of natural regulation and a normal number of copies of the gene in the cell by modification of genes in their native genomic setting; and
- A versatile and modular product engine that can target a different gene sequence with the same base editor and a different guide RNA.

Our base editing technology

Our proprietary DNA base editors have two principal components that may be fused together or incorporated into one another to form a single protein. The first component is a CRISPR associated protein. These proteins have been adapted and engineered to target specific genomic locations in human cells. The targeting ability of the CRISPR protein has been preserved, but the cutting ability has been modified such that the CRISPR protein does not make a double-stranded break in the DNA. The second component of our base editors is a human deaminase, a class of naturally occurring enzymes. Our Cytosine Base Editors, or CBEs, and our Adenine Base Editors, or ABEs, each use a different, engineered deaminase, which we have designed to act only on single-stranded DNA. The deaminase makes a predictable chemical modification, called deamination, of the amine group on either adenine (A) or cytosine (C) bases.

The deaminase in a CBE will convert an amine group of C, resulting in the formation of uracil (U), which is read by the DNA polymerase as a thymine (T) base. Once this strand has been edited, the intermediate DNA consists of an edited strand, containing a U at the target locus, and an unedited strand with a guanine (G) base. The U:G is a mismatch and in order to preserve the edit, we modify the CRISPR to cleave the unedited single strand of the DNA, referred to as nicking. Nicking is intended to increase the efficiency of editing by inducing the cell to use the newly edited strand, and not the unedited strand, as the template for repair, resulting in a U:A pair with minimal translocations. Upon DNA repair or replication, the U is read as a T, resulting in a T:A pair, thereby completing the permanent conversion of a C:G base pair to a T:A base pair. Analogously, when an ABE is used instead of a CBE, the conversion of an amine group of A results in the formation of inosine, which is read by the DNA polymerase as a G, which subsequently leads to an A-to-G change. As a result, an A:T pair is converted to a G:C pair. Because the DNA is double-stranded, by targeting the non-coding strand, we can also convert a T:A pair to a C:G and a G:C pair to a A:T pair in the coding strand.

The modular and individual components of our base editors have the potential to be customized for specific diseases, potentially allowing us to create new programs with significant efficiencies in development. For instance, by changing the guide RNA and/or CRISPR protein, we can retarget base editors to different genomic locations based on their gene sequences. By changing the deaminase, we can retarget which base is edited (e.g., C or A). As a result, we believe our base editing technology is highly versatile, efficient, and scalable for the discovery of new drug candidates in the future.

Our base editing platform

We believe the unique advantages of base editing – single base editing precision, predictable editing outcome, high editing efficiency, and the avoidance of double-stranded breaks – make it a compelling approach for a wide range of therapeutic applications. This includes gene correction, gene modification, gene silencing and gene activation, as well as multiplex editing of several genes simultaneously.

To complement our next-generation gene editing technologies, we are also making significant investments in a suite of delivery technologies designed to deliver gene editing or other nucleic acid payloads to the right cells and enable potentially curative therapy. These delivery technologies include *ex vivo* modalities, such as electroporation, as well as *in vivo* modalities, such as LNPs. In our pipeline, we have initially focused on applications of these technologies where their delivery capabilities have already been clinically-validated by third parties, such as *ex vivo* editing of blood stem cells and LNP delivery to the liver. Longer term, we are also investing in more innovative delivery options, including next-generation mRNA and non-viral delivery technology. We have also developed critical enabling capabilities such as mRNA manufacturing and cell processing for autologous and allogeneic cell therapy.

Due to the critical importance of high-quality manufacturing and control of production timing and know-how, we have also established a 100,000 square foot cGMP manufacturing facility in Research Triangle Park, North Carolina. The facility is designed to support manufacturing for our *ex vivo* cell therapy programs in hematology and our *in vivo* non-viral delivery programs for liver and liver-mediated diseases, with the capability to scale-up to support potential commercial supply. For our initial clinical trials, we are relying primarily on our internal manufacturing capabilities, along with CMOs with relevant manufacturing experience in genetic medicines. We believe this investment will maximize the value of our portfolio and capabilities, the probability of technical success of our programs, and the speed at which we can provide potentially life-long cures to patients.

In summary, we believe that building an integrated platform combining our gene editing capabilities with advanced delivery and manufacturing capabilities will give us the flexibility to develop a sustainable portfolio, featuring rapid development of new programs and lifecycle improvements in our core programs.

In addition to our internal pipeline, the breadth and depth of our integrated technology platform gives us the opportunity to create a hub for partnering with other companies, which is an important part of our business model. We believe this model will help us to

unlock the full potential of precision genetic medicine across a wider array of possible applications, including many outside our core areas of focus.

Our overall goal with these platform activities is to continue expanding our access to the technologies and teams in genetic medicine that will maximize our long-term value creation and impact on patients.

Our base editing portfolio

We are advancing multiple programs through clinical development in parallel. Our lead programs are focused on sickle cell disease and AATD, and we are also advancing programs in other genetic diseases.

The following table summarizes the status of our primary programs:

PROGRAM / DISEASE or TARGET		DELIVERY	EDITING APPROACH	RESEARCH	LEAD OPTIMIZATION	IND ENABLING	PHASE I/II	PIVOTAL
Risto-cel	Sickle cell disease (SCD)	*Ex vivo* HSC	Activation of fetal hemoglobin (HbF)					
BEAM-103	SCD Beta-thalassemia	*Ex vivo or in vivo* HSC	CD117 edit-antibody					
***In vivo* HSC editing**	SCD Beta-thalassemia	*In vivo* LNP	Activation of HbF					
BEAM-302	Alpha-1 antitrypsin deficiency (AATD)	*In vivo* LNP	Correction of E342K mutation					
BEAM-301	Glycogen storage disease Ia (GSDIa)	*In vivo* LNP	Correction of R83C mutation					
BEAM-304	Phenylketonuria (PKU)	*In vivo* LNP	Correction of multiple mutations					
Apellis collaboration	Autoimmune disorders and other undisclosed	*In vivo* LNP	Modification of FcRN and other undisclosed					
Pfizer collaboration	Undisclosed	*In vivo* LNP	Undisclosed					

LNP = Lipid Nanoparticle; HSC = Hematopoietic Stem Cell; ESCAPE: Engineered Stem Cell Antibody Paired Evasion; FcRN = neonatal Fc receptor

Pipeline does not include assets designated for partnering

Hematology

We are pursuing a long-term, staged development strategy for our base editing approach to treat hematological diseases, such as sickle cell disease and beta-thalassemia. Our initial wave consists of *ex vivo* programs in which HSCs are collected from a patient, edited using electroporation, a clinically validated technology for the delivery of therapeutic constructs into harvested cells, and then infused back into the patient following a conditioning regimen, such as treatment with busulfan, the standard of care in HSC transplantation today. Once reinfused, the HSCs begin repopulating a portion of the bone marrow in a process known as engraftment. The engrafted, edited HSCs give rise to progenitor cell types with the corrected gene sequences. We are deploying this *ex vivo* approach in our risto-cel program. We are also pursuing a next wave of *in vivo* base editing with delivery directly into HSCs of patients via LNPs, which has the potential to make base editing therapy accessible to many more patients with sickle cell disease. We believe this multi-wave strategy can maximize the potential applicability of our sickle cell disease programs to patients as well as create a platform for the treatment of many other severe genetic blood disorders.

Sickle cell disease, a severe inherited blood disease, is caused by a single point mutation, E6V, in the beta globin gene. This mutation causes the mutated form of HbS to aggregate into long, rigid molecules that bend red blood cells into a sickle shape under conditions of low oxygen. Sickled cells obstruct blood vessels and die prematurely, ultimately resulting in anemia, severe pain (crises), infections, stroke, organ failure, and early death. Sickle cell disease is the most common inherited blood disorder in the United States, affecting an estimated 100,000 individuals, of which a significant proportion are of African American descent (1:365 births). Beta-thalassemia is another inherited blood disorder characterized by severe anemia caused by reduced production of functional hemoglobin due to insufficient expression of the beta globin protein. Transfusion-dependent beta-thalassemia, or TDBT, is the most severe form of this disease, often requiring multiple transfusions per year. Patients with TDBT suffer from failure to thrive, persistent infections, and life-threatening anemia. The incidence of symptomatic beta-thalassemia is estimated to be 1:100,000 worldwide, including 1:10,000 in Europe. In the United States, based on affected birth incidence of 0.7 in 100,000 births, and increasing survival rates, we expect the population of individuals affected by this disease to be more than 1,400 and rising.

Ex vivo base editing via autologous transplant with risto-cel

We are using base editing to pursue the development of risto-cel for the treatment of sickle cell disease. Risto-cel is a patient-specific, autologous HSC investigational therapy designed to offer a potentially best-in-class profile, incorporating base edits that are intended to mimic single nucleotide polymorphisms seen in individuals with hereditary persistence of fetal hemoglobin, or HPFH. The beneficial effects of the fetal form of hemoglobin, or HbF, to compensate for mutations in adult hemoglobin were first identified in individuals with HPFH. Individuals who carry mutations that would have typically caused them to be beta-thalassemia or sickle cell disease patients, but who also have HPFH, are asymptomatic or experience a much milder form of their disease.

Risto-cel aims to alleviate the effects of sickle cell disease by increasing HbF, which is expected to increase functional hemoglobin production and, in the case of sickle cell disease, inhibit hemoglobin S polymerization.

We are conducting a Phase 1/2 clinical trial designed to assess the safety and efficacy of risto-cel for the treatment of sickle cell disease, which we refer to as our BEACON trial. The BEACON trial includes approximately 50 adults and adolescents with severe sickle cell disease who have received prior treatment with at least one disease-modifying agent with inadequate response or intolerance. Following mobilization, conditioning and treatment with risto-cel, patients are assessed for safety and tolerability, with safety endpoints including neutrophil and platelet engraftment. Patients are also assessed for efficacy, with efficacy endpoints including the change from baseline in severe vaso-occlusive events, transfusion requirements, HbF levels, and quality of life assessments. The adult and adolescent enrollment for BEACON is complete, and manufacturing of all doses was completed as of December 2025. The FDA has granted orphan drug designation and regenerative medicine advanced therapy designation to risto-cel. Risto-cel has also been accepted into the FDA's Chemistry, Manufacturing, and Controls Development and Readiness pilot program.

In December 2025, we presented updated data from the BEACON trial at the American Society of Hematology 2025 Annual Meeting, or ASH. The presentation contained preliminary data as of August 6, 2025, from 31 patients in the trial, with follow up ranging from 0.3 to 20.4 months. The presentation data included the following:

- Patients achieved mean HbF levels above 60% and a mean durable reduction in corresponding HbS below 40%. A pancellular distribution of HbF, reflecting expression across most of the circulating red blood cells, was observed, with mean per-cell HbF levels maintained above the sickling threshold throughout follow-up. Durable, high editing efficiency was observed in peripheral blood and bone marrow following treatment with risto-cel. Mean peripheral blood editing was 67.4% at Month 6 and 72.8% by Month 12.
- Patients required a median of one (range: 1-5) stem cell collection cycle, comprising a median of three (range: 1–13) total collection days for the risto-cel manufacturing process and back-up cell collection. The median time to neutrophil engraftment was 17.5 days (range: 12-30), with a median duration of severe neutropenia of seven days (range: 1-17). The median time to platelet engraftment was 19 days (range: 11-53). In addition, 29% of patients did not require any platelet transfusions following risto-cel treatment.
- Total Hb levels increased rapidly with all patients experiencing resolution of anemia after elimination of the transfused blood. Key markers of hemolysis, including indirect bilirubin, haptoglobin, lactate dehydrogenase, and reticulocytes, normalized or improved in all patients following risto-cel treatment. Erythropoietin levels also trended toward normal, indicating significant improvement in oxygen delivery to tissues. Sickling parameters all decreased in the blood following risto-cel treatment to levels comparable to those seen in individuals with sickle cell trait.
- The initial safety profile of risto-cel was consistent with busulfan conditioning, autologous HSCT and underlying sickle cell disease. The most common treatment-emergent adverse events were consistent with busulfan conditioning, including febrile neutropenia, stomatitis and decreased appetite. As previously reported, one patient died four months after risto-cel infusion due to respiratory failure that was determined by the investigator to be likely related to busulfan conditioning and deemed unrelated to risto-cel. No patients experienced any investigator-reported severe vaso-occlusive crises post-engraftment.

We expect to submit a BLA for risto-cel as early as year-end 2026.

In Vivo *Base Editing via HSC-targeted LNPs*

We continue to develop targeted LNPs for the *in vivo* delivery of gene editing payloads to HSCs. Based on recent advancements in this technology, we are now prioritizing *in vivo* delivery for our next wave approach to treating sickle cell disease. We have identified multiple targeted LNPs that have the potential for HSC delivery and are currently engaged in lead optimization. In parallel, we are also continuing development of our proprietary ESCAPE platform, which combines antibody-based conditioning with multiplex gene edited HSCs. ESCAPE has the potential to enable non-genotoxic treatment strategies that can be delivered either *ex vivo* or *in vivo*, including as part of any future *in vivo* program for sickle cell disease. We are conducting a Phase 1 healthy volunteer clinical trial of BEAM-103, an anti-CD117 monoclonal antibody that enables ESCAPE, and expect to complete dosing in the trial in the first half of 2026.

Genetic diseases

LNPs are a clinically validated technology for delivery of nucleic acid payloads to the liver. LNPs are multi-component particles that encapsulate the base editor mRNA and one or more guides and protect them from degradation while in an external environment, enabling the transient delivery of the base editor *in vivo*. All of the components of the LNP, as well as the mRNA encoding the base editor, are well-defined and can be manufactured synthetically, providing the opportunity for scalable manufacturing. We are currently using LNPs to advance BEAM-302, BEAM-304 and BEAM-301.

BEAM-302: In vivo LNP liver-targeting for AATD

BEAM-302 is a liver-targeting LNP formulation of base editing reagents designed to offer a one-time treatment to correct the E342K point mutation (PiZZ genotype) predominantly responsible for the severe form of AATD. AATD is an inherited genetic disorder that can cause early onset emphysema and liver disease. The most severe form of AATD arises when a patient has a point mutation in both copies of the SERPINA1 gene at amino acid 342 position (E342K, also known as the PiZ mutation or the "Z" allele). This point mutation causes Alpha-1 antitrypsin, or AAT, to misfold, accumulating inside liver cells rather than being secreted, resulting in very low levels (10%-15%) of circulating AAT. In addition to resulting in lower levels, the PiZ AAT protein variant is also less enzymatically effective compared to wildtype AAT protein. As a consequence, the lung is left unprotected from neutrophil elastase, resulting in progressive, destructive changes in the lung, such as emphysema, which can result in the need for lung transplants. The mutant AAT protein also accumulates in the liver, causing liver inflammation and cirrhosis, which can ultimately cause liver failure or cancer requiring patients to undergo a liver transplant. It is estimated that approximately 100,000 individuals in the United States have two copies of the Z allele. There are currently no curative treatments for patients with AATD.

We are conducting a Phase 1/2 open label, dose exploration and dose expansion clinical trial of BEAM-302 for the treatment of AATD. The trial will evaluate the safety, tolerability, pharmacodynamics, pharmacokinetics and efficacy of BEAM-302. Part A of the trial is designed to evaluate AATD patients with lung disease, and Part B will evaluate AATD patients with mild to moderate liver disease with or without lung disease.

Updated clinical data from the dose-escalation portions of Part A and Part B are expected to be shared in the first quarter of 2026, along with an updated clinical development plan for BEAM-302 in patients with AATD. We expect to finalize dose selection for registrational development based on the totality of data from the BEAM-302 trial. We have reached alignment with the FDA on a potential accelerated approval pathway for BEAM-302 based on AAT biomarkers evaluated over 12 months. To support a future BLA submission, we anticipate enrolling approximately 50 additional patients to be treated with the selected optimal biological dose of BEAM-302 in an expansion of the ongoing Phase 1/2 clinical trial.

BEAM-304: In vivo LNP liver-targeting for PKU

BEAM-304 is a newly announced liver-targeting LNP formulation of base editing reagents designed to correct disease-causing mutations responsible for phenylketonuria, or PKU. PKU is an autosomal recessive disorder caused by mutations in the phenylalanine hydroxylase, or PAH, gene that prevents the body from metabolizing the amino acid phenylalanine, or Phe. Elevated levels of Phe may result in severe neurological and neurocognitive impairments. Patients are generally identified via newborn screening, with the standard of care involving a Phe-restricted diet, as well as medicines that manage Phe levels. Initial clinical development will focus on base editors addressing the two most prevalent variants found in nearly half of patients with PKU, with ongoing research efforts to address additional pathogenic mutations. In preclinical studies, administration of BEAM-304 resulted in the normalization of Phe levels in mice at therapeutically relevant doses, even when consuming a standard diet. In 2026, we plan to submit a regulatory application for authorization to initiate an open-label, dose-ascending, Phase 1/2 trial of BEAM-304 in PKU patients with the R408W mutation. We believe that learnings from this trial have the potential to provide a predictable path to accelerated development of BEAM-304 for additional mutations, including as a result of novel FDA frameworks for platform medicines.

BEAM-301: In vivo LNP liver-targeting for GSDIa

BEAM-301 is a liver-targeting LNP formulation of base editing reagents designed to correct the R83C mutation, the most prevalent disease-causing mutation for, and the mutation which results in the most severe form of, GSDIa. GSDIa is an autosomal recessive disorder caused by mutations in the G6PC gene that disrupts a key enzyme, G6Pase, critical for maintaining glucose homeostasis. Inhibition of G6Pase activity results in low fasting blood glucose levels that can result in seizures and be fatal. Patients with this mutation typically require ongoing corn starch administration, without which they may enter into hypoglycemic shock within one to three hours.

We are conducting a Phase 1/2 clinical trial of BEAM-301 at a select number of sites in the United States. The trial is an open-label, multi-cohort, single-ascending dose evaluation of BEAM-301 for the treatment of GSDIa in patients with the R83C mutation. Key endpoints of the trial include safety and tolerability, time to hypoglycemia during fasting, and changes from baseline in corn starch supplementation. Dosing is complete in the first cohort and enrollment has been initiated in the second cohort. We expect to report initial data from the trial in 2026.

Collaborations

We believe our collection of base editing, gene editing and delivery technologies has significant potential across a broad array of genetic diseases. To fully realize this potential, we have established and plan to continue to seek out innovative collaborations, licenses, and strategic alliances with pioneering companies and with leading academic and research institutions. Additionally, we have and intend to continue to pursue relationships that potentially allow us to accelerate our preclinical research and development efforts. We believe these relationships will allow us to aggressively pursue our vision of maximizing the potential of base editing to provide life-long cures for patients suffering from serious diseases.

Pfizer

In December 2021, we entered into a four-year research collaboration agreement with Pfizer Inc., or Pfizer, focused on *in vivo* base editing programs for three targets for rare genetic diseases. In December 2025, at the completion of the research term, Pfizer opted in to an exclusive, worldwide license for a liver-targeted development candidate in the collaboration. The development candidate employs our proprietary, liver-targeting LNP to deliver base editing reagents. In connection with the opt-in, Pfizer will take an exclusive, worldwide license to the development candidate, after which it will be responsible for all development activities, as well as potential regulatory approvals, manufacturing and commercialization. We will be eligible for development, regulatory and commercial milestone payments and will have a right to opt in, at the end of Phase 1/2 clinical trials, upon the payment of an option exercise fee, to a global co-development and co-commercialization agreement pursuant to which we and Pfizer would share net profits as well as development and commercialization (including manufacturing) costs in a 35%/65% ratio (Beam/Pfizer).

Apellis Pharmaceuticals

In June 2021, we entered into a research collaboration agreement, or the Apellis Agreement, with Apellis Pharmaceuticals, Inc., or Apellis, focused on the use of our base editing technology to discover new treatments for complement system-driven diseases. Under the terms of the Apellis Agreement, we will conduct preclinical research on six base editing programs that target specific genes within the complement system in various organs, including the eye, liver, and brain. Apellis has an exclusive option to license any or all of the six programs and will assume responsibility for subsequent development. As of September 30, 2025, Apellis notified us of its decision to opt-in to the base editing program directed to FcRN. As a result of Apellis' decision to opt-in to the program, we received a cash opt-in fee of $3.8 million. We may elect to enter into a 50-50 U.S. co-development and co-commercialization agreement with Apellis with respect to one program licensed under the collaboration.

Verve Therapeutics and Eli Lilly and Company

We are party to a license agreement, or the Verve Agreement, with Verve Therapeutics, Inc., or Verve, pursuant to which we granted Verve exclusive worldwide licenses under our base editing technologies for human therapeutic applications against a total of three liver-mediated, cardiovascular disease targets, which consist of PCSK9, ANGPTL3 and an undisclosed target. In October 2023, we entered into a transfer and delegation agreement, or the Lilly Agreement, with Eli Lilly and Company, or Lilly, pursuant to which Lilly acquired certain assets and other rights under the Verve Agreement, including our opt-in rights to co-develop and co-commercialize each of Verve’s base editing programs. In addition, Lilly acquired the right to receive any future milestone or royalty payments payable to us under the Verve Agreement. Under the terms of the Lilly Agreement, we received a $200.0 million payment and are eligible to receive up to $350.0 million in potential future development-stage payments upon the completion of certain clinical, regulatory and alliance events, of which $25.0 million has been received through December 31, 2025. There were no milestone payments received during the year ended December 31, 2025. In July 2025, Lilly announced that it had completed an acquisition of Verve.

Orbital Therapeutics and Bristol Myers Squibb Company

We are party to a license agreement, or the Orbital Agreement, with Orbital, pursuant to which each of us have granted the other non-exclusive licenses to certain technology that is necessary or reasonably useful for the non-viral delivery or the design or manufacture of RNA for the prevention, treatment or diagnosis of human disease. Our license to Orbital is for all fields other than the Beam field, as described below, and also excludes the targets and substantially all of the indications that are the subject of our existing programs. The Beam field consists of all products and biologics that function in the process of gene editing or conditioning for use in cell transplantation, or that act in combination with any such products or biologics. Orbital’s license to us is for all fields other than the Orbital field, which consists of products and biologics that function as vaccines and also of therapeutic proteins, other than therapeutic proteins (i) that use gene editing, (ii) for use in conditioning, (iii) for use in regenerative medicine, (iv) for use as a CAR immune therapy that does not use gene editing (v) for use as a T-cell receptor therapy that does not use gene editing or (vi) that modulate certain immune responses.

In December 2025, Bristol-Myers Squibb Company completed an acquisition of Orbital, or the Acquisition. At the closing of the Acquisition, we held 75 million shares of Orbital common stock, which were cancelled and converted into $255.1 million in closing cash consideration, plus the right to receive up to approximately $26.3 million in additional cash consideration upon the release, if any, of certain escrows.

Prime Medicine

We are party to a collaboration and license agreement with Prime Medicine to research and develop a novel gene editing technology developed by one of our founders. Under the terms of the agreement, we granted Prime Medicine a non-exclusive license to certain CRISPR technology, delivery technology and certain other technology controlled by us to develop and commercialize gene editing products for the treatment of human diseases. Prime Medicine granted us an exclusive license to develop and commercialize prime gene editing technology for the creation or modification of any single base transition mutations, as well as any edits made for the treatment of sickle cell disease.

Competition

The pharmaceutical and biotechnology industries, including the genetic medicines field, are characterized by rapidly advancing technologies, intense competition, and a strong emphasis on intellectual property. While we believe that our differentiated technology, scientific expertise, and intellectual property position provide us with competitive advantages, we face potential competition from a variety of companies in these fields. Within these industries, we will compete with existing large pharmaceutical companies, specialty pharmaceutical companies, and biotechnology companies.

There are several other companies utilizing base editing technology, including Life Edit (an ElevateBio company), Metagenomi, Revvity, Aurora Therapeutics, Mammoth Biosciences, YolTech Therapeutics, HuidaGene Therapeutics and Intellia Therapeutics. In addition, we face competition from companies utilizing other gene editing modalities and various other genetic medicines.

Within the disease areas that we focus on, we are also aware of competing companies that have approved therapies, those with therapies in development, and others that may emerge in the future. For sickle cell disease, these companies include CRISPR Therapeutics, Vertex Pharmaceuticals, Genetix Biotherapeutics (formerly bluebird bio), YolTech Therapeutics, Novartis Pharmaceuticals, Kamau Therapeutics, Fulcrum Therapeutics, Tessera Therapeutics, Cimeio Therapeutics and Agios Pharmaceuticals. For our AATD targeted therapies, these include Wave Life Sciences, YolTech Therapeutics, Prime Medicine, CRISPR Therapeutics, Moderna, Korro Bio, Tessera Therapeutics and Arrowhead Pharmaceuticals.

Any product candidates that we successfully develop and commercialize will compete with existing therapies and new therapies that may become available in the future that are approved to treat the same diseases for which we may obtain approval for our product candidates. This may include other types of therapies, such as small molecule, antibody, and/or protein therapies.

In addition, many of our current or potential competitors, either alone or with their collaboration partners, have significantly greater financial resources and expertise in research and development, manufacturing, conducting preclinical studies and clinical trials and seeking approval for products than we do today. Mergers and acquisitions in the pharmaceutical, biotechnology and gene therapy 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 also compete with these companies in recruiting, hiring and retaining qualified scientific and management talent, establishing clinical trial sites and patient registration for clinical trials, obtaining manufacturing slots at CMOs, and in acquiring technologies complementary to, or necessary for, our programs. Our commercial opportunity could be reduced or eliminated if our competitors develop and commercialize products that are safer, more effective, particularly if they represent cures, have fewer or less severe side effects, are more convenient, or are less expensive than any products that we may develop. Our competitors also may obtain FDA or other regulatory approval for their products more rapidly than we may obtain approval for ours, which could result in our competitors establishing a strong market position before we are able to enter the market. The key competitive factors affecting the success of all of our programs are likely to be their efficacy, safety, convenience, and availability of reimbursement.

Intellectual property

Our success depends in part on our ability to obtain and maintain proprietary protection for our platform technology, our programs, and know-how related to our business, defend and enforce our intellectual property rights, in particular, our patent rights, preserve the confidentiality of our trade secrets, and operate without infringing, misappropriating or otherwise violating any valid and enforceable intellectual property rights of others. We seek to protect our proprietary position by, among other things, exclusively licensing and filing U.S. and certain foreign patent applications related to our platform technology, existing and planned programs, and improvements that are important to the development of our business, where patent protection is available. Notwithstanding these efforts, we cannot be sure that patents will be granted with respect to any patent applications we have licensed or filed or may license or file in the future, and we cannot be sure that any patents we have licensed or patents that may be licensed or granted to us in the future will not be challenged, invalidated, or circumvented or that such patents will be commercially useful in protecting our technology. For more information regarding the risks related to our intellectual property, please see Item 1A., *Risk factors—Risks related to our intellectual property*, in this Annual Report on Form 10-K.

Our wholly owned and our in-licensed patents and patent applications cover various aspects of our base editing platform and our programs, including various gene editors, guide RNA sequences, systems and methods for increasing the specificity of gene editing, therapeutic methods, and various modalities for delivery gene editors.

We also have an option to license patents and patent applications relating to CRISPR/Cas9 systems. We intend to continue to pursue, when possible, additional patent protection, including composition of matter, method of use, and process claims, directed to each component of our platform technology and the programs in our portfolio. As of December 31, 2025, our wholly-owned patent portfolio consisted of 12 issued U.S. patents, and 57 issued patents in jurisdictions outside the United States. We also have more than 550 pending patent applications, including PCT applications, provisional patent applications and counterparts to the foregoing U.S. and foreign patents. In addition, we co-own one issued U.S. patent and five issued patents in jurisdictions outside the United States. We also have approximately 24 pending patent applications between the Broad Institute, Inc., UCL Business, Ltd., and Apellis Pharmaceuticals, Inc. The patents and patent applications outside of the United States were filed in numerous jurisdictions, including Australia, Brazil, Canada, China, Europe, Hong Kong, India, Japan, Korea, Singapore and South Africa. Many of our owned patents and patent applications are related to our DNA base editing technology, including claims to base editor variants with enhanced activities or novel properties, methods of using such base editors, methods of using such base editors for therapeutic indications, guide RNAs that target base editors to therapeutically relevant DNA sequences, and methods for evaluating base editing specificity. Certain of our owned patents and patent applications are related to viral and non-viral delivery technologies. If issued as U.S. patents, and if the appropriate maintenance fees are paid, the U.S. patents would be expected to expire between 2039 and 2046, excluding any additional term for patent term adjustments or patent term extensions.

As of December 31, 2025, our in-licensed patent portfolio consisted of more than 75 issued U.S. patents, and more than 150 issued patents in jurisdictions outside the United States. We also have more than 300 pending patent applications, including PCT applications, provisional patent applications and counterparts to the foregoing U.S. and foreign patents. The patents and patent applications outside of the United States were filed in numerous jurisdictions, including Australia, Canada, China, Europe, Hong Kong, India, Israel, Japan, Korea, New Zealand, Russia and Singapore. The patents and applications from our in-licensed portfolio for DNA base editing include claims to novel base editors, claims to engineered deaminase enzymes (e.g., compositions including the base editor or engineered deaminase as a component, methods of using such base editors, including methods of using such base editors for therapeutic indications, and guide RNAs that target base editors to therapeutically relevant DNA sequences. The in-licensed patents and applications also cover various aspects related to the platform technology, including base editing systems that employ *S. pyogenes* Cas9, *S. aureus* Cas9, Cas9 PAM variants, inactive forms of Cas9, and/or Cas9 nickases, and systems for delivery of base editors. The patents and applications from our in-licensed portfolio for RNA base editing include claims to novel base editors, compositions including the base editor as a component, guide RNAs that target base editors to therapeutically relevant RNA sequences, and methods of using such base editors, including methods of using such base editors for therapeutic indications. The patents and applications from our in-licensed portfolio for delivery technologies include claims to novel lipid-based delivery systems and compositions, and methods of using such systems and compositions to deliver base editors. The patents and applications from our in-licensed portfolio for the balance of our platform include claims to compositions and methods for delivery of charged base editor proteins into cells, modification and improvements to the base editing systems including improvements to the nucleotide binding protein component, guide RNA component and base editing enzyme component of the base editing complex, methods for evaluating gene targeting and base editing efficiency and compositions and methods for prime editing. Our current in-licensed patents and patent applications, if the appropriate maintenance fees are paid, are expected to expire between 2034 and 2044, excluding any additional term for patent term adjustments or patent term extensions (or the corresponding foreign equivalent). For information related to our in-licensed intellectual property, see the subsection below titled "—Intellectual Property Licenses."

We also have a nonexclusive license to conduct research activities and an option to exclusively license certain patents and patent applications directed to Cas9 and Cas12a from Editas Medicine, Inc., or Editas, which in turn has licensed such patents from various academic institutions. In the case of Cas9, a number of the U.S. patents are subject to an interference declared by the Patent and Trademark office, and a number of the European patents are the subject of one or more oppositions. For more information regarding the risks related to our intellectual property, please see Item 1., *Business—Intellectual property—Intellectual property licenses* and Item 1A., *Risk factors—Risks related to our intellectual property*, in this Annual Report on Form 10-K.

The term of individual patents depends upon the legal term for patents in the countries in which they are granted. In most countries, including the United States, the patent term is 20 years from the earliest claimed filing date of a non-provisional patent application in the applicable country. However, the actual protection afforded by a patent varies from country to country, and depends upon many factors, including the type of patent, the scope of its coverage, the availability of regulatory-related extensions, the availability of legal remedies in a particular country and the validity and enforceability of the patent. In the United States, a patent's term may, in certain cases, be lengthened by patent term adjustment, or PTA, which compensates a patentee for administrative delays by the USPTO in examining and granting a patent, or may be shortened (e.g., if a patent is terminally disclaimed over a commonly owned patent having an earlier expiration date). In some instances, such a PTA may result in a U.S. patent term extending beyond 20 years from the earliest date of filing a non-provisional patent application related to the U.S. patent. Patent term extensions, or PTE, under the Drug Price Competition and Patent Term Restoration Act of 1984, commonly known as the Hatch-Waxman Act, are also possible for patents that cover an FDA-approved drug as compensation for the patent term lost during the FDA regulatory review process. The Hatch-Waxman Act permits a PTE of up to five years beyond the expiration of the patent. The length of the PTE is related to the length of time the drug is under regulatory review. PTE cannot extend the remaining term of a patent beyond a total of 14 years from the date of product approval and only one patent applicable to an approved drug, a method for using it, or a method of manufacturing it, may be extended. Similar provisions are available in Europe and certain other jurisdictions to extend the term of a patent that covers an approved drug. In the future, if our products receive regulatory approval, we may be eligible to apply for PTEs on patents covering such products, however there is no guarantee that the applicable authorities, including the FDA in the United States, will agree with our assessment of whether such PTE should be granted, and if granted, the length of such PTE. For more information regarding the risks related to our intellectual property, please see Item 1A., *Risk factors—Risks related to our Intellectual property*, in this Annual Report on Form 10-K.

We also rely on trade secrets, know-how, continuing technological innovation, and confidential information to develop and maintain our proprietary position and protect aspects of our business that are not amenable to, or that we do not consider appropriate for, patent protection. We seek to protect our proprietary technology and processes, in part, by confidentiality agreements with our employees, consultants, scientific advisors, and contractors. We also seek to preserve the integrity and confidentiality of our data and trade secrets by maintaining physical security of our premises and physical and electronic security of our information technology systems. While we have implemented measures to protect and preserve our trade secrets, such measures can be breached, and we may not have adequate remedies for any such breach. In addition, our trade secrets may otherwise become known or be independently discovered by competitors. For more information regarding the risks related to our intellectual property, please see Item 1A., *Risk factors—Risks related to our intellectual property*, in this Annual Report on Form 10-K.

We also rely on trademark protection for our company name and related designs. As of December 31, 2025, our registered trademark portfolio contained 35 registered/allowed trademarks and pending trademark applications in the United States and in certain overseas jurisdictions.

Intellectual property licenses

We are a party to a number of license agreements under which we license patents, patent applications, and other intellectual property from third parties. The licensed intellectual property covers, in part, CRISPR-related compositions of matter and their use for base editing. These licenses impose various diligence and financial payment obligations on us. We expect to continue to enter into these types of license agreements in the future. We consider the following license agreements to be material to our business.

License agreement with The President and Fellows of Harvard College

In June 2017, we entered into a license agreement with Harvard, as amended, or the Harvard License Agreement, pursuant to which we received an exclusive, worldwide, royalty-bearing, sublicensable license under certain patent rights owned or controlled by Harvard to make, have made, offer for sale, sell, have sold and import licensed products in the field of the prevention or treatment of any and all human diseases and conditions, excluding human germline modification and products for non-human animal and plant applications. We also received a non-exclusive, worldwide, royalty-bearing, sublicensable license to research, have researched, develop and have developed "enabled" products related to the Harvard patent rights which are not licensed products.

The licensed patents are directed, among other things, to C-to-T, A-to-G, and C-to-G base editors, for the treatment of certain diseases and conditions and to base editing, more generally.

Under the Harvard License Agreement, we are required to use commercially reasonable efforts to develop products incorporating the base editing technology covered in the licensed patents, in accordance with a development plan that we prepared and submitted to Harvard. The development plan includes certain development milestones that we are required to meet, as well as the timelines for the completion thereof, and we may update the development plan from time to time as we believe necessary, in our good faith judgment, for us to meet such milestones. If we are successfully able to gain regulatory approval in any country to introduce a licensed product into the commercial market in such country, then we are also required to use commercially reasonable efforts to commercialize such licensed product and make such licensed product reasonably available to the public. If we fail to meet any of the deadlines for the development milestones, then Harvard may, depending on the nature of the failure and the impacted milestones, either terminate the Harvard License Agreement or our licenses with respect to the applicable licensed product(s), subject to certain exceptions and

opportunities for us to cure such failure. Additionally, we are required to meet development milestones for the development of a licensed product covered by certain sub-categories of licensed patents. Failure to achieve milestones with respect to such sub-categories gives Harvard the right to grant third parties non-exclusive licenses under such failed sub-categories.

The licenses granted to us under the Harvard License Agreement are expressly subject to certain preexisting rights held by Harvard and certain third parties. For example, certain of the licensed patents were developed by employees of the Howard Hughes Medical Institute and were subsequently assigned to Harvard but remain subject to a non-exclusive license between Harvard and Howard Hughes, pursuant to which Howard Hughes received a license from Harvard under certain of the licensed patents for research purposes with the right to sublicense to non-profit and governmental entities. In addition, certain of the licensed patents claim or cover inventions resulting from research that was sponsored by the U.S. government, and the U.S. government retains certain rights with respect to such licensed patents under applicable U.S. law. Harvard additionally retains limited rights for itself and for other non-profit research organizations to practice the licensed patents for research, educational, and scholarly purposes. Furthermore, Harvard retains the right, beginning a certain period of time after regulatory approval of any licensed product in the U.S. or certain European countries, to grant third parties the non-exclusive right to develop, manufacture, have manufactured, import, have imported, offer for sale, sell, have sold or otherwise distribute or have distributed such licensed product or an equivalent thereof solely for sale on a locally-affordable basis in certain specified developing countries in which we do not have plans to seek regulatory approval.

Although the licenses granted to us under the Harvard License Agreement are exclusive, Harvard may grant a license to a third party under the licensed patents to research, develop, and commercialize a product directed to one or more particular targets, or a proposed product, in the field under limited circumstances. If a third party that is not a specified competitor of ours inquires with Harvard for such a license, and then attempts to enter into a sublicense agreement with us after being referred to us by Harvard and fails to do so after a certain period of time and presents to Harvard a proposal including certain information describing the proposed development and commercialization of such product, then Harvard may notify us of such proposal. If we are not researching, developing or commercializing such a proposed product, then we can notify Harvard as to whether we are interested in developing such proposed product, entering into a sublicense agreement with such third party to develop such proposed product, or entering into a sublicense with another third party to develop the same proposed product. If we inform Harvard that we are interested in developing such proposed product, then we will prepare a development plan, similar in scope to the development plan under the Harvard License Agreement, to develop such proposed product. If we inform Harvard that we are interested in entering into a sublicense agreement pursuant to which a third party would receive a sublicense from us under the licensed patents to develop such proposed product, then we will have a specified period of time to enter into such a sublicense agreement and provide reasonable evidence thereof. If we are not researching, developing, or commercializing such a proposed product, fail to provide a development plan, or fail to enter into a sublicense agreement with respect to such proposed product, in each case, within specified time periods, then Harvard may grant a license to the applicable third party under the licensed patents to research, develop, and commercialize such proposed product.

We are permitted to further sublicense our rights under the Harvard License Agreement to third parties, provided that any such sublicense agreement with a third party must remain in compliance with and be consistent with the terms of the Harvard License Agreement, and certain rights granted to us under the Harvard License Agreement can only be sublicensed to *bona fide* collaboration partners who are working with us to develop one or more licensed products. In addition, any such sublicense agreement must include certain provisions to ensure our ability to comply with the Harvard License Agreement. We are also responsible for any breaches of a sublicense agreement by the applicable sublicensee, if such breach results in a material breach of the Harvard License Agreement, provided that if we cure the breach or diligently enforce our rights to terminate the sublicense, we will not be subject to termination by Harvard for the sublicensee's breach, even if it resulted in a material breach of the Harvard Agreement.

In exchange for the licenses granted to us under the Harvard License Agreement, we initially issued to Harvard 101,363 shares of our common stock and subsequently issued 765,549 shares of our common stock pursuant to anti-dilution rights in the Harvard License Agreement. We are also required to pay to Harvard an annual license maintenance fee ranging from low-to-mid five figures to low six figures, depending on the particular calendar year. Harvard is also entitled to receive potential clinical and regulatory milestones in the mid-to-high eight figure range, and to receive success payments based on increases in the fair market value of our common stock. If we undergo a change of control during the term of the Harvard License Agreement, then certain of the milestone payments would be increased. We paid Harvard a total of $9.0 million upon the completion of our Series A and Series B financings.

In May 2021, the first success payment measurement occurred and amounts due to Harvard were calculated to be $15.0 million. We elected to make the payment in shares of our common stock and issued 174,825 shares of our common stock to settle this liability on June 10, 2021. We may additionally owe Harvard success payments of up to an additional $90.0 million.

With respect to the sale of licensed products by us, our affiliates or our sublicensees, Harvard is entitled to receive low single digit royalties on net sales of licensed products until, on a country-by-country basis, the latest of the expiration of (i) the last to expire valid claim of a licensed patent covering the applicable licensed product, (ii) the period of exclusivity associated with such licensed product in such country or (iii) a certain number of years after the first commercial sale of such licensed product in such country. We are entitled to certain reductions and offsets on these royalties with respect to a licensed product in a given country and certain increases in the event we, our affiliates or sublicensees bring patent challenges relating to any licensed patents (subject to an ability to delay and/or avoid such increases by diligently seeking to terminate and/or terminating the sublicense that has taken the applicable action). If we sublicense our rights to develop or commercialize a licensed product under the Harvard License Agreement to a third party and we receive non-royalty sublicense income, then Harvard is entitled to a percentage of such consideration, ranging from the high single digits to an amount in the first decile depending on the date in which such sublicense agreement is executed and the stage of development our licensed products at such time.

Harvard is responsible for the prosecution and maintenance of all licensed patents, provided that we have customary consultation, comment, and review rights with respect to such prosecution and maintenance activities. We are responsible for Harvard's documented out-of-pocket expenses with respect to such prosecution and maintenance, but if Harvard enters into a license agreement with a third party pursuant to which it grants such third party a license under the licensed patents outside of our field, then Harvard must use reasonable efforts to include a provision in such agreement that provides for an apportionment of prosecution and maintenance costs between us and such third party with respect to such licensed patents. If we choose to no longer pay for the prosecution and maintenance costs of a given licensed patent, then we will be relieved of such payment obligation, but our license with respect to such licensed patent will also terminate.

Unless earlier terminated, the Harvard License Agreement will remain in effect until the later of the last-to-expire valid claim of the licensed patents or the end of the last to expire royalty term. We may terminate the Harvard License Agreement at our convenience following written notice to Harvard. Either party may terminate the Harvard License Agreement for a material breach of the other party, subject to a notice and cure period. Harvard may also terminate the Harvard License Agreement in the event of our bankruptcy or insolvency or if we fail to procure and maintain insurance. Upon expiration or termination of the Harvard License Agreement, the licenses granted to us will terminate and all rights under the licensed patent rights will revert to Harvard.

License agreement with Editas Medicine, Inc.

In May 2018, we entered into a license agreement, or the Editas License Agreement, with Editas pursuant to which we received an exclusive (even as to Editas), royalty-bearing, sublicensable, worldwide license under certain patent rights owned or controlled by Editas related to certain base editing technologies and CRISPR technology to develop, commercialize, make, have made, use, offer for sale, sell and import certain base editing products for the treatment of human diseases or conditions. The license we received is non-exclusive with respect to certain specified targets. Our licensed field excludes the use of certain gene editing technologies in certain fields of use that have already been licensed to other partners of Editas, provided that our licensed field may expand if the fields licensed to other Editas partners are reduced or are otherwise modified as a result of any termination, expiration, or amendment to Editas' agreements with such partners. In addition, we received a royalty-free, non-sublicensable, non-exclusive license under a separate set of patent rights owned or controlled by Editas to conduct research activities in our licensed field and for which we have an option to obtain an exclusive license from Editas.

Certain of the patents licensed to us under the Editas License Agreement were licensed to Editas from Broad Institute and Harvard and certain of the patents for which we have an option to obtain a license were licensed to Editas from the Massachusetts General Hospital, or MGH. Accordingly, the licenses granted to us under the Editas License Agreement are subject to the terms and conditions set forth in each of the license agreements concerning the licensed patents between Broad Institute, Harvard and Editas, or the Broad/Harvard Head Licenses, and each of the license agreements concerning the patents for which we have an option to obtain a license between MGH and Editas, or the MGH Head Licenses.

As described above, Editas granted us an exclusive option to obtain an exclusive license under certain patents on a patent family-by-patent family basis. If we so exercise the option with respect to a patent family of such optioned patents, then we would receive an exclusive license to such patent family of the same scope as the other patents exclusively licensed to us under the Editas License Agreement. In order to exercise an option with respect to a patent family of these optioned patents we would pay an eight-figure option exercise fee, depending on the date in which particular option is exercised.

Under the Editas License Agreement, we are required to use commercially reasonable efforts to develop a licensed product in our licensed field in each of the United States, Japan, the United Kingdom, or U.K., Germany, France, Italy and Spain. If we are successfully able to gain regulatory approval in any country for a licensed product, then we are also required to use commercially reasonable efforts to commercialize such licensed product in such country. We also have sole control and responsibility over all regulatory activities with respect to the development of licensed products.

We are permitted to further sublicense certain of our rights under the Editas License Agreement to third parties, provided that any such sublicense agreement with a third party must remain in compliance with and be consistent with the terms of the Editas License Agreement and the Broad/Harvard Head Licenses and MGH Head Licenses, as applicable. We are also responsible for any breaches of a sublicense agreement by the applicable sublicensee and are responsible for all payments due under the Editas License Agreement by operation of any such sublicense. Following the signing of the Editas License Agreement, we obtained the right to further sublicense our rights to the licensed patents from Broad Institute and Harvard to third parties, provided that we comply with certain sublicensing requirements under each of the Broad/Harvard Head Licenses as if we were Editas, as well as certain other customary conditions. We have not obtained any such right from MGH allowing us to further sublicense our rights under the licensed patents from MGH to third parties and will require written consent in the event we wish to further sublicense such rights to a third party.

Upon the execution of the Editas License Agreement, we paid Editas an upfront fee of $180,000. We also issued to Editas 1,833,333 shares of our Series A-1 Preferred Stock and 1,222,222 shares of our Series A-2 Preferred Stock. In addition, if any of our commercial, regulatory, development or sales activities with respect to the licensed products triggers a milestone payment or sublicense income that Editas owes under the Broad/Harvard Head Licenses or the MGH Head Licenses, then we are required to pay Editas the full amount of such milestone payment or sublicense income, as applicable; provided that we will not pay Editas for any sublicense income due as a result of our payment of any option exercise fee to Editas. Aggregate milestone amounts under the Editas License Agreement could equal up to $68.8 million for each product developed and commercialized using rights related to certain base editing technologies and CRISPR technology; in the event we develop and commercialize products covered by claims from the additional patent families licensed or optioned to us under the Editas License Agreement, aggregate milestone payments could equal up to $74.0 million per product. The percentage of sublicense income we would owe under the Editas License Agreement ranges from none to amounts between 10% and 20%. In addition, we agreed to pay for a portion of the annual license maintenance fees and prosecution and maintenance costs that Editas incurs itself or owes under the Broad /Harvard Head Licenses and the MGH Head Licenses with respect to the licensed patents. The upfront fee, equity issuance, and option exercise payments we make to Editas under the Editas License Agreement constitute both consideration for the licenses granted to us under the Editas License Agreement and reimbursement for prosecution and maintenance costs for the licensed patents.

With respect to the sale of licensed products by us, our affiliates or our sublicensees, we are required to pay to Editas an amount equal to the royalty rates that it owes to Broad Institute, Harvard, or MGH under its applicable in-licenses, plus an additional low- to mid-single digit royalty on net sales of licensed products, depending on whether such licensed product is covered by an Editas-owned patent and based on the aggregate worldwide net sales of licensed products in a given calendar year. We are entitled to certain reductions and offsets on these royalties with respect to a licensed product in a given country and if Editas is entitled to receive any reductions or offsets in respect to its royalty payment obligations under the relevant Broad/Harvard Head Licenses or MGH Head Licenses, then Editas will use reasonable efforts to avail itself of such reductions, which in turn would reduce our royalty payment obligations under the Editas License Agreement. The royalty term expires on a licensed product-by-licensed product and country-by-country basis upon the later of (i) the last-to-expire royalty term in such country under any applicable Broad/Harvard Head License or MGH Head License, and, if such product is covered by a licensed Editas-owned patent, (ii) the date at which such product is no longer covered by a valid claim of a licensed Editas-owned patent in such country.

As between the parties, Editas is responsible for the prosecution and maintenance of all licensed patents, provided that we have certain information, comment, and review rights for certain of the licensed patents.

Unless earlier terminated, the Editas License Agreement will expire on a licensed product-by-licensed product and country-by-country basis on the expiration of the applicable royalty term with respect to such licensed product in such country. We may terminate the Editas License Agreement on written notice to Editas subject to a specified notice period. Either party may terminate the Editas License Agreement for a material breach of the other party, subject to a notice and cure period. Editas may also terminate the Editas License Agreement if we challenge the validity of any of the licensed patents, subject to customary carveouts. Upon expiration or termination of the Editas License Agreement in its entirety or with respect to a family of patents, the licenses granted to us will immediately terminate in its entirety or solely with respect to the expired or terminated patent family, as the case may be; however, if we have the right to terminate the Editas License Agreement due to Editas' material breach of the Editas License Agreement, then in lieu of so terminating the Editas License Agreement, we can elect to reduce our royalty payment obligations under the Editas License Agreement by certain specified percentages.

License agreement with Bio Palette Co., Ltd.

On March 27, 2019, we entered into a license agreement, or the Bio Palette License Agreement, with Bio Palette Co., Ltd., or Bio Palette, pursuant to which we received an exclusive (even as to Bio Palette and its affiliates), sublicensable license under certain patent rights related to base editing owned or controlled by Bio Palette to research, make, have made, import, export, distribute, use, have used, sell, have sold or offer for sale, and otherwise exploit products for the treatment of human disease throughout the world, but excluding products in the microbiome field in Asia. In addition, we granted Bio Palette an exclusive (even as to us and our affiliates) license under certain patent rights related to base editing and gene editing owned or controlled by us to research, make, have made, import, export, distribute, use, have used, sell, have sold or offer for sale, and otherwise exploit products in the microbiome field in Asia, subject to our right, in its sole discretion, to expand Bio Palette's license (and the applicable royalty obligations) to the entire

territory. Each party to the Bio Palette Agreement retains non-exclusive rights to develop and manufacture products in the microbiome field worldwide for the sole purpose of exploiting those products in its own territory. Each party agrees to certain coordination obligations in the microbiome field in the event that either party determines not to exploit their rights in such field.

If Bio Palette comes into the control of any other patent right that is useful for the treatment, diagnosis or prevention of any human diseases or conditions and intends to grant a license under that patent right in certain defined fields and in certain defined territories, we have the exclusive right of first negotiation for an exclusive license under that patent right in those fields and territories. If we come into the control of any other patent right that is useful in certain defined fields and intend to grant a license under that patent right in those fields in certain defined territories, Bio Palette has the exclusive right of first negotiation for an exclusive license under that patent right in those fields and territories.

We are required to use commercially reasonable efforts to develop a licensed product in the United States, Japan, the U.K., France, Germany, Italy and Spain. For any licensed product in our licensed field and territory that receives regulatory approval, we are required to use commercially reasonable efforts to commercialize that licensed product in the relevant country. Bio Palette is required to use commercially reasonable efforts to develop a licensed product in Japan. For any licensed product that receives regulatory approval, Bio Palette is required to use commercially reasonable efforts to commercialize such licensed product in the relevant country.

Certain of the patents, or the Kobe Patents, licensed to us under the Bio Palette License Agreement were licensed to Bio Palette from Kobe University under a license agreement we refer to as the Kobe Head License. Accordingly, the licenses granted to us under the Bio Palette License Agreement are subject to the terms and conditions set forth in the Kobe Head License, which include provisions providing for certain rights to be retained by third parties including governmental authorities.

We and Bio Palette are both permitted to sublicense the licensed patents to affiliates and third parties, provided that the applicable terms of the Bio Palette License Agreement and the Kobe Head License would apply to such affiliates and third parties. The sublicensing party is also responsible for any breaches of such terms by the applicable sublicensee and is responsible for all payments due under the Bio Palette License Agreement by operation of any such sublicense.

Upon the execution of the Bio Palette License Agreement, we paid Bio Palette an upfront fee of $0.5 million. In connection with the execution of the Bio Palette License Agreement, we issued to Bio Palette 16,725 shares of our common stock, with an agreement to issue additional shares of our common stock in the low six figures in the event that the referenced Bio Palette patent issues in the United States. Upon the issuance of a certain Bio Palette patent in the United States in June 2020, we made a milestone payment to Bio Palette of $2.0 million and, in July 2020, issued to Bio Palette 175,000 shares of our common stock valued at $0.3 million. We also agreed to pay a royalty at a fraction of a percent on net sales of products that are covered by the patents licensed by Bio Palette to us, and Bio Palette agreed to pay a royalty at a fraction of a percent on net sales of products that are covered by the patents licensed by us to Bio Palette. The royalty term for a product in a country will terminate on the later of the expiration of (i) patent-based exclusivity with respect to such licensed product in such country or (ii) regulatory exclusivity with respect to such licensed product in such country.

Any intellectual property arising out of activities under the Bio Palette License Agreement will be owned by the party inventing such intellectual property. Bio Palette is responsible for the prosecution and maintenance of all patents licensed by Bio Palette to us, provided that we have customary consultation, comment and review rights with respect to such prosecution and maintenance activities solely with respect to national entries of a certain specified PCT application. We have the sole right to prosecute and maintain patents licensed by us to Bio Palette.

Unless earlier terminated, the Bio Palette License Agreement will expire on a licensed product-by-licensed product and country-by-country basis upon the expiration of the applicable royalty term for each such licensed product and country. Each party has the right to terminate the Bio Palette License Agreement for convenience with respect to the license granted to such party subject to a specified notice period. Either party may terminate the Bio Palette License Agreement with respect to the license granted to the other party for a material breach by the other party, subject to a specified notice and cure period. Additionally, either party may also terminate the Bio Palette License Agreement in the event of the other party's bankruptcy or insolvency or if the other party, its affiliates or sublicensees brings a patent challenge relating to any licensed patents (but, in the case of such a patent challenge by a sublicensee, subject to a cure period for such party to terminate its agreement with the sublicensee that has taken the applicable action).

Standby License Agreement with Kobe University and Bio Palette

We are party to a standby license agreement, or the Standby License Agreement, with Kobe University and Bio Palette, dated February 6, 2026. Under the terms of the Standby License Agreement, if the Kobe Head License terminates for any reason other than (i) a termination by Kobe University due to Bio Palette's default of the Kobe Head License, which default by Bio Palette is a result of our material breach of the Bio Palette License Agreement, or (ii) a termination after the execution of a new license agreement for the Kobe Patents in connection with a change of control transaction involving Bio Palette, which new license agreement includes a standby license in favor of us of the same scope set forth in the Standby License Agreement, Kobe University grants, as of the effective time of such termination, or the Effective Time, directly to us, an exclusive (even as to Kobe University and its affiliates) license to practice the Kobe Patents of the same scope as the license granted to us pursuant to the Bio Palette License Agreement. As financial consideration for the Standby License Agreement, after the Effective Time we will pay Kobe University amounts that subsequently become payable by us to Bio Palette pursuant to Section 4.4 of the Bio Palette License Agreement.

Government regulation

Government authorities in the United States, at the federal, state and local level, and in other countries and jurisdictions, including the European Union, or EU, extensively regulate, among other things, the research, development, testing, manufacturing, packaging, labeling, storage, record keeping, reimbursement, advertising, promotion, distribution, post-approval monitoring and reporting and import and export, pricing and reimbursement of pharmaceutical products, including biological products. The failure to comply with the applicable regulatory requirements at any time during the product development process or post-approval may subject a sponsor for marketing approval to delays in development or approval, as well as administrative and judicial sanctions. The regulatory requirements applicable to product development, approval and marketing are subject to change, and regulations and administrative guidance often are revised or reinterpreted by government agencies in ways that may have a significant impact on our business.

The processes for obtaining marketing approvals in the United States and in foreign countries and jurisdictions and compliance with applicable statutes and regulatory requirements, both pre- and post-approval, and obtaining reimbursement status will continue to require the expenditure of substantial time and financial resources. The regulatory requirements applicable to biological product development, approval, and marketing are subject to change, and regulations and administrative guidance often are revised or reinterpreted by the agencies in ways that may have a significant impact on our business. Ethical, social and legal concerns about gene therapy, genetic testing and genetic research could result in additional regulations restricting or prohibiting the processes we may use.

Licensure and regulation of biologics in the United States

In the United States, our candidate products are regulated as biological products, or biologics, under the Public Health Service Act, or the PHSA, and the Federal Food, Drug and Cosmetic Act, or the FDCA, the implementing regulations of the FDA and other federal, state and local statutes and regulations.

The FDA must approve a product candidate for a therapeutic indication before it may be marketed in the United States. A company, institution, or organization which takes responsibility for the initiation and management of a clinical development program for such products is referred to as a sponsor. A sponsor seeking approval to market and distribute a new biologic in the United States generally must satisfactorily complete each of the following steps:

- preclinical laboratory tests, animal studies and formulation studies all performed in accordance with the FDA's Good Laboratory Practice, or GLP, regulations;
- completion of the manufacture, under cGMP conditions, of the drug substance and drug product that the sponsor intends to use in human clinical trials along with required analytical and stability testing;
- design of a clinical protocol and submission to the FDA of an investigational new drug, or IND, application for human clinical testing, which must become effective before human clinical trials may begin;
- approval by an independent institutional review board, or IRB, representing each clinical site before each clinical trial may be initiated;
- performance of adequate and well-controlled human clinical trials to establish the safety, potency, and purity of the product candidate for each proposed indication, in accordance with current Good Clinical Practices, or GCP;
- preparation and submission to the FDA of a BLA requesting marketing of the biological product for one or more proposed indications, including submission of detailed information on the manufacture and composition of the product and proposed labelling;
- review of the BLA by an FDA advisory committee, where applicable;
- satisfactory completion of one or more FDA inspections of the manufacturing facility or facilities, including those of third parties, at which the product, or components thereof, are produced to assess compliance with cGMP requirements; to assure that the facilities, methods, and controls are adequate to preserve the product's identity, strength, quality, and

purity; and, if applicable, the FDA's current Good Tissue Practice, or cGTP, requirements for the use of human cellular and tissue products;

- satisfactory completion of any FDA audits of the non-clinical and clinical trial sites to assure compliance with GLPs and GCPs and the integrity of clinical data in support of the BLA;
- payment of the application fee under the Prescription Drug User Free Act, or PDUFA, unless exempted; and
- FDA review and approval of the BLA, which may be subject to additional post-approval requirements, including the potential requirement to implement a Risk Evaluation and Mitigation Strategy, or REMS, and any post-approval studies required by the FDA.

Preclinical studies and investigational new drug application

Before testing any investigational biological product in humans, including a gene editing product candidate, the product candidate must undergo preclinical testing. Preclinical tests include laboratory evaluations of product chemistry, formulation and stability, as well as studies to evaluate the potential for efficacy and toxicity in animal studies. These studies are generally referred to as IND-enabling studies. The conduct of the preclinical tests and formulation of the compounds for testing must comply with federal regulations and requirements, including GLP regulations and standards and the United States Department of Agriculture's Animal Welfare Act, if applicable. With the passage of the FDA's Modernization Act 2.0 in December 2022, Congress eliminated provisions in both the FDCA and the Public Health Service Act, or PHSA, that required animal testing in support of an NDA or BLA. While animal testing may still be conducted, the FDA was authorized to rely on alternative non-clinical tests, including cell-based assays, microphysiological systems, or bioprinted or computer models. In April 2025, the FDA released a roadmap to replace animal testing in preclinical safety studies with scientifically validated new approach methodologies. The results of the preclinical tests, together with manufacturing information and analytical data, are submitted to the FDA as part of an IND application.

An IND is an exemption from the FDCA that allows an unapproved drug or biological product to be shipped in interstate commerce for use in an investigational clinical trial. Such authorization must be secured prior to interstate shipment and administration of any product candidate that is not the subject of an approved new drug application, or NDA. In support of a request for an IND, applicants must submit a protocol for each clinical trial and any subsequent protocol amendments must be submitted to the FDA as part of the IND. An IND automatically becomes effective 30 days after receipt by the FDA, unless before that time the FDA raises concerns or questions about the product or conduct of the proposed clinical trial, including concerns that human research subjects will be exposed to unreasonable health risks or any issues surrounding chemistry, manufacturing and controls, or CMC, for the proposed product. In that case, the IND sponsor and the FDA must resolve any outstanding FDA concerns before the clinical trials can begin. Preclinical or nonclinical testing typically continues even after the IND is submitted.

Following commencement of a clinical trial under an IND, the FDA may also place a clinical hold or partial clinical hold on that trial. A clinical hold is an order issued by the FDA to the sponsor to delay a proposed clinical investigation or to suspend an ongoing investigation. A partial clinical hold is a delay or suspension of only part of the clinical work requested under the IND. For example, a partial clinical hold might state that a specific protocol or part of a protocol may not proceed, while other parts of a protocol or other protocols may do so. No more than 30 days after the imposition of a clinical hold or partial clinical hold, the FDA will provide the sponsor a written explanation of the basis for the hold. Following the issuance of a clinical hold or partial clinical hold, a clinical investigation may only resume once the FDA has notified the sponsor that the investigation may proceed. The FDA will base that determination on information provided by the sponsor correcting the deficiencies previously cited or otherwise satisfying the FDA that the investigation can proceed or recommence.

Expanded access to an investigational drug for treatment use

Expanded access, sometimes called "compassionate use," is the use of investigational products outside of clinical trials to treat patients with serious or immediately life-threatening diseases or conditions when there are no comparable or satisfactory alternative treatment options. FDA regulations allow access to investigational products under an IND by the company or the treating physician for treatment purposes on a case-by-case basis for: individual patients (single-patient IND applications for treatment in emergency settings and non-emergency settings); intermediate-size patient populations; and larger populations for use of the investigational product under a treatment protocol or treatment IND application.

There is no requirement for a manufacturer to provide expanded access to an investigational product. However, if a manufacturer decides to make its investigational product available for expanded access, FDA reviews requests for expanded access and determines if treatment may proceed. Expanded access may be appropriate when all of the following criteria apply: patient(s) have a serious or immediately life-threatening disease or condition, and there is no comparable or satisfactory alternative therapy to diagnose, monitor, or treat the disease or condition; the potential patient benefit justifies the potential risks of the treatment and the potential risks are not unreasonable in the context or condition to be treated; and the expanded use of the investigational drug for the requested treatment will not interfere with initiation, conduct, or completion of clinical investigations that could support marketing approval of the product or otherwise compromise the potential development of the product.

Under the FDCA, sponsors of one or more investigational products for the treatment of a serious disease(s) or condition(s) must make publicly available their policy for evaluating and responding to requests for expanded access for individual patients. Sponsors are required to make such policies publicly available upon the earlier of initiation of a Phase 2 or Phase 3 study; or 15 days after the investigational drug or biologic receives designation as a breakthrough therapy, fast track product, or regenerative medicine advanced therapy.

In addition, on May 30, 2018, the Right to Try Act was signed into law. The law, among other things, provides an additional mechanism for patients with a life-threatening condition who have exhausted approved treatments and are unable to participate in clinical trials to access certain investigational products that have completed a Phase I clinical trial, are the subject of an active IND, and are undergoing investigation for FDA approval. Unlike the expanded access framework described above, the Right to Try Pathway does not require FDA to review or approve requests for use of the investigational product. There is no obligation for a manufacturer to make its investigational products available to eligible patients under the Right to Try Act.

Human clinical trials in support of a BLA

Clinical trials involve the administration of the investigational product candidate to healthy volunteers or patients with the disease to be treated under the supervision of qualified principal investigators, generally physicians not employed by or under the trial sponsor's control, in accordance with GCP requirements, which include the requirement that all research subjects provide their informed consent for their participation. Clinical trials are conducted under study protocols detailing, among other things, the objectives of the study, inclusion and exclusion criteria, the parameters to be used in monitoring safety, and the effectiveness criteria to be evaluated. A protocol for each clinical trial and any subsequent protocol amendments must be submitted to the FDA as part of the IND.

For clinical trials conducted in the United States, an IND is required, and each clinical trial must be reviewed and approved by an IRB either centrally or individually at each institution at which the clinical trial will be conducted. The IRB will consider, among other things, clinical trial design, patient informed consent, ethical factors, the safety of human subjects, and the possible liability of the institution. An IRB must operate in compliance with FDA regulations. Clinical trials must also comply with extensive GCP rules and the requirements for obtaining subjects' informed consent. The FDA, IRB, or the clinical trial sponsor may suspend or discontinue a clinical trial at any time for various reasons, including a finding that the clinical trial is not being conducted in accordance with FDA requirements, including GCP, or the subjects or patients are being exposed to an unacceptable health risk.

Additionally, some clinical trials are overseen by an independent group of qualified experts organized by the clinical trial sponsor, known as a data monitoring committee, or DMC. This group may recommend continuation of the study as planned, changes in study conduct, or cessation of the study at designated checkpoints based on access to certain data from the study. Finally, research activities involving infectious agents, hazardous chemicals, recombinant DNA, and genetically altered organisms and agents may be subject to review and approval of an Institutional Biosafety Committee, or IBC, in accordance with NIH Guidelines for Research Involving Recombinant or Synthetic Nucleic Acid Molecules.

Clinical trials typically are conducted in three sequential phases, but the phases may overlap or be combined. Additional studies may be required after approval.

- *Phase 1* clinical trials are initially conducted in a limited population to test the product candidate for safety, including adverse effects, dose tolerance, absorption, metabolism, distribution, excretion, and pharmacodynamics in healthy humans or, on occasion, in the case of some products for severe or life-threatening diseases, especially when the product may be too inherently toxic to ethically administer to healthy volunteers, in patients, such as cancer patients.
- *Phase 2* clinical trials are generally conducted in a limited patient population to identify possible adverse effects and safety risks, evaluate the efficacy of the product candidate for specific targeted indications and determine dose tolerance and optimal dosage. Multiple Phase 2 clinical trials may be conducted by the sponsor to obtain information prior to beginning larger and more costly Phase 3 clinical trials.
- *Phase 3* clinical trials proceed if the Phase 2 clinical trials demonstrate that a dose range of the product candidate is potentially effective and has an acceptable safety profile. Clinical trials are undertaken within an expanded patient population at multiple geographically dispersed clinical study sites to further evaluate dosage, provide substantial evidence of clinical efficacy, and further test for safety. A well-controlled, statistically robust Phase 3 trial may be designed to deliver the data that regulatory authorities will use to decide whether or not to approve, and, if approved, how to appropriately label a biologic; such Phase 3 studies are referred to as "pivotal."

A clinical trial may combine the elements of more than one phase and the FDA often requires more than one Phase 3 trial to support marketing approval of a product candidate. A company's designation of a clinical trial as being of a particular phase is not necessarily indicative that the study will be sufficient to satisfy the FDA requirements of that phase because this determination cannot be made until the protocol and data have been submitted to and reviewed by the FDA. Moreover, as noted above, a pivotal trial is a clinical trial that is believed to satisfy FDA requirements for the evaluation of a product candidate's safety, purity and potency such that it can be used, alone or with other pivotal or non-pivotal trials, to support regulatory approval. Generally, pivotal trials are Phase 3 trials, but

they may be Phase 2 trials if the design provides a well-controlled and reliable assessment of clinical benefit, particularly in an area of unmet medical need.

In some cases, the FDA may approve a BLA for a product candidate but require the sponsor to conduct additional clinical trials to further assess the product candidate's safety or effectiveness after approval. Such trials are typically referred to as post approval or post marketing clinical trials. These studies are used to gain additional experience from the treatment of patients in the intended therapeutic indication and to document a clinical benefit in the case of biologics approved under accelerated approval regulations. Failure to exhibit due diligence with regard to conducting post approval or post marketing clinical trials could result in withdrawal of approval for products. The FDA generally recommends that sponsors observe subjects for potential gene-therapy related delayed adverse events in a long-term follow-up study of fifteen years for integrating vectors, up to fifteen years for herpes virus vectors capable of establishing latency, up to fifteen years for microbial vectors known to establish persistent infection, up to fifteen years for gene editing products, and up to five years for AAV vectors. The FDA recommends that these long-term follow-up studies include, at a minimum, five years of annual physical examinations followed by annual queries, either in-person or by phone or written questionnaire, for the remaining observation period.

In December 2022, with the passage of Food and Drug Omnibus Reform Act, or FDORA, Congress required sponsors to develop and submit a diversity action plan, or DAP, for each Phase 3 clinical trial or any other "pivotal study" of a new drug or biological product. These plans are meant to encourage the enrollment of more diverse patient populations in late-stage clinical trials of FDA-regulated products. Specifically, actions plans must include the sponsor's goals for enrollment, the underlying rationale for those goals, and an explanation of how the sponsor intends to meet them. In addition to these requirements, the legislation directs the FDA to issue new guidance on diversity action plans. In June 2024, as mandated by FDORA, the FDA issued draft guidance outlining the general requirements for DAPs. Unlike most guidance documents issued by the FDA, the DAP guidance when finalized will have the force of law, because FDORA specifically dictates that the form and manner for submission of DAPs are specified in FDA guidance. On January 27, 2025, in response to an Executive Order issued by President Trump on January 21, 2025, on Diversity, Equity and Inclusion programs, the FDA removed this draft guidance from its website. Subsequently, in July 2025, pursuant to a court order, the FDA restored the draft DAP guidance to its website with a statement that "information on this page may be modified and/or removed in the future subject to the terms of the court's order and implemented consistent with applicable law." Accordingly, in light of these ongoing actions, there is considerable uncertainty surrounding the draft DAP guidance and how the FDA will consider DAPs in connection with its review of BLAs.

In September 2025, the FDA issued final guidance with updated recommendations for GCPs aimed at modernizing the design and conduct of clinical trials. The updates are intended to help pave the way for more efficient clinical trials to facilitate the development of medical products. The final guidance is adopted from the International Council for Harmonisation's, or ICH, recently updated E6(R3) final guideline that was developed to enable the incorporation of rapidly developing technological and methodological innovations into the clinical trial enterprise. In addition, the FDA issued final guidance outlining recommendations for the implementation of decentralized clinical trials.

In October 2025, the FDA issued final guidance that focuses on patient-focused drug development. The guidance outlines how stakeholders, such as patients, caregivers, researchers and medical product developers, can submit patient experience data in support of the development and approval of drug products. To that end, the guidance provides an overview of clinical outcome assessments, or COAs, in clinical trials, and the role that COAs may play in evaluating the clinical benefit of a medical product.

Progress reports detailing the results of clinical trials must be submitted at least annually to the FDA and more frequently if serious adverse events occur. In addition, IND safety reports must be submitted to the FDA for any of the following: serious and unexpected suspected adverse reactions; findings from other studies or animal or *in vitro* testing that suggest a significant risk in humans exposed to the product; and any clinically important increase in the occurrence of a serious suspected adverse reaction over that listed in the protocol or investigator brochure. Phase 1, Phase 2 and Phase 3 clinical trials may not be completed successfully within any specified period, or at all. The FDA will typically inspect one or more clinical sites to assure compliance with GCP and the integrity of the clinical data submitted.

Sponsors of clinical trials of certain FDA-regulated products, including prescription drugs, are required to register and disclose certain clinical trial information on a public registry maintained by the NIH. In particular, information related to the product, patient population, phase of investigation, study sites and investigators and other aspects of the clinical trial is made public as part of the registration of the clinical trial. Although sponsors are also obligated to disclose the results of their clinical trials after completion, disclosure of the results can be delayed in some cases for up to two years after the date of completion of the trial. The NIH's Final Rule on ClinicalTrials.gov registration and reporting requirements became effective in 2017. As of January 31, 2026, the FDA has issued eight notices of non-compliance, thereby signaling the government's willingness to begin enforcing these requirements against non-compliant clinical trial sponsors. While these notices did not result in civil monetary penalties, the failure to submit clinical trial information to clinicaltrials.gov is a prohibited act under the FDCA with violations subject to potential civil monetary penalties of up

to $10,000 for each day the violation continues. Violations may also result in injunctions and/or criminal prosecution or disqualification from federal grants.

Interactions with the FDA during the clinical development program

Following the clearance of an IND and the commencement of clinical trials, a sponsor is given the opportunity to meet with the FDA at certain points in the clinical development program. There are five types of meetings that occur between sponsors and the FDA. Type A meetings are those that are necessary for an otherwise stalled product development program to proceed or to address an important safety issue. Type B meetings include pre-IND and pre-NDA meetings as well as end of phase meetings such as end of Phase 2 meetings. A Type C meeting is any meeting other than a Type A or Type B meeting regarding the development and review of a product. A Type D meeting is focused on a narrow set of issues and should not require input from more than three disciplines or Divisions of FDA. Finally, INTERACT meetings are intended for novel products and development programs that present unique challenges in the early development of an investigational product. The FDA has indicated that its responses, as conveyed in meeting minutes and advice letters, only constitute mere recommendations and/or advice made to a sponsor and, as such, sponsors are not bound by such recommendations and/or advice. Nonetheless, from a practical perspective, a sponsor's failure to follow the FDA's recommendations for design of a clinical program may put the program at significant risk of failure.

Clinical studies outside the United States in support of FDA approval

In connection with our clinical development program, we are planning to conduct trials at sites outside the United States. When a foreign clinical trial is conducted under an IND, all IND requirements must be met unless waived. When a foreign clinical trial is not conducted under an IND, the sponsor must ensure that the trial complies with certain regulatory requirements of the FDA in order to use the trial as support for an IND or application for marketing approval. Specifically, the trials must be conducted in accordance with GCP, including undergoing review and receiving approval by an independent ethics committee, or IEC, and seeking and receiving informed consent from subjects. GCP requirements encompass both ethical and data integrity standards for clinical trials. The FDA's regulations are intended to help ensure the protection of human subjects enrolled in non-IND foreign clinical trials, as well as the quality and integrity of the resulting data. They further help ensure that non-IND foreign trials are conducted in a manner comparable to that required for IND trials.

The acceptance by the FDA of trial data from clinical trials conducted outside the United States in support of U.S. approval may be subject to certain conditions or may not be accepted at all. In cases where data from foreign clinical trials are intended to serve as the sole basis for marketing approval in the U.S., the FDA will generally not approve the application on the basis of foreign data alone unless (i) the data are applicable to the U.S. population and U.S. medical practice; (ii) the trials were performed by clinical investigators of recognized competence and pursuant to GCP regulations; and (iii) the data may be considered valid without the need for an on-site inspection by the FDA, or if the FDA considers such inspection to be necessary, the FDA is able to validate the data through an on-site inspection or other appropriate means.

In addition, even where the foreign trial data are not intended to serve as the sole basis for approval, the FDA will not accept the data as support for an application for marketing approval unless the trial is well-designed and well-conducted in accordance with GCP requirements and the FDA is able to validate the data from the trial through an onsite inspection if deemed necessary. Many foreign regulatory authorities have similar approval requirements. In addition, such foreign trials are subject to the applicable local laws of the foreign jurisdictions where the trials are conducted.

Pediatric studies

Under the Pediatric Research Equity Act of 2003, or PREA, a BLA or supplement thereto must contain data that are adequate to assess the safety and effectiveness 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. Sponsors must submit a pediatric study plan, or PSP, within 60 days of an end-of-Phase 2 meeting or as may be agreed between the sponsor and the FDA. The PSP outlines the proposed pediatric study or studies they plan to conduct, including study objectives and design, any deferral or waiver requests, and other information required by regulation. The FDA must then review the information submitted, consult with the sponsor, and agree upon a final plan. The FDA or the sponsor may request an amendment to the plan at any time. In May 2023, the FDA issued new draft guidance that further describes the pediatric study requirements under PREA.

For investigational products intended to treat a serious or life-threatening disease or condition, the FDA must, upon the request of a sponsor, meet to discuss preparation of the initial pediatric study plan or to discuss deferral or waiver of pediatric assessments. In addition, FDA will meet early in the development process to discuss pediatric study plans with sponsors and FDA must meet with sponsors by no later than the end-of-phase 1 meeting for serious or life-threatening diseases and by no later than 90 days after FDA's receipt of the study plan. The FDA may, on its own initiative or at the request of the sponsor, grant deferrals for submission of some or all pediatric data until after approval of the product for use in adults, or full or partial waivers from the pediatric data requirements, under specified circumstances. Unless otherwise required by regulation, the pediatric data requirements do not apply to products with orphan designation.

The FDA may, on its own initiative or at the request of the sponsor, grant deferrals for submission of some or all pediatric data until after approval of the product for use in adults, or full or partial waivers from the pediatric data requirements. A deferral may be granted for several reasons, including a finding that the product or therapeutic candidate is ready for approval for use in adults before pediatric trials are complete or that additional safety or effectiveness data needs to be collected before the pediatric trials begin. The law now requires the FDA to send a PREA Non-Compliance letter to sponsors who have failed to submit their pediatric assessments required under PREA, have failed to seek or obtain a deferral or deferral extension or have failed to request approval for a required pediatric formulation. It further requires the FDA to publicly post the PREA Non-Compliance letter and sponsor's response. Unless otherwise required by regulation, the pediatric data requirements do not apply to products with orphan designation, although FDA has recently taken steps to limit what it considers abuse of this statutory exemption in PREA by announcing that it does not intend to grant any additional orphan drug designations for rare pediatric subpopulations of what is otherwise a common disease. The FDA also maintains a list of diseases that are exempt from PREA requirements due to low prevalence of disease in the pediatric population. In May 2023, the FDA issued new draft guidance that further describes the pediatric study requirements under PREA.

Special regulations and guidance governing gene therapy products

It is possible that the procedures and standards applied to gene therapy products and cell therapy products may be applied to any CRISPR/Cas9 product candidates we may develop, but that remains uncertain at this point. The FDA has defined a gene therapy product as one that mediates its effects by transcription and/or translation of transferred genetic material and/or by integrating into the host genome and which are administered as nucleic acids, viruses, or genetically engineered microorganisms. The products may be used to modify cells *in vivo* or be transferred to cells *ex vivo* prior to administration to the recipient. The Center for Biologics Evaluation and Research, or CBER, at FDA regulates gene therapy products. Within CBER, the review of gene therapy and related products is consolidated in the Office of Therapeutic Products, or OTP, and the FDA has established the Cellular, Tissue and Gene Therapies Advisory Committee to advise CBER on its reviews.

The FDA has issued numerous guidance documents regarding gene therapies. Although the FDA's guidance documents are not legally binding, compliance with certain aspects of them is likely necessary to gain approval for any product candidate we may develop. The guidance documents provide recommendations and additional clarity as to factors that the FDA will consider at each stage of gene therapy development and relate to, among other things, the proper preclinical assessment of gene therapies; the CMC information that should be included in an IND application; the proper design of tests to measure product potency in support of an IND or BLA application; measures to observe delayed adverse effects in subjects who have been exposed to investigational gene therapies; and gene therapy products for the treatment of rare diseases. In addition, in November 2024, the FDA issued draft guidance to address frequently asked questions surrounding the development of cellular and gene therapy products.

If a gene therapy trial is conducted at, or sponsored by, institutions receiving any NIH funding for research involving recombinant or synthetic nucleic acid molecules, the trial must be conducted in accordance with the NIH Guidelines for Research Involving Recombinant DNA Molecules. Research conducted at such institutions that involves the transfer of recombinant or synthetic nucleic acid molecules, or DNA or RNA derived from recombinant or synthetic nucleic acid molecules, into human subjects must undergo review and approval by an IBC before it commences. Many companies and other institutions not otherwise subject to the NIH Guidelines voluntarily follow them.

Compliance with cGMP and cGTP requirements

The FDA's regulations require that pharmaceutical products be manufactured in specific approved facilities and in accordance with cGMPs. The cGMP regulations include requirements relating to organization of personnel, buildings and facilities, equipment, control of components and drug product containers and closures, production and process controls, packaging and labeling controls, holding and distribution, laboratory controls, records and reports and returned or salvaged products. Manufacturers and other entities involved in the manufacture and distribution of approved pharmaceuticals are required to register their establishments with the FDA and some state agencies and are subject to periodic unannounced inspections by the FDA for compliance with cGMPs and other requirements. Inspections must follow a "risk-based schedule" that may result in certain establishments being inspected more frequently.

Manufacturers may also have to provide, on request, electronic or physical records regarding their establishments. Delaying, denying, limiting, or refusing inspection by the FDA may lead to a product being deemed to be adulterated. Changes to the manufacturing process, specifications or container closure system for an approved product are strictly regulated and often require prior FDA approval before being implemented. FDA regulations also require, among other things, the investigation and correction of any deviations from cGMP and the imposition of reporting and documentation requirements upon the NDA sponsor and any third-party manufacturers involved in producing the approved product.

Before approving a BLA, the FDA typically will 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 full compliance with cGMP requirements and adequate to assure consistent production of the product within required specifications. The PHSA emphasizes the importance of manufacturing control for products like biologics whose attributes cannot be precisely defined. Material changes in manufacturing equipment, location, or process post-approval, may result in additional regulatory review and approval. The PREVENT Pandemics Act, which was enacted in December 2022, clarifies that foreign drug manufacturing establishments are subject to registration and listing requirements even if a drug or biologic undergoes further manufacture, preparation, propagation,

compounding, or processing at a separate establishment outside the United States prior to being imported or offered for import into the United States.

For a gene therapy product, the FDA also will not approve the product if the manufacturer is not in compliance with cGTP. These standards are found in FDA regulations and guidance documents that govern the methods used in, and the facilities and controls used for, the manufacture of human cells, tissues, and cellular and tissue based products, or HCT/Ps, which are human cells or tissue intended for implantation, transplant, infusion, or transfer into a human recipient. The primary intent of the GTP requirements is to ensure that cell and tissue-based products are manufactured in a manner designed to prevent the introduction, transmission, and spread of communicable disease. FDA regulations also require tissue establishments to register and list their HCT/Ps with the FDA and, when applicable, to evaluate donors through screening and testing.

Manufacturers and others involved in the manufacture and distribution of products must also register their establishments with the FDA and certain state agencies. Both domestic and foreign manufacturing establishments must register and provide additional information to the FDA upon their initial participation in the manufacturing process. Any product manufactured by or imported from a facility that has not registered, whether foreign or domestic, is deemed misbranded under the FDCA. The manufacturing facilities may be subject to periodic unannounced inspections by government authorities to ensure compliance with cGMPs and other laws. If a manufacturing facility is not in substantial compliance with the applicable regulations and requirements imposed when the product was approved, regulatory enforcement action may be taken, which may include a warning letter or an injunction against shipment of products from the facility and/or recall of products previously shipped.

In May 2025, the FDA disclosed plans to expand its use of unannounced inspections of foreign manufacturing facilities that produce drugs and biologics distributed in the United States. Subsequently, in August 2025, the FDA introduced a "PreCheck" program with the intention of supporting companies as they build new facilities in the United States. The PreCheck program provides manufacturers with more frequent FDA communication at critical development stages, including facility design, construction, and pre-production. These FDA initiatives flow from an Executive Order issued by President Trump on May 5, 2025, calling for actions to reduce regulatory barriers to pharmaceutical manufacturing in the United States.

Submission of a BLA

Assuming successful completion of the required clinical testing, the results of the preclinical studies and clinical trials, along with information relating to the product's CMC and proposed labeling, are submitted to the FDA as part of an application requesting approval to market the product candidate for one or more indications. The fee required for the submission of a BLA under the Prescription Drug User Fee Act, or PDUFA, is substantial (for example, for federal fiscal year 2026, this application fee is approximately $4.7 million), and the sponsor of an approved BLA is also subject to an annual program fee, set at $442,213 per eligible prescription drug product for federal fiscal year 2026. These fees are typically adjusted annually, and exemptions and waivers may be available under certain circumstances, such as where a waiver is necessary to protect the public health, where the fee would present a significant barrier to innovation, or where the sponsor is a small business submitting its first human therapeutic application for review.

The FDA conducts a preliminary review of the BLA within 60 days of receipt and must inform the sponsor by that time whether the application is sufficiently complete to permit substantive review. In pertinent part, the FDA's regulations for applications state that an application "shall not be considered as filed until all pertinent information and data have been received" by the FDA. In the event that the FDA determines that an application does not satisfy this standard, it will issue a Refuse to File, or RTF, determination to the sponsor. In October 2025, the FDA issued internal guidance clarifying that "materially incomplete or inadequately organized" applications that would not permit timely, efficient and complete review will be subjects of RTFs. The internal guidance also provides that the FDA will issue an RTF for an application that relies on a single adequate and well-controlled investigation to support approval if prior communications with the FDA determined the need for more than one clinical study and any justification for a single investigation is inadequate. The FDA may request additional information rather than accept a BLA for filing. In this event, the application must be resubmitted with the additional information. The resubmitted application is also subject to review before the FDA accepts it for filing.

After the submission is accepted for filing, the FDA begins an in-depth substantive review of the application. The FDA may inform the sponsor of certain requirements for information when it accepts the BLA or by the 74th day of the receipt of the BLA. Thereafter, the FDA may submit "information requests" to the sponsor in the course of the FDA's review of the BLA. The FDA reviews the BLA to determine, among other things, whether the proposed product is safe and effective for its intended use, whether it has an acceptable purity profile and whether the product is being manufactured in accordance with cGMP. Under the goals and policies agreed to by the FDA under PDUFA, the FDA has ten months from the filing date in which to complete its initial review of a standard application for an investigational product that is a new molecular entity, and six months from the filing date for an application with "priority review." The review process may be extended by the FDA for three additional months to consider new information or in the case of a clarification provided by the sponsor to address an outstanding deficiency identified by the FDA following the original submission. Despite these review goals, it is not uncommon for FDA review of a BLA to extend beyond the PDUFA goal date.

Before approving a BLA, the FDA may inspect the sponsor and one or more clinical trial sites to assure compliance with IND and GCP requirements and the integrity of the clinical data submitted to the FDA. With passage of FDORA, Congress clarified the FDA's

authority to conduct inspections by expressly permitting inspection of facilities involved in the preparation, conduct, or analysis of clinical and non-clinical studies submitted to FDA as well as other persons holding study records or involved in the study process.

Moreover, the FDA will review a sponsor's financial relationship with the principal investigators who conducted the clinical trials in support of the NDA. This is because under certain circumstances, principal investigators at a clinical trial site may also serve as scientific advisors or consultants to a sponsor and receive compensation in connection with such services. Depending on the level of that compensation and any other financial interest a principal investigator may have in a sponsor, the sponsor may be required to report these relationships to the FDA. The FDA will then evaluate that financial relationship and determine whether it creates a conflict of interest or otherwise affects the interpretation of the trial or the integrity of the data generated at the principal investigator's clinical trial site. If so, the FDA may exclude data from the clinical trial site in connection with its determination of safety, purity and potency of the investigational product.

Additionally, the FDA may refer the BLA, including applications for novel product candidates which present difficult questions of safety or efficacy, to an advisory committee for review, evaluation and recommendation as to whether the application should be approved and under what conditions. Typically, an advisory committee is a panel of independent experts, including clinicians and other scientific experts that reviews, evaluates and provides a recommendation as to whether the application should be approved and under what conditions. The FDA is not bound by the recommendation of an advisory committee, but it considers such recommendations when making final decisions on approval. Data from clinical trials are not always conclusive, and the FDA or its advisory committee may interpret data differently than the NDA sponsor interprets the same data. The FDA may also re-analyze the clinical trial data, which could result in extensive discussions between the FDA and the sponsor during the review process.

The FDA's decision on a BLA

The FDA reviews a BLA to determine, among other things, whether the product is safe, pure and potent. To reach this determination, the FDA must determine that the expected benefits of the proposed product outweigh its potential risks to patients. This "benefit-risk" assessment is informed by the extensive body of evidence about the product's safety, purity and potency in the BLA. This assessment is also informed by other factors, including: the severity of the underlying condition and how well patients' medical needs are addressed by currently available therapies; uncertainty about how the premarket clinical trial evidence will extrapolate to real-world use of the product in the post-market setting; and whether risk management tools are necessary to manage specific risks.

The FDA typically requires a robust safety database and substantial evidence of the efficacy of the product. The term "substantial evidence" has been interpreted by the FDA to require at least two adequate and well-controlled clinical investigations to establish effectiveness of a product. Under certain circumstances, however, the FDA has indicated that a single trial with certain characteristics and additional information may satisfy this standard. This approach was subsequently endorsed by Congress in 1998 with legislation providing, in pertinent part, that "If [the FDA] determines, based on relevant science, that data from one adequate and well-controlled clinical investigation and confirmatory evidence (obtained prior to or after such investigation) are sufficient to establish effectiveness, the FDA may consider such data and evidence to constitute substantial evidence." In December 2019, the FDA issued draft guidance further explaining the studies that are needed to establish substantial evidence of effectiveness. It has not yet finalized that guidance, but the FDA did issue draft guidance in September 2023 that outlines considerations for relying on confirmatory evidence in lieu of a second clinical trial to demonstrate efficacy. More recently, in December 2025, the FDA signaled that it is considering only requiring one clinical study for approval of most products. The FDA indicated at such time that it may issue guidance regarding this change through a press release or other means; the FDA has not yet, however, issued such guidance.

In addition, before approving an application, the FDA will determine whether the facility in which the product is manufactured, processed, packed or held meets standards designed to assure the product's continued safety. The approval process is lengthy and often difficult, and the FDA may refuse to approve a BLA if the applicable regulatory criteria are not satisfied or may require additional clinical or other data and information. After evaluating the application and all related information, including the advisory committee recommendations, if any, and inspection reports of manufacturing facilities and clinical trial sites, the FDA may issue either an approval letter or a Complete Response Letter, or CRL.

A CRL indicates that the review cycle of the application is complete, and the application will not be approved in its present form. A CRL generally outlines the deficiencies in the submission and may require substantial additional testing or information in order for the FDA to reconsider the application. The CRL may require additional clinical or other data, additional pivotal Phase 3 clinical trial(s) and/or other significant and time-consuming requirements related to clinical trials, preclinical studies or manufacturing. If a CRL is issued, the sponsor will have one year to respond to the deficiencies identified by the FDA, at which time the FDA can deem the application withdrawn or, in its discretion, grant the sponsor an additional six-month extension to respond. For those seeking to challenge the FDA's CRL decision, the FDA has indicated that sponsors may request a formal hearing on the CRL or they may file a request for reconsideration or a request for a formal dispute resolution. While CRLs were previously treated by the FDA as confidential and were only disclosed in action packages for approved products, the FDA announced in September 2025 that it will now release CRLs promptly after they are issued to sponsors. Since that announcement, the FDA has posted a number of CRLs on its website.

During the product approval process, the FDA also will determine whether a REMS is necessary to assure the safe use of the product. If the FDA concludes a REMS is needed, the sponsor of the BLA must submit a proposed REMS; the FDA will not approve the BLA without a REMS, if required.

An approval letter, on the other hand, authorizes commercial marketing of the product with specific prescribing information for specific indications. That is, the approval will be limited to the conditions of use (e.g., patient population or indication) described in the FDA-approved labeling. Further, depending on the specific risk(s) to be addressed, the FDA may require that contraindications, warnings or precautions be included in the product labeling, require that post-approval trials, including post-marketing clinical trials, be conducted to further assess a product's safety after approval, require testing and surveillance programs to monitor the product after commercialization or impose other conditions, including distribution and use restrictions or other risk management mechanisms under a REMS, which can materially affect the potential market and profitability of the product. The FDA may prevent or limit further marketing of a product based on the results of post-marketing trials or surveillance programs. After approval, some types of changes to the approved product, such as adding new indications, manufacturing changes and additional labeling claims, are subject to further testing requirements and FDA review and approval.

Fast track, breakthrough therapy, priority review and regenerative advanced therapy designations

The FDA has several programs designed to expedite the development and approval of drugs and biological products intended to treat serious or life-threatening diseases or conditions. These programs include fast track designation, breakthrough therapy designation, priority review designation, and regenerative medicine advanced therapy (RMAT) designation. These designations are not mutually exclusive, and a product candidate may qualify for one or more of these programs. While these programs are intended to expedite product development and approval, they do not alter the standards for FDA approval.

The FDA may grant a product fast track designation if it is intended for the treatment of a serious or life-threatening disease or condition, and nonclinical or clinical data demonstrate the potential to address an unmet medical need for such disease or condition. For fast track products, sponsors may have greater interactions with the FDA, and the FDA may initiate review of sections of a fast track product's marketing application before the application is complete in some circumstances. Fast track designation may be rescinded if FDA believes that the product no longer meets the qualifying criteria.

A product may be designated as a breakthrough therapy if it is intended to treat a serious or life-threatening disease or condition and preliminary clinical evidence indicates that the product may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints. The FDA may take certain actions with respect to breakthrough therapies, including holding meetings with the sponsor throughout the development process; providing timely advice to the product sponsor regarding development and approval; involving more senior staff in the review process; assigning a cross-disciplinary project lead for the review team; and taking other steps to aid sponsors in designing the clinical trials in an efficient manner. Breakthrough designation may be rescinded if a product no longer meets the qualifying criteria.

With passage of the 21st Century Cures Act in December 2016, Congress authorized an additional expedited program for regenerative medicine advanced therapies. A product is eligible for 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 has the potential to address unmet medical needs for such disease or condition. The benefits of RMAT designation include the benefits available to breakthrough therapies, including potential eligibility for priority review and accelerated approval based on surrogate or intermediate endpoints. RMAT designation may be rescinded if a product no longer meets the qualifying criteria.

FDA may designate a product for priority review if it is a product that treats a serious condition and, if approved, would provide a significant improvement in safety or effectiveness of the treatment, prevention, or diagnosis of such condition. A priority designation is intended to direct overall attention and resources to the evaluation of such applications, and it shortens the FDA's goal for taking action on a marketing application from ten months to six months.

On June 17, 2025, the FDA announced the creation of a new voucher program to expedite the development and approval of new drug products. Vouchers issued under the new program, which is known as the Commissioner's National Priority Voucher, or CNPV, Program, may reportedly be redeemed by sponsors to shorten the review time of an NDA from approximately 10-12 months to 1-2 months. The FDA has indicated that the new CNPV process will convene experts from the FDA's offices for a team-based review rather than using the standard review system of a drug application being sent to numerous FDA offices. Clinical information will be reviewed by a multidisciplinary team of physicians and scientists who will pre-review the submitted information and convene for a one-day meeting. Vouchers under this program will reportedly be given to companies aligned with U.S. national priorities. As with the FDA's other programs for expediting review and approval of new drug products, there is no guarantee it would result in approval of our marketing applications or that such approval, if granted, would be on an expedited basis.

In September 2025, the FDA introduced a framework intended to streamline the approval of new therapies for ultrarare diseases. The Rare Disease Evidence Principles, or RDEP, is intended to allow sponsors to rely on a single-arm trial in support of approval of drugs and biologics that treat rare diseases with very small patient populations and where the disease is linked to a known genetic defect and characterized by progressive functional deterioration leading to disability or death in a short period of time. The targeted diseases should also lack adequate alternative therapies.

Accelerated approval pathway

The FDA may grant accelerated approval to a product for a serious or life-threatening condition that provides meaningful therapeutic advantage to patients over existing treatments based upon a determination that the product has an effect on a surrogate endpoint that is reasonably likely to predict clinical benefit. The FDA may also grant accelerated approval for such a condition when the product has an effect on an intermediate clinical endpoint that can be measured earlier than an effect on irreversible morbidity or mortality, or IMM, and 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.

The accelerated approval pathway is most often used in settings in which the course of a disease is long, and an extended period of time is required to measure the intended clinical benefit of a product, even if the effect on the surrogate or intermediate clinical endpoint occurs rapidly. Thus, accelerated approval has been used extensively in the development and approval of products for treatment of a variety of cancers in which the goal of therapy is generally to improve survival or decrease morbidity and the duration of the typical disease course requires lengthy and sometimes large trials to demonstrate a clinical or survival benefit.

For drugs granted accelerated approval, FDA generally requires sponsors to conduct, in a diligent manner, additional post-approval confirmatory studies to verify and describe the product's clinical benefit. Failure to conduct required post-approval studies with due diligence, failure to confirm a clinical benefit during the post-approval studies, or dissemination of false or misleading promotional materials would allow the FDA to withdraw the product approval on an expedited basis. All promotional materials for product candidates approved under accelerated approval are subject to prior review by the FDA unless FDA informs the sponsor otherwise.

With passage of the FDORA in December 2022, Congress modified certain provisions governing accelerated approval of drug and biologic products. Specifically, the new legislation authorized the FDA to require (i) a sponsor to have its confirmatory clinical trial underway before accelerated approval is awarded; (ii) a sponsor of a product granted accelerated approval to submit progress reports on its post-approval studies to FDA every six months (until the study is completed); and (iii) use expedited procedures to withdraw accelerated approval of an NDA or BLA after the confirmatory trial fails to verify the product's clinical benefit. Further, FDORA requires the agency to publish on its website "the rationale for why a post-approval study is not appropriate or necessary" whenever it decides not to require such a study upon granting accelerated approval.

In March 2023, the FDA issued draft guidance that outlines its current thinking and approach to accelerated approval. The FDA indicated that the accelerated approval pathway is commonly used for approval of oncology drugs due to the serious and life-threatening nature of cancer. Although single-arm trials have been commonly used to support accelerated approval, a randomized controlled trial is the preferred approach as it provides a more robust efficacy and safety assessment and allows for direct comparisons to an available therapy. To that end, the FDA outlined considerations for designing, conducting, and analyzing data for trials intended to support accelerated approvals of oncology therapeutics. Subsequently, in December 2024 and January 2025, the FDA issued additional draft guidances relating to accelerated approval. These guidances describe the FDA's latest thinking on what it means to conduct a confirmatory trial with due diligence and how the agency plans to interpret whether such a study needs to be underway at the time of approval. While these guidances are currently only in draft form and will ultimately not be legally binding even when finalized, sponsors typically observe the FDA's guidance closely to ensure that their investigational products qualify for accelerated approval.

Post-approval regulation

If regulatory approval for marketing of a product or new indication for an existing product is obtained, the sponsor will be required to comply with all regular post-approval regulatory requirements as well as any post-approval requirements that the FDA has imposed as part of the approval process. The sponsor will be required to report certain adverse reactions and production problems to the FDA, provide updated safety, purity and potency information and comply with requirements concerning advertising and promotional labeling requirements. Manufacturers and certain of 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 ongoing regulatory requirements, including cGMP regulations, which impose certain procedural and documentation requirements upon manufacturers. Accordingly, the sponsor and its third-party manufacturers must continue to expend time, money and effort in the areas of production and quality control to maintain compliance with cGMP regulations and other regulatory requirements.

A product may also be subject to official lot release, meaning that the manufacturer is required to perform certain tests on each lot of the product before it is released for distribution. If the product is subject to official lot release, the manufacturer must submit samples of each lot, 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, to the FDA. The FDA may in addition perform certain confirmatory tests on lots of some products before releasing the lots for distribution. Finally, the FDA will conduct laboratory research related to the safety, purity, potency and effectiveness of pharmaceutical products.

Once an approval is granted, the FDA may withdraw the approval if compliance with regulatory requirements and standards is not maintained or if problems occur after the product reaches the market. Later discovery of previously unknown problems with a product, including 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 trials to assess new safety risks; or imposition of distribution or other restrictions under a REMS program. Other potential consequences include, among other things:

- restrictions on the marketing or manufacturing of the product, complete withdrawal of the product from the market or product recalls;
- safety alerts, Dear Healthcare Provider letters, press releases or other communications containing warnings or other safety information about a product;
- mandated modification of promotional materials and labeling and issuance of corrective information;
- fines, warning letters or holds on post-approval clinical trials;
- refusal of the FDA to approve pending applications or supplements to approved applications, or suspension or revocation of product license approvals;
- product recall, seizure or detention, or refusal to permit the import or export of products;
- injunctions or the imposition of civil or criminal penalties; and
- consent decrees, corporate integrity agreements, debarment, or exclusion from federal health care programs.

The FDA strictly regulates the advertising and labeling of prescription drug products, including biological products. This regulation includes, among other things, standards and regulations for direct-to-consumer advertising, communications regarding unapproved uses, industry-sponsored scientific and educational activities and promotional activities involving the Internet and social media. Promotional claims about a drug's safety or effectiveness are prohibited before the drug is approved. In addition, the sponsor of an approved drug in the United States may not promote that drug for unapproved, or off-label, uses, although a physician may prescribe a drug for an off-label use in accordance with the practice of medicine. It may be permissible, under very specific, narrow conditions, for a manufacturer to engage in nonpromotional, non-misleading communication regarding off-label information, such as distributing scientific or medical journal information.

On September 9, 2025, President Trump issued a memorandum directing HHS to ensure transparency and accuracy in direct-to-consumer, or DTC, prescription drug advertising, including by increasing the amount of information regarding any risks associated with the use of any such prescription drug required to be provided in prescription drug advertisements. To that end, the FDA announced that it is initiating a rulemaking process "to eliminate the 'adequate provision' loophole that allows pharmaceutical advertisements to hide safety information by placing it in another format or location." In this context, the FDA declared that it will no longer tolerate what it characterized as "deceptive practices" in prescription drug advertising and that the FDA would "aggressively deploy" its available enforcement tools, with "heightened scrutiny" of fair balance and disclosures in social media promotions. The FDA also issued a generic "notice letter" directing companies to "remove any noncompliant advertising and bring all promotional communications into compliance."

Moreover, with passage of the Pre-Approval Information Exchange Act, or PIE Act, in December 2022, sponsors of products that have not been approved may proactively communicate to payors certain information about products in development to help expedite patient access upon product approval. Previously, such communications were permitted under FDA guidance but the new legislation explicitly provides protection to sponsors who convey certain information about products in development to payors, including unapproved uses of approved products. In addition, in January 2025, the FDA published final guidance outlining its non-binding policies governing the distribution of scientific information on unapproved uses to healthcare providers. This final guidance calls for such communications to be truthful, non-misleading, factual, and unbiased and include all information necessary for healthcare providers to interpret the strengths and weaknesses and validity and utility of the information about the unapproved use.

If a company is found to have promoted off-label uses, it may become subject to administrative and judicial enforcement by the FDA, the DOJ, or the Office of the Inspector General of HHS, as well as state authorities. This could subject a company to a range of penalties that could have a significant commercial impact, including civil and criminal fines and agreements that materially restrict the manner in which a company promotes or distributes drug products. The federal government has levied large civil and criminal fines

against companies for alleged improper promotion and has also requested that companies enter into consent decrees or permanent injunctions under which specified promotional conduct is changed or curtailed.

After approval, some types of changes to the approved product, such as adding new indications or dosing regimens, manufacturing changes, or additional labeling claims, are subject to further FDA review and approval. In addition, the FDA may require testing and surveillance programs to monitor the effect of approved products that have been commercialized, and the FDA has the power to prevent or limit further marketing of a product based on the results of these post-marketing programs.

The FDA may withdraw product 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 issues with manufacturing processes, may result in revisions to the approved labeling to add new safety information; imposition of post-market studies or clinical trials to assess new safety signals; or imposition of distribution or other restrictions under a REMS program. Other potential consequences include, among other things:

- restrictions on the marketing or manufacturing of the product;
- fines, warning letters or holds on post-approval clinical trials;
- refusal of the FDA to approve pending applications or supplements to approved applications, or suspension or revocation of product license approvals;
- product recall, seizure, or detention, or refusal to permit the import or export of products; or
- injunctions or the imposition of civil or criminal penalties.

Finally, if there are any modifications to the product, including changes in indications, labeling or manufacturing processes or facilities, the sponsor may be required to submit and obtain FDA approval of a new BLA or a BLA supplement, which may require the sponsor to develop additional data or conduct additional preclinical studies and clinical trials. Securing FDA approval for new indications is similar to the process for approval of the original indication and requires, among other things, submitting data from adequate and well-controlled clinical trials to demonstrate the product's safety, purity and potency in the new indication. Even if such trials are conducted, the FDA may not approve any expansion of the labeled indications for use in a timely fashion, or at all. There also are continuing, annual user fee requirements that are now assessed as program fees for certain approved drugs.

Orphan drug designation and exclusivity

Orphan drug designation in the United States is designed to encourage sponsors to develop products intended for the treatment of rare diseases or conditions. In the United States, a rare disease or condition is statutorily defined as a condition that affects fewer than 200,000 individuals in the United States or that affects more than 200,000 individuals in the United States and for which there is no reasonable expectation that the cost of developing and making the product available for the disease or condition will be recovered from sales of the product in the United States.

Orphan drug designation qualifies a company for certain tax credits. In addition, if a drug candidate that has orphan drug designation subsequently receives the first FDA approval for that drug for the disease for which it has such designation, the product is entitled to orphan drug exclusivity, which means that the FDA may not approve any other applications to market the same drug for the same indication for seven years following product approval unless the subsequent product candidate is demonstrated to be clinically superior. Absent a showing of clinical superiority, FDA cannot approve the same product made by another manufacturer for the same indication during the market exclusivity period unless it has the consent of the sponsor or the sponsor is unable to provide sufficient quantities.

A sponsor may request orphan drug designation of a previously unapproved product or new orphan indication for an already marketed product. In addition, a sponsor of a product that is otherwise the same product as an already approved orphan drug may seek and obtain orphan drug designation for the subsequent product for the same rare disease or condition if it can present a plausible hypothesis that its product may be clinically superior to the first drug. More than one sponsor may receive orphan drug designation for the same product for the same rare disease or condition, but each sponsor seeking orphan drug designation must file a complete request for designation. To qualify for orphan exclusivity, however, the drug must be clinically superior to the previously approved product that is the same drug for the same condition.

Gene therapy products present novel issues for assessing when two products are the "same" for orphan exclusivity purposes. In September 2021, the FDA issued a final guidance document describing its current thinking on when a gene therapy product is the "same" as another product for the purpose of orphan exclusivity. Under the guidance, if either the transgene or vector differs between two gene therapy products in a manner that does not reflect "minor" differences, the two products would be considered different drugs for orphan drug exclusivity purposes. FDA will determine whether two vectors from the same viral class are the same on a case-by-case basis and may consider additional key features in assessing sameness. While the guidance provides some additional clarity on FDA's approach to assessing "sameness," significant ambiguity and uncertainty remain as to how FDA will assess viral vectors in the same class, what differences in vector or transgene are considered minor, and what additional features may be considered.

The period of exclusivity begins on the date that the marketing application is approved by the FDA and applies only to the indication for which the product has been designated. The FDA may approve a second application for the same product for a different use or a second application for a clinically superior version of the product for the same use. Orphan drug exclusivity will not bar approval of another product under certain circumstances, including if the company with orphan drug exclusivity is not able to meet market demand or the subsequent product with the same drug for the same condition is shown to be clinically superior to the approved product on the basis of greater efficacy or safety, or provide a major contribution to patient care. This is the case despite an earlier court opinion holding that the Orphan Drug Act unambiguously required the FDA to recognize orphan drug exclusivity regardless of a showing of clinical superiority. Under Omnibus legislation signed in December 2020, the requirement for a product to show clinical superiority applies to drugs and biologics that received orphan drug designation before enactment of the FDA Reauthorization Act of 2017, or FDARA, in 2017, but have not yet been approved or licensed by the FDA.

The FDA and Congress may further reevaluate the Orphan Drug Act and its regulations and policies. This may be particularly true in light of a decision from the Court of Appeals for the 11th Circuit in September 2021. In *Catalyst Pharms, Inc. v. Becerra*, or *Catalyst*, the court held that, for the purpose of determining the scope of orphan drug exclusivity, the term "same disease or condition" in the statute means the designated "rare disease or condition" and could not be interpreted by the FDA to mean the "indication or use." On January 23, 2023, the FDA announced that, in matters beyond the scope of the *Catalyst* court order, the FDA will continue to apply its existing regulations tying orphan-drug exclusivity to the uses or indications for which the orphan drug is approved. More recently however, on February 14, 2025, a federal district court in Washington, D.C., fully embraced the reasoning of the *Catalyst* decision in another decision challenging the scope of orphan drug exclusivity. On April 17, 2025, the FDA appealed this decision to the U.S. Court of Appeals for the D.C. Circuit. The implications of this decision, and its impact on the FDA's implementation of the Orphan Drug Act, are unclear at this point.

Pediatric exclusivity

Pediatric exclusivity is another type of non-patent regulatory exclusivity in the United States. Specifically, the Best Pharmaceuticals for Children Act provides for the attachment of an additional six months of exclusivity, which is added on to the term of any remaining regulatory exclusivity at the time the pediatric exclusivity is granted. This six-month exclusivity may be granted if a BLA sponsor submits pediatric data that fairly respond to a written request from the FDA for such data, even if the data do not show the product to be effective in the pediatric population studied.

Biosimilars and exclusivity

The 2010 Patient Protection and Affordable Care Act, or PPACA, which was signed into law in March 2010, included a subtitle called the Biologics Price Competition and Innovation Act of 2009, or BPCIA. The BPCIA established a regulatory scheme authorizing the FDA to approve biosimilars and interchangeable biosimilars.

Under the BPCIA, a manufacturer may submit an application for licensure of a biological product that is "biosimilar to" or "interchangeable with" a previously approved biological product or "reference product." In order for the FDA to approve a biosimilar product, it must find that there are no clinically meaningful differences between the reference product and proposed biosimilar product in terms of safety, purity, and potency. For the FDA to approve a biosimilar product as interchangeable with a reference product, the agency must find that the biosimilar product can be expected to produce the same clinical results as the reference product, and (for products administered multiple times) that the biologic and the reference biologic may be switched after one has been previously administered without increasing safety risks or risks of diminished efficacy relative to exclusive use of the reference biologic. In October 2025, the FDA issued draft guidance which proposes to eliminate the need for sponsors of biosimilar products to conduct comparative human clinical efficacy studies, allowing them to rely instead on analytical testing to demonstrate product differences from a reference product.

Under the BPCIA, an application for a biosimilar product may not be submitted to the FDA until four years following the date of approval of the reference product. The FDA may not approve a biosimilar product until 12 years from the date on which the reference product was first licensed. This 12-year exclusivity period is referred to as the reference product exclusivity period and bars approval of a biosimilar but notably does not prevent approval of a competing product pursuant to a full BLA (i.e., containing the sponsor's own preclinical data and data from adequate and well-controlled clinical trials to demonstrate the safety, purity, and potency of the product). The BPCIA also created certain exclusivity periods for biosimilars approved as interchangeable products. In December 2022, Congress clarified through FDORA that the FDA may approve multiple first interchangeable biosimilar biological products so long as the products are all approved on the first day on which such a product is approved as interchangeable with the reference product. The law also includes an extensive process for the innovator biologic and biosimilar manufacturer to litigate patent infringement, validity, and enforceability prior to the approval of the biosimilar.

Since the passage of the BPCIA, many states have passed laws or amendments to laws, including laws governing pharmacy practices, which are state-regulated, to regulate the use of biosimilars.

Patent term restoration and extension

A patent claiming a new biological product may be eligible for a limited patent term extension under the Hatch-Waxman Act, which permits a patent restoration of up to five years for a single patent for an approved product as compensation for patent term lost during product development and FDA regulatory review. The restoration period granted on a patent covering a product is typically one-half the time between the effective date a clinical investigation involving human beings is begun and the submission date of a marketing application less any dime during which the sponsor failed to exercise due diligence, plus the time between the submission date of an application and the ultimate approval date less any dime during which the sponsor failed to exercise due diligence. Patent term restoration cannot be used to extend the remaining term of a patent past a total of 14 years from the product's approval date. Only one patent applicable to an approved product is eligible for the extension, only those claims covering the approved drug, a method for using it, or a method for manufacturing it may be extended and the application for the extension must be submitted prior to the expiration of the patent in question. A patent that covers multiple products for which approval is sought can only be extended in connection with one of the approvals. The USPTO reviews and approves the application for any patent term extension or restoration in consultation with the FDA.

FDA approval of companion diagnostics

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, for novel drugs, a companion diagnostic device and its corresponding therapeutic should be approved or cleared contemporaneously by the FDA for the use indicated in the therapeutic product's labeling. Approval or clearance of the companion diagnostic device will ensure that the device has been adequately evaluated and has adequate performance characteristics in the intended population. In July 2016, the FDA issued a draft guidance intended to assist sponsors of the drug therapeutic and *in vitro* companion diagnostic device on issues related to co-development of the products.

Further, in April 2020, the FDA issued additional guidance which describes considerations for the development and labeling of companion diagnostic devices to support the indicated uses of multiple drug or biological oncology products, when appropriate. This guidance builds upon existing policy regarding the labeling of companion diagnostics. In its 2014 guidance, the FDA stated that if evidence is sufficient to conclude that the companion diagnostic is appropriate for use with a specific group of therapeutic products, the companion diagnostic's intended use/indications for use should name the specific group of therapeutic products, rather than specific products. The 2020 guidance expands on the policy statement in the 2014 guidance by recommending that companion diagnostic developers consider a number of factors when determining whether their test could be developed, or the labeling for approved companion diagnostics could be revised through a supplement, to support a broader labeling claim such as use with a specific group of oncology therapeutic products (rather than listing an individual therapeutic product(s)).

Under the FDCA, *in vitro* diagnostics, including companion diagnostics, are regulated as medical devices. In the United States, the FDCA and its implementing regulations, and other federal and state statutes and regulations govern, among other things, medical device design and development, preclinical and clinical testing, premarket clearance or approval, registration and listing, manufacturing, labeling, storage, advertising and promotion, sales and distribution, export and import, and post-market surveillance. Unless an exemption applies, diagnostic tests require marketing clearance or approval from the FDA prior to commercial distribution.

The FDA previously has required *in vitro* companion diagnostics intended to select the patients who will respond to the product candidate to obtain pre-market approval, or PMA, simultaneously with approval of the therapeutic product candidate. The PMA process, including the gathering of clinical and preclinical data and the submission to and review by the FDA, can take several years or longer. It involves a rigorous premarket review during which the sponsor must prepare and provide the FDA with reasonable assurance of the device's safety and effectiveness and information about the device and its components regarding, among other things, device design, manufacturing and labeling. PMA applications are subject to an application fee, which for federal fiscal year 2025 is $540,783 and the small business fee is $139,196.

A clinical trial is typically required for a PMA application and, in a small percentage of cases, the FDA may require a clinical study in support of a 510(k) submission. A manufacturer that wishes to conduct a clinical study involving the device is subject to the FDA's IDE regulation. The IDE regulation distinguishes between significant and non-significant risk device studies and the procedures for obtaining approval to begin the study differ accordingly. Also, some types of studies are exempt from the IDE regulations. A significant risk device presents a potential for serious risk to the health, safety, or welfare of a subject. Significant risk devices are devices that are substantially important in diagnosing, curing, mitigating, or treating disease or in preventing impairment to human health. Studies of devices that pose a significant risk require both FDA and an IRB approval prior to initiation of a clinical study. Many companion diagnostics are considered significant risk devices due to their role in diagnosing a disease or condition. Non-significant risk devices are devices that do not pose a significant risk to the human subjects. A non-significant risk device study requires only IRB approval prior to initiation of a clinical study.

After a device is placed on the market, it remains subject to significant regulatory requirements. Medical devices may be marketed only for the uses and indications for which they are cleared or approved. Device manufacturers must also establish registration and device listings with the FDA. A medical device manufacturer's manufacturing processes and those of its suppliers are required to comply with the applicable portions of the Quality System Regulation, which covers the methods and documentation of the design, testing, production, processes, controls, quality assurance, labeling, packaging and shipping of medical devices. Domestic facility records and manufacturing processes are subject to periodic unscheduled inspections by the FDA. The FDA also may inspect foreign facilities that export products to the United States.

Federal and state data privacy and security laws

There are multiple privacy and data security laws that may impact our business activities in the United States and in other countries where we conduct trials or where we may do business in the future. These laws are evolving and may increase both our obligations and our regulatory risks in the future. In the health care industry generally, under the federal Health Insurance Portability and Accountability Act of 1996, or HIPAA, HHS has issued regulations to protect the privacy and security of protected health information, or PHI, used or disclosed by covered entities including certain healthcare providers, health plans and healthcare clearinghouses. HIPAA also imposes certain obligations on the business associates of covered entities that obtain protected health information in providing services to or on behalf of covered entities. HIPAA may apply to us in certain circumstances and may also apply to our business partners in ways that may impact our relationships with them. Any clinical trials we conduct will be regulated by Subpart A of 45 CFR 46, also known as the Common Rule, which also includes specific privacy-related provisions. In addition to federal privacy regulations, there are a number of state laws governing confidentiality and security of health information that may be applicable to our business. In addition to possible federal civil and criminal penalties for HIPAA violations, state attorneys general are authorized to file civil actions for damages or injunctions in federal courts to enforce HIPAA and seek attorney's fees and costs associated with pursuing federal civil actions. In addition, state attorneys general (along with private plaintiffs) have brought civil actions seeking injunctions and damages resulting from alleged violations of HIPAA's privacy and security rules. State attorneys general also have authority to enforce state privacy and security laws. Moreover, new laws and regulations governing privacy and security may be adopted in the future as well.

In addition to potential enforcement by the HHS, we could also be potentially subject to privacy enforcement from the Federal Trade Commission, or the FTC. The FTC has been particularly focused on the unpermitted processing of health and genetic data through its recent enforcement actions and is expanding the types of privacy violations that it interprets to be "unfair" under Section 5 of the FTC Act, as well as the types of activities it views to trigger the Health Breach Notification Rule, which the FTC also has the authority to enforce. The FTC is also in the process of developing rules related to commercial surveillance and data security. We will need to account for the FTC's evolving rules and guidance for proper privacy and data security practices in order to mitigate risk for a potential enforcement action, which may be costly. Finally, both the FTC and HHS's enforcement priorities, as well as those of other federal regulators, may be impacted by the change in administration and new leadership. These shifts in enforcement priorities may also impact our business.

There are also increased restrictions at the federal level relating to transferring sensitive data outside of the United States to certain foreign countries. For example, in 2024, Congress passed H.B. 815, which included the Protecting Americans' Data from Foreign Adversaries Act of 2024. This law creates certain restrictions for entities that disclose sensitive data, including potential health data, to countries such as China. Failure to comply with these rules can lead to a potential FTC enforcement action. Additionally, the Department of Justice recently finalized a rule implementing Executive Order 14117, which creates similar restrictions related to the transfer of sensitive U.S. data to countries such as China. These data transfer restrictions, and others that may pass in the future, may create operational challenges and legal risks for our business.

At the state level, California has enacted legislation that has been dubbed the first "GDPR-like" law in the United States. Known as the California Consumer Privacy Act, or CCPA, it creates new individual privacy rights for consumers (as that word is broadly defined in the law) and places increased privacy and security obligations on entities handling personal data of consumers or households. The CCPA went into effect on January 1, 2020 and requires covered companies to provide new disclosures to California consumers, provide such consumers new ways to opt-out of certain sales of personal information, and allow for a new cause of action for data breaches. Additionally, effective starting on January 1, 2023, the California Privacy Rights Act, or CPRA, significantly modified the CCPA, including by expanding consumers' rights with respect to certain sensitive personal information. The CPRA also creates a new state agency that will be vested with authority to implement and enforce the CCPA and the CPRA. The CCPA and CPRA could impact our business activities depending on how it is interpreted and exemplifies the vulnerability of our business to not only cyber threats but also the evolving regulatory environment related to personal data and individually identifiable health information. These provisions may apply to some of our business activities.

In addition to California, at least eighteen other states have passed comprehensive privacy laws similar to the CCPA and CPRA. These laws are either in effect or will go into effect sometime before the end of 2026. Like the CCPA and CPRA, these laws create obligations related to the processing of personal information, as well as special obligations for the processing of "sensitive" data, which includes health data in some cases. Some of the provisions of these laws may apply to our business activities. There are also states that are strongly considering additional laws that could go into effect in 2026 and beyond, and Congress has also been debating passing a federal privacy law. There are also states that are specifically regulating health information that may affect our business. For

example, the State of Washington passed the My Health My Data Act in 2023 which specifically regulated health information that is not otherwise regulated by the HIPAA rules, and the law also has a private right of action, which further increases the relevant compliance risk. Connecticut and Nevada have also passed similar laws regulating consumer health data, and more states are considering such legislation. These laws may impact our business activities, including our identification of research subjects, relationships with business partners and ultimately the marketing and distribution of our products.

Plaintiffs' lawyers are also increasingly using privacy-related statutes at both the state and federal level to bring lawsuits against companies for their data-related practices. In particular, there have been a significant number of cases filed against companies for their use of pixels and other web trackers. These cases often allege violations of the California Invasion of Privacy Act and other state laws regulating wiretapping, as well as the federal Video Privacy Protection Act.

Regulation and procedures governing approval of medicinal products in the EU and the U.K.

In order to market any product outside of the United States, a company must also comply with numerous and varying regulatory requirements of other countries and jurisdictions regarding quality, safety, purity and potency, and governing, among other things, clinical trials, marketing authorization, commercial sales and distribution of products. Whether or not it obtains FDA approval for a product, a sponsor will need to obtain the necessary approvals by the comparable foreign regulatory authorities before it can commence clinical trials or marketing of the product in those countries or jurisdictions. Specifically, the process governing approval of medicinal products in the EU generally follows the same lines as in the United States. It entails satisfactory completion of preclinical studies and adequate and well-controlled clinical trials to establish the safety, purity and potency of the product for each proposed indication. It also requires the submission to the relevant competent authorities of a marketing authorization application, or MAA, and granting of a marketing authorization by these authorities before the product can be marketed and sold in the EU.

Non-clinical studies

Non-clinical studies are performed to demonstrate the health or environmental safety of new chemical or biological substances. Non-clinical (pharmaco-toxicological) studies must be conducted in compliance with the principles of good laboratory practice, or GLP, as set forth in EU Directive 2004/10/EC (unless otherwise justified for certain particular medicinal products – e.g., radio-pharmaceutical precursors for radio-labeling purposes). In particular, non-clinical studies, both *in vitro* and *in vivo*, must be planned, performed, monitored, recorded, reported and archived in accordance with the GLP principles, which define a set of rules and criteria for a quality system for the organizational process and the conditions for non-clinical studies. These GLP standards reflect the Organization for Economic Co-operation and Development requirements.

Clinical trial approval

On January 31, 2022, the new Clinical Trials Regulation (EU) No 536/2014, or CTR, became effective in the European Union and replaced the prior Clinical Trials Directive 2001/20/EC. The new regulation aims at simplifying and streamlining the authorization, conduct and transparency of clinical trials in the EU. Under the new coordinated procedure for the approval of clinical trials, the sponsor of a clinical trial to be conducted in more than one Member State of the EU, or EU Member State, will only be required to submit a single application for approval. The submission will be made through the Clinical Trials Information System, a new clinical trials portal overseen by the European Medicines Agency, or the EMA, and available to clinical trial sponsors, competent authorities of the EU Member States and the public.

Beyond streamlining the process, the CTR includes a single set of documents to be prepared and submitted for the application as well as simplified reporting procedures for clinical trial sponsors, and a harmonized procedure for the assessment of applications for clinical trials, which is divided in two parts. Part I is assessed by the competent authorities of all EU Member States in which an application for authorization of a clinical trial has been submitted, or the Member States Concerned. Part II is assessed separately by each Member State Concerned. Strict deadlines have been established for the assessment of clinical trial applications. The role of the relevant ethics committees in the assessment procedure will continue to be governed by the national law of the Member State Concerned. However, overall related timelines will be defined by the CTR.

The CTR did not change the preexisting requirement that a sponsor must obtain prior approval from the competent national authority of the EU Member State in which the clinical trial is to be conducted. If the clinical trial is conducted in different EU Member States, the competent authorities in each of these EU Member States must provide their approval for the conduct of the clinical trial. Furthermore, the sponsor may only start a clinical trial at a specific clinical site after the applicable ethics committee has issued a favorable opinion. As of January 31, 2026, all clinical trials (including those which are ongoing) became subject to the provisions of the CTR.

Parties conducting certain clinical trials must, as in the United States, post clinical trial information in the EU at the EU Clinical Trials Register (https://eudract.ema.europa.eu).

Marketing authorization

To obtain a marketing authorization, or MA, for a gene therapy product under the EU regulatory system, a sponsor must submit an application via the centralized procedure administered by the EMA. Specifically, the grant of an MA in the EU for products containing

viable human tissues or cells such as gene therapy medicinal products is governed by Regulation 1394/2007/EC on advanced therapy medicinal products, read in combination with Directive 2001/83/EC of the European Parliament and of the Council, commonly known as the Community code on medicinal products. Regulation 1394/2007/EC lays down specific rules concerning the authorization, supervision, and pharmacovigilance of gene therapy medicinal products, somatic cell therapy medicinal products, and tissue engineered products. Manufacturers of advanced therapy medicinal products must demonstrate the quality, safety, and efficacy of their products to the EMA's Committee for Advance Therapies which provides a draft opinion regarding the application for marketing authorization and which is subject to final approval by the EMA's Committee for Medicinal Products for Human Use. The European Commission grants or refuses marketing authorization in light of that final approval.

Under the centralized procedure in the EU, the maximum timeframe for the evaluation of an application for an MA is 210 days, excluding clock stops when additional information or written or oral explanation is to be provided by the sponsor in response to questions of the Committee for Medicinal Products for Human Use, or CHMP. Accelerated evaluation may be granted by the CHMP in exceptional cases, when a medicinal product is of major interest from the point of view of public health and, in particular, from the viewpoint of therapeutic innovation. If the CHMP accepts such a request, the time limit of 210 days will be reduced to 150 days, but it is possible that the CHMP may revert to the standard time limit for the centralized procedure if it determines that it is no longer appropriate to conduct an accelerated assessment.

Conditional marketing authorization

In specific circumstances, E.U. legislation (Article 14–a Regulation (EC) No 726/2004 (as amended by Regulation (EU) 2019/5 and Regulation (EC) No 507/2006 on Conditional MA for Medicinal Products for Human Use) enables sponsors to obtain a conditional MA prior to obtaining the comprehensive clinical data required for an application for a full marketing authorization. Such conditional approvals may be granted for product candidates (including medicines designated as orphan medicinal products) if (1) the product candidate is intended for the treatment, prevention or medical diagnosis of seriously debilitating or life-threatening diseases; (2) the product candidate is intended to meet unmet medical needs of patients; (3) the benefit of the immediate availability on the market of the medicinal product concerned outweighs the risk inherent in the fact that additional data are still required; (4) the risk-benefit balance of the product candidate is positive; and (5) it is likely that the sponsor will be in a position to provide the required comprehensive clinical trial data. A conditional marketing authorization may contain specific obligations to be fulfilled by the marketing authorization holder, including obligations with respect to the completion of ongoing or new studies and with respect to the collection of pharmacovigilance data. Conditional marketing authorizations are valid for one year, and may be renewed annually, if the risk-benefit balance remains positive, and after an assessment of the need for additional or modified conditions or specific obligations. The timelines for the centralized procedure described above also apply with respect to the review by the CHMP of applications for a conditional marketing authorization.

Exceptional circumstances

An MA may also be granted "under exceptional circumstances" when the applicant can show that it is unable to provide comprehensive data on the efficacy and safety under normal conditions of use even after the product has been authorized and subject to specific procedures being introduced. This may arise in particular when the intended indications are very rare and, in the present state of scientific knowledge, it is not possible to provide comprehensive information, or when generating data may be contrary to generally accepted ethical principles. This MA is close to the conditional MA as it is reserved to medicinal products to be approved for severe diseases or unmet medical needs and the applicant does not hold the complete data set legally required for the grant of a MA. However, unlike the conditional MA, the applicant does not, and will not in the future, have to provide the missing data. Although the MA "under exceptional circumstances" is granted definitively, the risk-benefit balance of the medicinal product is reviewed annually and the MA is withdrawn in case the risk-benefit ratio is no longer favorable. Under these procedures, before granting the MA, the EMA or the competent authorities of the member states make an assessment of the risk-benefit balance of the product on the basis of scientific criteria concerning its quality, safety, and efficacy. Except conditional MAs, MAs have an initial duration of five years. After these five years, the authorization may be renewed on the basis of a reevaluation of the risk-benefit balance.

Regulatory exclusivity

In the EU, new chemical entities approved on the basis of a complete independent data package qualify for eight years of data exclusivity upon marketing authorization and an additional two years of market exclusivity pursuant to Regulation (EC) No 726/2004, as amended, and Directive 2001/83/EC, as amended. Data exclusivity prevents regulatory authorities in the EU from referencing the innovator's data to assess a generic (abbreviated) application for a period of eight years. This also applies to biosimilars. During the additional two-year period of market exclusivity, a generic marketing authorization application can be submitted, and the innovator's data may be referenced, but no generic medicinal product can be marketed until the expiration of the market exclusivity. The overall ten-year period will be extended to a maximum of eleven years if, during the first eight years of those ten years, the marketing authorization holder obtains an authorization for one or more new therapeutic indications which, during the scientific evaluation prior to authorization, is held to bring a significant clinical benefit in comparison with existing therapies. In addition, if a pediatric investigation plan is accepted, then a further year of market exclusivity might be obtained (or in the alternative a patent extension (SPC) of a further 6 months). For orphan medicinal products, the periods are separate and different in that there is a total of 10-year data exclusivity and if they have a PIP, there is a further two-year extension to that 10-year period. Even if a compound is considered to be a new chemical or biological entity so that the innovator gains the prescribed period of data exclusivity, another company may market another version of the product if such company obtained marketing authorization based on an MAA with a complete independent data package of pharmaceutical tests, preclinical tests and clinical trials.

In this context, it should be noted that the EU pharmaceutical legislation is currently undergoing a complete review process, in the context of the Pharmaceutical Strategy for Europe initiative, launched by the European Commission in November 2020. The European Commission's proposal for revision of several legislative instruments related to medicinal products was published in April 2023 and includes, among other things, provisions that would potentially reduce the duration of regulatory data protection. The European Parliament requested several amendments in April 2024.

On June 4, 2025, after almost two years of negotiations among the EU Member States, the Council of the European Union adopted its position on the proposed overhaul of the EU general pharmaceutical legislative framework, which is known as the new Pharma Package. Thereafter, on December 11, 2025, the European Parliament and the Council reached a provisional political agreement on the legislation which is expected to be adopted by mid-2026. Key changes include updating regulatory data exclusivity to a new system with eight years of data exclusivity and reduced market exclusivity period to one year, which can be extended if specific conditions are fulfilled, adding launch/supply obligations, incentivizing antibiotic innovation with transferable vouchers, and streamlining approval procedures in the EU. There will likely be a transition period of 24 months, with these changes taking effect in mid-2028.

Periods of authorization and renewals

A marketing authorization is valid for five years, in principle, and it may be renewed after five years on the basis of a reevaluation of the risk-benefit balance by the EMA or by the competent authority of the authorizing member state. To that end, the marketing authorization holder must provide the EMA or the competent authority with a consolidated version of the file in respect of quality, safety, purity and potency, including all variations introduced since the marketing authorization was granted, at least six months before the marketing authorization ceases to be valid. Once renewed, the marketing authorization is valid for an unlimited period, unless the European Commission or the competent authority decides, on justified grounds relating to pharmacovigilance, to proceed with one additional five-year renewal period. Any authorization that is not followed by the placement of the drug on the EU market (in the case of the centralized procedure) or on the market of the authorizing member state within three years after authorization ceases to be valid.

Regulatory requirements after marketing authorization

Following approval, the holder of the marketing authorization is required to comply with a range of requirements applicable to the manufacturing, marketing, promotion and sale of the medicinal product. These include compliance with the EU's stringent pharmacovigilance or safety reporting rules, pursuant to which post-authorization studies and additional monitoring obligations can be imposed. In addition, the manufacturing of authorized products, must also be conducted in strict compliance with the EMA's GMP requirements and comparable requirements of other regulatory bodies in the EU, which mandate the methods, facilities, and controls used in manufacturing, processing and packing of drugs to assure their safety and identity. The marketing and promotion of authorized products, including industry-sponsored continuing medical education and advertising directed toward the prescribers of drugs and/or the general public, are strictly regulated in the EU under Directive 2001/83EC, as amended.

PRIME designation in the EU

The EU has a Priority Medicines, or PRIME, scheme that is intended to encourage drug development in areas of unmet medical need and provides accelerated assessment of products representing substantial innovation reviewed under the centralized procedure. Products from small- and medium-sized enterprises may qualify for earlier entry into the PRIME scheme than larger companies. Many benefits accrue to sponsors of product candidates with PRIME designation, including but not limited to, early and proactive regulatory dialogue with the EMA, frequent discussions on clinical trial designs and other development program elements, and accelerated marketing authorization application assessment once a dossier has been submitted.

Pediatric studies

Prior to obtaining a marketing authorization in the EU, sponsors must demonstrate compliance with all measures included in an EMA-approved PIP covering all subsets of the pediatric population, unless the EMA has granted a product-specific waiver, a class waiver, or a deferral for one or more of the measures included in the PIP. The respective requirements for all marketing authorization procedures are provided in Regulation (EC) No 1901/2006, the so-called Paediatric Regulation. This requirement also applies when a company wants to add a new indication, pharmaceutical form or route of administration for a medicine that is already authorized. The Paediatric Committee of the EMA, or PDCO, may grant deferrals for some medicines, allowing a company to delay development of the medicine for children until there is enough information to demonstrate its effectiveness and safety in adults. The PDCO may also grant waivers when development of a medicine for children is not needed or is not appropriate, such as for diseases that only affect the elderly population. Before an MAA can be filed, or an existing marketing authorization can be amended, the EMA determines that companies actually comply with the agreed studies and measures listed in each relevant PIP.

Orphan drug designation and exclusivity

Regulation (EC) No 141/2000 and Regulation (EC) No. 847/2000 provide that a product can be designated as an orphan drug by the European Commission if its sponsor can establish: that the product is intended for the diagnosis, prevention or treatment of (1) a life-threatening or chronically debilitating condition affecting not more than five in ten thousand persons in the EU when the application is made, or (2) a life-threatening, seriously debilitating or serious and chronic condition in the EU and that without incentives it is unlikely that the marketing of the drug in the EU would generate sufficient return to justify the necessary investment. For either of these conditions, the sponsor must demonstrate that there exists no satisfactory method of diagnosis, prevention, or treatment of the condition in question that has been authorized in the EU or, if such method exists, the drug will be of significant benefit to those affected by that condition.

Pediatric exclusivity

If a sponsor obtains a marketing authorization in all EU member states, or a marketing authorization granted in the centralized procedure by the European Commission, and the study results for the pediatric population are included in the product information, even when negative, the medicine is then eligible for an additional six-month period of qualifying patent protection through extension of the term of the Supplementary Protection Certificate, or SPC.

Patent term extensions in the EU and other jurisdictions

The EU also provides for patent term extension through SPCs. The rules and requirements for obtaining an SPC are similar to those in the United States. An SPC may extend the term of a patent for up to five years after its originally scheduled expiration date and can provide up to a maximum of fifteen years of marketing exclusivity for a drug. In certain circumstances, these periods may be extended for six additional months if pediatric exclusivity is obtained, which is described in detail below. Although SPCs are available throughout the EU, sponsors must apply on a country-by-country basis. Similar patent term extension rights exist in certain other foreign jurisdictions outside the EU.

Pricing decisions for approved products

In the EU, pricing and reimbursement schemes vary widely from country to country. Some countries provide that products may be marketed only after a reimbursement price has been agreed. Some countries may require the completion of additional studies that compare the cost-effectiveness of a particular product candidate to currently available therapies or so-called health technology assessments, in order to obtain reimbursement or pricing approval. For example, the EU provides options for EU Member States to restrict the range of products for which their national health insurance systems provide reimbursement and to control the prices of medicinal products for human use. EU Member States may approve a specific price for a product, or they may instead adopt a system of direct or indirect controls on the profitability of the company placing the product on the market. Other EU Member States allow companies to fix their own prices for products but monitor and control prescription volumes and issue guidance to physicians to limit prescriptions. Recently, many countries in the EU have increased the amount of discounts required on pharmaceuticals, and these efforts could continue as countries attempt to manage health care expenditures, especially in light of the severe fiscal and debt crises experienced by many countries in the EU. The downward pressure on health care 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 EU Member States and parallel trade (i.e., 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 products, if approved in those countries.

General Data Protection Regulation

Similar to the laws in the United States, there are significant privacy and data security laws that apply in Europe and other countries. The collection, use, disclosure, transfer, or other processing of personal data, including personal health data, regarding individuals who are located in the European Economic Area, or the EEA, and the processing of personal data that takes place in the EEA, is subject to the EU's General Data Protection Regulation, or GDPR, which became effective on May 25, 2018. The GDPR is wide-ranging in scope and imposes numerous requirements on companies that process personal data, and it imposes heightened requirements on companies that process health and other sensitive data, such as requiring in many situations that a company obtain the consent of the individuals to whom the sensitive personal data relate before processing such data. Examples of obligations imposed by the GDPR on companies processing personal data that fall within the scope of the GDPR include providing information to individuals regarding data processing activities, implementing safeguards to protect the security and confidentiality of personal data, appointing a data protection officer, providing notification of data breaches, and taking certain measures when engaging third-party processors. The GDPR also imposes strict rules on the transfer of personal data to countries outside the EEA, including the U.S., and permits data protection authorities to impose large penalties for violations of the GDPR, including potential fines of up to €20 million or 4% of annual global revenues, whichever is greater. The GDPR also 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. Compliance with the GDPR is a rigorous and time-intensive process that may increase the cost of doing business or require companies to change their business practices to ensure full compliance.

There are ongoing concerns about the ability of companies to transfer personal data from the EU to other countries. In July 2020, the Court of Justice of the European Union, or the CJEU, invalidated the EU-U.S. Privacy Shield framework, or Privacy Shield, one of the mechanisms used to legitimize the transfer of personal data from the EEA to the U.S. The CJEU decision also drew into question the long-term viability of an alternative means of data transfer, the standard contractual clauses, for transfers of personal data from the EEA to the U.S. While we were not self-certified under the Privacy Shield, this CJEU decision has led to increased scrutiny on data transfers from the EU to the U.S. generally and increase our costs of compliance with data privacy legislation as well as our costs of negotiating appropriate privacy and security agreements with our vendors and business partners.

Following the CJEU decision, in October 2022, President Biden signed an executive order to implement the EU-U.S. Data Privacy Framework, or DPF, which would serve as a replacement to the EU-U.S. Privacy Shield. The European Commission initiated the process to adopt an adequacy decision for the DPF in December 2022, and has now adopted an adequacy decision to permit data transfers from the EU to the United States going forward. This development permits data transfers at this point under this framework and more broadly has made international data transfers more straightforward, but these provisions are being challenged in court. There is currently one pending litigation against the DPF before the Court of Justice of the European Union (CJEU), C-703/25 P – *Latombe v Commission*. The recent election in the United States and the new administration may also impact whether the DPF remains an adequate data transfer framework. The continuing uncertainty around this issue may further impact our business operations in the EU.

On June 23, 2016, the electorate in the U.K. voted in favor of leaving the EU, commonly referred to as Brexit. As with other issues related to Brexit, there are open questions about how personal data will be protected in the U.K. and whether personal information can transfer from the EU to the U.K. Following the withdrawal of the U.K. from the EU, the U.K. Data Protection Act of 2018 applies to the processing of personal data that takes place in the U.K. and includes parallel obligations to those set forth by the GDPR. While the Data Protection Act of 2018 in the U.K. that "implements" and complements the GDPR has achieved Royal Assent on May 23, 2018 and is now effective in the U.K., it is unclear whether transfer of data from the EEA to the U.K. will remain lawful under the GDPR.

The U.K. government has already determined that it considers all European Union 27 and EEA member states to be adequate for the purposes of data protection, ensuring that data flows from the U.K. to the EU/EEA remain unaffected. In addition, a recent decision from the European Commission appears to deem the U.K. as being "essentially adequate" for purposes of data transfer from the EU to the U.K. On December 19, 2025, the European Commission renewed this decision until December 27, 2031. The U.K. and the United States also have agreed on a framework for personal data to be transferred between the U.K. and the United States, called the U.K.-U.S. Data Bridge. The U.K.-U.S. Data Bridge may be challenged in the future. Continuing uncertainty about these data transfers, including the possibility of future changes, may impact our business operations.

Beyond the GDPR, there are privacy and data security laws in a growing number of countries around the world. While many loosely follow the GDPR as a model, other laws contain different or conflicting provisions. These laws will impact our ability to conduct our business activities, including both our clinical trials and any eventual sale and distribution of commercial products.

Brexit and the regulatory framework in the U.K.

Brexit took place on January 31, 2020. The EU and the U.K. reached an agreement on their new partnership in the Trade and Cooperation Agreement, which entered into force on May 1, 2021.

The U.K. is no longer part of the European Single Market and EU Customs Union. As of January 1, 2025, the Medicines and Healthcare Products Regulatory Agency, or MHRA, is responsible for approving all medicinal products destined for the U.K. market (i.e., Great Britain and Northern Ireland). On April 28, 2025, the U.K. Parliament adopted amendments to improve and strengthen the U.K.'s clinical trials regulatory regime, and such amendments will take effect on April 28, 2026. These changes were needed since the current U.K. requirements are based upon the now-repealed EU Clinical Trials Directive (2001/20/EC), which has been replaced by the European Clinical Trials Regulation (Regulation EU No 536/2014). Since the U.K. left the EU prior to the date on which the EU CTR took effect, the U.K. legal framework did not benefit from the same revisions as occurred at the EU level.

Further, as of January 1, 2024, a new international recognition procedure, or IRP, applies, which intends to facilitate approval of pharmaceutical products in the U.K. The IRP is open to applicants that have already received an authorization for the same product from one of the MHRA's specified Reference Regulators, or RRs. The RRs notably include the EMA and regulators in the EEA member states for approvals in the EU centralized procedure and mutual recognition procedure, as well as the FDA for product approvals granted in the United States. The RR assessment must have undergone a full and standalone review. RR assessments based on reliance or recognition cannot be used to support an IRP application. A CHMP positive opinion or an MRDC positive end of procedure outcome is an RR authorization for the purposes of IRP.

Coverage, pricing, and reimbursement

Significant uncertainty exists as to the coverage and reimbursement status of any product candidates for which we may seek regulatory approval by the FDA or other government authorities. In the United States and markets in other countries, patients who are prescribed treatments for their conditions and providers performing the prescribed services generally rely on third-party payors to reimburse all or part of the associated healthcare costs. Patients are unlikely to use any product candidates we may develop unless coverage is provided and reimbursement is adequate to cover a significant portion of the cost of such product candidates. Sales of our products will depend, in significant part, on the availability of coverage and the adequacy of reimbursement from third-party payors.

Within the United States, third-party payors include government authorities or government healthcare programs, such as Medicare and Medicaid, and private entities, such as managed care organizations, private health insurers and other organizations. The process for determining whether a third-party payor will provide coverage for a product may be separate from the process for setting the reimbursement rate that the payor will pay for the drug product. Third-party payors may limit coverage to specific products on an approved list, or formulary, which might not include all of the FDA-approved products for a particular indication. Some third-party payors may manage utilization of a particular product by requiring pre-approval (known as "prior authorization") for coverage of particular prescriptions (to allow the payor to assess medical necessity). Moreover, a third-party payor's decision to provide coverage for a drug product does not imply that an adequate reimbursement rate will be approved. Adequate third-party reimbursement may not be available to enable us to maintain net price levels sufficient to realize an appropriate return on our investment in product development. Additionally, coverage and reimbursement for drug products can differ significantly from payor to payor. One third-party payor's decision to cover a particular drug product or service does not ensure that other payors will also provide coverage for the drug product or will provide coverage at an adequate reimbursement rate.

Third-party payors are increasingly challenging the price and examining the cost-effectiveness of new products and services in addition to their safety, purity and potency. To obtain or maintain coverage and reimbursement for any current or future product, we may need to conduct expensive pharmacoeconomic studies to demonstrate the medical necessity and cost-effectiveness of our product. These studies will be in addition to the studies required to obtain regulatory approvals. 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. Thus, obtaining and maintaining reimbursement status is time-consuming and costly.

As noted above, the marketability of any product candidates for which we receive regulatory approval for commercial sale may suffer if the government and other third-party payors fail to provide coverage and adequate reimbursement. There is an emphasis on cost containment measures in the United States and we expect the pressure on pharmaceutical pricing will increase. Coverage policies and third-party reimbursement rates may change at any time. Even if favorable coverage and reimbursement status is attained for one or more product candidates for which we receive regulatory approval from one or more third party payors, less favorable coverage policies and reimbursement rates may be implemented in the future.

If we obtain appropriate approval in the future to market any of our current product candidates in the United States, we may be required to provide discounts or rebates under government healthcare programs or to certain government and private purchasers in order to obtain coverage under federal healthcare programs such as Medicaid. Participation in such programs may require us to track and report certain drug prices. We may be subject to fines and other penalties if we fail to report such prices accurately.

Outside the United States, ensuring adequate coverage and payment for any product 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 EU, pricing and reimbursement schemes vary widely from country to country because this is not yet the subject of harmonized EU law. Many 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 and others with “peg” their pricing to a basket of other countries. EU member states may approve a specific price for a product, or it may instead adopt a system of direct or indirect controls on the profitability of the company placing the product on the market. Some member states, in addition to controlling pricing will monitor and control prescription volumes and issue guidance to physicians to limit prescriptions. Recently, many countries in the EU have increased the amount of discounts required on pharmaceuticals and these efforts could continue as countries attempt to manage healthcare expenditures, especially in light of the severe fiscal and debt crises experienced by many countries in the EU. The downward pressure on health care 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 EU member states, and parallel trade (arbitrage between low-priced and high-priced member states), can further reduce prices. There can be no assurance that any country that has price controls or reimbursement limitations for pharmaceutical products will allow favorable reimbursement and pricing arrangements for any of our products, if approved in those countries.

Healthcare law and regulation

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

- federal false claims, false statements and civil monetary penalties laws prohibiting, among other things, any person from knowingly presenting, or causing to be presented, a false claim for payment of government funds or knowingly making, or causing to be made, a false statement to get a false claim paid;
- federal healthcare program anti-kickback law, which prohibits, among other things, persons from soliciting, receiving or providing remuneration, directly or indirectly, to induce either the referral of an individual for the purchasing or ordering of a good or service, for which payment may be made under federal healthcare programs such as Medicare and Medicaid;
- the federal Health Insurance Portability and Accountability Act of 1996, or HIPAA, which, in addition to privacy protections applicable to healthcare providers and other entities, prohibits executing a scheme to defraud any healthcare benefit program or making false statements relating to healthcare matters;
- the federal Food, Drug, and Cosmetic Act, or the FDCA, which among other things, strictly regulates drug marketing, prohibits manufacturers from marketing such products for off-label use and regulates the distribution of samples;
- federal laws that require pharmaceutical manufacturers to report certain calculated product prices to the government or provide certain discounts or rebates to government authorities or private entities, often as a condition of reimbursement under government healthcare programs;
- the so-called “federal sunshine” law, which requires pharmaceutical and medical device companies to monitor and report certain financial interactions with certain healthcare providers and teaching hospitals to the Center for Medicare & Medicaid Services within HHS for re-disclosure to the public, as well as ownership and investment interests held by physicians and their immediate family members;
- state laws requiring pharmaceutical companies to comply with specific compliance standards, restrict financial interactions between pharmaceutical companies and healthcare providers or require pharmaceutical companies to report information related to payments to health care providers or marketing expenditures; and
- analogous state and foreign laws and regulations, such as state anti-bribery, anti-kickback and false claims laws, which may apply to healthcare items or services that are reimbursed by non-governmental third-party payors, including private insurers.

Health care and other reform

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

In March 2010, the United States Congress enacted the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act of 2010, or collectively the PPACA, which, among other things, includes changes to the coverage and payment for drug products under government healthcare programs. Other legislative changes have been proposed and adopted since the PPACA was enacted. In August 2011, the Budget Control Act of 2011, among other things, created measures for spending reductions by Congress. A Joint Select Committee on Deficit Reduction, tasked with recommending a targeted deficit reduction of at least $1.2 trillion for the years 2013 through 2021, was unable to reach required goals, thereby triggering the legislation’s automatic reduction to several government programs. These changes included aggregate reductions to Medicare payments to providers of up to 2% per fiscal year, which went into effect in April 2013 and will remain in effect through 2031.

Under current legislation the actual reduction in Medicare payments will vary from 1% in 2022 to up to 3% in the final fiscal year of this sequester. The Consolidated Appropriations Act, which was signed into law by President Biden in December 2022, made several changes to sequestration of the Medicare program. Section 1001 of the Consolidated Appropriations Act delays the 4% Statutory Pay-As-You-Go Act of 2010, or PAYGO, sequester for two years, through the end of calendar year 2024. Triggered by enactment of the American Rescue Plan Act of 2021, the 4% cut to the Medicare program would have taken effect in January 2023. The Consolidated Appropriations Act’s health care offset title includes Section 4163, which extends the 2% Budget Control Act of 2011 Medicare sequester for six months into fiscal 2032 and lowers the payment reduction percentages in fiscal 2030 and 2031.

The American Taxpayer Relief Act of 2012, among other things, reduced Medicare payments to several providers and increased the statute of limitations period for the government to recover overpayments to providers from three to five years. These laws may result in additional reductions in Medicare and other healthcare funding and otherwise affect the prices we may obtain for any of our product candidates for which we may obtain regulatory approval or the frequency with which any such product candidate is prescribed or used.

Since enactment of the PPACA, there have been, and continue to be, numerous legal challenges and Congressional actions to repeal and replace provisions of the law. For example, with enactment of the Tax Cuts and Jobs Act of 2017, which was signed by President Trump on December 22, 2017, Congress repealed the "individual mandate." The repeal of this provision, which requires most Americans to carry a minimal level of health insurance, became effective in 2019. On June 17, 2021, the U.S. Supreme Court dismissed the most recent judicial challenge to the PPACA brought by several states without specifically ruling on the constitutionality of the PPACA. Litigation and legislation over the PPACA are likely to continue, with unpredictable and uncertain results.

Pharmaceutical prices

The prices of prescription pharmaceuticals have also been the subject of considerable discussion in the United States. There have been several recent U.S. congressional inquiries, as well as proposed and enacted state and federal legislation designed to, among other things, bring more transparency to pharmaceutical pricing, review the relationship between pricing and manufacturer patient programs, and reduce the costs of pharmaceuticals under Medicare and Medicaid.

In addition, in October 2020, HHS and the FDA published a final rule allowing states and other entities to develop a Section 804 Importation Program, or SIP, to import certain prescription drugs from Canada into the United States. That regulation was challenged in a lawsuit by the Pharmaceutical Research and Manufacturers of America, or PhRMA, but the case was dismissed by a federal district court in February 2023 after the court found that PhRMA did not have standing to sue HHS. Several states have passed laws allowing for the importation of products from Canada. On January 5, 2023, the FDA approved Florida's plan for Canadian product importation. That state now has authority to import certain products from Canada for a period of two years once certain conditions are met. Florida will first need to submit a pre-import request for each product selected for importation, which must be approved by the FDA. The state will also need to relabel the products and perform quality testing of the products to meet FDA standards. On May 21, 2025, the FDA announced that it would offer individual states the opportunity to submit a draft proposal for pre-review and meet with the FDA to obtain initial feedback from FDA prior to formally submitting their section 804 importation program (SIP) proposal. The intent of these meetings is to assist states in developing their proposals by further clarifying requirements, enhancing the quality of proposals submitted to the FDA and ultimately shortening the review timeline.

Further, on November 20, 2020, HHS finalized a regulation removing safe harbor protection for price reductions from pharmaceutical manufacturers to plan sponsors under Part D, either directly or through pharmacy benefit managers, unless the price reduction is required by law. The rule also creates a new safe harbor for price reductions reflected at the point-of-sale, as well as a safe harbor for certain fixed fee arrangements between pharmacy benefit managers and manufacturers. Pursuant to court order, the removal and addition of the aforementioned safe harbors were delayed and recent legislation imposed a moratorium on implementation of the rule until January 1, 2026. The Inflation Reduction Act of 2022, or IRA, further delayed implementation of this rule to January 1, 2032.

On August 16, 2022, the IRA was signed into law by President Biden. The new legislation has implications for Medicare Part D, which is a program available to individuals who are entitled to Medicare Part A or enrolled in Medicare Part B to give them the option of paying a monthly premium for outpatient prescription drug coverage. Among other things, the IRA requires manufacturers of certain drugs to engage in price negotiations with Medicare (beginning in 2026), with prices that can be negotiated subject to a cap; imposes rebates under Medicare Part B and Medicare Part D to penalize price increases that outpace inflation (first due in 2023); and replaces the Part D coverage gap discount program with a new discounting program (beginning in 2025). The IRA permits the Secretary of HHS to implement many of these provisions through guidance, as opposed to regulation, for the initial years.

Specifically, with respect to price negotiations, Congress authorized Medicare to negotiate lower prices for certain costly single-source drug and biologic products that do not have competing generics or biosimilars and are reimbursed under Medicare Part B and Part D. CMS may negotiate prices for ten high-cost drugs paid for by Medicare Part D starting in 2026, followed by 15 Part D drugs in 2027, 15 Part B or Part D drugs in 2028 and 20 Part B or Part D drugs in 2029 and beyond. This provision applies to drug products that have been approved for at least 9 years and biologics that have been licensed for 13 years. Drugs and biologics that have been approved for a single rare disease or condition were originally categorically excluded from price negotiation. With passage of the One Big Beautiful Bill Act, or OBBBA, on July 3, 2025, which was signed into law on July 4, 2025, Congress extended this exemption to drugs and biologics with multiple orphan drug designations.

Further, the legislation subjects drug manufacturers to civil monetary penalties and a potential excise tax for failing to comply with the legislation by offering a price that is not equal to or less than the negotiated "maximum fair price" under the law or for taking price increases that exceed inflation. The legislation also requires manufacturers to pay rebates for drugs in Medicare Part D whose price increases exceed inflation. The new law also caps Medicare out-of-pocket drug costs at an estimated $4,000 a year in 2024 and, thereafter beginning in 2025, at $2,000 a year.

The first cycle of negotiations for the Medicare Drug Price Negotiation Program commenced in the summer of 2023. On August 15, 2024, the HHS published the results of the first Medicare drug price negotiations for ten selected drugs that treat a range of conditions, including diabetes, chronic kidney disease, and rheumatoid arthritis. The prices of these ten drugs became effective January 1, 2026. On January 17, 2025, CMS announced its selection of 15 additional drugs covered by Part D for the second cycle of negotiations by February 1, 2025. While there had been some questions about the Trump Administration's position on this program, CMS issued a public statement on January 29, 2025, declaring that lowering the cost of prescription drugs is a top priority of the new administration and CMS is committed to considering opportunities to bring greater transparency in the negotiation program. The second cycle of negotiations with participating drug companies will occur during 2025, and any negotiated prices for this second set of drugs will be effective starting January 1, 2027.

On June 6, 2023, Merck & Co. filed a lawsuit against HHS and CMS asserting that, among other things, the IRA's Drug Price Negotiation Program for Medicare constitutes an uncompensated taking in violation of the Fifth Amendment of the Constitution. Subsequently, a number of other parties, including the U.S. Chamber of Commerce, Bristol Myers Squibb Company, the Pharmaceutical Research and Manufacturers of America, Novo Nordisk, Inc., Janssen Pharmaceuticals, Inc., Novartis AG, AstraZeneca plc and Boehringer Ingelheim International GMBH, also filed lawsuits in various courts with similar constitutional claims against HHS and CMS. HHS has generally won the substantive disputes in these cases or succeeded in getting claims dismissed for lack of standing. Most of these cases are now on appeal. On October 30, 2024, the U.S. Court of Appeals for the Third Circuit heard oral arguments in three of these cases. In April 2025, the U.S. Court of Appeals for the Second Circuit and the U.S. Court of Appeals for the Third Circuit heard arguments in an additional three cases. On May 8, 2025, the U.S. Court of Appeals for the Third Circuit rejected AstraZeneca L.P.'s challenge to the Medicare price negotiation program, finding that the program did not violate the company's due process rights under the Constitution since there is no protected property interest in selling goods to Medicare beneficiaries at a price higher than what the government is willing to pay in reimbursement. Litigation involving these and other provisions of the IRA will continue with unpredictable and uncertain results.

The Trump Administration has taken a number of actions to reduce the costs of pharmaceutical products. For example, on April 15, 2025, President Trump issued an Executive Order which directs HHS to take steps to reduce the prices of pharmaceutical products. Such measures include streamlining the state drug importation program and modifying provisions of the 340B program. Further, on May 12, 2025, President Trump issued an additional Executive Order calling on pharmaceutical manufacturers to voluntarily reduce the prices of medicines in the United States. The order provides that if such actions do not lower the costs of pharmaceuticals, the Secretary of HHS will pursue other actions, including proposing a rulemaking that imposes most-favored-nation, or MFN, pricing in the United States. Thereafter, on July 31, 2025, President Trump issued letters to 17 pharmaceutical companies reiterating the requirements of the May 12, 2025 Executive Order and demanding that such companies extend MFN pricing to Medicaid patients, guarantee MFN pricing for newly-launched drug products, return increased revenues abroad to American patients and provide for direct purchasing at MFN pricing. Since that time, virtually all of these pharmaceutical companies have entered into agreements with the Trump Administration to provide for lower prices on certain pharmaceuticals.

At the state level, individual states are increasingly aggressive in passing legislation and implementing 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. In addition, regional healthcare organizations 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. These measures could reduce the ultimate demand for our products, once approved, or put pressure on our product pricing. We expect that additional state and federal healthcare reform measures will be adopted in the future, any of which could limit the amounts that federal and state governments will pay for healthcare products and services, which could result in reduced demand for our product candidates or additional pricing pressures. This may be increasingly true with respect to products approved pursuant to the accelerated approval pathway. State Medicaid programs and other payers are developing strategies and implementing significant coverage barriers, or refusing to cover these products outright, arguing that accelerated approval drugs have insufficient or limited evidence despite meeting the FDA's standards for accelerated approval.

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 drug candidate to currently available therapies or so-called health technology assessments, in order to obtain reimbursement or pricing approval. For example, the EU provides options for its member states to restrict the range of products for which their national health insurance systems provide reimbursement and to control the prices of medicinal products for human use. EU Member States may approve a specific price for a product or it may instead adopt a system of direct or indirect controls on the profitability of the company placing the product on the market. Other EU Member States allow companies to fix their own prices for products, but monitor and control prescription volumes and issue guidance to physicians to limit prescriptions. Recently, many countries in the EU have increased the amount of discounts required on pharmaceuticals and these efforts could continue as countries attempt to manage healthcare expenditures, especially in light of the severe fiscal and debt crises experienced by many countries in the EU. The downward pressure on health care costs in general, particularly prescription drugs, has become intense. As a result, increasingly high barriers are being erected to the entry of new products. Political, economic and

regulatory developments may further complicate pricing negotiations, and pricing negotiations may continue after reimbursement has been obtained. Reference pricing used by various EU Member States, and parallel trade, i.e., 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 products, if approved in those countries.

Human Capital Resources

As of December 31, 2025, we had 511 team members employed with us full-time, of which 118 had a M.D. or Ph.D. degree. Of these team members, 399 were engaged in research and development activities and 112 were in general and administrative roles. None of our team members are represented by a labor union or covered by a collective bargaining agreement. The human capital measures and objectives we focus on in managing our business are our employee retention rate and our internal engagement survey participation rate. We seek to maintain an annual retention rate of greater than 85% and a survey participation rate of greater than 90%.

Available Information

Our website address is www.beamtx.com, and our investor relations website is located at investors.beamtx.com. Information on our website is not incorporated by reference herein. We will make available on our website, free of charge, our Annual Report on Form 10-K, Quarterly Reports on Form 10-Q, Current Reports on Form 8-K and any amendments to those reports filed or furnished pursuant to Section 13(a) or 15(d) of the Exchange Act, as soon as reasonably practicable after we electronically file such material with, or furnish it to, the SEC. The SEC maintains an Internet site (http://www.sec.gov) containing reports, proxy and information statements, and other information regarding issuers that file electronically with the SEC.

Investors and others should note that we announce material information to our investors using one or more of the following: SEC filings, press releases and our corporate website, including without limitation the "Investors & Media" section of our website. We use these channels, as well as social media channels such as X and LinkedIn, in order to achieve broad, non-exclusionary distribution of information to the public and for complying with our disclosure obligations under Regulation FD. It is possible that the information we post on our corporate website or other social media could be deemed to be material information. Therefore, we encourage investors, the media, and others interested in our company to review the information we post on the "Investor Center" section of our corporate website and on our social media channels. The contents of our corporate website and social media channels are not, however, a part of this Annual Report on Form 10-K.

Item 1A. Risk Factors.

You should carefully consider the risks and uncertainties described below together with all of the other information contained in this Annual Report on Form 10-K, including our consolidated financial statements and related notes appearing at the end of this Annual Report on Form 10-K, in evaluating our company. If any of the events or developments described below were to occur, our business, prospects, operating results and financial condition could suffer materially, and the trading price of our common stock could decline. The risks and uncertainties described below are not the only ones we face. Additional risks and uncertainties not presently known to us or that we currently believe to be immaterial may also adversely affect our business.

Risks related to our financial position and need for additional capital

We have incurred significant losses since inception. We expect to incur losses for the foreseeable future and may never achieve or maintain profitability.

Since inception, we have incurred significant operating losses. Our net loss was $80.0 million, $376.7 million and $132.5 million for the years ended December 31, 2025, 2024 and 2023, respectively. As of December 31, 2025, we had an accumulated deficit of $1.6 billion. We have financed our operations primarily through private placements of our preferred stock, proceeds from sales of our common stock, collaboration revenue and our credit facility with Sixth Street Lending Partners. We have devoted substantially all of our efforts to research and development. We expect to continue to incur significant expenses and increasing operating losses for the foreseeable future. The net losses we incur may fluctuate significantly from quarter to quarter. We anticipate that our expenses will increase substantially if and as we:

- advance clinical trials of our product candidates;
- continue our research programs and our preclinical development of other product candidates from our research programs;
- seek to identify additional research programs and additional product candidates;
- initiate preclinical testing and clinical trials for any other product candidates we identify and develop;
- maintain, expand, enforce, defend and protect our intellectual property portfolio and provide reimbursement of third-party expenses related to our patent portfolio;
- seek marketing approvals for any of our product candidates that successfully complete clinical trials;

- establish a sales, marketing, and distribution infrastructure to commercialize any medicines for which we may obtain marketing approval;
- further develop our base editing platform;
- hire additional personnel, including research and development, clinical, and commercial personnel;
- add operational, financial, and management information systems and personnel, including personnel to support our product development;
- acquire or in-license products, intellectual property, medicines, and technologies; and
- maintain and operate a commercial-scale cGMP manufacturing facility.

We have not completed any pivotal clinical trials of any product candidates and do not, and may never, have a product candidate approved for commercialization. To become and remain profitable, we must develop and, either directly or through collaborators, eventually commercialize a medicine or medicines with significant market potential. This will require us to be successful in a range of challenging activities, including identifying product candidates, completing preclinical studies and clinical trials of product candidates, obtaining marketing approval for these product candidates, manufacturing, marketing, and selling those medicines for which we may obtain marketing approval, and satisfying any post-marketing requirements. We may never succeed in these activities and, even if we do, may never generate revenues that are significant or large enough to achieve profitability. Because of the numerous risks and uncertainties associated with developing base editing product candidates, we are unable to predict the extent of any future losses or when we will become profitable, if at all. If we do 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 decrease the value of our company and could impair our ability to raise capital, maintain our research and development efforts, expand our business, or continue our operations.

We will need substantial additional funding. If we are unable to raise capital when needed, we would be forced to delay, reduce, or eliminate our research and product development programs or future commercialization efforts.

We expect our operating expenses to increase as we continue research and development activities, expand clinical trials, and seek marketing approval for product candidates. In addition, if we obtain marketing approval for any product candidates we may develop, we expect to incur significant commercialization expenses related to product sales, marketing, manufacturing, and distribution to the extent that such sales, marketing, manufacturing, and distribution are not the responsibility of a collaborator. Accordingly, we will need to obtain substantial additional funding in connection with our continuing operations. We have in the past delayed, reduced and eliminated certain research and product development programs to decrease operating expenses. If in the future we are unable to raise capital when needed or on attractive terms, we may again be forced to delay, reduce, or eliminate programs or curtail commercialization efforts.

At December 31, 2025, our cash, cash equivalents, and marketable securities were $1.2 billion. We believe that our existing cash, cash equivalents, and marketable securities will enable us to fund our operating expenses and capital expenditure requirements for at least the next 12 months. However, our operating plan may change as a result of factors currently unknown to us, and we may need to seek additional funding sooner than planned. Our future capital requirements will depend on many factors, including:

- the cost of continuing to build our base editing platform;
- the costs of acquiring licenses for the delivery modalities that will be used with our product candidates;
- the scope, progress, results, and costs of discovery, preclinical development, laboratory testing, manufacturing, and clinical trials for the product candidates we may develop;
- the costs of preparing, filing, and prosecuting patent applications, maintaining and enforcing our intellectual property and proprietary rights, and defending intellectual property-related claims;
- the costs, timing, and outcome of regulatory review of the product candidates we may develop;
- the costs of preparing for and undertaking future activities, including product sales, medical affairs, marketing, manufacturing, distribution, coverage and reimbursement for any product candidates for which we receive regulatory approval to commercialize;
- the success of our license agreements and our collaborations;
- our ability to establish and maintain additional license agreements and collaborations on favorable terms, if at all;
- the achievement of milestones or occurrence of other developments that trigger payments under any additional license agreements or collaboration agreements we obtain;
- the payment of success liabilities to Harvard and Broad Institute, should we choose to pay in cash;
- the extent to which our contingent liabilities require cash expenditures;
- the extent to which we acquire or in-license products, intellectual property and technologies;
- the costs of operating and expanding our manufacturing capacity; and
- the costs of establishing a sales, marketing, and distribution infrastructure to commercialize any product candidates for which we may obtain marketing approval.

Identifying potential product candidates and conducting preclinical studies and clinical trials is a time-consuming, expensive, and uncertain process that takes years to complete, and we may never generate the necessary data or results required to obtain marketing approval and achieve product sales. In addition, even if we successfully identify and develop product candidates and those product candidates are approved, we may not achieve commercial success. Our commercial revenues, if any, will be derived from sales of medicines that we do not expect to be commercially available for years, if ever. Accordingly, we will need to continue to rely on additional financing to achieve our business objectives. Adequate additional financing may not be available to us on acceptable terms, or at all.

Any additional fundraising efforts may divert the attention of our management from their day-to-day activities, which may adversely affect our ability to develop and, if approved, commercialize our product candidates. We cannot be certain that additional funding will be available to us on acceptable terms, or at all. Other than our credit facility with Sixth Street Lending Partners, we have no committed source of additional capital and, if we are unable to raise additional capital in sufficient amounts or on terms acceptable to us on a timely basis, we may have to significantly delay, scale back or discontinue the development or, if approved, commercialization of our product candidates or other research and development initiatives. For example, in October 2023, we announced a portfolio reprioritization and strategic restructuring, including cost-reduction initiatives which resulted in the pausing or elimination of certain pipeline programs.

Our current and any future license agreements and collaboration agreements may also be terminated if we are unable to meet the payment or other obligations under the agreements. We could be required to seek collaborators for product candidates we may develop at an earlier stage than otherwise would be desirable or on terms that are less favorable than might otherwise be available or relinquish or license on unfavorable terms our rights to product candidates we may develop in markets where we otherwise would seek to pursue development or commercialization ourselves.

If we are unable to obtain funding on a timely basis, we may also be unable to expand our operations or otherwise capitalize on our business opportunities, as desired, which could materially affect our business, financial condition and results of operations. Any of the above events could significantly harm our business, prospects, financial condition and results of operations and cause the price of our common stock to decline.

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

Until such time, if ever, as we can generate substantial product revenues, we expect to finance our cash needs through a combination of equity offerings, debt financings, collaborations, strategic alliances, and licensing arrangements. Other than our credit facility with Sixth Street Lending Partners, we do not have any committed external source of capital. To the extent that we raise additional capital through the sale of equity or convertible debt securities, your ownership interest will be diluted. The terms of these securities may include liquidation or other preferences that adversely affect your rights as a common stockholder. Our financing agreement with Sixth Street Lending Partners includes covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures, declaring dividends, and other restrictions, and future debt financings may contain similar restrictions.

If we raise funds through additional collaborations, strategic alliances, or licensing arrangements with third parties, we may have to relinquish valuable rights to our technologies, future revenue streams, research programs, or product candidates we may develop, or we may have to grant licenses on terms that may not be favorable to us. 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. In addition, we have and may in the future enter collaboration and acquisition agreements, pursuant to which we are required to issue additional shares of our common stock in connection with future milestone payment obligations. These and other future issuances to our partners and collaborators may cause substantial dilution to our stockholders.

Our short operating history may make it difficult for you to evaluate the success of our business to date and to assess our future viability.

We are an early-stage company. We were founded in January 2017 and began operations in July 2017. Our operations to date have been limited to organizing and staffing our company, business planning, raising capital, acquiring and developing our platform and technology, identifying potential product candidates, undertaking preclinical studies, operating clinical trials and preparing for the potential commercial launch of product candidates. Many of our product development programs are still in the early clinical, preclinical or research stage of development, and their risk of failure is high. We have not yet demonstrated an ability to successfully complete any pivotal clinical trials, obtain marketing approvals, manufacture a commercial-scale medicine, or arrange for a third party to do so on our behalf, or conduct sales and marketing activities necessary for successful commercialization. Typically, it takes about 10 to 15 years to develop a new medicine from the time it is discovered to when it is available for treating patients. 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.

Our limited operating history, particularly in light of the rapidly evolving base editing and gene editing field, may make it difficult to evaluate our technology and industry and predict our future performance. Our short history as an operating company makes any assessment of our future success or viability subject to significant uncertainty. We will encounter risks and difficulties frequently experienced by early-stage companies in rapidly evolving fields. If we do not address these risks successfully, our business will suffer.

In addition, as a new business, we may encounter other unforeseen expenses, difficulties, complications, delays, and other known and unknown factors. We are currently transitioning from a company with a research focus to a company capable of supporting commercial activities. We may not be successful in such a transition.

We have never generated revenue from product sales and may never become profitable.

Our ability to generate revenue from product sales and achieve profitability depends on our ability, alone or with collaborative partners, to successfully complete the development of, and obtain the regulatory approvals necessary to commercialize, product candidates we may identify for development. We do not anticipate generating revenues from product sales for the next several years, if ever. Our ability to generate future revenues from product sales depends heavily on our, or our collaborators', ability to successfully:

- identify product candidates and complete research and preclinical and clinical development of the product candidates we or our collaborators may identify;
- seek and obtain regulatory and marketing approvals for any of our product candidates for which we or our collaborators successfully complete clinical trials;
- launch and commercialize any of our product candidates for which we obtain regulatory and marketing approval by establishing a sales force, marketing, and distribution infrastructure or, alternatively, collaborating with a commercialization partner;
- qualify for adequate coverage and reimbursement by government and third-party payors for our product candidates for which we or our collaborators obtain regulatory and marketing approval;
- develop, maintain, and enhance a sustainable, scalable, reproducible, and transferable manufacturing process for the product candidates we or our collaborators may develop;
- maintain and operate a commercial-scale cGMP manufacturing facility;
- establish and maintain supply and manufacturing relationships with third parties that can provide adequate, in both amount and quality, products, and services to support clinical development and the market demand for our product candidates for which we or our collaborators obtain regulatory and marketing approval;
- obtain market acceptance of any product candidates we or our collaborators may develop as viable treatment options;
- address competing technological and market developments;
- implement internal systems and infrastructure, as needed;
- negotiate favorable terms in any collaboration, licensing, or other arrangements into which we may enter and performing our obligations in such collaborations, licensing or other arrangements;
- maintain, protect, enforce, defend, and expand our portfolio of intellectual property rights, including patents, trade secrets, and know-how;
- avoid and defend against third-party interference, infringement, and other intellectual property claims; and
- attract, hire, and retain qualified personnel.

Even if one or more of the product candidates we or our collaborators may develop are approved for commercial sale, we anticipate incurring significant costs associated with commercializing any approved product candidate. Our expenses could increase beyond expectations if we are required by the FDA, the EMA, or other regulatory authorities to perform clinical and other studies in addition to those that we currently anticipate. Even if we are able to generate revenues from the sale of any approved products, we may not become profitable and may need to obtain additional funding to continue operations.

Even if we do 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 decrease the value of our company and could impair our ability to raise capital, maintain our research and development efforts, expand our business or continue our operations.

Our future ability to utilize our net operating loss carryforwards and certain other tax attributes may be limited.

We have incurred substantial losses during our history, and we may never achieve profitability. To the extent that we continue to generate taxable losses, any unused U.S. federal losses generated after 2017 will carry forward indefinitely to offset future taxable income; any U.S. federal taxable losses generated prior to 2018 will carry forward for 20 tax years from the year of generation. Additionally, we continue to generate business tax credits, including U.S. federal research and development tax credits, which generally may be carried forward 20 tax years from the year of generation to offset a portion of our future tax liability, if any. Additionally, Sections 382 and 383 of the Internal Revenue Code of 1986, as amended, limit a corporation's ability to utilize tax attributes to the extent the corporation experiences an "ownership change," generally defined as a greater than 50 percentage point change in ownership, measured by value, among 5% or greater shareholders over a rolling three-year testing period. To the extent a corporation experiences an ownership change, utilization of pre-ownership change tax attributes (e.g., net operating losses and general business tax credits) to offset post-ownership change taxable income or taxes, is subject to an annual limitation, generally calculated as the pre-ownership change equity value of the corporation, subject to certain prescribed adjustments, multiplied by the long-term tax exempt rate published monthly by the Internal Revenue Service. We completed a Section 382 study as of December 31, 2024, and determined that no historical ownership changes occurred since December 2021. However, we may have experienced ownership changes since December 31, 2024 and may experience additional ownership changes in the future as a result of shifts in our stock ownership, some of which are outside of our control. As a result, if we earn net taxable income, our ability to use our pre-ownership change net operating losses or other tax attributes to offset U.S. federal taxable income or taxes may be subject to limitations, which could potentially result in increased future tax liability to us. There may also be limitations on our ability to use net operating losses and business tax credits at the state level. Additional limitations on our ability to utilize our net operating losses and other tax attributes to offset future taxable income or taxes may arise as a result of our corporate structure, whereby net operating losses or other tax attributes generated by certain of our subsidiaries or controlled entities may not be available to offset taxable income or taxes of other subsidiaries or controlled entities.

There is also a risk that due to regulatory changes or other unforeseen reasons, our existing net operating losses or business tax credits could expire or otherwise become unavailable to offset future income tax liabilities. At the state level, there may also be periods during which the use of net operating losses or business tax credits is suspended or otherwise limited, which could accelerate or permanently increase state taxes owed. In addition, state net operating losses generated in one state cannot be used to offset income generated in another state. For these reasons, we may not be able to realize a tax benefit from the use of our net operating losses or tax credits, even if we attain profitability.

The terms of our financing agreement with Sixth Street Lending Partners and our indebtedness could adversely affect our operations and limit our ability to plan for or respond to changes in our business. If we are unable to comply with restrictions in the financing agreement, the repayment of our existing indebtedness could be accelerated.

On February 24, 2026, we entered into a financing agreement with Sixth Street Lending Partners as the administrative agent and collateral agent for several lenders, or the Financing Agreement. The Financing Agreement establishes a senior secured term loan facility of $500.0 million, or the Credit Facility, consisting of $100.0 million funded on the closing date; up to $300 million available upon the achievement of certain clinical, regulatory and commercial milestones for risto-cel; and an additional $100 million available at our option, subject to mutual agreement between the parties, during the seven-year term of the Financing Agreement.

The Financing Agreement contains customary representations and warranties and affirmative and negative covenants. For example, it requires us to make certain payments over time and, subject to certain exceptions, restricts our ability to incur additional indebtedness, grant liens, make investments (including acquisitions), effectuate mergers or consolidations, engage in asset sales and licensing transactions, pay dividends, modify material agreements, pay subordinated indebtedness, and undertake other matters customarily restricted in such agreements. Among other requirements of the Financing Agreement, we and our subsidiaries party to the Financing Agreement must maintain certain minimum liquidity requirements if our market capitalization falls below an agreed threshold. We are also subject to restrictions on sales and licensing transactions with respect to our core intellectual property and product assets, including, but not limited to, risto-cel and BEAM-302, subject to certain exceptions. These and other terms in the Financing Agreement could restrict our ability to grow our business or enter into transactions that we believe would be beneficial to our business. Additionally, if we fail to comply with the covenants under the Financing Agreement, it will result in an event of default. Upon the occurrence of an event of default, and subject to any specified cure periods, all amounts owed under the Financing Agreement may be declared immediately due and payable by the lenders, and the lenders may foreclose on the collateral, which could have a material adverse effect on our business, financial condition and results of operations.

Our current and potential indebtedness could affect our business in the following ways, among other things: make it more difficult for us to satisfy our contractual and commercial commitments; require us to use a substantial portion of our cash flow subject to mandatory prepayments to pay interest and principal when due, which would reduce funds available for working capital, capital expenditures and other general corporate purposes; limit our ability to obtain additional financing for working capital, capital expenditures, acquisitions and other investments or general corporate purposes; heighten our vulnerability to downturns in our business, our industry or in the general economy; place us at a disadvantage compared to our competitors that may have

proportionately less debt; limit management's discretion in operating our business; and limit our flexibility in planning for, or reacting to, changes in our business, the industry in which we operate or the general economy.

Our business may not generate cash flows from operations in the future that are sufficient to service our debt and support our growth strategies. In addition, our ability to generate sufficient cash flows to meet our debt obligations depends upon several factors, such as the ability of us and our licensees to timely complete clinical trials and obtain marketing approval for our clinical-stage product candidates, to successfully commercialize our clinical-stage product candidates, our receipt of regulatory approval for risto-cel and our other product candidates, and our future performance, which is subject to financial, business, and other impacts on our operations, many of which are beyond our control. If we are unable to generate sufficient cash flows, we may be required to adopt one or more alternatives, such as obtaining additional equity capital on terms that may be onerous or highly dilutive, selling assets, or restructuring debt. Our ability to refinance our indebtedness will depend on the capital markets and our financial condition at such time. We may not be able to engage in any of these activities or engage in these activities on desirable terms, which could result in a default on our debt obligations.

Risks related to discovery, development, and commercialization

Base editing is a novel technology that has only recently been clinically validated for human therapeutic use. The approaches we are taking to discover and develop novel therapeutics are early in their development and may never lead to marketable products.

We are focused on developing potentially curative medicines utilizing base editing technology. Although there have been significant advances in the field of gene therapy, which typically involves introducing a copy of a gene into a patient's cell, and gene editing in recent years, including the approval of an *ex vivo* nuclease editing product for treatment of sickle cell disease, base editing technologies are new and early in their development. The technologies that we have licensed and are developing have not yet completed any clinical trials. The scientific evidence to support the feasibility of developing product candidates based on these technologies is both preliminary and limited, and base editing and delivery modalities for it are novel. Successful development of product candidates by us will require solving a number of issues, including safely delivering a therapeutic into target cells within the human body or in an *ex vivo* setting, optimizing the efficiency and specificity of such product candidates, and ensuring the therapeutic selectivity of such product candidates. Several biological steps are required for delivery of base editing medicines to translate into therapeutically active medicines. These processing steps may differ between individuals and differ based on the targeted tissue. These differences could lead to variable levels of therapeutic protein, variable activity, immunogenicity, or variable distribution to tissues further increasing the risk inherent in the development of base editing medicines. There can be no assurance we will be successful in solving any or all of these issues, or that we will be able to progress our preclinical studies or clinical trials in accordance with anticipated timelines.

We have only recently brought therapeutics to the clinic, and our future success is highly dependent on the successful development of base editing technologies, cellular delivery methods and therapeutic applications of that technology. While other gene editing technologies have progressed through clinical trials, they continue to suffer from various limitations, and such limitations may affect our future success. We may decide to alter or abandon our initial programs as new data become available and we gain experience in developing base editing therapeutics. We cannot be sure that our technologies will yield satisfactory products that are safe and effective, scalable or profitable in our initial indications or any other indication we pursue.

Development activities in the field of base editing are currently subject to a number of risks related to the ownership and use of certain intellectual property rights that are subject to patent interference proceedings in the United States and opposition proceedings in Europe. For additional information regarding the risks that may apply to our and our licensors' intellectual property rights, see the section entitled "—Risks related to our intellectual property".

We are early in our development efforts. Our product candidates are still in preclinical or clinical development and we have not, and may never, commercialize a product candidate. If we are unable to advance our product candidates through clinical development, obtain regulatory approval and ultimately commercialize our product candidates, or experience significant delays in doing so, our business will be materially harmed.

We are early in our development efforts and our future success depends heavily on the successful development of our base editing product candidates and the results of our clinical trials, none of which have yet been completed. Our ability to generate product revenue will depend heavily on the successful development and, if approved, eventual commercialization of our product candidates, which may never occur. We currently generate no revenue from sales of any product, and we may never be able to develop or commercialize a marketable product.

Commencing clinical trials in the United States is subject to acceptance by the FDA of our investigational new drug applications, or INDs, and finalizing the trial design based on discussions with the FDA and other regulatory authorities. Similarly, in the European Union, or EU, a CTA must be obtained from each member state's national competent authority where the study is conducted, and a positive opinion of an independent ethics committee.

Even after we receive and incorporate guidance from these regulatory authorities, the FDA, European national competent authority, or other regulatory authorities could disagree that we have satisfied their requirements to commence our clinical trial or change their

position on the acceptability of our data, trial design or the clinical endpoints selected, which may require us to complete additional preclinical studies or clinical trials or impose stricter requirements for approval than we currently expect.

In addition, changes in marketing approval policies during the development period, changes in or the enactment or promulgation of additional statutes, regulations or guidance or changes in regulatory review for each submitted product application, may cause delays in the approval or rejection of an application. For example, in December 2022, with the passage of FDORA, Congress required sponsors to develop and submit a DAP for each Phase 3 clinical trial or any other "pivotal study" of a new drug or biological product. These plans are meant to encourage the enrollment of more diverse patient populations in late-stage clinical trials of FDA-regulated products. In June 2024, as mandated by FDORA, the FDA issued draft guidance outlining the general requirements for DAPs. Unlike most guidance documents issued by the FDA, the DAP guidance when finalized will have the force of law because FDORA specifically dictates that the form and manner for submission of DAPs are specified in FDA guidance. On January 27, 2025, in response to an Executive Order issued by President Trump on January 21, 2025, on Diversity, Equity and Inclusion programs, the FDA removed this draft guidance from its website. Thereafter, following litigation, the FDA was directed by a federal district court to restore the draft guidance to the FDA website. When the FDA did so, it stated that "information on this page may be modified and/or removed in the future subject to the terms of the court's order and implemented consistent with applicable law." Accordingly, in light of these ongoing actions, there is considerable uncertainty surrounding the draft DAP guidance and how the FDA will consider diversity action plans in connection with its review of BLAs.

Further, in January 2022, the new Clinical Trials Regulation (EU) No 536/2014 became effective in the European Union and replaced the prior Clinical Trials Directive 2001/20/EC. This regulation aims at simplifying and streamlining the authorization, conduct and transparency of clinical trials in the European Union. Under the coordinated procedure for the approval of clinical trials, the sponsor of a clinical trial to be conducted in more than one EU Member State will only be required to submit a single application for approval. The submission will be made through the Clinical Trials Information System, a clinical trials portal overseen by the European Medicines Agency, or EMA, and available to clinical trial sponsors, competent authorities of the EU Member States and the public.

Commercialization of our product candidates we may develop will require additional preclinical and clinical development; regulatory and marketing approval in any jurisdictions where our product candidates would be marketed, including by the FDA for the U.S. market and the European Commission upon a positive benefit/risk assessment provided by the EMA in the EEA; obtaining or creating manufacturing supply, capacity and expertise; building of a commercial organization; and significant marketing efforts. The success of product candidates we identify and develop will depend on many factors, including the following:

- sufficiency of our financial and other resources to complete the necessary preclinical studies, IND/CTA-enabling studies, and clinical trials;
- regulatory clearance of IND applications, CTAs or comparable foreign applications that allow commencement of our planned clinical trials or future clinical trials for our product candidates;
- successful enrollment in, and completion of, clinical trials in accordance with all applicable current Good Clinical Practice guidelines, or GCPs, current Good Laboratory Practice guidelines adopted by the International Conference on Harmonization of Technical Requirements for Pharmaceuticals for Human Use, or ICH, and other regulatory requirements from foreign regulatory authorities;
- receipt of marketing approvals and, where required, pricing and reimbursement decisions from applicable regulatory authorities;
- establishment of arrangements with third-party manufacturers for clinical supply and commercial manufacturing and, where applicable, commercial manufacturing capabilities;
- successful development of our internal manufacturing processes and transfer to larger-scale facilities operated by either a contract manufacturing organization, or CMO, or by us;
- obtaining and maintaining patent, trade secret, and other intellectual property protection and non-patent exclusivity for our medicines;
- launching commercial sales of the medicines, if and when approved, whether alone or in collaboration with others;
- acceptance of the products, if and when approved, by patients, the medical community, and third-party payors;
- effectively competing with other therapies and treatment options;
- a continued acceptable safety profile of the medicines following approval;
- enforcing and defending intellectual property and proprietary rights and claims; and
- supplying the product at a price that is acceptable to the pricing or reimbursement authorities in different countries.

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

If any of the product candidates we may develop, or the delivery modalities we rely on to administer them, cause serious adverse events, undesirable side effects, or unexpected characteristics, such events, side effects or characteristics could delay or prevent regulatory approval of the product candidates, limit the commercial potential, or result in significant negative consequences following any potential marketing approval.

We have not completed pivotal clinical trials of any of our product candidates. Moreover, there have been only a limited number of clinical trials involving the use of base editing technology similar to our technology. We do not yet know whether any product candidates we develop will prove safe in humans. In the genetic medicine field, there have been several significant adverse events from gene therapy treatments in the past, including reported cases of leukemia, serious blood disorders and death. There can be no assurance that base editing technologies, or components of our product candidates or methods of delivery, will not cause undesirable side effects, as improper editing of a patient's DNA and other effects could lead to lymphoma, leukemia, or other cancers, other serious conditions or syndromes or other aberrantly functioning cells.

A significant risk in any base editing product candidate is that "off-target" edits may occur, which could cause serious adverse events, undesirable side effects or unexpected characteristics. For example, Erwei Zuo et al. reported that cytosine base editors generated substantial off-target edits, that is, edits in unintended locations on the DNA, when tested in mouse embryos. Such unintended edits are referred to as "spurious deamination." We cannot be certain that off-target editing will not occur in any of our planned or future clinical studies, and the lack of observed side effects in preclinical studies does not guarantee that such side effects will not occur in human clinical studies. We have developed assays that can detect off-target edits, even when such edits occur at very low frequencies. Using these assays, we have observed off-target edits in our base editing product candidates. As the sensitivity of these assays increases, it is possible that we will continue to detect more such off-target edits. While we do not believe that the off-target edits we have observed to date have had a material adverse impact on the safety or benefit of our product candidates, if, in the future, we detect off-target edits for a product candidate that negatively impact safety or efficacy, our ability to develop the product candidate as a therapeutic could be adversely affected.

There is also the potential risk of delayed adverse events following exposure to base editing therapy due to the permanence of edits to DNA or due to other components of product candidates used to carry the genetic material. Further, because base editing makes a permanent change, the therapy cannot be withdrawn, even after a side effect is observed. In addition, Rees et al. and Grunewald et al. have reported that the deaminases we currently use in our C base editors and our A base editors for use in DNA base editing also cause unintended mutations in RNA for as long as the editor is present in the cell.

Although we and others have demonstrated the ability to engineer base editors to improve the specificity of their edits in a laboratory setting, we cannot be sure that our engineering efforts will be effective in any product candidates that we may develop. For example, we might not be able to engineer an editor to make the desired change or a by-stander edit could diminish the effectiveness of an edit that we make.

In certain of our programs, we plan to use LNPs to deliver our base editors. LNPs have been shown to induce oxidative stress in the liver at certain doses, as well as initiate systemic inflammatory responses that can be fatal in some cases. While we aim to continue to optimize our LNPs, there can be no assurance that our LNPs will not have undesired effects. Our LNPs could contribute, in whole or in part, to one or more of the following: immune reactions; infusion reactions; complement reactions; opsonization reactions; antibody reactions including IgA, IgM, IgE or IgG or some combination thereof; or reactions to the polyethylene glycol from some lipids or polyethylene glycol otherwise associated with the LNP. Certain aspects of our investigational medicines may induce immune reactions from either the mRNA or the lipid as well as adverse reactions within liver pathways or degradation of the mRNA or the LNP, any of which could lead to significant adverse events in one or more of our current or future clinical trials. Many of these types of side effects have been seen for legacy LNPs. There may be uncertainty as to the underlying cause of any such adverse event, which would make it difficult to accurately predict side effects in future clinical trials and would result in significant delays in our programs.

In addition to side effects and adverse events caused by our product candidates, the conditioning administration process or related procedures used in risto-cel and potentially other *ex vivo* product candidates also can cause adverse side effects and adverse events. For example, a patient in our BEACON trial for the treatment of sickle cell disease died due to respiratory failure four months after busulfan conditioning and infusion with risto-cel. While the investigator determined that the patient's death was likely related to busulfan conditioning and deemed the event unrelated to risto-cel, we cannot be certain of these conclusions, nor that other patients will not experience adverse events from the trial's conditioning regimen or otherwise. If in the future we are unable to demonstrate that such adverse events were not caused by the conditioning regimens used, or administration process or related procedure, the FDA, the European Commission, EMA or other regulatory authorities could order us to cease further development of, or deny or limit approval of, our product candidates using such regimens, processes or procedures for any or all target indications. Even if we are able to demonstrate that adverse events are not related to the product candidate or the administration of such product candidate, such occurrences could affect patient recruitment, the ability of enrolled patients to complete the clinical trial, or the commercial viability of any product candidates that obtain regulatory approval.

If any product candidates we develop are associated with serious adverse events, undesirable side effects, or unexpected characteristics, we may need to abandon their development or limit development to certain uses or subpopulations in which the serious

adverse events, undesirable side effects or other characteristics are less prevalent, less severe, or more acceptable from a risk-benefit perspective, any of which would have a material adverse effect on our business, financial condition, results of operations, and prospects. Many product candidates that initially showed promise in early stage testing for treating cancer or other diseases have later been found to cause side effects that prevented further clinical development of the product candidates.

If in the future we are unable to demonstrate that any of the above adverse events were caused by factors other than our product candidate, the FDA, the EMA or other regulatory authorities could order us to cease further development of, or deny approval of, any product candidates we are able to develop for any or all targeted indications. Even if we are able to demonstrate that all future 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 delay, suspend or terminate any clinical trial of any product candidate we may develop, 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 identify and develop product candidates, and may harm our business, financial condition, result of operations, and prospects significantly.

If we successfully develop a product candidate and it receives marketing approval, we are required to present a comprehensive, concise, and critical analysis of new or emerging information on the risks of the approved product and on its benefit in approved indications, to enable an appraisal of benefit-risk profile in a Periodic Benefit Risk Evaluation Report, or PBRER, according to an internationally harmonized standard. Additionally, the FDA or a comparable regulatory authority such as the EMA could require us to adopt a Risk Evaluation and Mitigation Strategy, or REMS, or similar requirement such as Risk Management Plan, or RMP, to ensure that the benefits of treatment with such product candidate outweigh the risks for each potential patient, which may include, among other things, a medication guide outlining the risks of the product for distribution to patients, a communication plan to health care practitioners, extensive patient monitoring, or distribution systems and processes that are highly controlled, restrictive, and more costly than what is typical for the industry. FDA, EMA, or other comparable regulatory authorities may require specific post-approval trials to be carried out to further characterize the clinical efficacy and/or safety of the product candidate. Furthermore, if we or others later identify undesirable side effects caused by any product candidate that we develop, several potentially significant negative consequences could result, including:

- regulatory authorities may suspend or withdraw approvals of such product candidate;
- regulatory authorities may require additional warnings on the label or limit the approved use of such product candidate;
- we may be required to conduct additional clinical trials;
- we could be sued and held liable for harm caused to patients; and
- our reputation may suffer.

Any of these events could prevent us from achieving or maintaining market acceptance of any product candidates we may identify and develop and could have a material adverse effect on our business, financial condition, and results of operations.

We have only recently begun testing our proposed delivery modalities and product candidates in clinical trials and any favorable preclinical results, or initial clinical results, are not necessarily predictive of results that may be observed in later clinical trials.

There is a high failure rate for drugs and biologics proceeding through clinical trials. Even if initial clinical trials in any of our product candidates are successful, these product candidates may fail to show the desired safety, purity and potency in later stages of clinical development despite having successfully advanced through preclinical studies and initial clinical trials. Data obtained from preclinical and clinical activities are also subject to varying interpretations, and the FDA, EMA, or other regulatory authorities may disagree with our interpretations, which may delay, limit, or prevent regulatory approval. In addition, regulatory delays or rejections may be encountered as a result of many factors, including changes in regulatory policy during the period of product development.

Any such adverse events may cause us to delay, limit, or terminate ongoing or planned clinical trials, any of which would have a material adverse effect on our business, financial condition, results of operations, and prospects.

If we experience delays or difficulties in the enrollment or treatment of patients in clinical trials, our receipt of necessary regulatory approvals could be delayed or prevented.

We or our collaborators may not be able to initiate or continue clinical trials for any product candidates we identify or develop if we are unable to locate, enroll, and treat a sufficient number of eligible patients in these trials as required by the FDA, the EMA or other analogous regulatory authorities outside the United States, or as needed to provide appropriate statistical power for a given trial. Enrollment may be particularly challenging for some of the rare genetically defined diseases we are targeting in our most advanced programs, as well as for some of our product candidates for pediatric populations, due to a number of factors, including small patient populations as well as screening and testing requirements that limit patient eligibility. In addition, if patients are unwilling to participate in our base editing trials because of negative publicity from adverse events related to the biotechnology, gene therapy, or gene editing fields, competitive clinical trials for similar patient populations, clinical trials in competing products, or for other reasons, the timeline for recruiting patients, conducting studies, and obtaining regulatory approval of any product candidates we may develop

may be delayed. Moreover, some of our competitors currently and may in the future have ongoing clinical trials for product candidates that treat the same indications as product candidates we are developing and may develop in the future, and patients who would otherwise be eligible for our clinical trials may instead enroll in clinical trials of our competitors' product candidates. Treatment of enrolled patients may also be delayed or prevented due to a number of factors, including the complexity of our trials.

Clinical trial patient enrollment is also affected by other factors, including:

- severity of the disease under investigation;
- size of the patient population and process for identifying patients;
- design of the trial protocol;
- availability and efficacy of approved medications for the disease under investigation;
- availability of genetic testing for potential patients;
- ability to obtain and maintain patient informed consent;
- risk that enrolled patients will drop out before completion of the trial;
- eligibility and exclusion criteria for the trial in question;
- perceived risks and benefits of the product candidate under trial;
- perceived risks and benefits of base editing as a therapeutic approach;
- efforts to facilitate timely enrollment in clinical trials;
- patient referral practices of physicians;
- ability to monitor patients adequately during and after treatment; and
- proximity and availability of clinical trial sites for prospective patients, especially for those conditions which have small patient pools.

Our ability to successfully initiate, enroll, and complete a clinical trial in any foreign country is subject to numerous risks unique to conducting business in foreign countries, including:

- difficulty in establishing or managing relationships with contract research organizations, or CROs, and physicians;
- different standards for the conduct of clinical trials;
- different standard-of-care for patients with a particular disease;
- difficulty in locating qualified local consultants, physicians, and partners; and
- potential burden of complying with a variety of foreign laws, medical standards, and regulatory requirements, including the regulation of pharmaceutical and biotechnology products and treatment and of gene editing technologies.

Enrollment or treatment delays in our clinical trials may result in increased development costs for any product candidates we may develop, which would cause the value of our company to decline and limit our ability to obtain additional financing. If we or our collaborators have difficulty enrolling or treating a sufficient number of patients to conduct our clinical trials as planned, we may need to delay, limit, or terminate ongoing or planned clinical trials, any of which would have an adverse effect on our business, financial condition, results of operations, and prospects.

If clinical trials of any product candidates we identify and develop fail to demonstrate safety, purity and potency to the satisfaction of regulatory authorities or do not otherwise produce positive results, we may incur additional costs or experience delays in completing, or ultimately be unable to complete, the development and commercialization of such product candidates.

Before obtaining marketing approval from regulatory authorities for the sale of any product candidates we identify and develop, we must complete preclinical development and then conduct extensive clinical trials to demonstrate their safety, purity and potency in humans. Clinical testing is expensive, difficult to design and implement, can take many years to complete, and is uncertain as to outcome. A failure of one or more clinical trials can occur at any stage of testing. The outcome of preclinical studies and early clinical trials may not be predictive of the success of later clinical trials, and interim results of a clinical trial do not necessarily predict final results.

Moreover, preclinical and clinical data are often susceptible to varying interpretations and analyses. Many companies that have believed their product candidates performed satisfactorily in preclinical studies and clinical trials have nonetheless failed to obtain marketing approval of their product candidates.

We and our collaborators 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 any product candidates we identify and develop, including:

- delays in reaching a consensus with regulators on trial design and endpoints;
- regulators, institutional review boards, or IRBs, or independent ethics committees may not authorize us or our investigators to commence a clinical trial or conduct a clinical trial at a prospective trial site;
- delays in reaching or failing to reach agreement on acceptable clinical trial contracts or clinical trial protocols with prospective CROs and clinical trial sites;
- clinical trials of any product candidates we may develop may produce negative or inconclusive results, and we may decide, or regulators may require us, to conduct additional clinical trials or abandon product development or research programs;
- difficulty in designing well-controlled clinical trials due to ethical considerations which may render it inappropriate to conduct a trial with a control arm that can be effectively compared to a treatment arm;
- difficulty in designing clinical trials and selecting endpoints for diseases that have not been well-studied and for which the natural history and course of the disease is poorly understood;
- the number of patients required for clinical trials of any product candidates we develop may be larger than we anticipate; enrollment of suitable participants in these clinical trials, which may be particularly challenging for some of the rare genetically defined diseases we are targeting in our most advanced programs, may be delayed or slower than we anticipate; or patients 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;
- regulators, IRBs, or independent ethics committees may require that we or our investigators suspend or terminate clinical research or clinical trials of any product candidates we develop for various reasons, including noncompliance with regulatory requirements, a finding of undesirable side effects or other unexpected characteristics, or that the participants are being exposed to unacceptable health risks or after an inspection of our clinical trial operations or trial sites;
- the cost of clinical trials of any product candidates we may develop may be greater than we anticipate;
- the supply or quality of any product candidates we may develop or other materials necessary to conduct clinical trials of any product candidates we develop may be insufficient or inadequate, including as a result of delays in the testing, validation, manufacturing, and delivery of any product candidates we may develop to the clinical sites by us or by third parties with whom we have contracted to perform certain of those functions;
- delays in having patients complete participation in a trial or return for post-treatment follow-up;
- delays in or cancellations of clinical trials as a result of efforts by the Trump Administration to reduce research funding by the NIH of medical research;
- clinical trial sites dropping out of a trial;
- selection of clinical endpoints that require prolonged periods of clinical observation or analysis of the resulting data;
- occurrence of serious adverse events associated with any product candidates we may develop that are viewed to outweigh their potential benefits;
- occurrence of serious adverse events in trials of the same class of agents conducted by other sponsors;
- disruption to the operations of the FDA, EMA or other relevant regulatory authority; and
- changes in regulatory requirements and guidance that require amending or submitting new clinical protocols or otherwise complying with additional requirements.

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

- be delayed in obtaining marketing approval for any such product candidates we may develop or not obtain marketing approval at all;
- obtain approval for indications or patient populations that are not as broad as intended or desired;
- obtain approval with labeling that includes significant use or distribution restrictions or safety warnings, including boxed warnings;
- be subject to changes in the way the product is administered;
- be required to perform additional clinical trials to support approval or be subject to additional post-marketing testing requirements;
- have regulatory authorities withdraw, or suspend, their approval of the product or impose restrictions on its distribution in the form of a REMS or through modification to an existing REMS;
- be sued; or
- experience damage to our reputation.

Product development costs will also increase if we or our collaborators experience delays in clinical trials or other testing or in obtaining marketing approvals. We do not know whether any clinical trials will begin as planned, will need to be restructured, or will be completed on schedule, or at all. Significant clinical trial delays also could shorten any periods during which we may have the exclusive right to commercialize any product candidates we may develop, could allow our competitors to bring products to market before we do, and could impair our ability to successfully commercialize any product candidates we may develop, any of which may harm our business, financial condition, results of operations, and prospects.

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

Because we have limited financial, scientific and managerial resources, we focus on research programs and product candidates that we identify for specific indications among many potential options. As a result, we may forego or delay pursuit of opportunities with other product candidates or for other indications that later prove to have greater commercial potential. Our resource allocation decisions may cause us to fail to capitalize on viable commercial products or profitable market opportunities. Our spending on current and future research and development programs and product candidates for specific indications may not yield any commercially viable medicines. 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. For example, in October 2023, we implemented a strategic restructuring to prioritize development of our *ex vivo* and *in vivo* sickle cell disease programs, as well as our *in vivo* base editor BEAM-302. While we may identify new collaboration partners who can progress some of these programs, we may not be successful in doing so in a timely manner, on acceptable terms or at all. We may otherwise fail to raise sufficient additional capital in order to progress these programs ourselves or we may determine, for internal resource allocation purposes or for other reasons, to abandon development of these programs. As a result, we could miss valuable opportunities to capitalize on the potential of the programs. We may also allocate internal resources to a product candidate in a therapeutic area in which it would have been more advantageous to enter into a collaboration or that does not prove to have viable commercial opportunities. Any failure to use our financial and human resources efficiently could harm our business and operations.

We are conducting clinical trials at sites outside the United States. The FDA may not accept data from trials conducted in such locations, and the conduct of trials outside the United States could subject us to additional delays and expense.

We are conducting clinical trials with one or more trial sites that are located outside the United States. The acceptance by the FDA or other regulatory authorities of trial data from clinical trials conducted outside their jurisdiction may be subject to certain conditions or may not be accepted at all. In cases where data from foreign clinical trials are intended to serve as the sole basis for marketing approval in the U.S., the FDA will generally not approve the application on the basis of foreign data alone unless (i) the data are applicable to the U.S. population and U.S. medical practice; (ii) the trials were performed by clinical investigators of recognized competence and pursuant to GCP regulations; and (iii) the data may be considered valid without the need for an on-site inspection by the FDA, or if the FDA considers such inspection to be necessary, the FDA is able to validate the data through an on-site inspection or other appropriate means.

In addition, even where the foreign trial data are not intended to serve as the sole basis for approval, the FDA will not accept the data as support for an application for marketing approval unless the trial is well-designed and well-conducted in accordance with GCP requirements and the FDA is able to validate the data from the trial through an onsite inspection if deemed necessary. Many foreign regulatory authorities have similar approval requirements. In addition, such foreign trials would be subject to the applicable local laws of the foreign jurisdictions where the trials are conducted. There can be no assurance that the FDA or any comparable foreign regulatory authority will accept data from trials conducted outside of the U.S. or the applicable jurisdiction. If the FDA or any

comparable foreign regulatory authority does not accept such data, it would result in the need for additional trials, which could be costly and time-consuming, and which may result in current or future product candidates that we may develop not receiving approval for commercialization in the applicable jurisdiction.

Conducting clinical trials outside the U.S. also exposes us to additional risks, including risks associated with:

- additional foreign regulatory requirements;
- foreign exchange fluctuations;
- compliance with foreign manufacturing, customs, shipment and storage requirements;
- cultural differences in medical practice and clinical research;
- diminished protection of intellectual property in some countries; and
- interruptions or delays in our trials resulting from geopolitical events, such as war or terrorism.

Even if we complete the necessary clinical trials, we cannot predict when, or if, we will obtain regulatory approval to commercialize a product candidate we develop in the United States or any other jurisdiction, and any such approval may be for a narrower indication than we seek.

We cannot commercialize a product candidate until the appropriate regulatory authorities have reviewed and approved the product candidate. Even if any product candidates we may develop meet their safety, purity and potency endpoints in clinical trials, the regulatory authorities may not complete their review processes in a timely manner, or we may not be able to obtain regulatory approval. Additional delays may result if an FDA Advisory Committee, EMA's Committee for Medicinal Products for Human Use, or CHMP, or other regulatory authority recommends non-approval or restrictions on approval. In addition, we may experience delays or rejections based upon additional government regulation from future legislation or administrative action, or changes in regulatory authority policy during the period of product development, clinical trials, and the review process.

Regulatory authorities also may approve a product candidate for more limited indications than requested or they may impose significant limitations in the form of narrow indications, warnings or a REMS or an RMP. These regulatory authorities may require labeling that includes precautions or contra-indications with respect to conditions of use, or they may grant approval subject to the performance of costly post-marketing clinical trials. In addition, regulatory authorities may not approve the labeling claims that are necessary or desirable for the successful commercialization of any product candidates we may develop. Any of the foregoing scenarios could materially harm the commercial prospects for any product candidates we may develop and materially adversely affect our business, financial condition, results of operations, and prospects.

Marketing approval by the FDA in the United States, or by the EMA in the EEA, if obtained, does not ensure approval by regulatory authorities in other countries or jurisdictions. 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 candidate testing and validation and additional administrative review periods. Seeking foreign regulatory approval could result in difficulties and 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 product candidates we may develop in those countries. The foreign regulatory approval process involves all of the risks associated with FDA approval. We do not have any product candidates approved for sale in any jurisdiction, including international markets, and we do not have experience in obtaining regulatory approval 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 our product candidates will be unrealized.

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

The commercial success of any of our product candidates we may develop will depend upon its degree of market acceptance by physicians, patients, third-party payors, and others in the medical community. Ethical, social, and legal concerns about genetic medicines generally and base editing technologies specifically could result in additional regulations restricting or prohibiting the marketing of our product candidates we may develop. Even if any product candidates we may develop receive marketing approval, they may nonetheless fail to gain sufficient market acceptance by physicians, patients, healthcare payors, and others in the medical community. The degree of market acceptance of any product candidates we may develop, if approved for commercial sale, will depend on a number of factors, including:

- the efficacy and safety of such product candidates as demonstrated in clinical trials;
- the potential and perceived advantages compared to alternative treatments;

- the limitation to our targeted patient population and limitations or warnings contained in approved labeling by the FDA, EMA, or other regulatory authorities;
- the ability to offer our medicines for sale at competitive prices;
- convenience and ease of administration compared to alternative treatments;
- the clinical indications for which the product candidate is approved by the FDA, the EMA, or other regulatory agencies;
- public attitudes regarding genetic medicine generally and gene editing and base editing technologies specifically;
- the willingness of the target patient population to try novel therapies and of physicians to prescribe these therapies, as well as their willingness to accept a therapeutic intervention that involves the editing of the patient's gene;
- product labeling or product insert requirements of the FDA, the European Commission, the EMA, or other regulatory authorities, including any limitations or warnings contained in a product's approved labeling;
- relative convenience and ease of administration;
- the timing of market introduction of competitive products;
- publicity concerning our products or competing products and treatments;
- the strength of marketing and distribution support;
- sufficient third-party coverage or reimbursement; and
- the prevalence and severity of any side effects.

Even if any of our product candidates we may develop are approved, such products may not achieve an adequate level of acceptance, we may not generate significant product revenues, and we may not become profitable.

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

We are early in our commercial readiness activities, do not yet have a sales or marketing infrastructure and do not have experience in the sale, marketing, or distribution of pharmaceutical products. To achieve commercial success for any approved medicine for which we retain sales and marketing responsibilities, we must either develop a sales and marketing organization or outsource these functions to third parties. In the future, we may choose to build a focused sales, marketing, and commercial support infrastructure to sell, or participate in sales activities with our collaborators for, some of our product candidates we develop if and when they are approved.

There are risks involved with both establishing our own commercial capabilities and entering into arrangements with third parties to perform these services. For example, recruiting and training a sales force or reimbursement specialists is expensive and time consuming and could delay any product launch. If the commercial launch of a product candidate for which we recruit a sales force and establish marketing and other commercialization capabilities is delayed or does not occur for any reason, we would have prematurely or unnecessarily incurred these commercialization expenses. This may be costly, and our investment would be lost if we cannot retain or reposition our commercialization personnel.

Factors that may inhibit our efforts to commercialize our product candidates we may develop on our own include:

- our inability to recruit and retain adequate numbers of effective sales, marketing, reimbursement, customer service, medical affairs, and other support personnel;
- the inability of sales personnel to obtain access to physicians or persuade adequate numbers of physicians to prescribe any future medicines;
- the inability of reimbursement professionals to negotiate arrangements for formulary access, reimbursement, and other acceptance by payors;
- restricted or closed distribution channels that make it difficult to distribute our product candidates we may develop to segments of the patient population;
- the lack of complementary medicines to be offered by sales personnel, which may put us at a competitive disadvantage relative to companies with more extensive product lines; and
- unforeseen costs and expenses associated with creating an independent commercialization organization.

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

We face significant competition in an environment of rapid technological change, and there is a possibility that our competitors may achieve regulatory approval before us or develop therapies that are safer or more advanced or effective than ours, which may harm our financial condition and our ability to successfully market or commercialize any product candidates we may develop.

The development and commercialization of new drug products is highly competitive. Moreover, the base editing and delivery technology fields are characterized by rapidly changing technologies, significant competition, and a strong emphasis on intellectual property. We will face competition with respect to any product candidates that we may seek to develop or commercialize in the future from major pharmaceutical companies, specialty pharmaceutical companies, and biotechnology companies worldwide. Potential competitors also include academic institutions, government agencies, and other public and private research organizations that conduct research, seek patent protection, and establish collaborative arrangements for research, development, manufacturing, and commercialization.

There are a number of large pharmaceutical and biotechnology companies that currently market and sell products or are pursuing the development of products for the treatment of the disease indications for which we have research programs. Some of these competitive products and therapies are based on scientific approaches that are the same as or similar to our approach, and others are based on entirely different approaches.

There are several other companies utilizing base editing technology, including Life Edit (an ElevateBio company), Metagenomi, Revvity, Aurora Therapeutics, Mammoth Biosciences, YolTech Therapeutics, HuidaGene Therapeutics and Intellia Therapeutics. In addition, we face competition from companies utilizing other gene editing modalities and various other genetic medicines. Within the disease areas that we focus on, we are also aware of competing companies that have approved therapies, those with therapies in development, and others that may emerge in the future. For sickle cell disease, these companies include CRISPR Therapeutics, Vertex Pharmaceuticals, Editas Medicine, Genetix Biotherapeutics (formerly bluebird bio), YolTech Therapeutics, Novartis Pharmaceuticals, Kamau Therapeutics, Fulcrum Therapeutics, Tessera Therapeutics, Cimeio Therapeutics and Agios Pharmaceuticals. For our AATD targeted therapies, these include Wave Life Sciences, YolTech Therapeutics, Prime Medicine, CRISPR Therapeutics, Moderna, Korro Bio, Tessera Therapeutics and Arrowhead Pharmaceuticals.

Any product candidates that we successfully develop and commercialize will compete with existing therapies and new therapies that may become available in the future that are approved to treat the same diseases for which we may obtain approval for our product candidates we may develop. This may include other types of therapies, such as small molecule, antibody, and/or protein therapies.

Many of our current or potential competitors, either alone or with their collaboration partners, may have significantly greater financial resources and expertise in research and development, manufacturing, preclinical testing, conducting clinical trials, obtaining regulatory approvals, and marketing approved products than we do. Mergers and acquisitions in the pharmaceutical, biotechnology, and gene therapy industries may result in even more resources being concentrated among a smaller number of our competitors. Smaller or early-stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies. These competitors also compete with us in recruiting and retaining qualified scientific and management personnel and establishing clinical trial sites and patient registration for clinical trials, as well as in acquiring technologies complementary to, or necessary for, our programs. Our commercial opportunity could be reduced or eliminated if our competitors develop and commercialize product candidates that are safer, more effective, have fewer or less severe side effects, are more convenient, or are less expensive than any product candidates that we may develop or that would render any product candidates that we may develop obsolete or non-competitive. Our competitors also may obtain FDA, EMA, or other regulatory approval for their product candidates more rapidly than we may obtain approval for ours, which could result in our competitors establishing a strong market position before we are able to enter the market. Additionally, technologies developed by our competitors may render our potential product candidates uneconomical or obsolete, and we may not be successful in marketing any product candidates we may develop against competitors.

In addition, as a result of the expiration or successful challenge of our patent rights, we could face more litigation with respect to the validity and/or scope of patents relating to our competitors' products. The availability of our competitors' products could limit the demand, and the price we are able to charge, for any product candidates that we may develop and commercialize.

Adverse public perception of genetic medicines, and gene editing and base editing in particular, may negatively impact regulatory approval of, and/or demand for, our potential products.

Our potential therapeutic products involve editing the human genome. The clinical and commercial success of our potential products will depend in part on public understanding and acceptance of the use of gene editing therapy for the prevention or treatment of human diseases. Public attitudes may be influenced by claims that gene editing is unsafe, unethical, or immoral, and, consequently, our product candidates 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.

In addition, gene editing technology is subject to public debate and heightened regulatory scrutiny due to ethical concerns relating to the application of gene editing technology to human embryos or the human germline.

Although we do not use our technologies to edit human embryos or the human germline, such public debate and heightened regulatory scrutiny could prevent or delay our development of product candidates. More restrictive government regulations or negative public opinion would have a negative effect on our business or financial condition and may delay or impair our development and commercialization of product candidates or demand for any product candidates we may develop. Adverse events in our preclinical studies or clinical trials or those of our competitors or of academic researchers utilizing gene editing technologies, even if not ultimately attributable to product candidates we may identify and develop, and the gene publicity could result in increased governmental regulation, unfavorable public perception, potential regulatory delays in the testing or approval of potential product candidates we may identify and develop, stricter labeling requirements for those product candidates that are approved, and a decrease in demand for any such product candidates. Use of gene editing technology by a third party or government to develop biological agents or products that threaten U.S. national security could similarly result in such negative impacts to us.

Even if we are able to commercialize any product candidates, such products may become subject to unfavorable pricing regulations, third-party reimbursement practices, or healthcare reform initiatives, which would harm our business.

The regulations that govern marketing approvals, pricing, and reimbursement for new medicines vary widely from country to country. Some countries require approval of the sale price of a medicine before it can be marketed. In many countries, the pricing review period begins after marketing or product licensing approval is granted. In some foreign markets, prescription pharmaceutical pricing remains subject to continuing governmental control even after initial approval is granted. As a result, we might obtain marketing approval for a medicine in a particular country, but then be subject to price regulations that delay or might even prevent our commercial launch of the medicine, possibly for lengthy time periods, and negatively impact the revenues we are able to generate from the sale of the medicine in that country. Adverse pricing limitations may hinder our ability to recoup our investment in one or more product candidates we may develop, even if any product candidates we may develop obtain marketing approval.

Our ability to commercialize any medicines successfully also will depend in part on the extent to which reimbursement for these medicines and related treatments will be available from government authorities or healthcare programs, private health plans, and other organizations. Government authorities and third-party payors, such as private health plans, decide which medications they will pay for and establish reimbursement levels. A primary trend in the U.S. healthcare industry and elsewhere is cost containment. Government authorities and third-party payors have attempted to control costs by limiting coverage and the amount of reimbursement for particular medications. For example, the Inflation Reduction Act of 2022, or IRA, includes several provisions that may impact our business, including provisions that impose new manufacturer financial liability on all drugs in Medicare Part D, allow the U.S. government to negotiate Medicare Part B and Part D pricing for certain high-cost drugs and biologics without generic or biosimilar competition, require companies to pay rebates to Medicare for drug prices that increase faster than inflation, and delay the rebate rule that would require pass through of pharmacy benefit manager rebates to beneficiaries. We cannot yet predict the effect the IRA will have on our business and the healthcare industry in general.

Increasingly, third-party payors are also challenging the prices charged for medical products and requiring that drug companies provide them with predetermined discounts from list prices. Novel medical products, if covered at all, may be subject to enhanced utilization management controls designed to ensure that the products are used only when medically necessary. Such utilization management controls may discourage the prescription or use of a medical product by increasing the administrative burden associated with its prescription or creating coverage uncertainties for prescribers and patients. We cannot be sure that reimbursement will be available for any medicine that we commercialize and, if reimbursement is available, that the level of reimbursement will be adequate. Reimbursement may impact the demand for, or the price of, any product candidate for which we obtain marketing approval. If reimbursement is not available or is available only to limited levels, we may not be able to successfully commercialize any product candidate for which we obtain marketing approval.

There may be significant delays in obtaining reimbursement for newly approved medicines, and coverage may be more limited than the purposes for which the medicine is approved by the FDA, the EMA or other regulatory authorities outside the United States. Moreover, eligibility for reimbursement does not imply that any medicine will be paid for in all cases or at a rate that covers our costs, including research, development, manufacture, sale, and distribution. Interim reimbursement levels for new medicines, if applicable,

may also not be sufficient to cover our costs and may not be made permanent. Reimbursement rates may vary according to the use of the medicine and the clinical setting in which it is used, may be based on reimbursement levels already set for lower cost medicines and may be incorporated into existing payments for other services. Net prices for medicines may be reduced by mandatory discounts or rebates required by government healthcare programs or private payors and by any future relaxation of laws that presently restrict imports of medicines from countries where they may be sold at lower prices than in the United States. Our inability to promptly obtain coverage and profitable payment rates from both government-funded and private payors for any approved medicines we may develop could have a material adverse effect on our operating results, our ability to raise capital needed to commercialize medicines, and our overall financial condition.

Due to the novel nature of our technology and the potential for any product candidates we may develop to offer therapeutic benefit in a single administration or limited number of administrations, we face uncertainty related to pricing and reimbursement for these product candidates.

Our initial target patient populations are relatively small, as a result of which the pricing and reimbursement of any product candidates we may develop, if approved, must be adequate to support the necessary commercial infrastructure. If we are unable to obtain adequate levels of reimbursement, our ability to successfully market and sell any such product candidates will be adversely affected. The manner and level at which reimbursement is provided for services related to any product candidates we may develop (e.g., for administration of our product candidate to patients) is also important. Inadequate reimbursement for such services may lead to physician and payor resistance and adversely affect our ability to market or sell our product candidates we may develop. In addition, we may need to develop new reimbursement models in order to realize adequate value. Payors may not be able or willing to adopt such new models, and patients may be unable to afford that portion of the cost that such models may require them to bear. If we determine such new models are necessary but we are unsuccessful in developing them, or if such models are not adopted by payors, our business, financial condition, results of operations, and prospects could be adversely affected.

We expect the cost of a single administration of the genetic medicines we are seeking to develop to be substantial, when and if they achieve regulatory approval. We expect that coverage and reimbursement by government and private payors will be essential for most patients to be able to afford these treatments. Accordingly, sales of any such product candidates will depend substantially, both domestically and abroad, on the extent to which the costs of any product candidates we may develop will be paid by government authorities, private health plans, and other third-party payors. Payors may not be willing to pay high prices for a single administration. Coverage and reimbursement by a third-party payor may depend upon several factors, including the third-party payor's determination that use of a product is:

- a covered benefit under its health plan;
- safe, effective, and medically necessary;
- appropriate for the specific patient;
- cost-effective; and
- neither experimental nor investigational.

Obtaining coverage and reimbursement for a product from third-party payors is a time-consuming and costly process that could require us to provide to the payor supporting scientific, clinical, and cost-effectiveness data. There is significant uncertainty related to third-party coverage and reimbursement of newly approved products. We may not be able to provide data sufficient to gain acceptance with respect to coverage and reimbursement. If coverage and reimbursement are not available, or are available only at limited levels, we may not be able to successfully commercialize any product candidates we may develop. Even if coverage is provided, the approved reimbursement amount may not be adequate to realize a sufficient return on our investment.

Moreover, the downward pressure on healthcare costs in general, particularly prescription drugs and surgical procedures and other treatments, has become intense. As a result, increasingly high barriers are being erected to the entry of new product candidates such as ours. If we are unable to obtain adequate levels of reimbursement, our ability to successfully market and sell any product candidates we may develop will be harmed.

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

We focus a substantial portion of our research and product development on treatments for rare genetically defined diseases. Many of our product candidates we may develop are expected to target a single mutation; as a result, the relevant patient population may therefore be small. Our projections of both the number of people who have these diseases, as well as the subset of people with these diseases who have the potential to benefit from treatment with product candidates we may develop, are based on estimates. These estimates may prove to be incorrect and new studies may change the estimated incidence or prevalence of these diseases. The number

of patients in the United States, Europe, and elsewhere may turn out to be lower than expected, and patients may not be amenable to treatment with our product candidates we may develop, or may become increasingly difficult to identify or gain access to, all of which would adversely affect our business, financial condition, results of operations, and prospects. Additionally, because of the potential that any product candidates we develop could cure a target disease, we may not receive recurring revenues from patients and may deplete the patient population prevalence through curative therapy.

If we are unable to successfully identify patients who are likely to benefit from therapy with any product candidates we develop, or experience significant delays in doing so, we may not realize the full commercial potential of any medicines we may develop.

Our success may depend, in part, on our ability to identify patients who are likely to benefit from therapy with any medicines we may develop, which requires those potential patients to have their DNA analyzed for the presence or absence of a particular sequence. If we, or any third parties that we engage to assist us, are unable to successfully identify such patients, or experience delays in doing so, then:

- our ability to develop any product candidates may be adversely affected if we are unable to appropriately select patients for enrollment in our clinical trials; and
- we may not realize the full commercial potential of any product candidates we develop that receive marketing approval if, among other reasons, we are unable to appropriately select patients who are likely to benefit from therapy with our medicines.

Any product candidates we develop may require use of a companion diagnostic to identify patients who are likely to benefit from therapy. If safe and effective use of any of our product candidates we may develop depends on a companion diagnostic, we may not receive marketing approval, or marketing approval may be delayed, if we are unable to or are delayed in developing, identifying, or obtaining regulatory approval or clearance for the companion diagnostic product for use with our product candidate. Identifying a manufacturer of the companion diagnostic and entering into an agreement with the manufacturer could also delay the development of our product candidates.

As a result of these factors, we may be unable to successfully develop and realize the commercial potential of any product candidates we may identify and develop, and our business, financial condition, results of operations, and prospects would be materially adversely affected.

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

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

- decreased demand for any product candidates or medicines that we may develop;
- injury to our reputation and significant negative media attention;
- withdrawal of clinical trial participants;
- significant time and costs to defend the related litigation;
- substantial monetary awards to trial participants or patients;
- loss of revenue; and
- the inability to commercialize any medicines that we may develop.

Although we maintain product liability insurance coverage, it may not be adequate to cover all liabilities that we may incur. We anticipate that we will need to increase our insurance coverage as we continue clinical trials and if we successfully commercialize any medicine. 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.

We are subject to regulatory and operational risks associated with our internal manufacturing facility.

We have an approximately 100,000 square foot manufacturing facility in Research Triangle Park, North Carolina intended to support clinical and commercial production of certain of our product candidates and components thereof. We aim to follow cGMP processes necessary to release product for our clinical trials and to meet all requirements from regulatory agencies, including the FDA, to allow us to support research, clinical and, if approved, commercial production of certain of our product candidates and components thereof. However, we can provide no assurances that our facility will be able to support our intended internal manufacturing capabilities and/or needs or comply with regulatory agency requirements. Furthermore, while the design of our facility is based on current standards for

biotechnology facilities, it was not pre-approved by any regulatory agency, nor has the facility been inspected by any regulatory agency such as the FDA. If we are unable to operate our facility at the intended capacity, or if our facility is determined not to comply with applicable regulatory requirements, we may be unable to manufacture our products or product candidates in a timely manner, which could significantly impair our ability to develop, obtain approval for, or commercialize our product candidates. Additionally, we have incurred substantial expenditures in constructing our facility, and expect to incur significant additional expenditures operating our facility in the future. To the extent our facility does not operate at capacity or comply with regulatory requirements, we may incur expenditures beyond those we currently contemplate, including costs associated with engaging third parties to meet our manufacturing needs or undertaking remediation efforts to bring our facility into compliance with regulatory standards. Any such delays, interruptions or increased costs arising from the operation of our manufacturing facility could materially harm our business, operating results, financial condition and prospects.

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

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

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

In addition, we may incur substantial costs in order to comply with current or future environmental, health, and safety laws, regulations, and permitting requirements. These current or future laws, regulations, and permitting requirements may impair our research, development, or production efforts. Failure to comply with these laws, regulations, and permitting requirements also may result in substantial fines, penalties, or other sanctions or business disruption, which could have a material adverse effect on our business, financial condition, results of operations, and prospects.

Any third-party contract manufacturers and suppliers we engage will also be subject to these and other environmental, health, and safety laws and regulations. Liabilities they incur pursuant to these laws and regulations could result in significant costs or an interruption in operations, which could have a material adverse effect on our business, financial condition, results of operations, and prospects.

Genetic medicines are novel, and any product candidates we develop may be complex and difficult to manufacture. We could experience delays in satisfying regulatory authorities or production problems that result in delays in our development or commercialization programs, limit the supply of our product candidates we may develop, or otherwise harm our business.

Any product candidates we may develop will likely 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 the product candidates we intend to develop generally cannot be fully characterized. As a result, assays of the finished product candidate may not be sufficient to ensure that the product candidate will perform in the intended manner. Problems with the manufacturing process, even minor deviations from the normal process, could result in product defects or manufacturing failures that result in lot failures, product recalls, product liability claims, insufficient inventory, or potentially delay progression of our potential regulatory filings. If we successfully develop product candidates, we may encounter problems achieving adequate quantities and quality of clinical-grade materials that meet FDA, EMA or other comparable applicable foreign standards or specifications with consistent and acceptable production yields and costs. In addition, our product candidates we may develop will require complicated delivery modalities, such as electroporation and LNPs, each of which will introduce additional complexities in the manufacturing process.

In addition, the FDA, the 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, the 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 clinical trials or product launches, which could be costly to us and otherwise harm our business, financial condition, results of operations, and prospects.

Furthermore, we intend to use novel technologies to deliver the base editor and guide RNA constructs of product candidates, however scientific evidence to support the feasibility of developing product candidates based on these technologies is both preliminary and limited.

We also may encounter problems hiring and retaining the experienced scientific, quality control, and manufacturing personnel needed to manage our manufacturing process, which could result in delays in our production or difficulties in maintaining compliance with applicable regulatory requirements.

Given the nature of biologics manufacturing, there is a risk of contamination during manufacturing. Any contamination could materially harm our ability to produce product candidates on schedule and could harm our results of operations and cause reputational damage. Some of the raw materials that we anticipate will be 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 any product candidates we may develop could adversely impact or disrupt the commercial manufacturing or the production of clinical material, which could materially harm our development timelines and our business, financial condition, results of operations, and prospects.

Any problems in our manufacturing process or the facilities with which we contract could make us a less attractive collaborator for potential partners, including larger pharmaceutical companies and academic research institutions, which could limit our access to additional attractive development programs. Problems in third-party manufacturing process or facilities also could restrict our ability to ensure sufficient clinical material for any clinical trials we may be conducting or are planning to conduct and meet market demand for any product candidates we develop and commercialize.

Risks related to our relationships with third parties

We rely on and expect to continue to rely on third parties to manufacture components of our product candidates we may develop, conduct our clinical trials and some aspects of our research and preclinical testing, and those third parties may not perform satisfactorily, including failing to meet deadlines for the completion of such trials, research, or testing.

We rely on and expect to continue to rely on third parties, such as CMOs, CROs, clinical data management organizations, medical institutions, and clinical investigators, to conduct some aspects of our research and preclinical testing, to manufacture components of our product candidates and to conduct our clinical trials. Any of these third parties may terminate their engagements with us at any time under certain criteria. If we need to enter into alternative arrangements, it may delay our product development activities.

Our reliance on these third parties for research and development activities reduces our control over these activities but does not relieve us of our responsibilities. For example, we remain responsible for ensuring that each of our clinical trials is conducted in accordance with the general investigational plan and protocols for the trial. Moreover, the FDA, EMA and other regulatory authorities require us to comply with standards, commonly referred to as Good Clinical Practices, 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. In the United States, we also are required to register ongoing clinical trials and post the results of completed clinical trials on a government-sponsored database, ClinicalTrials.gov, within certain timeframes. Failure to do so can result in fines, adverse publicity, and civil and criminal sanctions.

Although we design the clinical trials for our product candidates, we rely on and expect to continue to rely on CROs to conduct some or all of the clinical trials. As a result, many important aspects of our development programs, including their conduct and timing, will be outside of our direct control. Our reliance on third parties to conduct preclinical studies and clinical trials also results in less direct control over the management of data developed through preclinical studies and clinical trials than would be the case if we were relying entirely upon our own staff. Communicating with outside parties can also be challenging, potentially leading to mistakes as well as difficulties in coordinating activities. Outside parties may:

- have staffing difficulties;
- fail to comply with contractual obligations;
- experience regulatory compliance issues;
- undergo changes in priorities or become financially distressed; or
- form relationships with other entities, some of which may be our competitors.

These factors may materially adversely affect the willingness or ability of third parties to conduct our preclinical studies and clinical trials and may subject us to unexpected cost increases that are beyond our control. If the CROs and other third parties do not perform preclinical studies and future clinical trials in a satisfactory manner, breach their obligations to us or fail to comply with regulatory requirements, the development, regulatory approval and commercialization of our product candidates may be delayed, we may not be able to obtain regulatory approval and commercialize our product candidates, or our development programs may be materially and irreversibly harmed. If we are unable to rely on preclinical and clinical data collected by our CROs and other third parties, we could be required to repeat, extend the duration of, or increase the size of any preclinical studies or clinical trials we conduct and this could significantly delay commercialization and require greater expenditures.

We contract with third parties for the manufacture and supply of materials for our research programs, preclinical studies and clinical trials and expect to continue to do so for at least a portion of our future research programs, preclinical studies and clinical trials and for commercialization of any product candidates that we may develop. This reliance on third parties increases the risk that we will not have sufficient quantities of such materials, product candidates, or any medicines that we may develop and commercialize, or that such supply will not be available to us at an acceptable cost, which could delay, prevent, or impair our development or commercialization efforts.

We currently rely on third-parties for the manufacture and supply of materials for a portion of our preclinical studies and clinical trials, and may continue to do so for future research programs, preclinical studies, clinical testing and for commercial supply of any product candidates that we may develop and for which we or our collaborators obtain marketing approval.

While we have built a manufacturing facility designed to support manufacturing for our *ex vivo* cell therapy programs in hematology and *in vivo* non-viral delivery programs for liver diseases in Research Triangle Park, North Carolina, we cannot be certain that we will be able to maintain cGMP compliance, expand our internal manufacturing capacity, or meet the planned manufacturing needs of our programs.

We may be unable to establish long-term supply agreements with third-party suppliers or to do so on acceptable terms. Even if we are able to establish long-term supply agreements with third-parties, reliance on third-parties entails additional risks, including:

- the possible breach of the manufacturing or supply agreement by the third party;
- the possible termination or nonrenewal of the agreement by the third party at a time that is costly or inconvenient for us;
- reliance on the third party for regulatory compliance, quality assurance, safety, and pharmacovigilance and related reporting; and
- the possible inability of third-party suppliers to supply and/or transport materials, components and products to us in a timely manner as a result of disruptions to the global supply chain or other factors.

Third-party suppliers may not be able to comply with cGMP regulations or other regulatory requirements outside the United States. Our failure, or the failure of third-party suppliers, to comply with applicable regulations could result in sanctions being imposed on us, including fines, injunctions, civil penalties, delays, suspension or withdrawal of approvals, license revocations, seizures or recalls of product candidates or medicines, operating restrictions, and criminal prosecutions, any of which could significantly and adversely affect supplies of our medicines and harm our business, financial condition, results of operations, and prospects.

For example, we rely on various CROs to obtain non-human primates, or NHPs, for use in preclinical development work. While we believe we currently have access to a supply of NHPs adequate to meet our near-term needs, such supply may nevertheless be adversely affected by supply chain limitations. If we are unable to secure adequate supply of NHPs certain of our preclinical development efforts will be delayed, and the cost of conducting discovery projects and preclinical development activities may substantially increase. Such delays or cost increases could materially adversely affect our discovery and preclinical development activities and our business.

Any medicines that we develop may compete with other product candidates and products for access to manufacturing facilities or supplies. There are a limited number of manufacturers that operate under cGMP regulations and that might be capable of manufacturing drug components and drug product necessary for gene editing. Any performance failure on the part of our existing or future suppliers could delay preclinical or clinical development or marketing approval. We do not currently have arrangements in place for redundant supply of all drug components and drug products necessary for our gene editing product candidates. If any one of our current contract manufacturers or suppliers cannot perform as agreed, we may be required to replace that manufacturer or supplier. Although we believe that there are several potential alternative suppliers to support any product candidates we may develop, we may incur added costs and delays in identifying and qualifying any such replacement.

Our current and anticipated future dependence upon others for the manufacture of any product candidates we may develop may adversely affect our future profit margins and our ability to commercialize any medicines that receive marketing approval on a timely and competitive basis.

As our drug development pipeline increases and matures, the increased demand for clinical supplies from our facilities and third parties may impact our ability to operate. We will require increased capacity across our entire supply chain. Furthermore, we rely on many service providers, including those that provide manufacturing or testing services, all of whom have inherent risks in their operations that may adversely impact our operations.

Completion of our clinical trials and commercialization of our product candidates require access to, or development of, facilities to manufacture our product candidates at sufficient yields and, if approved, at commercial-scale. We have limited experience manufacturing any of our product candidates in the volumes that are necessary to support clinical trials and no experience manufacturing at volumes that are necessary to support commercial sales. Efforts to establish these capabilities may not meet initial expectations as to scheduling, scale-up, reproducibility, yield, purity, cost, potency or quality. In addition, other companies, many with substantial resources, compete with us for access to the materials needed to manufacture our product candidates.

We currently utilize, and expect to continue to utilize, third parties to, among other things, manufacture raw materials, components, parts, and consumables, and to perform quality testing. If the field of base editing and other genetic medicines continues to expand, we may encounter increasing competition for these materials and services. Demand for third-party manufacturing or testing facilities may grow at a faster rate than their existing capacity, which could disrupt our ability to find and retain third-party manufacturers capable of producing sufficient quantities of such raw materials, components, parts, and consumables required to manufacture our product candidates. The use of service providers and suppliers could expose us to risks, including, but not limited to:

- termination or non-renewal of supply and service agreements with third parties in a manner or at a time that is costly or damaging to us;
- disruptions to the operations of these suppliers and service providers caused by conditions unrelated to our business or operations, including the bankruptcy of the supplier or service provider; and
- inspections of third-party facilities by regulatory authorities that could have a negative outcome and result in delays to or termination of their ability to supply our requirements.

Our reliance on third-party manufacturers may adversely affect our operations or result in unforeseen delays or other problems beyond our control. Because of contractual restraints and the limited number of third-party manufacturers with the expertise, required regulatory approvals and facilities to manufacture our product candidates on a clinical and, if approved, a commercial scale, replacement of a manufacturer may be expensive and time-consuming and may cause interruptions in the production of our product candidates. A third-party manufacturer may also encounter difficulties in production. These problems may include:

- difficulties with production costs, scale up and yields;
- availability of raw materials and supplies;
- quality control and assurance;
- shortages of qualified personnel;
- compliance with strictly enforced federal, state and foreign regulations that vary in each country where products might be sold; and
- lack of capital funding.

As a result, any delay or interruption could have a material adverse effect on our business, financial condition, or results of operations.

We have and may in the future enter into collaborations with third parties for the research, development, and commercialization of certain of the product candidates we develop. If any such collaborations are not successful, we may not be able to capitalize on the market potential of those product candidates.

We have and may in the future seek third-party collaborators for the research, development, and commercialization of certain of the product candidates we develop. Under the agreements we have entered into and any agreements we may enter into in the future with any third parties, we have and will likely have limited control over the amount and timing of resources that our collaborators dedicate to the development or commercialization of any product candidates we seek to develop with them. Our ability to generate revenues from these arrangements will depend on our collaborators' abilities to successfully perform the functions assigned to them in these arrangements. We cannot predict the success of any collaboration that we enter into.

Collaborations involving our research programs or any product candidates we may develop pose numerous risks to us, including the following:

- Collaborators have significant discretion in determining the efforts and resources that they will apply to these collaborations.

- Collaborators may not pursue development and commercialization of any product candidates we develop or may elect not to continue or renew development or commercialization programs based on clinical trial results, changes in the collaborator's strategic focus or available funding or external factors such as an acquisition that diverts resources or creates 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 medicines or product candidates we develop 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.
- Collaborators with marketing and distribution rights to one or more medicines may not commit sufficient resources to the marketing and distribution of such medicine or medicines.
- Collaborators may not properly obtain, maintain, enforce, or defend our intellectual property or proprietary rights or may use our proprietary information in such a way as to invite litigation that could jeopardize or invalidate our proprietary information or expose us to potential litigation.
- Disputes may arise between the collaborators and us that result in the delay or termination of the research, development, or commercialization of our medicines or product candidates or that result in costly litigation or arbitration that diverts management attention and resources.
- We may lose certain valuable rights under circumstances identified in our collaborations, including if we undergo a change of control.
- Collaborations may be terminated and, if terminated, may result in a need for additional capital to pursue further development or commercialization of the applicable product candidates we may develop.
- Collaboration agreements may not lead to development or commercialization of product candidates in the most efficient manner or at all. If a present or future collaborator of ours were to be involved in a business combination, the continued pursuit and emphasis on our product development or commercialization program under such collaboration could be delayed, diminished, or terminated.

If our collaborations do not result in the successful development and commercialization of product candidates, or if one of our collaborators terminates its agreement with us, we may not receive any future research funding or milestone or royalty payments under the collaboration. Furthermore, even if we receive such payments, they will likely result in payment obligations under license agreements with our licensors, which could be substantial. If we do not receive the funding we expect under these collaboration agreements, or if the funding is substantially offset by payment obligations to our licensors, our development of product candidates could be delayed, and we may need additional resources to develop product candidates. In addition, if one of our collaborators terminates its agreement with us, we may find it more difficult to find a suitable replacement collaborator or attract new collaborators, and our development programs may be delayed or the perception of us in the business and financial communities could be adversely affected. All of the risks relating to product development, regulatory approval, and commercialization described in this Annual Report on Form 10-K apply to the activities of our collaborators.

These relationships, or those like them, may require us to incur non-recurring and other charges, increase our near- and long-term expenditures, issue securities that dilute our existing stockholders, or disrupt our management and business. In addition, we could face significant competition in seeking appropriate collaborators, and the negotiation process is time-consuming and complex. Our ability to reach a definitive collaboration agreement 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 several factors. If we license rights to any product candidates we or our collaborators may develop, we may not be able to realize the benefit of such transactions if we are unable to successfully integrate them with our existing operations and company culture.

If conflicts arise between us and our collaborators or strategic partners, these parties may act in a manner adverse to us and could limit our ability to implement our strategies.

If conflicts arise between our corporate or academic collaborators or strategic partners and us, the other party may act in a manner adverse to us and could limit our ability to implement our strategies. Some of our collaborators and strategic partners are conducting multiple product development efforts within each area that is the subject of the collaboration with us. Our collaborators or strategic partners, however, may develop, either alone or with others, products in related fields that are competitive with the product candidates we may develop that are the subject of these collaborations with us. Competing products, either developed by the collaborators or

strategic partners or to which the collaborators or strategic partners have rights, may result in the withdrawal of partner support for our product candidates we may develop.

Some of our collaborators or strategic partners could also become our competitors in the future. Our collaborators or strategic partners could develop competing products, preclude us from entering into collaborations with their competitors, fail to obtain timely regulatory approvals, terminate their agreements with us prematurely, or fail to devote sufficient resources to the development and commercialization of products. Any of these developments could harm our product development efforts.

If we are not able to establish collaborations on commercially reasonable terms, we may have to alter our development and commercialization plans.

Our product development and research programs and the potential commercialization of any product candidates we may develop will require substantial additional cash to fund expenses. For some of the product candidates we may develop, we may decide to collaborate with other pharmaceutical and biotechnology companies for the development and potential commercialization of those product candidates.

We face significant competition in seeking appropriate collaborators. Whether we reach a definitive agreement for a 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, the EMA 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 indications that may be available to collaborate on and whether such a collaboration could be more attractive than the one with us.

We may also be restricted under existing collaboration agreements from entering into future agreements on certain terms with potential collaborators. Collaborations are complex and time-consuming to negotiate and document. In addition, there have been a significant number of recent business combinations among large pharmaceutical companies that have resulted in a reduced number of potential future collaborators.

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

Risks related to our intellectual property

If we are unable to obtain and maintain patent and other intellectual property protection for any product candidates we develop and for our platform technologies, or if the scope of the patent and other intellectual property protection obtained is not sufficiently broad, our competitors could develop and commercialize products and technology similar or identical to ours, and our ability to successfully commercialize any product candidates we may develop, and our platform technologies may be adversely affected.

Our commercial success will depend in large part on our ability to obtain and maintain patent, trademark, trade secret and other intellectual property protection of our base editing platform technology, product candidates and other technology, including delivery platform technology methods used to manufacture them and methods of treatment, as well as successfully defending our patent and other intellectual property rights against third-party challenges. It is difficult and costly to protect our base editing platform technology and protect candidates, and we may not be able to ensure their protection. Our ability to stop unauthorized third parties from making, using, selling, offering to sell, importing or otherwise commercializing our product candidates we may develop is dependent upon the extent to which we have rights under valid and enforceable patents or trade secrets that cover these activities.

We seek to protect our proprietary position by in-licensing intellectual property relating to our platform technology and filing patent applications in the United States and abroad related to our base editing platform technology, delivery platform technology and product candidates that are important to our business. If we or our licensors are unable to obtain or maintain patent protection with respect to our base editing platform technology, delivery platform technology and product candidates we may develop, or if the scope of the patent protection secured is not sufficiently broad, our competitors could develop and commercialize products and technology similar or identical to ours and our ability to commercialize any product candidates we may develop may be adversely affected.

The patent prosecution process is expensive, time-consuming, and complex, and we may not be able to file, prosecute, maintain, enforce, or license all necessary or desirable patent applications at a reasonable cost or in a timely manner. In addition, we may not

pursue or obtain patent protection in all relevant markets. It is also possible that we will fail to identify patentable aspects of our research and development output in time to obtain patent protection. Although we enter into non-disclosure 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, CROs, contract manufacturers, consultants, advisors, and other third parties, any of these parties may breach the agreements and disclose such output before a patent application is filed, thereby jeopardizing our ability to seek patent protection. In addition, our ability to obtain and maintain valid and enforceable patents depends on whether the differences between our inventions and the prior art allow our inventions to be patentable over the prior art. Furthermore, publications of discoveries in the scientific literature often lag behind the actual discoveries, and patent applications in the United States and other jurisdictions are typically not published until 18 months after filing, or in some cases not at all. Therefore, we cannot be certain that we or our licensors were the first to make the inventions claimed in our owned or any licensed patents or pending patent applications, or that we or our licensors were the first to file for patent protection of such inventions.

The patent position of biotechnology and pharmaceutical companies generally is highly uncertain, involves complex legal and factual questions, and has been the subject of much litigation in recent years. The field of gene editing, especially in the area of base editing technology, has been the subject of extensive patenting activity and litigation. As a result, the issuance, scope, validity, enforceability, and commercial value of our patent rights are highly uncertain, and we may become involved in complex and costly litigation. Our pending and future patent applications may not result in patents being issued which protect our base editing platform technology, delivery platform technology and product candidates we may develop, or which effectively prevent others from commercializing competitive technologies and product candidates.

No consistent policy regarding the scope of claims allowable in the field of gene editing, including base editing technology, has emerged in the United States. The scope of patent protection outside of the United States is also uncertain. Changes in either the patent laws or their interpretation in the United States and other countries may diminish our ability to protect our inventions, obtain, maintain, enforce and defend our intellectual property rights and, more generally, could affect the value of our intellectual property or narrow the scope of our owned and licensed patent rights. With respect to both in-licensed and owned intellectual property, we cannot predict whether the patent applications we and our licensors are currently pursuing will issue as patents in any particular jurisdiction or whether the claims of any issued patents will be valid and enforceable and provide sufficient protection from competitors.

Moreover, the coverage claimed in a patent application can be significantly reduced before the patent is issued, and its scope can be reinterpreted after issuance. 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 own, or in-license, may be challenged, narrowed, circumvented, or invalidated by third parties. Consequently, we do not know whether any of our platform advances and product candidates we may develop will be protectable or remain protected by valid and enforceable patents. Our competitors or other third parties may be able to circumvent our patents by developing similar or alternative technologies or products in a non-infringing manner.

In addition, 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 candidates are commercialized. As a result, our intellectual property may not provide us with sufficient rights to exclude others from commercializing products similar or identical to ours. Moreover, some of our owned and in-licensed patents and patent applications are, and may in the future be, co-owned by us with third parties. For example, a patent application directed to our potential HBG1 and HBG2 product candidates is co-owned by us, the President and Fellows of Harvard College, or Harvard, and Broad Institute. At present, we do not have a license to the ownership interest of Harvard or Broad Institute. If we are unable to obtain an exclusive license to such third-party co-owners' interest in such patents or patent applications, such co-owners may be able to license their rights to other third parties, including our competitors, and our competitors could market competing products and technology. In addition, we may need the cooperation of any such co-owners of our patents in order to enforce such patents against third parties, and such cooperation may not be provided to us. Any of the foregoing could have a material adverse effect on our competitive position, business, financial conditions, results of operations, and prospects.

Our rights to develop and commercialize our base editing platform technology and product candidates are subject, in part, to the terms and conditions of licenses granted to us by others.

We depend on intellectual property licensed from third parties, and our licensors may not always act in our best interest. If we fail to comply with our obligations under our intellectual property licenses, if the licenses are terminated, or if disputes regarding these licenses arise, we could lose significant rights that are important to our business.

We have licensed and are dependent on certain patent rights and proprietary technology from third parties that are important or necessary to the development of our base editing technology and product candidates. For example, we are a party to license agreements with Broad Institute, Editas, Harvard, and Bio Palette, and others, pursuant to which we in-license key patents and patent applications for our base editing platform technology and product candidates (the Editas License Agreement, the Harvard License Agreement and the Bio Palette License Agreement, respectively). These license agreements impose various diligence, milestone payment, royalty, insurance, and other obligations on us. If we fail to comply with these obligations, our licensors may have the right to terminate our license, in which event we would not be able to develop or market our base editing platform or any other technology or product candidates covered by the intellectual property licensed under these agreements. For example, under the Harvard License Agreement, we are required to meet certain development milestones for the development of a licensed product covered by certain sub-categories of licensed patents. If we fail to meet such milestones, our rights with respect to the sub-category of licensed patents will terminate.

These and other licenses may not provide exclusive rights to use such intellectual property and technology in all relevant fields of use and in all territories in which we may wish to develop or commercialize our base editing platform technology and product candidates in the future. Some licenses granted to us are expressly subject to certain preexisting rights held by the licensor or certain third parties. As a result, we may not be able to prevent competitors from developing and commercializing competitive products in certain territories or fields. For example, certain licensed patents developed by employees of the Howard Hughes Medical Institute, or HHMI, and subsequently assigned to Harvard and licensed to us under the Harvard License Agreement remain subject to a non-exclusive license between Harvard and HHMI. The Editas License Agreement provides that our field of use excludes the use of certain gene editing technologies for the diagnosis, treatment, and prevention of human cancers through certain engineered T-cells, which are licensed to Juno Therapeutics, Inc. (a subsidiary of Bristol-Myers Squibb Company). If we determine that rights to such excluded field are necessary to commercialize any of our product candidates or maintain our competitive advantage, we may need to obtain a license from such third party in order to continue developing, manufacturing or marketing our product 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.

In addition, pursuant to our license agreement with Harvard, under certain specific circumstances Harvard may grant a license to the patents that are the subject of such license agreement to a third party in the same field as such patents are licensed to us. Such third party may then have full rights that are the subject of the Harvard License Agreement, which could impact our competitive position and enable a third party to commercialize products similar to our potential future product candidates and technology. Any grant of rights to a third party in this scenario would narrow the scope of our exclusive rights to the patents and patent applications we have in-licensed from Broad Institute and/or Harvard, as applicable.

We do not have complete control in the preparation, filing, prosecution, maintenance, enforcement, and defense of patents and patent applications covering the technology that we license from third parties. For example, pursuant to each of our intellectual property licenses with Broad Institute, Harvard, Editas and Bio Palette, our licensors retain control of preparation, filing, prosecution, and maintenance, and, in certain circumstances, enforcement and defense of their patents and patent applications. It is possible that our licensors' enforcement of patents against infringers or defense of such patents against challenges of validity or claims of enforceability may be less vigorous than if we had conducted them ourselves, or may not be conducted in accordance with our best interests. We cannot be certain that these patents and patent applications will be prepared, filed, prosecuted, maintained, enforced, and defended in a manner consistent with the best interests of our business. If our licensors fail to prosecute, maintain, enforce, and defend such patents, or lose rights to those patents or patent applications, the rights we have licensed may be reduced or eliminated, our right to develop and commercialize any of our product candidates we may develop that are the subject of such licensed rights could be adversely affected and we may not be able to prevent competitors from making, using, and selling competing products.

Our licensors may have relied on third-party consultants or collaborators or on funds from third parties such that our licensors are not the sole and exclusive owners of the patents we in-licensed. If other third parties have ownership rights to our in-licensed patents, the license granted to us in jurisdictions where the consent of a co-owner is necessary to grant such a license may not be valid and such co-owners may be able to license such patents to our competitors, and our competitors could market competing products and technology. In addition, our rights to our in-licensed patents and patent applications are dependent, in part, on inter-institutional or other operating agreements between the joint owners of such in-licensed patents and patent applications. If one or more of such joint owners breaches such inter-institutional or operating agreements, our rights to such in-licensed patents and patent applications may be adversely affected. Any of these events could have a material adverse effect on our competitive position, business, financial conditions, results of operations, and prospects.

Furthermore, inventions contained within some of our in-licensed patents and patent applications were made using U.S. government funding. We rely on our licensors to ensure compliance with applicable obligations arising from such funding, such as timely reporting, an obligation associated with our in-licensed patents and patent applications. The failure of our licensors to meet their obligations may lead to a loss of rights or the unenforceability of relevant patents. For example, the U.S. government could have certain rights in such in-licensed patents, including a non-exclusive license authorizing the U.S. government to use the invention or to have others use the invention on its behalf. If the U.S. government decides to exercise these rights, it is not required to engage us as its

contractor in connection with doing so. The U.S. government's rights may also permit it to disclose the funded inventions and technology to third parties and to exercise march-in rights to use or allow third parties to use the technology we have licensed that was developed using U.S. government funding. The U.S. government may also exercise its march-in rights if it determines that action is necessary because we or our licensors failed to achieve practical application of the U.S. government-funded technology, because action is necessary to alleviate health or safety needs, to meet requirements of federal regulations, or to give preference to U.S. industry. In addition, our rights in such in-licensed U.S. government-funded inventions may be subject to certain requirements to manufacture product candidates embodying such inventions in the United States. Any of the foregoing could harm our business, financial condition, results of operations, and prospects significantly.

In the event any of our third-party licensors determine that, in spite of our efforts, we have materially breached a license agreement or have failed to meet certain obligations thereunder, it may elect to terminate the applicable license agreement or, in some cases, one or more licenses under the applicable license agreement and such termination would result in us no longer having the ability to develop and commercialize product candidates and technology covered by that license agreement or license. In the event of such termination of a third-party in-license, or if the underlying patents under a third-party in-license fail to provide the intended exclusivity, competitors would have the freedom to seek regulatory approval of, and to market, products identical to ours. Any of these events could have a material adverse effect on our competitive position, business, financial conditions, results of operations, and prospects.

Our owned and in-licensed patents and patent applications may not provide sufficient protection of our platform technologies, our product candidates and our future product candidates or result in any competitive advantage.

We have in-licensed a number of issued U.S. patents and patent applications that cover base editing and gene targeting technologies, as well as our delivery platform technology. We have applied for provisional patent applications or Patent Cooperation Treaty, or PCT, applications intended to specifically cover our base editing platform technology and uses with respect to treatment of particular diseases and conditions, and currently own three issued U.S. patents. We have applied for provisional patent applications or PCT applications intended to specifically cover our delivery platform technology but do not currently own any issued U.S. patents. Each U.S. provisional patent application is not eligible to become an issued patent until, among other things, we file a non-provisional patent application within 12 months of the filing date of the applicable provisional patent application. Any failure to file a non-provisional patent application within this timeline could cause us to lose the ability to obtain patent protection for the intentions disclosed in the associated provisional patent applications. We cannot be certain that any of these patent applications will issue as patents, and if they do, that such patents will cover or adequately protect our base editing platform technology, delivery platform technology or our product candidates, or that such patents will not be challenged, narrowed, circumvented, invalidated or held unenforceable. Any failure to obtain or maintain patent protection with respect to our base editing platform technology, delivery platform technology and product candidates could have a material adverse effect on our business, financial condition, results of operations and growth prospects.

Our owned patents and patent applications and our in-licensed patents and patent applications contain claims directed to compositions of matter on our base editing product candidates, as well as methods directed to the use of such product candidates for gene therapy treatment. Method-of-use 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. 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, providers may recommend that patients use these products off-label, or patients may do so themselves.

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 foreign countries. For example, while our patent applications are pending, we may be subject to a third-party pre-issuance submission of prior art to the United States Patent and Trademark Office, or USPTO, or become involved in interference or derivation proceedings, or equivalent proceedings in foreign jurisdictions. Even if patents do successfully issue, third parties may challenge their inventorship, validity, enforceability or scope, including through opposition, revocation, reexamination, post-grant and *inter partes* review proceedings. An adverse determination in any such submission, proceeding or litigation could reduce the scope of, or invalidate or render unenforceable, our owned or in-licensed patent rights, allow third parties to commercialize our technology or product candidates 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. Moreover, we, or one of our licensors, may have to participate in interference proceedings declared by the USPTO to determine priority of invention or in post-grant challenge proceedings, such as oppositions in a foreign patent office, that challenge our or our licensor's priority of invention or other features of patentability with respect to our owned or in-licensed patents and patent applications. Such challenges may result in loss of patent rights, loss of exclusivity, or in patent claims being narrowed, invalidated, or held unenforceable, which could limit our ability to stop others from using or commercializing similar or identical technology and products, or limit the duration of the patent protection of our technology and product candidates. Furthermore, even if they are unchallenged, our patents and patent applications may not adequately protect our intellectual property or prevent others from designing around our claims. If the breadth or strength of protection provided by the patents and patent applications we own or the patents and patent applications we in-license with respect to our base editing platform technology, delivery platform technology and product candidates is threatened, it could dissuade companies from collaborating with us to develop, and threaten our ability to commercialize, our product candidates. Further, if we encounter delays in development, testing, and regulatory review of new product candidates, the period of time during which we could market our product candidates under patent protection would be reduced.

Given that 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 or our licensors were in the past or will be in the future the first to file any patent application related to our base editing technology, delivery platform technology or product candidates. In addition, some patent applications in the United States may be maintained in secrecy until the patents are issued. As a result, there may be prior art of which we or our licensors are not aware that may affect the validity or enforceability of a patent claim, and we or our licensors may be subject to priority disputes. For our in-licensed patent portfolios, we rely on our licensors to determine inventorship, and obtain and file inventor assignments of priority applications before their conversion as PCT applications. A failure to do so in a timely fashion may give rise to a challenge to entitlement of priority for foreign applications nationalized from such PCT applications. For example, the European Patent Office, or the EPO, Opposition Division, or the EPO Opposition Division, has revoked our optioned Broad Institute patent European Patent No. EP2771468 following a third-party challenge to its priority rights. The patent was revoked due to loss of priority. We or our licensors are subject to and may in the future become a party to proceedings or priority disputes in Europe or other foreign jurisdictions. The loss of priority for, or the loss of, these European patents could have a material adverse effect on the conduct of our business.

We may be required to disclaim part or all of the term of certain patents or 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 or our licensors are aware, but which we or our licensors 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. No assurance can be given that, if challenged, our patents would be declared by a court, patent office or other governmental authority to be valid or enforceable or that even if found valid and enforceable, a competitor's technology or product would be found by a court to infringe our patents. We may analyze patents or patent applications of our competitors that we believe are relevant to our activities, and consider that we are free to operate in relation to our product candidates, but our competitors may achieve issued claims, including in patents we consider to be unrelated, that block our efforts or potentially result in our product candidates or our activities infringing such claims. It is possible that our competitors may have filed, and may in the future file, patent applications covering our products or technology similar to ours. Those patent applications may have priority over our owned patent applications and in-licensed patent applications or patents, which could require us to obtain rights to issued patents covering such technologies. The possibility also exists that others will develop products that have the same effect as our product candidates on an independent basis that do not infringe our patents or other intellectual property rights, or will design around the claims of our patent applications or our in-licensed patents or patent applications that cover our product candidates.

Likewise, our currently owned patents and patent applications, if issued as patents, and in-licensed patents and patent applications, if issued as patents, directed to our proprietary base editing technologies and our product candidates are expected to expire from 2034 through 2046, without taking into account any possible patent term adjustments or extensions. Our owned or in-licensed patents may expire before, or soon after, our first product candidate achieves marketing approval in the United States or foreign jurisdictions. Additionally, no assurance can be given that the USPTO or relevant foreign patent offices will grant any of the pending patent applications we own or in-license currently or in the future. Upon the expiration of our current in-licensed patents, we may lose the right to exclude others from practicing these inventions. The expiration of these patents could also have a similar material adverse effect on our business, financial condition, results of operations and prospects.

Our owned patents and patent applications and in-licensed patents and patent applications and other intellectual property may be subject to priority disputes or to inventorship disputes and similar proceedings. If we or our licensors are unsuccessful in any of these proceedings, we may be required to obtain licenses from third parties, which may not be available on commercially reasonable terms or at all, or to cease the development, manufacture, and commercialization of one or more of the product candidates we may develop, which could have a material adverse impact on our business.

Although we have an option to exclusively license certain patents and patent applications directed to Cas9 and Cas12a from Editas, who in turn has licensed such patents from various academic institutions including Broad Institute, we do not currently have a license to such patents and patent applications. Certain of the U.S. patents and one U.S. patent application to which we hold an option are co-owned by Broad Institute and MIT, and in some cases co-owned by Broad Institute, MIT, and Harvard, which we refer to together as the Boston Licensing Parties, and were involved in U.S. interference No. 106,048 with one U.S. patent application co-owned by the University of California, the University of Vienna, and Emmanuelle Charpentier, which we refer to together as the University of California. On September 10, 2018, the Court of Appeals for the Federal Circuit, or the CAFC, affirmed the Patent Trial and Appeal Board of the USPTO's, or PTAB's, holding that there was no interference-in-fact. An interference is a proceeding within the USPTO to determine priority of invention of the subject matter of patent claims filed by different parties.

On June 24, 2019, the PTAB declared an interference (U.S. Interference No. 106,115) between ten U.S. patent applications ((U.S. Serial Nos. 15/947,680; 15/947,700; 15/947,718; 15/981,807; 15/981,808; 15/981,809; 16/136,159; 16/136,165; 16/136,168; and 16/136,175) that are co-owned by the University of California, and 13 U.S. patents and one U.S. patent application (U.S. Patent Nos. 8,697,359; 8,771,945; 8,795,965; 8,865,406; 8,871,445; 8,889,356; 8,895,308; 8,906,616; 8,932,814; 8,945,839; 8,993,233; 8,999,641; and 9,840,713, and U.S. Serial No. 14/704,551)) that are co-owned by the Boston Licensing Parties, which we have an option to under the Editas License Agreement. In the declared interference, the University of California has been designated as the junior party and the Boston Licensing Parties have been designated as the senior party.

As a result of the declaration of interference, an adversarial proceeding in the USPTO before the PTAB has been initiated, which is declared to ultimately determine priority, specifically and which party was first to invent the claimed subject matter. An interference is typically divided into two phases. The first phase is referred to as the motions or preliminary motions phase while the second is referred to as the priority phase. In the first phase, each party may raise issues including but not limited to those relating to the patentability of a party's claims based on prior art, written description, and enablement. A party also may seek an earlier priority benefit or may challenge whether the declaration of interference was proper in the first place. Priority, or a determination of who first invented the commonly claimed invention, is determined in the second phase of an interference. The ten University of California patent applications and the 13 U.S. patents and one U.S. patent application co-owned by the Boston Licensing Parties involved in U.S. Interference No. 106,115 generally relate to CRISPR/Cas9 systems or eukaryotic cells comprising CRISPR/Cas9 systems having fused or covalently linked RNA and the use thereof in eukaryotic cells. On February 28, 2022, the PTAB issued a decision that the Boston Licensing Parties have priority of invention over University of California with respect to a single RNA CRISPR-Cas9 system that functions in eukaryotic cells. This decision is being appealed. There can be no assurance that the U.S. interference will be resolved in favor of the Boston Licensing Parties on appeal. If the U.S. interference resolves in favor of University of California, or if the Boston Licensing Parties' patents and patent application are narrowed, invalidated, or held unenforceable, we may lose the ability to license the optioned patents and patent application and our ability to commercialize our product candidates may be adversely affected if we cannot obtain a license to relevant third party patents that cover our product candidates. 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 nonexclusive, thereby giving our competitors and other third parties access to the same technologies licensed to us, and it could require us to make substantial licensing and royalty payments. If we are unable to obtain a necessary license to a third-party patent on commercially reasonable terms, we may be unable to commercialize our base editing platform technology or product candidates or such commercialization efforts may be significantly delayed, which could in turn significantly harm our business.

We or our licensors may be subject to similar interferences in the future with the same risks as described above. For example, on December 14, 2020, the PTAB declared an interference (U.S. Interference No. 106,126) between 14 U.S. patents and two U.S. patent applications (U.S. Patent Nos. 8,697,359; 8,771,945; 8,795,965; 8,865,406; 8,871,445; 8,889,356; 8,889,418; 8,895,308; 8,906,616; 8,932,814; 8,945,839; 8,993,233; 8,999,641; and 9,840,713, and U.S. Serial Nos. 14/704,551 and 15/330,876) that are co-owned by the Boston Licensing Parties, which we have an option to under the Editas License Agreement, and one U.S. patent application (U.S. Serial Nos. 14/685,510) that is owned by Toolgen, Inc, or Toolgen. In the declared interference, the Boston Licensing Parties have

been designated as the junior party and Toolgen has been designated as the senior party. On September 28, 2022, the PTAB issued an order suspending proceedings in the priority phase of the interference. We cannot predict with any certainty when a decision will be made. The 14 U.S. patents and two U.S. patent applications co-owned by the Boston Licensing Parties involved in U.S. Interference No. 106,126 generally relate to CRISPR/Cas9 systems or eukaryotic cells comprising CRISPR/Cas9 systems having fused or covalently linked RNA and the use thereof in eukaryotic cells.

On June 21, 2021, the PTAB declared an interference (U.S. Interference No. 106,133) between the same 14 U.S. patents and two U.S. patent applications (U.S. Patent Nos. 8,697,359; 8,771,945; 8,795,965; 8,865,406; 8,871,445; 8,889,356; 8,889,418; 8,895,308; 8,906,616; 8,932,814; 8,945,839; 8,993,233; 8,999,641; and 9,840,713, and U.S. Serial Nos. 14/704,551 and 15/330,876, co-owned by the Boston Licensing Parties) as named in the interference with Toolgen, and one U.S. patent application (U.S. Serial Nos. 15/456,204) that is owned by Sigma-Aldrich Co., LLC, or Sigma-Aldrich. In the declared interference, the Boston Licensing Parties have been designated as the junior party and Sigma-Aldrich has been designated as the senior party. On December 14, 2022, the PTAB issued an order suspending proceedings in the priority phase of the interference. We cannot predict with any certainty when a decision will be made.

We or our licensors may also be subject to claims that former employees, collaborators, or other third parties have an interest in our owned patents or patent applications or in-licensed patents or patent applications or other intellectual property as an inventor or co-inventor. If we are unable to obtain an exclusive license to any such third-party co-owners' interest in such patents or patent applications, such co-owners may be able to license their rights to other third parties, including our competitors. In addition, we may need the cooperation of any such co-owners to enforce any patents that issue from such patent applications against third parties, and such cooperation may not be provided to us.

If we or our licensors are unsuccessful in any interference proceedings or other priority, validity (including any patent oppositions), or inventorship disputes to which we or they are subject, we may lose valuable intellectual property rights through the loss of one or more of our owned, licensed, or optioned patents (for example, European Patent No. EP3,115,457 B1, which we sublicensed from Bio Palette and which was subsequently revoked), or such patent claims may be narrowed (for example, European Patent No. EP3,604,511 B1, which we licensed from Harvard and which was subsequently narrowed), invalidated, or held unenforceable, or through loss of exclusive ownership of or the exclusive right to use our owned or in-licensed patents. In the event of loss of patent rights as a result of any of these disputes, we may be required to obtain and maintain licenses from third parties, including parties involved in any such interference proceedings or other priority or inventorship disputes. Such licenses may not be available on commercially reasonable terms or at all, or may be non-exclusive. If we are unable to obtain and maintain such licenses, we may need to cease the development, manufacture, and commercialization of one or more of the product candidates we may develop. The loss of exclusivity or the narrowing of our patent claims could limit our ability to stop others from using or commercializing similar or identical technology and product candidates. Even if we or our licensors are successful in an interference proceeding or other similar priority or inventorship disputes, it could result in substantial costs and be a distraction to management and other employees. Any of the foregoing could result in a material adverse effect on our business, financial condition, results of operations, or prospects.

We have limited foreign intellectual property rights and may not be able to protect our intellectual property and proprietary rights throughout the world.

We have limited intellectual property rights outside the United States. Filing, prosecuting, and defending patents on product candidates in all countries throughout the world would be prohibitively expensive, and our intellectual property rights in some countries outside the United States can be less extensive than those in the United States. In addition, the laws of foreign countries do not protect intellectual property rights to the same extent as federal and state laws of the United States. In addition, our intellectual property license agreements may not always include worldwide rights. Consequently, we may not be able to prevent third parties from practicing our inventions in all countries outside the United States, or from selling or importing products made using our inventions in and into the United States or other jurisdictions. Competitors may use our technologies in jurisdictions where we have not obtained patent protection to develop their own products and, further, may export otherwise infringing products to territories where we have patent protection but where enforcement is not as strong as that in the United States. These products may compete with our product candidates and our patents or other intellectual property rights may not be effective or sufficient to prevent them from competing.

Many companies have encountered significant problems in protecting and defending intellectual property rights in foreign jurisdictions. The legal systems of certain countries, particularly certain developing countries, do not favor the enforcement of patents, trade secrets, and other intellectual property protection, particularly those relating to biotechnology and pharmaceutical products, which could make it difficult for us to stop the infringement of our patents or marketing of competing products against third parties in violation of our intellectual property and proprietary rights generally. Proceedings to enforce our patents and intellectual property rights in foreign jurisdictions could result in substantial costs and divert our efforts and attention from other aspects of our business, could put our patents at risk of being invalidated or interpreted narrowly and our patent applications at risk of not issuing, and could provoke third parties to assert claims against us. We may not prevail in any lawsuits that we initiate, and the damages or other remedies awarded, if any, may not be commercially meaningful. Moreover, the initiation of proceedings by third parties to challenge the scope or validity of our patent rights in foreign jurisdictions could result in substantial cost and divert our efforts and attention

from other aspects of our business. Accordingly, our efforts to enforce our intellectual property and proprietary rights around the world may be inadequate to obtain a significant commercial advantage from the intellectual property that we develop or license.

Many countries have compulsory licensing laws under which a patent owner may be compelled to grant licenses to third parties. In addition, many countries limit the enforceability of patents against government agencies or government contractors. In these countries, the patent owner may have limited remedies, which could materially diminish the value of such patent. If we or any of our licensors is forced to grant a license to third parties with respect to any patents relevant to our business, our competitive position may be impaired, and our business, financial condition, results of operations, and prospects may be adversely affected.

If we fail to comply with our obligations in the agreements under which we license intellectual property rights from third parties or otherwise experience disruptions to our business relationships with our licensors, we could lose license rights that are important to our business.

We have entered into license agreements with third parties and may need to obtain additional licenses from our existing licensors and others to advance our research or allow commercialization of product candidates we may develop. It is possible that we may be unable to obtain any additional licenses at a reasonable cost or on reasonable terms, if at all. In either event, 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 product candidates, which could harm our business, financial condition, results of operations, and prospects significantly. We cannot provide any assurances that third-party patents do not exist which might be enforced against our current technology, including base editing technology, delivery platform technology, manufacturing methods, product candidates, or future methods or products resulting in either an injunction prohibiting our manufacture or future sales, or, with respect to our future sales, an obligation on our part to pay royalties and/or other forms of compensation to third parties, which could be significant.

In each of our license agreements, we are generally responsible for bringing any actions against any third party for infringing on the patents we have licensed. Certain of our license agreements also require us to meet development thresholds to maintain the license, including establishing a set timeline for developing and commercializing products. In spite of our efforts, our licensors might conclude that we have materially breached our obligations under such license agreements and might therefore terminate the license agreements, thereby removing or limiting our ability to develop and commercialize products and technology covered by these license agreements. If these in-licenses are terminated, or if the underlying patents fail to provide the intended exclusivity, competitors or other third parties would have the freedom to seek regulatory approval of, and to market, products identical to ours and we may be required to cease our development and commercialization of or base editing platform technology, delivery platform technology or product candidates. Any of the foregoing could have a material adverse effect on our competitive position, business, financial conditions, results of operations, and growth prospects. Disputes may arise regarding intellectual property subject to a licensing agreement, including:

- the scope of rights granted under the license agreement and other interpretation-related issues;
- the extent to which our technology and processes infringe on intellectual property of the licensor that is not subject to the licensing agreement;
- the sublicensing of patent and other rights to third parties under our collaborative development relationships;
- our diligence obligations under the license agreement 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 and ownership of inventions and know-how resulting from the joint creation or use of intellectual property by our licensors and us and our partners; and
- the priority of invention of patented technology.

In addition, 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 broaden what we believe to be the scope of the licensor's rights to our intellectual property and technology, or increase what we believe to be our financial or other obligations under the relevant agreement, any of which could have a material adverse effect on our business, financial condition, results of operations, and prospects. Moreover, if disputes over intellectual property that we have licensed prevent or impair our ability to maintain our current licensing arrangements on commercially acceptable terms, we may be unable to successfully develop and commercialize the affected product candidates. As a result, any termination of or disputes over our intellectual property licenses could result in the loss of our ability to develop and commercialize our base editing platform, delivery platform, or other product candidates or we could lose other significant rights, any of which could have a material adverse effect on our business, financial conditions, results of operations, and prospects. It is also possible that a third party could be granted limited licenses to some of the same technology, in certain circumstances.

We may not be successful in acquiring or in-licensing necessary rights to key technologies or any product candidates we may develop.

We currently have rights to intellectual property, through licenses from third parties, to identify and develop product candidates, and we expect to seek to expand our product candidate pipeline in part by in-licensing the rights to key technologies. The future growth of our business will depend in part on our ability to in-license or otherwise acquire the rights to additional product candidates and technologies. Although we have succeeded in licensing technologies from third party licensors, including Harvard, Broad Institute, Editas, and Bio Palette in the past, we cannot assure you that we will be able to in-license or acquire the rights to any product candidates or technologies from third parties on acceptable terms or at all.

For example, our agreements with certain of our third-party licensors provide that our field of use excludes particular fields, for example, the use of certain gene editing technologies for the diagnosis, treatment, and prevention of human cancers through certain engineered T-cells, which are licensed exclusively or non-exclusively to a third-party licensee. If we determine that rights to such fields are necessary to commercialize our drug candidates or maintain our competitive advantage, we may need to obtain a license from such third party in order to 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 drug candidates or allow our competitors or others the chance to access technology that is important to our business.

Furthermore, there has been extensive patenting activity in the fields of gene editing and delivery technologies, and pharmaceutical companies, biotechnology companies, and academic institutions are competing with us or are expected to compete with us in the fields of gene editing and delivery technologies and filing patent applications potentially relevant to our business and we are aware of certain third-party patents, as well as patent applications that, if issued, may allow the third party to circumvent our patent rights. For example, we are aware of several third-party patents, and patent applications that, if issued, may be construed to cover our base editing technology, delivery technology and product candidates. In order to market our product candidates, we may find it necessary or prudent to obtain licenses from such third-party intellectual property holders. However, we may be unable to secure such licenses or otherwise acquire or in-license any compositions, methods of use, processes, or other intellectual property rights from third parties that we identify as necessary for product candidates we may develop and base editing and delivery technologies. We may also require licenses from third parties for additional non-base editing technologies, including additional delivery methods that we are evaluating for use with product candidates we are developing and may develop in the future. In addition, some of our owned patents and patent applications and in-licensed patents and patent applications are co-owned with third parties. With respect to any patents co-owned with third parties, we may require licenses to such co-owners' interest to such patents. If we are unable to obtain an exclusive license to any such third-party co-owners' interest in such patents or patent applications, such co-owners may be able to license their rights to other third parties, including our competitors, and our competitors could market competing products and technology. In addition, we may need the cooperation of any such co-owners of our patents in order to enforce such patents against third parties, and such cooperation may not be provided to us.

Additionally, we may collaborate with academic institutions to accelerate our preclinical research or development under written agreements with these institutions. In certain cases, these institutions provide us with an option to negotiate a license to any of the institution's rights in technology resulting from the collaboration. Even if we hold such an option, we may be unable to negotiate a license from the institution within the specified timeframe or under terms that are acceptable to us. If we are unable to do so, the institution may offer the intellectual property rights to others, potentially blocking our ability to pursue our program.

In addition, the licensing or acquisition of third-party intellectual property rights is a highly competitive area, and a number of more established companies are also pursuing strategies to license or acquire third party intellectual property rights that we may consider attractive or necessary. These established companies may have a competitive advantage over us due to their size, capital resources and greater clinical development and commercialization capabilities. In addition, companies that perceive us to be a competitor may be unwilling to assign or license rights to us. We also may be unable to license or acquire third party intellectual property rights on terms that would allow us to make an appropriate return on our investment or at all. If we are unable to successfully obtain rights to required third party intellectual property rights or maintain the existing intellectual property rights we have, we may have to abandon development of the relevant program or product candidate, which could have a material adverse effect on our business, financial condition, results of operations, and prospects.

The intellectual property landscape around gene editing technology, including base editing and delivery technology, is highly dynamic, and third parties may initiate legal proceedings alleging that we are infringing, misappropriating, or otherwise violating their intellectual property rights, the outcome of which would be uncertain and may prevent, delay or otherwise interfere with our product discovery and development efforts.

The field of gene editing, especially in the area of base editing technology, is still in its infancy, and no base editing product candidates have reached the market. Due to the intense research and development that is taking place by several companies, including us and our competitors, in this field and in the field of delivery technology, the intellectual property landscape is evolving and in flux, and it may remain uncertain for the coming years. There may be significant intellectual property related litigation and proceedings relating to our owned and in-licensed, and other third party, intellectual property and proprietary rights in the future.

Our commercial success depends upon our ability and the ability of our collaborators and licensors to develop, manufacture, market, and sell any product candidates that we may develop and use our proprietary technologies without infringing, misappropriating, or otherwise violating the intellectual property and proprietary rights of third parties. The biotechnology and pharmaceutical industries are characterized by extensive litigation regarding patents and other intellectual property rights as well as administrative proceedings for challenging patents, including interference, derivation, inter partes review, post grant review, and reexamination proceedings before the USPTO or oppositions and other comparable proceedings in foreign jurisdictions. We may be subject to and may in the future become party to, or threatened with, adversarial proceedings or litigation regarding intellectual property rights with respect to our base editing platform technology, delivery platform technology and any product candidates we may develop, including interference proceedings, post-grant review, inter partes review, and derivation proceedings before the USPTO and similar proceedings in foreign jurisdictions such as oppositions before the EPO. Numerous U.S. and foreign issued patents and pending patent applications that are owned by third parties exist in the fields in which we are developing our product candidates and they may assert infringement claims against us based on existing patents or patents that may be granted in the future, regardless of their merit.

As the biotechnology and pharmaceutical industries expand and more patents are issued, the risk increases that our base editing platform technology, delivery platform technology and 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, which patents cover various types of therapies, products or their methods of use or manufacture. We are aware of certain third-party patents and patent applications that, if issued, may be construed to cover our base editing technology, delivery technology and product candidates. There may also be third-party patents of which we are currently unaware with claims to technologies, methods of manufacture or methods for treatment related to the use or manufacture of our product candidates. Because patent applications can take many years to issue, there may be currently pending patent applications that may later result in issued patents that our product candidates may infringe. In addition, third parties may obtain patents in the future and claim that use of our technologies infringes upon these patents.

Numerous third-party U.S. and foreign issued patents and pending patent applications exist in the fields in which we are developing product candidates. Our product candidates make use of CRISPR-based technology, which is a field that is highly active for patent filings. The extensive patent filings related to CRISPR and Cas make it difficult for us to assess the full extent of relevant patents and pending applications that may cover our base editing platform technology and product candidates and their use or manufacture. There may be third-party patents or patent applications with claims to materials, formulations, methods of manufacture or methods for treatment related to the use or manufacture of our base editing platform technology and product candidates. For example, we are aware of a patent portfolio that is co-owned by the University of California, University of Vienna and Emmanuelle Charpentier, or the University of California Portfolio, which contains multiple patents and pending applications directed to gene editing. The University of California portfolio includes, for example, U.S. Patent Nos. 10,266,850; 10,227,611; 10,000,772; 10,113,167; 10,301,651; 10,308,961; 10,337,029; 10,351,878; 10,407,697; 10,358,659; 10,358,658; 10,385,360; 10,400,253; 10,421,980; 10,415,061; 10,428,352; 10,443,076; 10,487,341; 10,513,712; 10,519,467; 10,526,619; 10,533,190; 10,550,407; 10,563,227; 10,570,419; 10,577,631; 10,597,680; 10,612,045; 10,626,419; 10,640,791; 10,669,560; 10,676,759; 10,752,920; 10,774,344; 10,793,878; 10,900,054; 10,982,230; 10,982,231; 10,988,780; 10,988,782; 11,001,863; 11,008,589; 11,008,590; 11,028,412; 11,186,849; 11,242,543; 11,274,318; 11,293,034; 11,332,761; 11,401,532; 11,473,108; 11,479,794; 11,549,127; 11,634,730; 11,674,159; 11,814,645; 11,970,711; 12,123,015; 12,180,503; 12,180,504; 12,215,343, which are expected to expire around March 2033, excluding any additional term for patent term adjustment, or PTA, or patent term extension, or PTE, and any disclaimed term for terminal disclaimers. The University of California portfolio also includes numerous additional pending patent applications. If these patent applications issue as patents, they are expected to expire around March 2033, excluding any PTA, PTE, and any disclaimed term for terminal disclaimers. As discussed above, certain applications in the University of California Portfolio are currently subject to U.S. Interference No. 106,115 with certain U.S. patents and one U.S. patent application that are co-owned by the Boston Licensing Parties to which we have an option under the Editas License Agreement. Although we have an option to exclusively license certain patents and patent applications directed to Cas9 and Cas12a from Editas, who in turn has licensed such patents from various academic institutions including Broad Institute, we do not currently have a license to such patents and patent applications. Certain members of the University of California Portfolio have been or are being opposed in Europe by multiple parties. For example, European Patent Nos. EP2,800,811 B1, and EP3,241,902 B1, EP3,401,400 B1 and EP3,597,749 B1 have been opposed, which patents are estimated to expire in March 2033 (excluding any patent term adjustments or extensions). The opposition procedure before the EPO allows one or more third parties to challenge the validity of a granted European patent within nine months after grant date of the European patent. Opposition proceedings may involve issues including, but not limited to, priority, patentability of the claims involved, and procedural formalities related to the filing of the patent application. As a result of the opposition proceedings, the Opposition Division can revoke a patent, maintain the patent as granted, or maintain the patent in an amended form. In April 2021, the claims of European patent EP3,241,902 B1 were revoked in their entirety by the Opposition Division, and that decision was not appealed. In November 2024, European patents EP2,800,811 B1 and EP3,401,400 B1 were revoked by the Boards of Appeal of the European Patent Office. In November 2025, the claims of European patent EP3,597,749 B1 were revoked in their entirety by the Opposition Division, and that decision is being appealed. It is uncertain how oppositions filed against EP3,597,749 B1 will be resolved. If this patent is maintained by the Boards of Appeal with claims similar to those that were opposed, our ability to commercialize our product candidates may be adversely affected if we do not obtain a license to these patents. 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 nonexclusive, thereby giving our competitors and other

third parties access to the same technologies licensed to us, and it could require us to make substantial licensing and royalty payments. If we are unable to obtain a necessary license to a third-party patent on commercially reasonable terms, we may be unable to commercialize our base editing platform technology or product candidates or such commercialization efforts may be significantly delayed, which could in turn significantly harm our business.

Numerous other patents and patent applications have been filed by other third parties directed to gene editing, guide nucleic acids, PAM sequence variants, split inteins, Cas12b or gene editing in the context of immune therapy or chimeric antigen receptors.

Because of the large number of patents issued and patent applications filed in our field, third parties may allege they have patent rights encompassing our product candidates, technologies or methods. Third parties may assert that we are employing their proprietary technology without authorization and may file patent infringement claims or lawsuit against us, and if we are found to infringe such third-party patents, we may be required to pay damages, cease commercialization of the infringing technology, or obtain a license from such third parties, which may not be available on commercially reasonable terms or at all.

Our ability to commercialize our product candidates in the United States and abroad may be adversely affected if we cannot obtain a license on commercially reasonable terms to relevant third-party patents that cover our product candidates, delivery platform technology or base editing platform technology. Even if we believe third-party intellectual property claims are without merit, there is no assurance that a court would find in our favor on questions of infringement, validity, enforceability, or priority. A court of competent jurisdiction could hold that these third-party patents are valid, enforceable, and infringed, which could materially and adversely affect our ability to commercialize any product candidates we may develop and any other product candidates or technologies covered by the asserted third-party patents. In order to successfully challenge the validity of any such U.S. patent in federal court, we would need to overcome a presumption of validity. As this burden is a high one requiring us to present clear and convincing evidence as to the invalidity of any such U.S. patent claim, there is no assurance that a court of competent jurisdiction would invalidate the claims of any such U.S. patent. If we are found to infringe a third party's intellectual property rights, and we are unsuccessful in demonstrating that such patents are invalid or unenforceable, we could be required to obtain a license from such third party to continue developing, manufacturing, and marketing any product candidates we may develop and our technology. However, 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 and other third parties access to the same technologies licensed to us, and it could require us to make substantial licensing and royalty payments. If we are unable to obtain a necessary license to a third-party patent on commercially reasonable terms, we may be unable to commercialize our base editing platform technology, delivery platform technology or product candidates or such commercialization efforts may be significantly delayed, which could in turn significantly harm our business. We also could be forced, including by court order, to cease developing, manufacturing, and commercializing the infringing technology or product candidates. 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 or other intellectual property right. Claims that we have misappropriated the confidential information or trade secrets of third parties could have a similar material adverse effect on our business, financial condition, results of operations, and prospects.

Defense of third-party claims of infringement of misappropriation, or violation of intellectual property rights involves substantial litigation expense and would be a substantial diversion of management and employee time and resources from our business. Some third parties may be able to sustain the costs of complex patent litigation more effectively than we can because they have substantially greater resources. In addition, any uncertainties resulting from the initiation and continuation of any litigation could have a material adverse effect on our ability to raise the funds necessary to continue our operations or could otherwise have a material adverse effect on our business, financial condition, results of operations and prospects. There could also 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, it could have a substantial adverse effect on the price of our common stock. Any of the foregoing events could have a material adverse effect on our business, financial condition, results of operations and prospects.

We may become involved in lawsuits to protect or enforce our patents, future patents or the patents of our licensors, which could be expensive, time consuming, and unsuccessful and could result in a finding that such patents are unenforceable or invalid.

Competitors may infringe our patents, future patents or the patents of our licensing partners, or we may be required to defend against claims of infringement. In addition, our patents, future patents or the patents of our licensing partners also are, and may in the future become, involved in inventorship, priority, validity or enforceability disputes. Countering or defending against such claims can be expensive and time consuming. In an infringement proceeding, a court may decide that a patent owned or in-licensed by us is invalid or unenforceable, or may refuse to stop the other party from using the technology at issue on the grounds that our owned and in-licensed patents do not cover the technology in question. An adverse result in any litigation proceeding could put one or more of our owned or in-licensed patents at risk of being invalidated or interpreted narrowly.

In patent litigation in the United States, defendant counterclaims alleging invalidity and/or unenforceability are commonplace, and there are numerous grounds upon which a third party can assert invalidity or unenforceability of a patent. Third parties may also raise similar claims before administrative bodies in the United States or abroad, even outside the context of litigation. These types of mechanisms include re-examination, post-grant review, *inter partes* review, interference proceedings, derivation proceedings, and

equivalent proceedings in foreign jurisdictions (e.g., opposition proceedings). These types of proceedings could result in revocation or amendment to our patents such that they no longer cover our product candidates. The outcome for any particular patent 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 licensors, 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, or if we are otherwise unable to adequately protect our rights, we would lose at least part, and perhaps all, of the patent protection on our technology and/or product candidates. Defense of these types of claims, regardless of their merit, would involve substantial litigation expense and would be a substantial diversion of employee resources from our business.

Conversely, we may choose to challenge the patentability of claims in a third party's U.S. patent by requesting that the USPTO review the patent claims in re-examination, post-grant review, *inter partes* review, interference proceedings, derivation proceedings, and equivalent proceedings in foreign jurisdictions (e.g., opposition proceedings). We are currently challenging, and in the future may choose to challenge, third party patents in patent opposition proceedings in the EPO or another foreign patent office. Even if successful, the costs of these opposition proceedings could be substantial, and may consume our time or other resources. If we fail to obtain a favorable result at the USPTO, EPO or other patent office then we may be exposed to litigation by a third party alleging that the patent may be infringed by our product candidates, base editing platform technology, delivery platform technology or other or proprietary technologies.

For example, as discussed above, elements of the University of California patent portfolio are being opposed in Europe by multiple parties and we are participating in the opposition proceedings. The EPO Opposition Division, or the Opposition Division, has initiated opposition proceedings against European patents estimated to expire in March 2033 (excluding any patent term adjustments or extensions) and co-owned by the University of California. The opposition procedure before the EPO allows one or more third parties to challenge the validity of a granted European patent within nine months after grant date of the European patent. Opposition proceedings may involve issues including, but not limited to, priority, patentability of the claims involved, and procedural formalities related to the filing of the patent application. As a result of the opposition proceedings, the Opposition Division can revoke a patent, maintain the patent as granted, or maintain the patent in an amended form. It is uncertain when or in what manner the Opposition Division will act on the opposition proceedings of these European patents. If these patents are maintained by the Opposition Division with claims similar to those that are currently opposed, our ability to commercialize our product candidates may be adversely affected if we do not obtain a license to these patents. 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 nonexclusive, thereby giving our competitors and other third parties access to the same technologies licensed to us, and it could require us to make substantial licensing and royalty payments. If we are unable to obtain a necessary license to a third-party patent on commercially reasonable terms, we may be unable to commercialize our base editing platform technology, delivery platform technology or product candidates or such commercialization efforts may be significantly delayed, which could in turn significantly harm our business.

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

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 applications are due to be paid to the USPTO and foreign patent agencies outside of the United States over the lifetime of our owned or licensed patents and applications. In certain circumstances, we rely on our licensing partners to pay these fees due to U.S. and non-U.S. patent agencies. The USPTO and foreign patent agencies require compliance with several procedural, documentary, fee payment, and other similar provisions during the patent application process. We are also dependent on our licensors to take the necessary action to comply with these requirements with respect to our licensed intellectual property. While an inadvertent lapse 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 a partial or complete loss of patent rights in the relevant jurisdiction. Were a noncompliance event to occur, our competitors might be able to

enter the market with similar or identical products or technology, which could have a material adverse effect on our business, financial condition, results of operations, and prospects.

Changes in patent law in the United States and in non-U.S. jurisdictions could diminish the value of patents in general, thereby impairing our ability to protect our platform technologies and product candidates.

As is the case with other biotech and pharmaceutical companies, our success is heavily dependent on intellectual property, particularly patents. Obtaining and enforcing patents in the biopharmaceutical industry involve both technological and legal complexity, and is therefore costly, time-consuming and inherently uncertain.

Changes in either the patent laws or interpretation of the patent laws could increase the uncertainties and costs surrounding the prosecution of patent applications and the enforcement or defense of our issued patents. For example, in March 2013, under the Leahy-Smith America Invents Act, or the America Invents Act, the United States transitioned from a "first to invent" to a "first-to-file" patent system. Under a "first-to-file" system, assuming that other requirements for patentability are met, the first inventor to file a patent application generally will be entitled to a patent on an invention regardless of whether another inventor had made the invention earlier. A third party that files a patent application in the USPTO after March 2013, but before us could therefore be awarded a patent covering an invention of ours even if we had made the invention before it was made by such third party. This will require us to be cognizant going forward of the time from invention to filing of a patent application. Since patent applications in the United States and most other countries are confidential for a period of time after filing or until issuance, we cannot be certain that we or our licensors were the first to either file any patent application related to our technology or product candidates or invent any of the inventions claimed in our or our licensor's patents or patent applications. The America Invents Act also includes a number of other significant changes to U.S. patent law, including provisions that affect the way patent applications are prosecuted, allowing third party submission of prior art and establish a new post-grant review system including post-grant review, *inter partes* review, and derivation proceedings. Because of a lower evidentiary standard in USPTO proceedings compared to the evidentiary standard in United States 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. The effects of some of these changes are currently unclear as the USPTO continues to promulgate new regulations and procedures in connection with the America Invents Act. In addition, the courts have yet to address many of these provisions and the applicability of the act and new regulations on the specific patents discussed in this filing have not been determined and would need to be reviewed. However, the America Invents Act and its implementation could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents.

In addition, recent U.S. Supreme Court rulings have narrowed the scope of patent protection available in certain circumstances and weakened the rights of patent owners in certain situations. In addition to increasing uncertainty with regard to our ability to obtain patents in the future, this combination of events has created uncertainty with respect to the validity and enforceability of patents, once obtained. Depending on future actions by the U.S. Congress, the federal courts, and the USPTO, the laws and regulations governing patents could change in unpredictable ways that could weaken our ability to obtain new patents or to enforce our existing patents and patents that we might obtain in the future. For example, in the case, *Assoc. for Molecular Pathology v. Myriad Genetics, Inc.,* the U.S. Supreme Court held that certain claims to DNA molecules are not patentable. We cannot predict how this and future decisions by the courts, the U.S. Congress or the USPTO may impact the value of our patents. Any similar adverse changes in the patent laws of other jurisdictions could also have a material adverse effect on our business, financial condition, results of operations and prospects.

Patent terms may be inadequate to protect our competitive position on our product candidates for an adequate amount of time.

Patents have a limited lifespan. The terms of individual patents depend upon the legal term for patents in the countries in which they are granted. In most countries, including the United States, if all maintenance fees are timely paid, the natural expiration of a patent is generally 20 years from its earliest non-provisional filing date in the applicable country. However, the actual protection afforded by a patent varies from country to country, and depends upon many factors, including the type of patent, the scope of its coverage, the availability of regulatory-related extensions, the availability of legal remedies in a particular country and the validity and enforceability of the patent. Various extensions including PTE and PTA, may be available, but the life of a patent, and the protection it affords, is limited. Even if patents covering our product candidates are obtained, once the patent life has expired, we may be open to competition from competitive products, including generics. Given the amount of time required for the development, testing and regulatory review of new product candidates, patents protecting our product candidates might expire before or shortly after we or our partners commercialize those candidates. As a result, our owned and licensed patent portfolio may not provide us with sufficient rights to exclude others from commercializing products similar or identical to ours.

If we do not obtain PTE and data exclusivity for any product candidates we may develop, our business may be materially harmed.

Depending upon the timing, duration and specifics of any FDA marketing approval of any product candidates we may develop, one or more of our U.S. patents may be eligible for limited PTE under the Drug Price Competition and Patent Term Restoration Act of 1984, or the Hatch-Waxman Amendments. The Hatch-Waxman Amendments PTE term of up to five years as compensation for patent term lost during the FDA regulatory review process. A PTE cannot extend the remaining term of a patent beyond a total of 14 years from the date of product approval, only one patent per product 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, even if we were to seek a PTE, it may not be granted because of, for example, the failure to exercise due diligence during the testing phase or regulatory review process, the failure to apply within applicable deadlines, the failure to apply prior to expiration of relevant patents, or any other failure to satisfy applicable requirements. Moreover, the applicable time period or the scope of patent protection afforded could be less than we request. If we are unable to obtain PTE or term of any such extension is less than we request, our competitors may obtain approval of competing products following our patent expiration, and our business, financial condition, results of operations, and prospects could be materially harmed.

If we are unable to protect the confidentiality of our trade secrets, our business and competitive position would be harmed.

In addition to seeking patents for our technology and product candidates, we also rely on know-how and trade secret protection, as well as confidentiality agreements, non-disclosure agreements and invention assignment agreements with our employees, consultants and third parties, to protect our confidential and proprietary information, especially where we do not believe patent protection is appropriate or obtainable.

It is our policy to require our employees, corporate collaborators, outside scientific collaborators, CROs, contract manufacturers, consultants, advisors, and other third parties to execute confidentiality agreements upon the commencement of employment or consulting relationships with us. These agreements provide that all confidential information concerning our business or financial affairs developed by or made known to the individual or entity during the course of the party's relationship with us is to be kept confidential and not disclosed to third parties, except in certain specified circumstances. In the case of employees, the agreements provide that all inventions conceived by the individual, and that are related to our current or planned business or research and development or made during normal working hours, on our premises or using our equipment or proprietary information, are our exclusive property. In the case of consultants and other third parties, the agreements provide that all inventions conceived in connection with the services provided are our exclusive property. However, we cannot guarantee that we have entered into such agreements with each party that may have or have had access to our trade secrets or proprietary technology and processes. Additionally, the assignment of intellectual property rights may not be self-executing, or the assignment agreements may be breached, and we may be forced to bring claims against third parties, or defend claims that they may bring against us, to determine the ownership of what we regard as our intellectual property. Any of these parties may breach the agreements and disclose our proprietary information, including our trade secrets, and we may not be able to obtain adequate remedies for such breaches. Enforcing a claim that a party illegally disclosed or misappropriated a trade secret is difficult, expensive, and time-consuming, and the outcome is unpredictable.

In addition to contractual measures, we try to protect the confidential nature of our proprietary information through other appropriate precautions, such as physical and technological security measures. However, trade secrets and know-how can be difficult to protect. These measures may not, for example, in the case of misappropriation of a trade secret by an employee or third party with authorized access, provide adequate protection for our proprietary information. Our security measures may not prevent an employee or consultant from misappropriating our trade secrets and providing them to a competitor, and any recourse we might take against this type of misconduct may not provide an adequate remedy to protect our interests fully. In addition, trade secrets may be independently developed by others in a manner that could prevent us from receiving legal recourse. If any of our confidential or proprietary information, such as our trade secrets, were to be disclosed or misappropriated, or if any of that information was independently developed by a competitor, our competitive position could be harmed.

In addition, some courts inside and outside the United States are sometimes less willing or unwilling to protect trade secrets. If we choose to go to court to stop a third party from using any of our trade secrets, we may incur substantial costs. Even if we are successful, these types of lawsuits may consume our time and other resources. Any of the foregoing could have a material adverse effect on our business, financial condition, results of operations and prospects.

Third parties have asserted and may in the future assert that we, our employees, consultants, or advisors have wrongfully used or disclosed confidential information or misappropriated trade secrets.

As is common in the biotechnology and pharmaceutical industries, we employ individuals that are currently or were previously employed at universities, research institutions or other biotechnology or pharmaceutical companies, including our competitors or potential competitors. In addition, we regularly enter into non-disclosure and confidentiality agreements to protect the proprietary positions of third parties, such as research institutions, outside scientific collaborators, CROs, third-party manufacturers, consultants, advisors, potential partners and other third parties in order to evaluate technology for potential development. Although we try to ensure that we and our employees, consultants, and advisors do not use the proprietary information or know-how of others, we have received and may in the future be subject to claims that we or these individuals have inadvertently or otherwise wrongfully used or disclosed intellectual property, including trade secrets or other proprietary information, of third parties, including any such individual's current or former employer. Also, we have in the past and may in the future be subject to claims that these individuals are violating non-compete agreements with their former employers. We may then have to pursue litigation to defend against any of these claims. If we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights or personnel. Even if we are successful in defending against such claims, litigation could result in substantial costs and be a distraction to 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, and we may not have sufficient financial or other resources to adequately conduct this type of litigation or proceedings. For example, 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. In any case, 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.

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 registered or unregistered trademarks or trade names may be challenged, infringed, circumvented or declared generic or determined to be infringing on other marks. We may not be able to protect our rights to these trademarks and trade names, which we need to build name recognition among potential partners or customers in our markets of interest. At times, competitors 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. Over the long term, if we are unable to establish name recognition based on our trademarks and trade names, then we may not be able to compete effectively, and our business may be adversely affected. Our efforts to enforce or protect our proprietary rights related to trademarks, trade secrets, domain names, copyrights or other intellectual property may be ineffective and could result in substantial costs and diversion of resources and could adversely affect our business, financial condition, results of operations and growth prospects.

Intellectual property rights do not necessarily address all potential threats.

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

- any product candidates we may develop will eventually become commercially available in generic or biosimilar product forms;
- others may be able to make gene therapy products that are similar to any product candidates we may develop or utilize similar base editing technology but that are not covered by the claims of the patents that we license or may own in the future;
- we, or our license partners or current or future collaborators, might not have been the first to make the inventions covered by the issued patent or pending patent application that we license or may own in the future;
- we, or our license partners or current or future collaborators, might not have been the first to file patent applications covering certain of our or their inventions;
- we, or our license partners or current or future collaborators, may fail to meet our obligations to the U.S. government regarding any in-licensed patents and patent applications funded by U.S. government grants, leading to the loss or unenforceability of patent rights;
- others may independently develop similar or alternative technologies or duplicate any of our technologies without infringing our owned or licensed intellectual property rights;

- it is possible that our pending, owned or licensed patent applications or those that we may own in the future will not lead to issued patents;
- it is possible that there are prior public disclosures that could invalidate our owned or in-licensed patents, or parts of our owned or in-licensed patents;
- it is possible that there are unpublished applications or patent applications maintained in secrecy that may later issue with claims covering our product candidates or technology similar to ours;
- it is possible that our owned or in-licensed patents or patent applications omit individual(s) that should be listed as inventor(s) or include individual(s) that 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;
- issued patents that we hold rights to may be held invalid, unenforceable, or narrowed in scope, including as a result of legal challenges by our competitors;
- the claims of our owned or in-licensed issued patents or patent applications, if and when issued, may not cover our product candidates;
- the laws of foreign countries may not protect our proprietary rights or the proprietary rights of license partners or current or future collaborators to the same extent as the laws of the United States;
- the inventors of our owned or in-licensed patents or patent applications may become involved with competitors, develop products or processes that design around our patents, or become hostile to us or the patents or patent applications on which they are named as inventors;
- our competitors might conduct research and development activities in countries where we do not have patent rights and then use the information learned from such activities to develop competitive products for sale in our major commercial markets;
- 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 develop additional proprietary technologies that are patentable;
- any product candidates we develop may be covered by third parties' patents or other exclusive rights;
- the patents of others may harm our business; or
- we may choose not to file a patent in order to maintain certain trade secrets or know-how, and a third party may subsequently file a patent covering such intellectual property.

Should any of these events occur, they could have a material adverse effect on our business, financial condition, results of operations, and prospects.

If approved, our product candidates that are licensed and regulated as biologics may face competition from biosimilars approved through an abbreviated regulatory pathway.

The Biologics Price Competition and Innovation Act of 2009, or BPCIA, was enacted as part of the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Affordability Reconciliation Act, or collectively, the ACA, to establish an abbreviated pathway for the approval of biosimilar and interchangeable biological products. The regulatory pathway establishes legal authority for the FDA to review and approve biosimilar biologics, including the possible designation of a biosimilar as "interchangeable" based on its similarity to an approved biologic. Under the BPCIA, a reference biological product is granted 12 years of data exclusivity from the time of first licensure of the product, and the FDA will not accept an application for a biosimilar or interchangeable product based on the reference biological product until four years after the date of first licensure of the reference product. In addition, the licensure 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 develop and receive approval of a competing biologic, so long as its BLA does not reply on the reference product, sponsor's data or submit the application as a biosimilar application.

In December 2022, Congress clarified through FDORA that the FDA may approve multiple first interchangeable biosimilar biological products so long as the products are all approved on the first day on which such a product is approved as interchangeable with the reference product and the exclusivity period may be shared amongst multiple first interchangeable products. There have been recent government proposals to reduce the 12-year reference product exclusivity period, but none has been enacted to date. At the same time, since passage of the BPCIA, many states have passed laws, or amendments to laws, which address pharmacy practices involving biosimilar products.

We believe that any of the product candidates we develop as a biological product under a BLA should qualify for the 12-year period of exclusivity. However, there is a risk that this exclusivity could be shortened due to congressional action or otherwise, or that the FDA will not consider the subject product candidates to be reference products for competing products, potentially creating the opportunity for biosimilar competition sooner than anticipated. For example, in October 2025, the FDA issued draft guidance which proposes to eliminate the need for sponsors of biosimilar products to conduct comparative human clinical efficacy studies, allowing them to rely instead on analytical testing to demonstrate product differences from a reference product. Moreover, the extent to which a biosimilar, once approved, will be substituted for any one of the reference products in a way that is similar to traditional generic substitution for non-biological products will depend on a number of marketplace and regulatory factors that are still developing. Nonetheless, the approval of a biosimilar to our product candidates may have a material adverse impact on our business due to increased competition and pricing pressure.

Risks related to regulatory and other legal compliance matters

Regulatory requirements governing genetic medicines, and in particular any novel genetic medicines we may develop, have changed frequently and may continue to change in the future.

Regulatory requirements governing genetic and cellular medicines, and in particular any novel genetic medicine products we may develop, have changed frequently and may continue to change in the future. We are aware of a limited number of genetic medicines that have received marketing authorization from the FDA and EMA. Even with respect to more established products in the genetic medicine field, the regulatory landscape is still developing. For example, the FDA has established the Office of Tissues and Advanced Therapies (formerly the Office of Cellular, Tissue and Gene Therapies) within CBER to consolidate the review of genetic medicines and related products, and the Cellular, Tissue and Gene Therapies Advisory Committee to advise CBER on its review. Genetic medicine clinical trials conducted at institutions that receive funding for recombinant DNA research from the NIH also are potentially subject to review by the Office of Biotechnology Activities' Recombinant DNA Advisory Committee, or the RAC; however, the NIH announced that the RAC will only publicly review clinical trials if the trials cannot be evaluated by standard oversight bodies and pose unusual risks.

The same applies in the European Union, or EU. The EMA's Committee for Advanced Therapies, or CAT, is responsible for assessing the quality, safety, purity and potency of advanced-therapy medicinal products. The role of the CAT is to prepare a draft opinion on an application for marketing authorization for a genetic medicinal candidate that is submitted to the CHMP before CHMP adopts its final opinion. In the EU, the development and evaluation of a genetic medicinal 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 genetic medicinal products and require that we comply with these new guidelines. As a result, the procedures and standards applied to genetic medicines and cell therapy products may be applied to any product candidates we may develop, but that remains uncertain at this point.

These regulatory review committees and advisory groups and the new guidelines they promulgate may lengthen the regulatory review process, require us to perform additional studies, increase our development costs, lead to changes in regulatory positions and interpretations, delay or prevent approval and commercialization of any product candidates we may develop or lead to significant post-approval limitations or restrictions. As we advance any product candidates we may develop, we will be required to consult with these regulatory and advisory groups and comply with applicable guidelines. If we fail to do so, we may be required to delay or discontinue development of these product candidates. Delay or failure to obtain, or unexpected costs in obtaining, the regulatory approval necessary to bring a potential product to market could decrease our ability to generate sufficient product revenue to maintain our business.

Although the FDA decides whether individual genetic medicine protocols may proceed, the RAC public review process, if undertaken, can delay the initiation of a clinical trial, even if the FDA has reviewed the trial design and details and approved its initiation. Conversely, the FDA can put an IND on a clinical hold even if the RAC has provided a favorable review or an exemption from in-depth, public review. If we were to engage an NIH-funded institution to conduct a clinical trial, that institution's IBC as well as its IRB would need to review the proposed clinical trial to assess the safety of the trial. In addition, adverse developments in clinical trials of genetic medicine products conducted by others may cause the FDA or other oversight bodies to change the requirements for approval of any product candidates we may develop. Similarly, the EMA may issue new guidelines concerning the development and marketing authorization for genetic medicine products and require that we comply with these new guidelines.

As we are initially seeking to identify and develop product candidates to treat diseases using novel technologies, there is heightened risk that the FDA, the EMA or other regulatory authority may not consider the clinical trial endpoints that we propose to provide clinically meaningful results. Even if the endpoints are deemed clinically meaningful, we may not achieve these endpoints to a degree

of statistical significance, particularly because many of the diseases we are targeting with our platform have small patient populations, making development of large and rigorous clinical trials more difficult. Further, even if we do achieve the pre-specified criteria, we may produce results that are unpredictable or inconsistent with the results of the non-primary endpoints or other relevant data. The FDA also weighs the benefits of a product against its risks, and the FDA may view the efficacy results in the context of safety as not being supportive of regulatory approval. Other regulatory authorities in the EU and other countries may make similar comments with respect to these endpoints and data. Any product candidates we may develop will be based on a novel technology that makes it difficult to predict the time and cost of development and of subsequently obtaining regulatory approval. No gene editing therapeutic product has been approved in the United States or in Europe, and only a limited number of gene therapy products have received marketing authorization or marketing approval from the European Commission or the FDA. Some of these products have taken years to register and have had to address significant issues in their post-marketing experience.

Adverse developments in post-marketing experience or in clinical trials conducted by others of genetic medicines or cell therapy products may cause the FDA, the EMA, and other regulatory bodies to revise the requirements for development or approval of any product candidates we may develop or limit the use of products utilizing non-viral genetic medicinal technologies, either of which could materially 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, purity and potency 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 novel product candidates such as the product candidates we may develop can be more expensive and take longer than for other, better known or more extensively studied pharmaceutical or other product candidates. Regulatory agencies administering existing or future regulations or legislation may not allow production and marketing of products utilizing non-viral genetic medicine 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.

In addition, ethical, social and legal concerns about genetic medicine, genetic testing and genetic research could result in additional regulations or prohibiting the processes we may use. Federal and state agencies, congressional committees and foreign governments have expressed their intentions to further regulate biotechnology. More restrictive regulations or claims that any product candidates we may develop are unsafe or pose a hazard could prevent us from commercializing any products. New government requirements may be established that could delay or prevent regulatory approval of any product candidates we may develop under development. It is impossible to predict whether legislative changes will be enacted, regulations, policies or guidance changed, or interpretations by agencies or courts changed, or what the impact of such changes, if any, may be.

As we advance any product candidates we develop through clinical development, we will be required to consult with these regulatory and advisory groups, and comply with applicable guidelines. These regulatory review committees and advisory groups and any new guidelines they promulgate may lengthen the regulatory review process, require us to perform additional studies, increase our development costs, lead to changes in regulatory positions and interpretations, delay or prevent approval and commercialization of any product candidates we may develop or lead to significant post-approval limitations or restrictions. Delay or failure to obtain, or unexpected costs in obtaining, the regulatory approval necessary to bring a potential product to market could decrease our ability to generate sufficient product revenue.

Even if we complete the necessary preclinical studies and clinical trials, the marketing approval process is expensive, time-consuming, and uncertain and may prevent us from obtaining approvals for the commercialization of any product candidates we may develop. If we are not able to obtain, or if there are delays in obtaining, required regulatory approvals, we will not be able to commercialize, or will be delayed in commercializing, product candidates we may develop, and our ability to generate revenue will be materially impaired.

Any product candidates we may develop and the activities associated with their development and commercialization, including their design, testing, manufacture, recordkeeping, labeling, storage, approval, advertising, promotion, sale, import, export, and distribution, are subject to comprehensive regulation by the FDA, the EMA and other regulatory authorities in the United States and by comparable authorities in other countries. Failure to obtain marketing approval for a product candidate will prevent us from commercializing the product candidate in a given jurisdiction. We have not received approval to market any product candidates from regulatory authorities in any jurisdiction. We have only limited experience in filing and supporting the applications necessary to gain marketing approvals and expect to rely on third parties to assist us in this process. Securing regulatory approval requires the submission of extensive preclinical and clinical data and supporting information to the various regulatory authorities for each therapeutic indication to establish the biological product candidate's safety, purity, and potency. Securing regulatory approval also requires the submission of extensive information about the product manufacturing process, and inspection of manufacturing facilities by, the relevant regulatory authority. Any product candidates we develop may not be effective, may be only moderately effective, or may prove to have undesirable or unintended side effects, toxicities, or other characteristics that may preclude our obtaining marketing approval or prevent or limit commercial use.

The process of obtaining marketing approvals, both in the United States and abroad, is expensive, may take many years if approval is obtained at all, and can vary substantially based upon a variety of factors, including the type, complexity, and novelty of the product candidates involved. Changes in marketing approval policies during the development period, changes in or the enactment of additional

statutes or regulations, or changes in regulatory review for each submitted product application, may cause delays in the approval or rejection of an application. The FDA and comparable authorities in other countries have substantial discretion in the approval process and may refuse to accept any application or may decide that our data is insufficient for approval and require additional preclinical, clinical, or other studies. In addition, varying interpretations of the data obtained from preclinical and clinical testing could delay, limit, or prevent marketing approval of a product candidate. Any marketing approval we ultimately obtain may be limited or subject to restrictions or post-approval commitments that render the approved medicine not commercially viable.

Moreover, principal investigators for our future clinical trials may serve as scientific advisors or consultants to us and receive compensation in connection with such services. Under certain circumstances, we may be required to report some of these relationships to the FDA or comparable foreign regulatory authorities. The FDA or a comparable foreign regulatory authority may conclude that a financial relationship between us and a principal investigator has created a conflict of interest or otherwise affected interpretation of the study. The FDA or comparable foreign regulatory authority 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 or comparable foreign regulatory authority, as the case may be, and may ultimately lead to the denial of marketing approval of one or more of our product candidates.

Further, under the Pediatric Research Equity Act, or PREA, a BLA or supplement to a BLA for certain biological products must contain data to assess the safety and effectiveness of the biological product in all relevant pediatric subpopulations and to support dosing and administration for each pediatric subpopulation for which the product is safe and effective, unless the sponsor receives a deferral or waiver from the FDA. A deferral may be granted for several reasons, including a finding that the product or therapeutic candidate is ready for approval for use in adults before pediatric trials are complete or that additional safety or effectiveness data needs to be collected before the pediatric trials begin. The applicable legislation in the EU also requires sponsors to either conduct clinical trials in a pediatric population in accordance with a Pediatric Investigation Plan approved by the Pediatric Committee of the EMA or to obtain a waiver or deferral from the conduct of these studies by this Pediatric Committee. For any of our product candidates for which we are seeking regulatory approval in the U.S. or the EU, we cannot guarantee that we will be able to obtain a waiver or alternatively complete any required studies and other requirements in a timely manner, or at all, which could result in associated reputational harm and subject us to enforcement action.

In addition, we could be adversely affected by several significant administrative law cases decided by the U.S. Supreme Court in 2024. In *Loper Bright Enterprises v. Raimondo*, for example, the court overruled *Chevron U.S.A., Inc. v. Natural Resources Defense Council, Inc*., which for 40 years required federal courts to defer to permissible agency interpretations of statutes that are silent or ambiguous on a particular topic. The U.S. Supreme Court stripped federal agencies of this presumptive deference and held that courts must exercise their independent judgment when deciding whether an agency such as the FDA acted within its statutory authority under the Administrative Procedure Act, or the APA. Additionally, in *Corner Post, Inc. v. Board of Governors of the Federal Reserve System*, the court held that actions to challenge a federal regulation under the APA can be initiated within six years of the date of injury to the plaintiff, rather than the date the rule is finalized. The decision appears to give prospective plaintiffs a personal statute of limitations to challenge longstanding agency regulations. Another decision, *Securities and Exchange Commission v. Jarkesy*, overturned regulatory agencies' ability to impose civil penalties in administrative proceedings. These decisions could introduce additional uncertainty into the regulatory process and may result in additional legal challenges to actions taken by federal regulatory agencies, including the FDA and CMS, that we rely on. In addition to potential changes to regulations as a result of legal challenges, these decisions may result in increased regulatory uncertainty and delays and other impacts, any of which could adversely impact our business and operations.

Further, our ability to develop and market new drug products may be impacted by litigation challenging the FDA's approval of another company's drug product. In April 2023, the U.S. District Court for the Northern District of Texas invalidated the approval by the FDA of mifepristone, a drug product which was originally approved in 2000 and whose distribution is governed by various measures adopted under a REMS. The Court of Appeals for the Fifth Circuit declined to order the removal of mifepristone from the market but did hold that plaintiffs were likely to prevail in their claim that changes allowing for expanded access of mifepristone, which the FDA authorized in 2016 and 2021, were arbitrary and capricious. In June 2024, the Supreme Court reversed that decision after unanimously finding that the plaintiffs (anti-abortion doctors and organizations) did not have standing to bring this legal action against the FDA. On October 11, 2024, the Attorneys General of three states (Missouri, Idaho and Kansas) filed an amended complaint in the district court in Texas challenging the FDA's actions. On January 16, 2025, the district court agreed to allow these states to file an amended complaint and continue to pursue this challenge. Thereafter, on September 30, 2025, the district court declined to dismiss the case and, instead, transferred it to the federal district court in the Eastern District of Missouri. Depending on the outcome of this litigation, our ability to develop new drug product candidates and to maintain approval of existing drug products could be delayed, undermined or subject to protracted litigation.

Finally, with the change in presidential administrations in 2025, there continues to be substantial uncertainty as to how the new administration will seek to modify or revise the requirements and policies of the FDA and other regulatory agencies with jurisdiction over our product candidates. The uncertainty could present new challenges or potential opportunities as we navigate the clinical development and approval process for our product candidates.

If we experience delays in obtaining approval or if we fail to obtain approval of any product candidates we may develop, the commercial prospects for those product candidates may be harmed, and our ability to generate revenues will be materially impaired.

Failure to obtain marketing approval in foreign jurisdictions would prevent any product candidates we may develop from being marketed in such jurisdictions, which, in turn, would materially impair our ability to generate revenue.

In order to market and sell any product candidates we may develop in the EU and other foreign jurisdictions, we or our third-party collaborators must obtain separate marketing approvals (a single one for the EU) and comply with numerous and varying regulatory requirements. The approval procedure varies among countries and can involve additional testing. The time required to obtain approval may differ substantially from that required to obtain FDA approval. The regulatory approval process outside the United States generally includes all of the risks associated with obtaining FDA approval. In addition, in many countries outside the United States, it is required that the product candidate be approved for reimbursement before the product candidate can be approved for sale in that country. We or these third parties may not obtain approvals from regulatory authorities outside the United States on a timely basis, if at all. Approval by the FDA does not ensure approval by regulatory authorities in other countries or jurisdictions, and approval by one regulatory authority outside the United States does not ensure approval by regulatory authorities in other countries or jurisdictions or by the FDA. We may not be able to file for marketing approvals and may not receive necessary approvals to commercialize our medicines in any jurisdiction, which would materially impair our ability to generate revenue.

Additionally, we could face heightened risks with respect to obtaining marketing authorization in the U.K. as a result of the withdrawal of the U.K. from the EU, commonly referred to as Brexit. The U.K. is no longer part of the European Single Market and EU Customs Union. As of January 1, 2025, the Medicines and Healthcare Products Regulatory Agency, or MHRA, is responsible for approving all medicinal products destined for the U.K. market (i.e., Great Britain and Northern Ireland). On April 28, 2025, the U.K. Parliament adopted amendments to improve and strengthen the U.K.'s clinical trials regulatory regime, and such amendments will take effect on April 28, 2026. These changes were needed since the current U.K. requirements are based upon the now-repealed EU Clinical Trials Directive (2001/20/EC), which has been replaced by the European Clinical Trials Regulation (Regulation EU No 536/2014). Since the U.K. left the EU prior to the date on which the EU CTR took effect, the U.K. legal framework did not benefit from the same revisions as occurred at the EU level.

At the same time, as of January 1, 2025, a new international recognition procedure, or IRP, applies, which intends to facilitate approval of pharmaceutical products in the U.K. The IRP is open to applicants that have already received an authorization for the same product from one of the MHRA's specified Reference Regulators, or RRs. The RRs notably include the EMA and regulators in the EU/European Economic Area, or EEA, member states for approvals in the EU centralized procedure and mutual recognition procedure, as well as the FDA for product approvals granted in the U.S. However, the concrete functioning of the IRP is currently unclear. Any delay in obtaining, or an inability to obtain, any marketing approvals may force us to restrict or delay efforts to seek regulatory approval in the U.K. for our product candidates, which could significantly and materially harm our business.

In addition, foreign regulatory authorities may change their approval policies and new regulations may be enacted. For instance, the EU pharmaceutical legislation is currently undergoing a complete review process, in the context of the Pharmaceutical Strategy for Europe initiative, launched by the European Commission in November 2020. The European Commission's proposal for revision of several legislative instruments related to medicinal products (potentially reducing the duration of regulatory data protection, revising the eligibility for expedited pathways, etc.) was published on April 26, 2023. On June 4, 2025, after almost two years of negotiations among the EU Member States, the Council of the European Union adopted its position on the proposed overhaul of the EU general pharmaceutical legislative framework, which is known as the new Pharma Package. Thereafter, on December 11, 2025, the European Parliament and the Council reached a provisional political agreement on the legislation which is expected to be adopted by mid-2026. The revisions may have a significant impact on the pharmaceutical industry and our business. They would, among other things, set a baseline period of eight years of data exclusivity and one year of market exclusivity with possible extensions for new indications, up to a maximum of 11 years total. There will likely be a transition period of 24 months, with the changes taking effect in mid-2028.

Any regulatory approval to market our products will be limited by indication. If we fail to comply or are found to be in violation of FDA regulations restricting the promotion of our products for unapproved uses, we could be subject to criminal penalties, substantial fines or other sanctions and damage awards.

The regulations relating to the promotion of products for unapproved uses are complex and subject to substantial interpretation by the FDA, EMA, MHRA and other government agencies. In September 2021, the FDA published final regulations which describe the types of evidence that the agency will consider in determining the intended use of a drug product. Physicians may nevertheless prescribe our products off-label to their patients in a manner that is inconsistent with the approved label. We intend to implement compliance and training programs designed to ensure that any of our sales and marketing practices comply with applicable regulations. Notwithstanding these programs, the FDA or other government agencies may allege or find that our practices constitute prohibited promotion of our products for unapproved uses. We also cannot be sure that our employees will comply with company policies and applicable regulations regarding the promotion of products for unapproved uses.

For example, on September 9, 2025, the President issued a memorandum directing HHS to "ensure transparency and accuracy in DTC prescription drug advertising, including by increasing the amount of information regarding any risks associated with the use of any

such prescription drug required to be provided in prescription drug advertisements." To that end, the FDA announced that it is initiating a rulemaking process "to eliminate the 'adequate provision' loophole that allows pharmaceutical advertisements to hide safety information by placing it in another format or location." In this context, the FDA declared that it will no longer tolerate what it characterized as "deceptive practices" in prescription drug advertising and that the FDA would "aggressively deploy" its available enforcement tools, with "heightened scrutiny" of fair balance and disclosures in social media promotions. The FDA also issued a generic "notice letter" directing companies to "remove any noncompliant advertising and bring all promotional communications into compliance."

Notwithstanding the regulatory restrictions on off-label promotion, the FDA and other regulatory authorities allow companies to engage in truthful, non-misleading, and non-promotional scientific communications concerning their products in certain circumstances. For example, in January 2025, the FDA published final guidance outlining its non-binding policies governing the distribution of scientific information on unapproved uses to healthcare providers. This final guidance calls for such communications to be truthful, non-misleading, factual, and unbiased and include all information necessary for healthcare providers to interpret the strengths and weaknesses and validity and utility of the information about the unapproved use. In addition, under some relatively recent guidance from the FDA and the Pre-Approval Information Exchange Act, or PIE Act, signed into law as part of the Consolidated Appropriations Act of 2023, companies may also promote information that is consistent with the prescribing information and proactively speak to formulary committee members of payors regarding data for an unapproved drug or unapproved uses of an approved drug. We may engage in these discussions and communicate with healthcare providers, payors and other constituencies in compliance with all applicable laws, regulatory guidance and industry best practices. We will need to carefully navigate the FDA's various regulations, guidance and policies, along with recently enacted legislation, to ensure compliance with restrictions governing promotion of our products.

In recent years, a significant number of pharmaceutical and biotechnology companies have been the target of inquiries and investigations by various federal and state regulatory, investigative, prosecutorial and administrative entities in connection with the promotion of products for unapproved uses and other sales practices, including the Department of Justice and various U.S. Attorneys' Offices, the Office of Inspector General of the Department of Health and Human Services, the FDA, the Federal Trade Commission, or the FTC, and various state Attorneys General offices. These investigations have alleged violations of various federal and state laws and regulations, including claims asserting antitrust violations, violations of the FDCA, the False Claims Act, the Prescription Drug Marketing Act and anti-kickback laws and other alleged violations in connection with the promotion of products for unapproved uses, pricing and Medicare and/or Medicaid reimbursement. Many of these investigations originate as "*qui tam*" actions under the False Claims Act. Under the False Claims Act, any individual can bring a claim on behalf of the government alleging that a person or entity has presented a false claim or caused a false claim to be submitted to the government for payment. The person bringing a *qui tam* suit is entitled to a share of any recovery or settlement. *Qui tam* suits, also commonly referred to as "whistleblower suits," are often brought by current or former employees. In a *qui tam* suit, the government must decide whether to intervene and prosecute the case. If it declines, the individual may pursue the case alone.

If the FDA or any other governmental agency initiates an enforcement action against us or if we are the subject of a *qui tam* suit and it is determined that we violated prohibitions relating to the promotion of products for unapproved uses, we could be subject to substantial civil or criminal fines or damage awards and other sanctions such as consent decrees and corporate integrity agreements pursuant to which our activities would be subject to ongoing scrutiny and monitoring to ensure compliance with applicable laws and regulations. Any such fines, awards or other sanctions would have an adverse effect on our revenue, business, financial prospects and reputation.

Any product for which we obtain marketing approval in the future could be subject to post-marketing restrictions or withdrawal from the market and we may be subject to substantial penalties if we fail to comply with regulatory requirements or if we experience unanticipated problems with any such product following approval.

Any product for which we obtain marketing approval, as well as the manufacturing processes, post-approval studies and measures, labeling, advertising and promotional activities for such product, among other things, will be subject to ongoing requirements of and review by the FDA and other regulatory authorities. These requirements include submissions of safety and other post-marketing information and reports, registration and listing requirements, 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. Even if marketing approval of a product is granted, the approval may be subject to limitations on the indicated uses for which the product may be marketed or to the conditions of approval, including the requirement to implement a REMS. The FDA may also impose requirements for costly post-marketing studies or clinical trials and surveillance to monitor the safety or efficacy of a product.

The FDA and other agencies, including the Department of Justice, closely regulate and monitor the post-approval marketing and promotion of products to ensure that they are manufactured, marketed and distributed only for the approved indications and in accordance with the provisions of the approved labeling. The FDA imposes stringent restrictions on manufacturers' communications regarding off-label use and if we market any product for an indication that is not approved, we may be subject to warnings or enforcement action for off-label marketing. Violation of the FDCA and other statutes, including the False Claims Act, relating to the

promotion and advertising of prescription drugs may lead to investigations or allegations of violations of federal and state health care fraud and abuse laws and state consumer protection laws.

In addition, later discovery of previously unknown adverse events or other problems with any product for which we may obtain marketing approval and its manufacturers or manufacturing processes or failure to comply with regulatory requirements, may yield various results, including:

- restrictions on such product, manufacturers or manufacturing processes;
- restrictions on the labeling or marketing of the product;
- restrictions on product distribution or use;
- requirements to conduct post-marketing studies or clinical trials;
- warning letters or untitled letters;
- withdrawal of the product from the market;
- refusal to approve pending applications or supplements to approved applications that we submit;
- recall of the product;
- restrictions on coverage by third-party payors;
- fines, restitution or disgorgement of profits or revenues;
- suspension or withdrawal of marketing approvals;
- refusal to permit the import or export of the product;
- product seizure; or
- injunctions or the imposition of civil or criminal penalties.

Similar restrictions apply to the approval of our products in the EU. The holder of a marketing authorization is required to comply with a range of requirements applicable to the manufacturing, marketing, promotion and sale of medicinal products. These include: compliance with the EU's stringent pharmacovigilance or safety reporting rules, which can impose post-authorization studies and additional monitoring obligations; the manufacturing of authorized medicinal products, for which a separate manufacturer's license is mandatory; and the marketing and promotion of authorized drugs, which are strictly regulated in the EU. and are also subject to EU. Member State laws. The failure to comply with these and other EU requirements can also lead to significant penalties and sanctions.

Fast track, breakthrough, or regenerative medicine advanced therapy designation by the FDA may not actually lead to a faster development or regulatory review or approval process and does not assure FDA approval of any product candidates we may develop.

FDA's fast track, breakthrough, and regenerative medicine advanced therapy, or RMAT, programs are intended to expedite the development of certain qualifying products 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 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. 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 condition, and preliminary clinical evidence indicates that the product candidate has the potential to address an unmet medical need for such condition. While we have in the past sought, and may again in the future seek, fast track, breakthrough, and/or RMAT designation, 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, or RMAT designation does not ensure that the product candidate will receive marketing approval or that approval will be granted within any particular timeframe. In addition, the FDA may withdraw fast track, breakthrough, or RMAT designation if it believes that the designation is no longer supported by data from our clinical development program. Fast track, breakthrough, and/or RMAT designation alone do not guarantee qualification for the FDA's priority review procedures.

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 any product candidates we may develop.

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 seek PRIME Designation in the EU for our product candidates, but we might not receive such designations, and even if we do, such designations may not lead to a faster development or regulatory review or approval process.

In the EU, we may seek PRIME designation for some of our product candidates in the future. PRIME is a voluntary program aimed at enhancing the EMA's role to reinforce scientific and regulatory support in order to optimize development and enable accelerated assessment of new medicines that are of major public health interest with the potential to address unmet medical needs. The program focuses on medicines that target conditions for which there exists no satisfactory method of treatment in the EU or even if such a method exists, it may offer a major therapeutic advantage over existing treatments. PRIME is limited to medicines under development and not authorized in the EU and where the sponsor intends to apply for an initial marketing authorization application through the centralized procedure. To be accepted for PRIME, a product candidate must meet the eligibility criteria in respect of its major public health interest and therapeutic innovation based on information that is capable of substantiating the claims. The benefits of a PRIME designation include the appointment of a CHMP rapporteur to provide continued support and help to build knowledge ahead of a marketing authorization application, early dialogue and scientific advice at key development milestones, and the potential to qualify products for accelerated review, meaning reduction in the review time for an opinion on approvability to be issued earlier in the application process. PRIME enables a sponsor to request parallel EMA scientific advice and health technology assessment advice to facilitate timely market access. Even if we receive PRIME designation for any of our product candidates, the designation may not result in a materially faster development process, review or approval compared to conventional EMA procedures. Further, obtaining PRIME designation does not assure or increase the likelihood of EMA's grant of a marketing authorization.

We may seek designation for our platform technology as a designated platform technology, but we might not receive such designation, and even if we do, such designation may not lead to a faster regulatory review or approval process.

We may seek designation for our platform technology as a designated platform technology. Under FDORA, a platform technology incorporated within or utilized by a drug or biological product is eligible to be a designated platform technology if (1) the platform technology is incorporated in, or utilized by, a drug approved under a BLA; (2) preliminary evidence submitted by the sponsor of the approved or licensed drug, or a sponsor that has been granted a right of reference to data submitted in the application for such drug, demonstrates that the platform technology has the potential to be incorporated in, or utilized by, more than one drug without an adverse effect on quality, manufacturing, or safety; and (3) data or information submitted by the applicable person indicates that incorporation or utilization of the platform technology has a reasonable likelihood to bring significant efficiencies to the drug development or manufacturing process and to the review process. A sponsor may request the FDA to designate a platform technology as a designated platform technology concurrently with, or at any time after, submission of an IND application for a drug that incorporates or utilizes the platform technology that is the subject of the request. If so designated, the FDA may expedite the development and review of any subsequent original BLA for a drug that uses or incorporates the platform technology. Even if we believe our platform technology meets the criteria for such designation, the FDA may disagree and instead determine not to grant such designation. In addition, the receipt of such designation for a platform technology does not ensure that a drug will be developed more quickly or receive FDA approval. Moreover, the FDA may revoke a designation if the FDA determines that a designated platform technology no longer meets the criteria for such designation.

Inadequate funding for the FDA, the SEC and other government agencies, including from government shut downs, or other disruptions to these agencies' operations, could hinder their ability to hire and retain key leadership and other personnel, prevent new products and services from being developed or commercialized in a timely manner or otherwise prevent those agencies from performing normal business functions on which the operation of our business may rely, which could negatively impact our business.

The ability of the FDA to review and approve new products can be affected by a variety of factors, including government budget and funding levels, ability to hire and retain key personnel and accept the payment of user fees, and statutory, regulatory and policy changes. Average review times at the FDA have fluctuated in recent years as a result. Disruptions at the FDA and other agencies may also slow the time necessary for new product candidates to be reviewed and/or approved by necessary government agencies, which would adversely affect our business. In addition, government funding of the SEC and other government agencies on which our operations may rely, including those that fund research and development activities, is subject to the political process, which is inherently fluid and unpredictable.

Disruptions at the FDA, EMA and other agencies may also slow the time necessary for new drugs to be reviewed and/or approved by necessary government agencies, which would adversely affect our business. For example, in recent years, the U.S. government shut

down and certain regulatory agencies, such as the FDA and the SEC, had to furlough critical employees and stop critical activities. In addition, disruptions may also result from events similar to the COVID-19 pandemic. During the COVID-19 pandemic, a number of companies announced receipt of complete response letters due to the FDA's inability to complete required inspections for their applications. In the event of a similar public health emergency in the future, the FDA may not be able to continue its current pace and review timelines could be extended. Regulatory authorities outside the United States facing similar circumstances may adopt similar restrictions or other policy measures in response to a similar public health emergency and may also experience delays in their regulatory activities.

Further, while the FDA's review of marketing applications and other activities for new drugs and biologics is largely funded through the user fee program established under PDUFA, it remains unclear how the current administration's reduction in force and budget cuts will impact this program and the ability of the FDA to provide guidance and review our product candidates in a timely manner. There also continues to be uncertainty as to how other measures being implemented by the current administration across the government will impact our activities and those of the FDA and its operations. For example, the potential loss of FDA personnel could lead to further disruptions and delays in FDA review of our product candidates.

If an emergency related disruption occurs, it could significantly impact the ability of the FDA to timely review and process our regulatory submissions, which could have a material adverse effect on our business. Future emergency-related disruptions could also affect other government agencies such as the SEC, which may also impact our business by delaying review of our public filings, to the extent such review is necessary, and our ability to access the public markets.

We may not be able to obtain orphan drug exclusivity for one or more of our product candidates, and even if we do, that exclusivity may not prevent the FDA or the EMA from approving other competing products.

Under the Orphan Drug Act, the FDA may designate a product candidate as an orphan drug if it is a drug or biologic intended to treat a rare disease or condition. A similar regulatory scheme governs approval of orphan product candidates by the EMA in the EU. Generally, if a product with an orphan drug designation subsequently receives the first marketing approval for the indication for which it has such designation, the product is entitled to a period of marketing exclusivity, which precludes the FDA or the EMA from approving another marketing application for another product candidate for the same orphan therapeutic indication for that time period. The applicable period is seven years in the United States and ten years in the EU. The exclusivity period in the EU can be reduced to six years if a product no longer meets the criteria for orphan drug designation, in particular if the product is sufficiently profitable so that market exclusivity is no longer justified.

While we have received orphan drug designation for a product candidate in the past, the FDA's standards for granting orphan drug exclusivity in the gene therapy context remain unclear and evolving. For example, in September 2021, the FDA issued final guidance describing its current thinking on when a gene therapy product is the "same" as another product for purposes of orphan exclusivity. Under the guidance, if either the transgene or vector differs between two gene therapy products in a manner that does not reflect "minor" differences, the two products would be considered different drugs for orphan drug exclusivity purposes. The FDA will determine whether two vectors from the same viral class are the same on a case-by-case basis and may consider additional key features in assessing sameness. In addition, in order for the FDA to grant orphan drug exclusivity to one of our product candidates, the FDA must find that the product candidate is indicated for the treatment of a condition or disease that affects fewer than 200,000 individuals in the United States or that affects more than 200,000 individuals in the United States and for which there is no reasonable expectation that the cost of developing and making the product candidate available for the disease or condition will be recovered from sales of the product in the United States. The FDA may conclude that the condition or disease for which we seek orphan drug exclusivity does not meet this standard. Even if we obtain orphan drug exclusivity for a product candidate, that exclusivity may not effectively protect the product candidate from competition because different product candidates can be approved for the same condition. Orphan drug exclusivity may also be lost if the FDA or EMA determines that the request for designation was materially defective or if the manufacturer is unable to assure sufficient quantity of the product to meet the needs of the patients with the rare disease or condition. In addition, even after an orphan drug is approved, the FDA can subsequently approve the same drug for the same condition if the FDA concludes that the later product candidate is clinically superior in that it is shown to be safer, more effective or makes a major contribution to patient care compared with the product that has orphan exclusivity.

On August 3, 2017, the Congress passed the FDA Reauthorization Act of 2017, or FDARA. FDARA, among other things, codified the FDA's pre-existing 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 new legislation reverses 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. Further, under Omnibus legislation signed in December 2020, the requirement for a product to show clinical superiority applies to drugs and biologics that received orphan drug designation before enactment of FDARA in 2017, but have not yet been approved or licensed by the FDA.

The FDA may further reevaluate the Orphan Drug Act and its regulations and policies. This may be particularly true in light of a decision from the Court of Appeals for the 11th Circuit in September 2021. In *Catalyst Pharms, Inc. v. Becerra*, or *Catalyst*, the court held that, for the purpose of determining the scope of orphan drug exclusivity, the term "same disease or condition" in the statute

means the designated "rare disease or condition" and could not be interpreted by the FDA to mean the "indication or use." On January 23, 2023, the FDA announced that, in matters beyond the scope of the *Catalyst* court order, the FDA will continue to apply its existing regulations tying orphan-drug exclusivity to the uses or indications for which the orphan drug is approved. More recently however, on February 14, 2025, a federal district court in Washington, D.C., fully embraced the reasoning of the *Catalyst* decision in another decision challenging the scope of orphan drug exclusivity. On April 17, 2025, the FDA appealed this decision to the U.S. Court of Appeals for the D.C. Circuit. The implications of this decision, and its impact on the FDA's implementation of the Orphan Drug Act, are unclear at this point.

Our relationships with healthcare providers, physicians, and third-party payors will be subject to applicable anti-kickback, fraud and abuse, anti-bribery and other healthcare laws and regulations, which could expose us to criminal sanctions, civil penalties, contractual damages, reputational harm, and diminished profits and future earnings.

Healthcare providers, physicians, and third-party payors play a primary role in the recommendation and prescription of any product candidates that we may develop for which we obtain marketing approval. Our future arrangements with third-party payors and customers may expose us to broadly applicable fraud and abuse and other healthcare laws and regulations that may constrain the business or financial arrangements and relationships through which we market, sell, and distribute our medicines for which we obtain marketing approval. Restrictions under applicable federal and state healthcare laws and regulations, including certain laws and regulations applicable only if we have marketed products, include the following:

- federal false claims, false statements and civil monetary penalties laws prohibiting, among other things, any person from knowingly presenting, or causing to be presented, a false claim for payment of government funds or knowingly making, or causing to be made, a false statement to get a false claim paid;
- federal healthcare program anti-kickback law, which prohibits, among other things, persons from offering, soliciting, receiving or providing remuneration, directly or indirectly, to induce either the referral of an individual for, or the purchasing or ordering of a good or service, for which payment may be made under federal healthcare programs such as Medicare and Medicaid;
- the federal Health Insurance Portability and Accountability Act of 1996, or HIPAA, which, in addition to privacy protections applicable to healthcare providers and other entities, prohibits executing a scheme to defraud any healthcare benefit program or making false statements relating to healthcare matters;
- the federal Food, Drug, and Cosmetic Act, or the FDCA, which among other things, strictly regulates drug marketing, prohibits manufacturers from marketing such products for off-label use and regulates the distribution of samples;
- federal laws that require pharmaceutical manufacturers to report certain calculated product prices to the government or provide certain discounts or rebates to government authorities or private entities, often as a condition of reimbursement under government healthcare programs;
- the so-called "federal sunshine" law under the Healthcare Reform Act, which requires pharmaceutical and medical device companies to monitor and report certain financial interactions with certain healthcare providers to the Center for Medicare & Medicaid Services within the U.S. Department of Health and Human Services for re-disclosure to the public, as well as ownership and investment interests held by physicians and their immediate family members;
- state laws also requiring pharmaceutical companies to comply with specific compliance standards, restrict financial interactions between pharmaceutical companies and healthcare providers or require pharmaceutical companies to report information related to payments to health care providers or marketing expenditures; and
- analogous state and foreign laws and regulations, such as state anti-kickback, anti-bribery and false claims laws, which may apply to healthcare items or services that are reimbursed by non-governmental third-party payors, including private insurers.

Efforts to ensure that our business arrangements with third parties will comply with applicable healthcare laws and regulations will involve substantial costs. Given the breadth of the laws and regulations, limited guidance for certain laws and regulations and evolving government interpretations of the laws and regulations, governmental authorities may possibly conclude that our business practices may not comply with healthcare laws and regulations. If our operations are found to be in violation of any of the laws described above or any other government regulations that apply to us, we may be subject to penalties, including civil and criminal penalties, damages, fines, exclusion from participation in government health care programs, such as Medicare and Medicaid, imprisonment, and the curtailment or restructuring of our operations, any of which could adversely affect our business, financial condition, results of operations, and prospects.

The provision of benefits or advantages to physicians to induce or encourage the prescription, recommendation, endorsement, purchase, supply, order, or use of medicinal products is prohibited in the EU. The provision of benefits or advantages to physicians is

also governed by the national anti-bribery laws of EU Member States, such as the U.K. Bribery Act 2010. Infringement of these laws could result in substantial fines and imprisonment.

Payments made to physicians in certain EU Member States must be publicly disclosed. Moreover, agreements with physicians often must be the subject of prior notification and approval by the physician's employer, his or her competent professional organization, and/or the regulatory authorities of the individual EU Member States. These requirements are provided in the national laws, industry codes, or professional codes of conduct applicable in the EU Member States. Failure to comply with these requirements could result in reputational risk, public reprimands, administrative penalties, fines or imprisonment.

Recently enacted and future legislation may increase the difficulty and cost for us and any future collaborators to obtain marketing approval of and commercialize our product candidates and affect the prices we, or they, may obtain.

In the United States and some foreign jurisdictions, there have been a number of legislative and regulatory changes and proposed changes regarding the healthcare system that could, among other things, prevent or delay marketing approval of any product candidates we may develop, restrict or regulate post-approval activities and affect our ability, or the ability of any future collaborators, to profitably sell any products for which we, or they, obtain marketing approval. We expect that current laws, as well as other healthcare reform measures that may be adopted in the future, may result in more rigorous coverage criteria and in additional downward pressure on the price that we, or any future collaborators, may receive for any approved products.

In March 2010, the United States Congress enacted the 2010 Patient Protection and Affordable Care Act, or the PPACA. In addition, other legislative changes have been proposed and adopted since the PPACA was enacted. In August 2011, the Budget Control Act of 2011, among other things, created measures for spending reductions by Congress. A Joint Select Committee on Deficit Reduction, tasked with recommending a targeted deficit reduction of at least $1.2 trillion for the years 2013 through 2021, was unable to reach required goals, thereby triggering the legislation's automatic reduction to several government programs. These changes included aggregate reductions to Medicare payments to providers of up to 2% per fiscal year, which went into effect in April 2013 and will remain in effect through 2031.

The American Taxpayer Relief Act of 2012, among other things, reduced Medicare payments to several providers and increased the statute of limitations period for the government to recover overpayments to providers from three to five years. These new laws may result in additional reductions in Medicare and other healthcare funding and otherwise affect the prices we may obtain for any of our product candidates for which we may obtain regulatory approval or the frequency with which any such product candidate is prescribed or used.

Other legislative changes have been adopted since the PPACA was enacted, including aggregate reductions to Medicare payments to providers of up to 2% per fiscal year, which went into effect in April 2013 and will remain in effect through 2031. Under current legislation, the actual reductions in Medicare payments may vary up to 4%. The Consolidated Appropriations Act, or CAA, which was signed into law by President Biden in December 2022, made several changes to sequestration of the Medicare program. Section 1001 of the CAA delays the 4% Statutory Pay-As-You-Go Act of 2010, or PAYGO, sequester for two years, through the end of calendar year 2024. Triggered by the enactment of the American Rescue Plan Act of 2021, the 4% cut to the Medicare program would have taken effect in January 2023. The CAA's health care offset title includes Section 4163, which extends the 2% Budget Control Act of 2011 Medicare sequester for six months into fiscal year 2032 and lowers the payment reduction percentages in fiscal years 2030 and 2031.

Since enactment of the ACA, there have been and continue to be, numerous legal challenges and Congressional actions to repeal and replace provisions of the law. For example, with enactment of the Tax Act in 2017, Congress repealed the "individual mandate." The repeal of this provision, which requires most Americans to carry a minimal level of health insurance, became effective in 2019. Further, in June 2021, The U.S. Supreme Court dismissed a lawsuit challenging the constitutionality of the ACA after finding that the plaintiffs do not have standing to bring the litigation. Litigation and legislation over the ACA are likely to continue, with unpredictable and uncertain results.

In the EU, on December 13, 2021, Regulation No 2021/2282 on Health Technology Assessment, or HTA, amending Directive 2011/24/EU, was adopted. While the regulation entered into force in January 2022, it will only begin to apply from January 2025 onwards, with preparatory and implementation-related steps to take place in the interim. Once applicable, it will have a phased implementation depending on the concerned products. The regulation intends to boost cooperation among EU Member States in assessing health technologies, including new medicinal products as well as certain high-risk medical devices, and provide the basis for cooperation at the EU level for joint clinical assessments in these areas. It will permit EU Member States to use common HTA tools, methodologies, and procedures across the EU, working together in four main areas, including joint clinical assessment of the innovative health technologies with the highest potential impact for patients, joint scientific consultations whereby developers can seek advice from HTA authorities, identification of emerging health technologies to identify promising technologies early, and continuing voluntary cooperation in other areas. Individual EU member states will continue to be responsible for assessing non-clinical (e.g., economic, social, ethical) aspects of health technology, and making decisions on pricing and reimbursement.

We expect that these healthcare reforms, as well as other healthcare reform measures that may be adopted in the future, may result in additional reductions in Medicare and other healthcare funding, more rigorous coverage criteria, new payment methodologies and additional downward pressure on the price that we receive for any approved product and/or the level of reimbursement physicians receive for administering any approved product we might bring to market. Reductions in reimbursement levels may negatively impact the prices we receive or the frequency with which our products are prescribed or administered. Any reduction in reimbursement from Medicare or other government programs may result in a similar reduction in payments from private payors. Accordingly, such reforms, if enacted, could have an adverse effect on anticipated revenue from product candidates that we may successfully develop and for which we may obtain marketing approval and may affect our overall financial condition and ability to develop or commercialize product candidates.

The prices of prescription pharmaceuticals in the United States and foreign jurisdictions are subject to considerable legislative and executive actions and could impact the prices we obtain for our products, if and when licensed.

The prices of prescription pharmaceuticals have been the subject of considerable discussion in the United States. There have been several U.S. congressional inquiries, as well as proposed and enacted state and federal legislation designed to, among other things, bring more transparency to pharmaceutical pricing, review the relationship between pricing and manufacturer patient programs, and reduce the costs of pharmaceuticals under Medicare and Medicaid.

In October 2020, HHS and the FDA published a final rule allowing states and other entities to develop a Section 804 Importation Program, or SIP, to import certain prescription drugs from Canada into the United States. That regulation was challenged in a lawsuit by the Pharmaceutical Research and Manufacturers of America, or PhRMA, but the case was dismissed by a federal district court in February 2023 after the court found that PhRMA did not have standing to sue HHS. Several states have passed laws allowing for the importation of products from Canada. On May 21, 2025, the FDA announced that it would offer individual states the opportunity to submit a draft proposal for pre-review and meet with the FDA to obtain initial feedback from FDA prior to formally submitting their SIP proposal. The intent of these meetings is to assist states in developing their proposals by further clarifying requirements, enhancing the quality of proposals submitted to the FDA and ultimately shortening the review timeline.

Further, on November 20, 2020, HHS finalized a regulation removing safe harbor protection for price reductions from pharmaceutical manufacturers to plan sponsors under Part D, either directly or through pharmacy benefit managers, unless the price reduction is required by law. The rule also creates a new safe harbor for price reductions reflected at the point-of-sale, as well as a safe harbor for certain fixed fee arrangements between pharmacy benefit managers and manufacturers. Pursuant to court order, the removal and addition of the aforementioned safe harbors were delayed and recent legislation imposed a moratorium on implementation of the rule until January 1, 2026. The IRA further delayed implementation of this rule to January 1, 2032.

On August 16, 2022, the IRA was signed into law by President Biden. The new legislation has implications for Medicare Part D, which is a program available to individuals who are entitled to Medicare Part A or enrolled in Medicare Part B to give them the option of paying a monthly premium for outpatient prescription drug coverage. Among other things, the IRA requires manufacturers of certain drugs to engage in price negotiations with Medicare (beginning in 2026), with prices that can be negotiated subject to a cap; imposes rebates under Medicare Part B and Medicare Part D to penalize price increases that outpace inflation (first due in 2023); and replaces the Part D coverage gap discount program with a new discounting program (beginning in 2025). The IRA permits the Secretary of the Department of Health and Human Services (HHS) to implement many of these provisions through guidance, as opposed to regulation, for the initial years.

Specifically, with respect to price negotiations, Congress authorized Medicare to negotiate lower prices for certain costly single-source drug and biologic products that do not have competing generics or biosimilars and are reimbursed under Medicare Part B and Part D. CMS may negotiate prices for ten high-cost drugs paid for by Medicare Part D starting in 2026, followed by 15 Part D drugs in 2027, 15 Part B or Part D drugs in 2028 and 20 Part B or Part D drugs in 2029 and beyond. This provision applies to drug products that have been approved for at least 9 years and biologics that have been licensed for 13 years. Drugs and biologics that have been approved for a single rare disease or condition were originally categorically excluded from price negotiation. With passage of the OBBBA on July 3, 2025, which was signed into law on July 4, 2025, Congress extended this exemption to drugs and biologics with multiple orphan drug designations. Nonetheless, since CMS may establish a maximum price for these products in price negotiations, we would be fully at risk of government action if our products are the subject of Medicare price negotiations. Moreover, given the risk that could be the case, these provisions of the IRA may also further heighten the risk that we would not be able to achieve the expected return on our drug products or full value of our patents protecting our products if prices are set after such products have been on the market for nine years.

The first cycle of negotiations for the Medicare Drug Price Negotiation Program commenced in the summer of 2023. On August 15, 2024, the HHS published the results of the first Medicare drug price negotiations for ten selected drugs that treat a range of conditions, including diabetes, chronic kidney disease, and rheumatoid arthritis. The prices of these ten drugs became effective January 1, 2026. On January 17, 2025, CMS announced its selection of 15 additional drugs covered by Part D for the second cycle of negotiations by February 1, 2025. While there had been some questions about the Trump Administration's position on this program, CMS issued a public statement on January 29, 2025, declaring that lowering the cost of prescription drugs is a top priority of the new

administration and CMS is committed to considering opportunities to bring greater transparency in the negotiation program. The second cycle of negotiations with participating drug companies will occur during 2025, and any negotiated prices for this second set of drugs will be effective starting January 1, 2027.

Further, the legislation subjects drug manufacturers to civil monetary penalties and a potential excise tax for failing to comply with the legislation by offering a price that is not equal to or less than the negotiated "maximum fair price" under the law or for taking price increases that exceed inflation. The legislation also requires manufacturers to pay rebates for drugs in Medicare Part D whose price increases exceed inflation. The new law also caps Medicare out-of-pocket drug costs at an estimated $4,000 a year in 2024 and, thereafter beginning in 2025, at 2,000 a year. In addition, the IRA potentially raises legal risks with respect to individuals participating in a Medicare Part D prescription drug plan who may experience a gap in coverage if they required coverage above their initial annual coverage limit before they reached the higher threshold, or "catastrophic period" of the plan. Individuals requiring services exceeding the initial annual coverage limit and below the catastrophic period, must pay 100% of the cost of their prescriptions until they reach the catastrophic period. Among other things, the IRA contains many provisions aimed at reducing this financial burden on individuals by reducing the co-insurance and co-payment costs, expanding eligibility for lower income subsidy plans, and price caps on annual out-of-pocket expenses, each of which could have potential pricing and reporting implications.

On June 6, 2023, Merck & Co. filed a lawsuit against HHS and CMS asserting that, among other things, the IRA's Drug Price Negotiation Program for Medicare constitutes an uncompensated taking in violation of the Fifth Amendment of the Constitution. Subsequently, a number of other parties, including the U.S. Chamber of Commerce, Bristol Myers Squibb Company, the Pharmaceutical Research and Manufacturers of America, Novo Nordisk, Inc., Janssen Pharmaceuticals, Inc., Novartis AG, AstraZeneca plc and Boehringer Ingelheim International GMBH, also filed lawsuits in various courts with similar constitutional claims against HHS and CMS. There have been various decisions by the courts considering these cases since they were filed. HHS has generally won the substantive disputes in these cases or succeeded in getting claims dismissed for lack of standing. Most of these cases are now on appeal. On October 30, 2024, the U.S. Court of Appeals for the Third Circuit heard oral arguments in three of these cases. In April 2025, the U.S. Court of Appeals for the Second Circuit and the U.S. Court of Appeals for the Third Circuit heard arguments in an additional three cases. On May 8, 2025, the U.S. Court of Appeals for the Third Circuit rejected AstraZeneca L.P.'s challenge to the Medicare price negotiation program, finding that the program did not violate the company's due process rights under the Constitution since there is no protected property interest in selling goods to Medicare beneficiaries at a price higher than what the government is willing to pay in reimbursement. Litigation involving these and other provisions of the IRA will continue with unpredictable and uncertain results.

At the state level, individual states are increasingly aggressive in passing legislation and implementing 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. This is increasingly true with respect to products approved pursuant to the accelerated approval pathway. State Medicaid programs and other payers are developing strategies and implementing significant coverage barriers, or refusing to cover these products outright, arguing that accelerated approval drugs have insufficient or limited evidence despite meeting the FDA's standards for accelerated approval. In addition, regional healthcare organizations 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. These measures could reduce the ultimate demand for our products, once approved, or put pressure on our product pricing. We expect that additional state and federal healthcare reform measures will be adopted in the future, any of which could limit the amounts that federal and state governments will pay for healthcare products and services, which could result in reduced demand for our product candidates or additional pricing pressures. This may be increasingly true with respect to products approved pursuant to the accelerated approval pathway. State Medicaid programs and other payers are developing strategies and implementing significant coverage barriers, or refusing to cover these products outright, arguing that accelerated approval drugs have insufficient or limited evidence despite meeting the FDA's standards for accelerated approval.

In the EU, similar political, economic and regulatory developments may affect our ability to profitably commercialize our product candidates, if approved. In markets outside of the United States and the EU, reimbursement and healthcare payment systems vary significantly by country, and many countries have instituted price ceilings on specific products and therapies. In some countries, particularly the countries of the EU, the pricing of prescription pharmaceuticals is subject to governmental control. In these countries, pricing negotiations with governmental authorities can take considerable time after the receipt of marketing approval for a product. To obtain reimbursement or pricing approval in some countries, we may be required to conduct a clinical trial that compares the cost-effectiveness of our product candidate to other available therapies. If reimbursement of our products is unavailable or limited in scope or amount, or if pricing is set at unsatisfactory levels, our business could be harmed, possibly materially.

Our employees, principal investigators, consultants, and commercial partners may engage in misconduct or other improper activities, including non-compliance with regulatory standards and requirements and insider trading.

We are exposed to the risk of fraud or other misconduct by our employees, consultants, principal investigators and any commercial partners we may have. Misconduct by these parties could include intentional failures to comply with FDA regulations or the regulations applicable in the EU and other jurisdictions, provide accurate information to the FDA, the EMA, and other regulatory

authorities, comply with healthcare fraud and abuse laws and regulations in the United States and abroad, report financial information or data accurately, or disclose unauthorized activities to us. In particular, sales, marketing, and business arrangements in the healthcare industry are subject to extensive laws and regulations intended to prevent fraud, misconduct, kickbacks, self-dealing and other abusive practices. These laws and regulations restrict or prohibit a wide range of pricing, discounting, marketing and promotion, sales commission, customer incentive programs, and other business arrangements. Such misconduct also could involve the improper use of information obtained in the course of clinical trials or interactions with the FDA, the EMA or other regulatory authorities, which could result in regulatory sanctions and cause serious harm to our reputation. We have adopted a code of conduct applicable to all of our employees, but it is not always possible to identify and deter employee misconduct, and the precautions we take to detect and prevent this activity may not be effective in controlling unknown or unmanaged risks or losses or in protecting us from government investigations or other actions or lawsuits stemming from a failure to comply with these laws or regulations. If any such actions are instituted against us, and we are not successful in defending ourselves or asserting our rights, those actions could have a significant impact on our business, financial condition, results of operations, and prospects, including the imposition of significant fines or other sanctions.

Laws and regulations governing international operations may preclude us from developing, manufacturing and selling certain product candidates outside of the United States and require us to develop and implement costly compliance programs.

We are subject to numerous laws and regulations in each jurisdiction outside the United States in which we operate. The creation, implementation and maintenance of international business practices compliance programs is costly and such programs are difficult to enforce, particularly where reliance on third parties is required.

The Foreign Corrupt Practices Act, or FCPA, prohibits any U.S. individual or business from paying, offering, authorizing payment or offering of anything of value, directly or indirectly, to any foreign official, political party or candidate for the purpose of influencing any act or decision of the foreign entity in order to assist the individual or business in obtaining or retaining business. The FCPA also obligates companies whose securities are listed in the United States to comply with certain accounting provisions requiring us to maintain books and records that accurately and fairly reflect all transactions of the corporation, including international subsidiaries, and to devise and maintain an adequate system of internal accounting controls for international operations. The anti-bribery provisions of the FCPA are enforced primarily by the Department of Justice. The SEC is involved with enforcement of the books and records provisions of the FCPA.

Similarly, the U.K. Bribery Act 2010 has extra-territorial effect for companies and individuals having a connection with the U.K. The U.K. Bribery Act prohibits inducements both to public officials and private individuals and organizations. Compliance with the FCPA and the U.K. Bribery Act is expensive and difficult, particularly in countries in which corruption is a recognized problem. In addition, the FCPA presents particular challenges in the pharmaceutical industry, because, in many countries, hospitals are operated by the government, and doctors and other hospital employees are considered foreign officials. Certain payments to hospitals in connection with clinical trials and other work have been deemed to be improper payments to government officials and have led to FCPA enforcement actions.

Various laws, regulations and executive orders also restrict the use and dissemination outside of the United States, or the sharing with certain non-U.S. nationals, of information classified for national security purposes, as well as certain products and technical data relating to those products. Our expansion outside of the United States has required, and will continue to require, us to dedicate additional resources to comply with these laws, and these laws may preclude us from developing, manufacturing, or selling certain drugs and drug candidates outside of the United States, which could limit our growth potential and increase our development costs. The failure to comply with laws governing international business practices may result in substantial penalties, including suspension or debarment from government contracting. Violation of the FCPA can result in significant civil and criminal penalties. Indictment alone under the FCPA can lead to suspension of the right to do business with the U.S. government until the pending claims are resolved. Conviction of a violation of the FCPA can result in long-term disqualification as a government contractor. The termination of a government contract or relationship as a result of our failure to satisfy any of our obligations under laws governing international business practices would have a negative impact on our operations and harm our reputation and ability to procure government contracts. The SEC also may suspend or bar issuers from trading securities on U.S. exchanges for violations of the FCPA's accounting provisions.

Changes in and uncertainty surrounding U.S. and international trade policies may adversely impact our business and operating results.

In the spring of 2025, the U.S. government initiated a series of tariff-related actions against U.S. trading partners. On April 2, 2025, President Trump issued an executive order announcing a "baseline" reciprocal tariff of 10% on all U.S. trading partners effective April 5, 2025, and higher individualized reciprocal tariffs on 57 countries (with certain product exemptions for pharmaceutical-related products, among others). Previously, the Trump Administration had imposed a 25% tariff on Canada and Mexico for goods not covered by the United States-Mexico-Canada Agreement, or USMCA, and tariffs due to drug trafficking equaling 20% on imports from China. In response, several countries threatened retaliatory measures, including Canada and China, which then imposed retaliatory tariffs. Prior to when the country-specific reciprocal tariffs were scheduled to take effect, the Trump Administration delayed the effective date of such tariffs for all countries except China to August 1, 2025. Later, the United States and China reached a framework agreement that ultimately resulted in the suspension of the higher reciprocal tariffs on China until November 10, 2025.

Shortly before that expiration date, the United States and China reached a one-year agreement with an expiration of November 10, 2026, that includes the continued suspension of the heightened reciprocal tariffs on China and delayed enforcement of new U.S. export rules targeting affiliates of blacklisted firms.

Since the April 2025 reciprocal tariffs announcement, the European Union, Japan, South Korea, Switzerland and the United Kingdom, among others, have reached deals with the United States that include reduced tariff rates to varying levels and other measures. On July 31, 2025, President Trump issued an Executive Order detailing new reciprocal tariff rates for individual countries that took effect on August 7, 2025. The new reciprocal rates, which are consistent with the rates reflected in the trade deals already announced, range from 10% to 41%. The new rates do not apply to Canada, China, Mexico and a few other countries. For China, the 10% baseline reciprocal tariff announced in April remains in effect, in addition to a minimum of a 10% tariff due to drug trafficking. Regarding Canada and Mexico, the rate remains 25% for goods that are not covered by the USMCA for Mexico and, effective August 1, 2025, was increased to 35% on imports from Canada that are not covered by the USMCA. President Trump also announced a further 10% increase on non-USMCA goods from Canada, but it is unclear when such increase will take effect. The European Union, Japan, South Korea, Switzerland (and Liechtenstein), the United Kingdom and others have reached agreements with the United States that cap pharmaceutical tariffs at 15%. In addition, an agreement with Malaysia provides a zero percent tariff exemption for pharmaceutical products that are not patented in the United States and are used in pharmaceutical applications and an agreement with Switzerland and Liechtenstein caps tariffs on pharmaceuticals imported from Switzerland and Liechtenstein at 15%. Finally, the United States signed an agreement with Taiwan on January 15, 2026 that eliminates U.S. tariffs on generic pharmaceuticals, and their active ingredients, imported from Taiwan.

The reciprocal tariffs and the drug trafficking tariffs were imposed by President Trump pursuant to the International Emergency Economic Powers Act, or IEEPA. These tariffs were found to be unconstitutional by multiple federal courts in the spring and summer of 2025. The U.S. Supreme Court agreed to review those decisions on an expedited schedule and is expected to issue its ruling in early 2026. In the meantime, the lower court decisions were stayed, which means that the tariffs remain in effect pending the U.S. Supreme Court's decision.

Sustained uncertainty about, or the further escalation of, trade and political tensions between the United States and China could result in a disadvantageous research and manufacturing environment in China, particularly for U.S.-based companies, including retaliatory restrictions that hinder or potentially inhibit our ability to rely on CMOs and other service providers that operate in China.

Separately, in April 2025, the Department of Commerce initiated an investigation under Section 232 of the Trade Expansion Act of 1962 into the impact on U.S. national security of the imports of pharmaceuticals and pharmaceutical ingredients, including finished drug products, medical countermeasures, critical inputs such as active pharmaceutical ingredients, and key starting materials, and derivative products of those items. On September 25, 2025 President Trump announced, via a post on Truth Social, that, beginning on October 1, 2025, all branded or patented drugs imported into the United States would face a 100% tariff. At the same time, President Trump indicated that these tariffs could be avoided by building pharmaceutical manufacturing facilities in the United States. Thereafter, President Trump delayed the October 1, 2025 effective date of the tariffs on branded or patented pharmaceutical products, announcing that the administration had now "begun preparing" tariffs on manufacturers that do not build in the United States or enter into an MFN drug pricing agreement with the Trump Administration.

As a result of changes in tariffs that have been announced and/or implemented, and the underlying uncertainty currently surrounding international trade, we could experience a negative impact on our costs of materials and production processes, and supply chain disruptions and delays due to new or revised tariff policies or trade restrictions. If we are unable to obtain necessary raw materials or product components in sufficient quantities and in a timely manner due to disruptions in the global supply chain caused by macroeconomic events and conditions, the development, testing and clinical trials of our product candidates may be delayed or infeasible, and regulatory approval or commercial launch of any resulting product may be delayed or not obtained, which could significantly harm our business. We cannot yet predict the effect of the U.S. tariffs on imports, or the extent to which other countries will impose quotas, duties, tariffs, taxes or other similar restrictions upon imports or exports in the future, nor can we predict future trade policy or the terms of any renegotiated trade agreements and their impact on our business.

We are subject to stringent privacy laws, information security laws, regulations, policies and contractual obligations related to data privacy and security and changes in such laws, regulations, policies and contractual obligations, and failure to comply with such requirements could subject us to significant fines and penalties and other consequences, which may have a material adverse effect on our business, financial condition or results of operations.

We are subject to data privacy and protection laws and regulations that apply to the collection, transmission, storage and use of personally-identifying information, which among other things, impose certain requirements relating to the privacy, security and transmission of personal information, including comprehensive regulatory systems in the U.S., EU, U.K. and other countries in which we may conduct business. The legislative and regulatory landscape for privacy and data protection continues to evolve in jurisdictions worldwide, and there has been an increasing focus on privacy and data protection issues with the potential to affect our business. Failure to comply with any of these laws and regulations could result in enforcement action against us, including fines, imprisonment

of company officials and public censure, claims for damages by affected individuals, damage to our reputation and loss of goodwill, any of which could have a material adverse effect on our business, financial condition, results of operations or prospects.

There are numerous U.S. federal and state laws and regulations related to the privacy and security of personal information. In particular, regulations promulgated pursuant to HIPAA establish privacy and security standards that limit the use and disclosure of individually identifiable health information, or protected health information, and require the implementation of administrative, physical and technological safeguards to protect the privacy of protected health information and ensure the confidentiality, integrity and availability of electronic protected health information. Determining whether protected health information has been handled in compliance with applicable privacy standards and our contractual obligations can be complex and may be subject to changing interpretation.

If we fail to comply with applicable privacy laws, including applicable HIPAA privacy and security standards, we could face civil and criminal penalties. HHS enforcement activity can result in financial liability and reputational harm, and responses to such enforcement activity can consume significant internal resources. In recent months, the Officer of Civil Rights, or OCR, has been especially active in enforcing the HIPAA rules. In addition, state attorneys general are authorized to bring civil actions seeking either injunctions or damages in response to violations that threaten the privacy of state residents. We cannot be sure how these regulations will be interpreted, enforced or applied to our operations. In addition to the risks associated with enforcement activities and potential contractual liabilities, our ongoing efforts to comply with evolving laws and regulations at the federal and state level may be costly and require ongoing modifications to our policies, procedures and systems. Additionally, OCR is looking to amend the HIPAA Security Rule, which, if and when finalized, could create additional compliance obligations and risk for our business.

In addition to potential enforcement by the HHS, we could also be potentially subject to privacy enforcement from the FTC. The FTC has been particularly focused on the unpermitted processing of health and genetic data through its recent enforcement actions and is expanding the types of privacy violations that it interprets to be "unfair" under Section 5 of the FTC Act, as well as the types of activities it views to trigger the Health Breach Notification Rule, which the FTC also has the authority to enforce. The FTC is also in the process of developing rules related to commercial surveillance and data security. We will need to account for the FTC's evolving rules and guidance for proper privacy and data security practices in order to mitigate risk for a potential enforcement action, which may be costly. Finally, both the FTC and HHS's enforcement priorities, as well as those of other federal regulators, may be impacted by the change in administration and new leadership. These shifts in enforcement priorities may also impact our business.

There are also increased restrictions at the federal level relating to transferring sensitive data outside of the U.S. to certain foreign countries. For example, in 2024, Congress passed H.B. 815, which included the Protecting Americans' Data from Foreign Adversaries Act of 2024. This law creates certain restrictions for entities that disclose sensitive data (including potential health data) to countries such as China. Failure to comply with these rules can lead to a potential FTC enforcement action. Additionally, the Department of Justice recently finalized a rule implementing Executive Order 14117, which creates similar restrictions related to the transfer of sensitive U.S. data to countries such as China. These data transfer restrictions, and others that may pass in the future, may create operational challenges and legal risks for our business.

States are also active in creating specific rules relating to the processing of personal information. In 2018, California passed into law the California Consumer Privacy Act or CCPA, which took effect on January 1, 2020 and imposed many requirements on businesses that process the personal information of California residents. Many of the CCPA's requirements are similar to those found in the European General Data Protection Regulation, or GDPR, which is further described below, including requiring businesses to provide notice to data subjects regarding the information collected about them and how such information is used and shared, and providing data subjects the right to request access to such personal information and, in certain cases, request the erasure of such personal information. The CCPA also affords California residents the right to opt-out of "sales" of their personal information. The CCPA contains significant penalties for companies that violate its requirements.

In November 2020, California voters passed a ballot initiative for the California Privacy Rights Act, or CPRA, which went into effect on January 1, 2023 and significantly expanded the CCPA to incorporate additional GDPR-like provisions, including requiring that the use, retention and sharing of personal information of California residents be reasonably necessary and proportionate to the purposes of collection or processing, granting additional protections for sensitive personal information, and requiring greater disclosures related to notice to residents regarding retention of information. The CPRA also created a new enforcement agency – the California Privacy Protection Agency – the sole responsibility of which is to enforce the CPRA and other California privacy laws, which will further increase compliance risk. The provisions in the CPRA may apply to some of our business activities.

In addition to California, at least eighteen other states have passed comprehensive privacy laws similar to the CCPA and CPRA. These laws are either in effect or will go into effect sometime before the end of 2026. Like the CCPA and CPRA, these laws create obligations related to the processing of personal information, as well as special obligations for the processing of "sensitive" data, which includes health data in some cases. Some of the provisions of these laws may apply to our business activities. There are also states that are strongly considering additional laws that could go into effect in 2026 and beyond, and Congress has also been debating passing a federal privacy law. There are also states that are specifically regulating health information that may affect our business. For example, Washington state passed the My Health My Data Act in 2023 that specifically regulates the collection and sharing of health

information that is not otherwise regulated by the HIPAA rules, and the law also has a private right of action, which further increases the relevant compliance risk. Connecticut and Nevada have also passed similar laws regulating consumer health data, and more states are considering such legislation. These laws may impact our business activities, including our identification of research subjects, relationships with business partners and ultimately the marketing and distribution of our products.

Plaintiffs' lawyers are also increasingly using privacy-related statutes at both the state and federal level to bring lawsuits against companies for their data-related practices. In particular, there have been a significant number of cases filed against companies for their use of pixels and other web trackers. These cases often allege violations of the California Invasion of Privacy Act and other state laws regulating wiretapping, as well as the federal Video Privacy Protection Act. The rise in these types of lawsuits creates potential risk for our business.

Similar to the laws in the U.S., there are significant privacy and data security laws that apply in Europe and other countries. The collection, use, disclosure, transfer, or other processing of personal data, including personal health data, regarding individuals who are located in the EEA, and the processing of personal data that takes place in the EEA, is regulated by the GDPR, which went into effect in May 2018 and which imposes obligations on companies that operate in our industry with respect to the processing of personal data and the cross-border transfer of such data. The GDPR imposes onerous accountability obligations requiring data controllers and processors to maintain a record of their data processing and policies. If our or our partners' or service providers' privacy or data security measures fail to comply with the GDPR requirements, we may be subject to litigation, regulatory investigations, enforcement notices requiring us to change the way we use personal data and/or fines of up to 20 million Euros or up to 4% of the total worldwide annual turnover of the group of companies of the preceding financial year, whichever is higher, as well as compensation claims by affected individuals, negative publicity, reputational harm and a potential loss of business and goodwill.

The GDPR places restrictions on the cross-border transfer of personal data from the EU to countries that have not been found by the EC to offer adequate data protection legislation. There are ongoing concerns about the ability of companies to transfer personal data from the EU to other countries. In July 2020, the Court of Justice of the EU, or CJEU, invalidated the EU-U.S. Privacy Shield, one of the mechanisms used to legitimize the transfer of personal data from the EEA to the U.S. The CJEU decision also drew into question the long-term viability of an alternative means of data transfer, the standard contractual clauses, for international transfers of personal data from the EEA. This CJEU decision resulted in increased scrutiny on data transfers and increased our costs of compliance with data privacy legislation, as well as our costs of negotiating appropriate privacy and security agreements with our vendors and business partners.

Some privacy advocacy groups have already suggested that they will be challenging the DPF, and there is currently one pending litigation against the DPF before the Court of Justice of the European Union (CJEU), C-703/25 P – *Latombe v Commission*. If these challenges are successful, they may not only impact the DPF, but also further limit the viability of the so-called standard contractual clauses and other data transfer mechanisms.

In October 2022, President Biden signed an executive order to implement the EU-U.S. Data Privacy Framework, which serves as a replacement to the EU-U.S. Privacy Shield. The EC adopted the adequacy decision on July 10, 2023. The adequacy decision permits U.S. companies who self-certify to the EU-U.S. Data Privacy Framework to rely on it as a valid data transfer mechanism for data transfers from the EU to the U.S. However, some privacy advocacy groups have already suggested that they will be challenging the EU-U.S. Data Privacy Framework. If these challenges are successful, they may not only impact the EU-U.S. Data Privacy Framework, but also further limit the viability of the standard contractual clauses and other data transfer mechanisms. The uncertainty around this issue has the potential to impact our business. Following the withdrawal of the U.K. from the EU, the U.K. Data Protection Act of 2018 applies to the processing of personal data that takes place in the U.K. and includes parallel obligations to those set forth by the GDPR. In relation to data transfers, both the U.K. and the EU have determined, through separate "adequacy" decisions, that data transfers between the two jurisdictions are in compliance with the U.K. Data Protection Act of 2018 and the GDPR, respectively. The U.K. and the U.S. have also agreed to a U.S.-U.K. "Data Bridge," which functions similarly to the EU-U.S. Data Privacy Framework and provides an additional legal mechanism for companies to transfer data from the U.K. to the U.S.

Switzerland has also approved an adequacy decision in relation to the Swiss-U.S. Data Privacy Framework (which functions similarly to the EU-U.S. Data Privacy Framework and the U.S.-U.K. Data Bridge in relation to data transfers from Switzerland to the U.S.). Any changes or updates to these developments have the potential to impact our business.

Beyond GDPR, there are privacy and data security laws in a growing number of countries around the world. While many loosely follow GDPR as a model, other laws contain different or conflicting provisions. These laws will impact our ability to conduct our business activities, including both our clinical trials and the sale and distribution of commercial products, through increased compliance costs, costs associated with contracting and potential enforcement actions.

While we continue to address the implications of the recent changes to data privacy regulations, data privacy remains an evolving landscape at both the domestic and international level, with new regulations coming into effect and continued legal challenges, and our efforts to comply with the evolving data protection rules may be unsuccessful. It is possible that these laws may be interpreted and applied in a manner that is inconsistent with our practices. We must devote significant resources to understanding and complying with this changing landscape. Failure to comply with laws regarding data protection would expose us to risk of enforcement actions taken

by data protection authorities in the EEA and elsewhere and carries with it the potential for significant penalties if we are found to be non-compliant. Similarly, failure to comply with federal and state laws in the U.S. regarding privacy and security of personal information could expose us to penalties under such laws. Any such failure to comply with data protection and privacy laws could result in government-imposed fines or orders requiring that we change our practices, claims for damages or other liabilities, regulatory investigations and enforcement action, litigation and significant costs for remediation, any of which could adversely affect our business. Even if we are not determined to have violated these laws, government investigations into these issues typically require the expenditure of significant resources and generate negative publicity, which could harm our business, financial condition, results of operations or prospects.

Social media platforms present new risks and challenges to our business.

As social media continues to expand, it also presents us with new risks and challenges. Social media is increasingly being used to communicate information about us, our programs and the diseases our product candidates are being developed to treat. Social media practices in the pharmaceutical and biotechnology industries are evolving, which creates uncertainty and risk of noncompliance with regulations applicable to our business. For example, patients may use social media platforms to comment on the effectiveness of, or adverse experiences with, a product or a product candidate, which could result in reporting obligations or other consequences. Further, the accidental or intentional disclosure of non-public information by our workforce or others through media channels could lead to information loss. In addition, there is a risk of inappropriate disclosure of sensitive information or negative or inaccurate posts or comments about us, our products, or our product candidates on any social media platform. If any of these events were to occur or we otherwise fail to comply with applicable regulations, we could incur liability, face restrictive regulatory actions or incur other harm to our business including quick and irreversible damage to our reputation, brand image and goodwill.

Risks related to employee matters, managing growth and information technology

Our future growth may depend on our ability to identify and acquire businesses or technologies, and if we do not successfully do so, or otherwise fail to integrate any new businesses or technologies into our operations, we may have limited growth opportunities and it could result in significant impairment charges or other adverse financial consequences.

We are continuing to seek to acquire businesses or technologies that we believe are a strategic fit with our business strategy. Future acquisitions, however, may entail numerous operational and financial risks, including:

- a reduction of our current financial resources;
- incurrence of substantial debt or dilutive issuances of securities to pay for acquisitions and in connection with future milestone payment obligations under such acquisition agreements;
- difficulty or inability to secure financing to fund development activities for those acquired or in-licensed technologies;
- higher than expected acquisition and integration costs;
- disruption of our business, customer base and diversion of our management’s time and attention to develop acquired technologies; and
- exposure to unknown liabilities.

We may not have sufficient resources to acquire businesses and technologies and integrate them into our current infrastructure. In particular, we may compete with larger biotechnology companies and other competitors in our efforts to establish new collaborations and in-licensing opportunities. These competitors likely will have access to greater financial resources than we do and may have greater expertise in identifying and evaluating new opportunities. Furthermore, there may be overlap between our product candidates and the companies which we acquire that may create conflicts in relationships or other commitments detrimental to the integrated businesses. Additionally, the time between our expenditures to acquire or in-license new technologies or businesses and the subsequent generation of revenues from those acquired technologies or businesses (or the timing of revenue recognition related to licensing agreements and/or strategic collaborations) could cause fluctuations in our financial performance from period to period. Finally, if we devote resources to potential acquisition opportunities that are never completed, or if we fail to realize the anticipated benefits of those efforts, we could incur significant impairment charges or other adverse financial consequences.

Our future success depends on our ability to retain our Chief Executive Officer, President and other key executives and to attract, retain, and motivate qualified personnel.

We are highly dependent on John Evans, our Chief Executive Officer, and Dr. Giuseppe Ciaramella, our President, as well as the other principal members of our management and scientific teams. Mr. Evans, Dr. Ciaramella and such other principal members are employed “at will,” meaning we or they may terminate their employment at any time. We do not maintain “key person” insurance for any of our executives or other employees. The loss of the services of any of these persons could impede the achievement of our research, development, and commercialization objectives.

Recruiting and retaining qualified scientific, clinical, manufacturing, and sales and marketing personnel will also be critical to our success. Our competitors may seek to use these transitions and the related potential disruptions to gain a competitive advantage over us. There is currently a shortage of highly qualified personnel in our industry, which is likely to continue. Additionally, this shortage of highly qualified personnel is particularly acute in the area where we are located.

We may not be able to attract and retain these 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 research and development and commercialization strategy. Our consultants and advisors, including our scientific co-founders, 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. The inability to recruit, or loss of services of certain executives, key employees, consultants, or advisors, may impede the progress of our research, development, and commercialization objectives and have a material adverse effect on our business, financial condition, results of operations, and prospects.

We expect to expand our development, regulatory, and future sales and marketing capabilities, and as a result, we may encounter difficulties in managing our growth, which could disrupt our operations.

In connection with the growth and advancement of our pipeline, we expect to increase the number of our employees and the scope of our operations in the areas of drug development, regulatory affairs, and sales and marketing. To manage our anticipated future growth in these areas, 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 expected expansion of our operations or recruit and train additional qualified personnel. Moreover, the expected physical 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.

As an early-stage biotechnology company, we are actively pursuing new platforms and product candidates in many therapeutic areas and across a wide range of diseases. Successfully developing product candidates for and fully understanding the regulatory and manufacturing pathways to all of these therapeutic areas and disease states requires a significant depth of talent, resources and corporate processes in order to allow simultaneous execution across multiple areas. Due to our limited resources, we may not be able to effectively manage this simultaneous execution and the expansion of our operations or recruit and train additional qualified personnel. This may result in weaknesses in our infrastructure, give rise to operational mistakes, legal or regulatory compliance failures, loss of business opportunities, loss of employees and reduced productivity among remaining employees. The physical expansion of our operations may lead to significant costs and may divert financial resources from other projects, such as the development of our product candidates. If our management is unable to effectively manage any future growth, our expenses may increase more than expected, our ability to generate or increase our revenue could be reduced and we may not be able to implement our business strategy. Our future financial performance and our ability to compete effectively and commercialize our product candidates, if approved, will depend in part on our ability to effectively manage the future development and expansion of our company.

Risks related to our common stock

The market price of our common stock may be volatile and fluctuate substantially, which could result in substantial losses for purchasers of our common stock and subject us to securities class action litigation.

Our stock price has been, and in the future, may be, subject to substantial volatility. 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, you may not be able to sell your common stock at or above your initial purchase price. The market price for our common stock may be influenced by many factors, including:

- the success of existing or new competitive product candidates or technologies;
- the timing and results of preclinical studies and clinical trials for any product candidates that we develop;
- failure or discontinuation of any of our product development and research programs;
- results of preclinical studies, clinical trials, or regulatory approvals of product candidates of our competitors, or announcements about new research programs or product candidates of our competitors;
- developments or changing views regarding the use of genetic medicines, including those that involve gene editing;
- commencement or termination of collaborations for our product development and research programs;
- regulatory or legal developments in the United States and other countries;
- developments or disputes concerning patent applications, issued patents, or other proprietary rights;

- the recruitment or departure of key personnel;
- the level of expenses related to any of our research programs, clinical development programs, or product candidates that we may develop;
- the results of our efforts to develop additional product candidates or products;
- actual or anticipated changes in estimates as to financial results, development timelines, or recommendations by securities analysts;
- announcement or expectation of additional financing efforts;
- sales of our common stock by us, our insiders or other stockholders;
- expiration of any future market stand-off or lock-up agreements;
- variations in our financial results or those of companies that are perceived to be similar to us;
- changes in estimates or recommendations by securities analysts, if any, that cover our stock;
- changes in the structure of healthcare payment systems;
- market conditions in the pharmaceutical and biotechnology sectors;
- the effects of pandemics and public health emergencies;
- general economic, industry, and market conditions; and
- the other factors described in this "Risk Factors" section.

Following periods of such volatility in the market price of a company's securities, securities class action litigation has often been brought against that company. Because of the potential volatility of our stock price, we may become the target of securities litigation in the future. Securities litigation could result in substantial costs and divert management's attention and resources from our business.

If we fail to establish and maintain proper and effective internal control over financial reporting, our operating results and our ability to operate our business could be harmed.

Maintaining adequate internal financial and accounting controls and procedures to ensure that we can produce accurate financial statements on a timely basis is a costly and time-consuming effort that needs to be re-evaluated frequently. Our internal control over financial reporting is a process designed to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements in accordance with generally accepted accounting principles. To comply with the requirements of being a public company, we have undertaken certain actions, such as documenting, reviewing and improving our internal controls and procedures for compliance with Section 404 of the Sarbanes-Oxley Act of 2002, or SOX, which requires annual management assessment of the effectiveness of our internal control over financial reporting and an annual report on and attestation to such assessment by our registered public accounting firm. Notwithstanding such actions, we may not be effective in maintaining the adequacy of our internal controls, and any failure to maintain that adequacy, or consequent inability to produce accurate financial statements on a timely basis, or any disagreement with our auditors on whether we have maintained such adequacy, could increase our operating costs and harm our business. In addition, investors' perceptions that our internal controls are inadequate or that we are unable to produce accurate financial statements on a timely basis may harm our common stock price and make it more difficult for us to effectively market and sell our service to new and existing customers.

We have incurred and expect to continue to incur increased costs as a result of operating as a public company, and our management is required to devote substantial time to new compliance initiatives and corporate governance practices.

As a public company, we have incurred and expect to continue to incur significant legal, accounting, and other expenses. The Sarbanes-Oxley Act of 2002, the Dodd-Frank Wall Street Reform and Consumer Protection Act, the listing requirements of The Nasdaq Stock Market, and other applicable securities rules and regulations impose various requirements on public companies, including establishment and maintenance of effective disclosure and financial controls and corporate governance practices. Our management and other personnel devote a substantial amount of time towards maintaining compliance with these requirements. These requirements have increased our legal and financial compliance costs and make some activities more time-consuming and costly. For example, as a public company it is more difficult and more expensive for us to maintain director and officer liability insurance, which could make it more difficult for us to attract and retain qualified members of our board of directors. These rules and regulations are often subject to varying interpretations, in many cases due to their lack of specificity, and, as a result, their application in practice may evolve over time as new guidance is provided by regulatory and governing bodies. This could result in continuing uncertainty regarding compliance matters and higher costs necessitated by ongoing revisions to disclosure and governance practices.

We do not expect to pay any dividends for the foreseeable future. Investors may never obtain a return on their investment unless they sell our common stock for a price higher than which they paid for it.

You should not rely on an investment in our common stock to provide dividend income. We do not anticipate that we will pay any dividends to holders of our common stock in the foreseeable future. Instead, we plan to retain any earnings to maintain and expand our existing operations. In addition, the terms of the Financing Agreement preclude us from paying dividends, and any future credit facility may contain terms prohibiting or limiting the amount of dividends that may be declared or paid on our common stock. Accordingly, investors must rely on sales of their common stock after price appreciation, which may never occur, as the only way to realize any return on their investment.

Provisions in our fourth amended certificate of incorporation, our second amended and restated by-laws, and Delaware law may have anti-takeover effects that could discourage an acquisition of us by others, even if an acquisition would be beneficial to our stockholders, and may prevent attempts by our stockholders to replace or remove our current management.

Our fourth amended certificate of incorporation, or our certificate of incorporation, and our second amended and restated by-laws, or our by-laws, and Delaware law contain provisions that may have the effect of discouraging, delaying or preventing a change in control of us or changes in our management that stockholders may consider favorable, including transactions in which you might otherwise receive a premium for your shares. Our certificate of incorporation and by-laws include provisions that:

- authorize "blank check" preferred stock, which could be issued by our board of directors without stockholder approval and may contain voting, liquidation, dividend and other rights superior to our common stock;
- create a classified board of directors whose members serve staggered three-year terms;
- specify that special meetings of our stockholders can be called only by our board of directors;
- prohibit stockholder action by written consent;
- establish an advance notice procedure for stockholder approvals to be brought before an annual meeting of our stockholders, including proposed nominations of persons for election to our board of directors;
- provide that vacancies on our board of directors may be filled only by a majority of directors then in office, even though less than a quorum;
- provide that our directors may be removed only for cause;
- specify that no stockholder is permitted to cumulate votes at any election of directors;
- expressly authorize our board of directors to make, alter, amend or repeal our by-laws; and
- require supermajority votes of the holders of our common stock to amend specified provisions of our certificate of incorporation and by-laws.

These provisions, alone or together, could delay or prevent hostile takeovers and changes in control or changes in our management. These provisions could also limit the price that investors might be willing to pay for shares of our common stock, thereby depressing the market price of our common stock.

In addition, because we are incorporated in the State of Delaware, we are governed by the provisions of Section 203 of the General Corporation Law of the State of Delaware, or 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.

Any provision of our certificate of incorporation, by-laws or Delaware law that has the effect of delaying or deterring a change in control could limit the opportunity for our stockholders to receive a premium for their shares of our common stock, and could also affect the price that some investors are willing to pay for our common stock.

Our certificate of incorporation and by-laws designate the state or federal courts within the State of Delaware as the exclusive forum for certain types of actions and proceedings that may be initiated by 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 certificate of incorporation provides that, subject to limited exceptions, the state or federal courts within the State of Delaware will be exclusive forums for (1) any derivative action or proceeding brought on our behalf, (2) any action asserting a claim of breach of a fiduciary duty owed by any of our directors, officers or other employees to us or our stockholders, (3) any action asserting a claim against us arising pursuant to any provision of the DGCL, our certificate of incorporation or our by-laws, (4) any action to interpret, apply, enforce or determine the validity of our certificate of incorporation or our by-laws or (5) any other action asserting a claim against us that is governed by the internal affairs doctrine. Furthermore, our by-laws also provide that unless we consent in writing to the selection of an alternative forum, the federal district courts of the United States shall be the exclusive forum for the resolution of

any complaint asserting a cause of action arising under the Securities Act. Any person or entity purchasing or otherwise acquiring any interest in shares of our capital stock shall be deemed to have notice of and to have consented to the provisions of our certificate of incorporation and by-laws described above. These choice of 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 such lawsuits against us and our directors, officers and employees. Alternatively, if a court were to find these provisions of our certificate of incorporation or by-laws inapplicable to, or unenforceable in respect of, one or more of the specified types of actions or proceedings, we may incur additional costs associated with resolving such matters in other jurisdictions, which could adversely affect our business and financial condition. For example, the Court of Chancery of the State of Delaware recently determined that a provision stating that federal district courts of the United States are the exclusive forum for resolving any complaint asserting a cause of action under the Securities Act is not enforceable. However, this decision may be reviewed and ultimately overturned by the Delaware Supreme Court.

General risk factors

Public health emergencies or epidemics could adversely impact our business.

Public health emergencies or epidemics could materially and adversely affect our business and our financial results and could cause a disruption to the development of our product candidates. The extent to any such public health emergency may impact our business, results of operations and future growth prospects will depend on a variety of factors, including the duration, scope and severity of the emergency, the existence and extent of travel restrictions and social distancing in the U.S. and other countries, business closures or business disruptions, the effectiveness of actions taken in the U.S. and other countries, and the availability of therapeutic interventions.

Some factors from public health emergencies that could delay or otherwise adversely affect the completion of our preclinical and clinical activities and, depending on the duration of the outbreak, the initiation of any future clinical trials, as well as our business generally, include:

- business disruptions caused by potential workplace, laboratory and office closures and an increased reliance on employees working from home, disruptions to or delays in ongoing laboratory experiments and operations, staffing shortages, travel limitations, cyber security and data accessibility, or communication or mass transit disruptions, any of which could adversely impact our business operations or delay necessary interactions with local regulators, ethics committees, manufacturing sites, research sites and other important agencies and contractors;
- limitations on the availability of preclinical and clinical trial sites, researchers and investigators, regulatory agency personnel, and materials;
- limitations on our business operations by local, state, or the federal government that could impact our ability to conduct our preclinical and clinical activities;
- limitations on travel that could hinder our timelines;
- interruption in global shipping affecting the transport of key materials;
- interruption of, or delays in receiving, key materials from our CMOs due to staffing shortages, production slowdowns or stoppages, increased demand from third parties for key materials and disruptions in delivery systems; and
- disruptions to our third-party suppliers, including through the effects of facility closures, reductions in operating hours, staggered shifts and other social distancing efforts, labor shortages, decreased productivity and unavailability of materials or components.

Public health emergencies may also have the effect of heightening many of the other risks described in this section titled "Item 1A. Risk Factors," such as risks related to our need to raise additional funding, fluctuation of our quarterly financial results, and our ability to obtain and maintain regulatory approvals.

Changes in tax laws or in their implementation or interpretation could adversely affect our business and financial condition.

Income, sales, use or other tax laws, statutes, rules, or regulations could be enacted or amended at any time, which could affect our business or financial condition, including causing potentially adverse impacts to our effective tax rate, tax liabilities and cash tax obligations. For example, the IRA was signed into law in August 2022, and the One Big Beautiful Bill Act, or OBBBA, was signed into law in July 2025. The IRA introduced new tax provisions, including a one percent excise tax imposed on certain stock repurchases by publicly traded companies. The one percent excise tax generally applies to any acquisition of stock by the publicly traded company (or certain of its affiliates) from a stockholder of the company in exchange for money or other property (other than stock of the company itself), subject to certain exceptions. Thus, the excise tax could apply to certain transactions that are not traditional stock repurchases. The OBBBA contains numerous tax provisions which may significantly affect our business or financial condition. The recent changes under the OBBBA include tax rate extensions and changes to the business interest deduction limitation, the expensing of domestic research and development expenditures (in contrast to the continued capitalization and amortization of foreign research and development expenditures), the bonus depreciation deduction rules, and the international tax framework.

Regulatory guidance under the IRA, the OBBBA, and other tax-related legislation is and continues to be forthcoming, and such guidance could ultimately increase or lessen the impact of these laws on our business and financial condition. In addition, it is uncertain if and to what extent various states will conform to changes to federal tax legislation.

Unstable market and economic conditions may have serious adverse consequences on our business, financial condition and stock price.

Global credit and financial markets have experienced extreme volatility and disruptions in the past several years, including severely diminished liquidity and credit availability, declines in consumer confidence, declines in economic growth, increases in unemployment rates and uncertainty about economic stability, disruptions impacting global supply, the conflict between Russia and Ukraine and related sanctions against Russia, increasing inflation rates and interest rate changes. There can be no assurance that further deterioration in credit and financial markets and confidence in economic conditions will not occur. Our general business strategy may be adversely affected by any such economic downturn, volatile business environment or continued unpredictable and unstable market conditions. If the current equity and credit markets deteriorate, or do not improve, it may make any necessary debt or equity financing more difficult, more costly, and more dilutive. Furthermore, our stock price may decline due in part to the volatility of the stock market and the general economic downturn.

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 stock price and could require us to delay, scale back or discontinue the development and commercialization of one or more of our product candidates or delay our pursuit of potential in-licenses or acquisitions. In addition, there is a risk that one or more of our current service providers, manufacturers and other partners may not survive these difficult economic times, which could directly affect our ability to attain our operating goals.

If securities analysts do not publish research or reports about our business or if they publish negative evaluations of our stock, the price of our stock could decline.

The trading market for our common stock relies in part on the research and reports that industry or financial analysts publish about us or our business. If one or more of these analysts ceases coverage of our company or fails to publish reports on us regularly, we could lose visibility in the financial markets, which in turn could cause our stock price or trading volume to decline. Moreover, if any of the analysts who cover us issue an adverse or misleading opinion regarding us, our business model or our stock performance, or if our operating results fail to meet the expectations of the investor community, one or more of the analysts who cover our company may change their recommendations regarding our company, and our stock price could decline.

Our internal computer systems, or those of our third-party vendors, collaborators or other contractors or consultants, may fail or suffer security breaches, which could result in a material disruption of our product development programs, compromise sensitive information related to our business or prevent us from accessing critical information, potentially exposing us to liability or otherwise adversely affecting our business.

Our internal computer systems and those of our current and any future third-party vendors, collaborators and other contractors or consultants are vulnerable to damage or interruption from computer viruses, computer hackers, malicious code, employee theft or misuse, denial-of-service attacks, sophisticated nation-state and nation-state-supported actors, unauthorized access, natural disasters, terrorism, war and telecommunication and electrical failures. While we seek to protect our information technology systems from system failure, accident and security breach through our information security program and relevant contractual agreements with our business partners, if such an event were to occur and cause interruptions in our operations, it could result in a disruption of our development programs and our business operations, whether due to a loss of our trade secrets or other proprietary information or other disruptions, including the possible loss of personal data. For example, the loss of clinical trial data from future clinical trials could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data, as well as subject us to obligations and risks related to the potential loss of personal data. If we were to experience a significant cybersecurity breach of our information systems or data, the costs associated with the investigation, remediation and potential notification of the breach to counterparties and data subjects could be material, in addition to potential costs related to regulatory investigations in the United States or other countries. In addition, our remediation efforts may not be successful. If we do not allocate and effectively manage the resources necessary to build and sustain the proper technology and cybersecurity infrastructure, we could suffer significant business disruption, including transaction errors, supply chain or manufacturing interruptions, processing inefficiencies, data loss or the loss of or damage to intellectual property or other proprietary information.

To the extent that any disruption or security breach were to result in a loss of, or damage to, our or our third-party vendors', collaborators' or other contractors' or consultants' data or applications, or inappropriate disclosure of confidential or proprietary information, we could incur liability including litigation exposure, penalties and fines, we could become the subject of regulatory action or investigation, our competitive position could be harmed and the further development and commercialization of our product candidates could be delayed. Any of the above could have a material adverse effect on our business, financial condition, results of operations or prospects.

Artificial intelligence presents risks and challenges that can impact our business including by posing security risks to our confidential information, proprietary information, and personal data.

Issues in the development and use of artificial intelligence, combined with an uncertain regulatory environment, may result in reputational harm, liability, or other adverse consequences to our business operations. As with many technological innovations, artificial intelligence presents risks and challenges that could impact our business. We may adopt and integrate generative artificial intelligence tools into our systems for specific use cases reviewed by legal and information security. Our vendors may incorporate generative artificial intelligence tools into their offerings without disclosing this use to us, and the providers of these generative artificial intelligence tools may not meet existing or rapidly evolving regulatory or industry standards with respect to privacy and data protection and may inhibit our or our vendors' ability to maintain an adequate level of service and experience. If we, our vendors, or our third-party partners experience an actual or perceived breach or privacy or security incident because of the use of generative artificial intelligence, we may lose valuable intellectual property and confidential information and our reputation and the public perception of the effectiveness of our security measures could be harmed. Further, bad actors around the world use increasingly sophisticated methods, including the use of artificial intelligence, to engage in illegal activities involving the theft and misuse of personal information, confidential information, and intellectual property. Additionally, there are increased rules and regulations governing the use and development of artificial intelligence at the state, federal, and international levels, which creates potential compliance and enforcement risk. These developments could damage our reputation, result in the loss of valuable property and information, and adversely impact our business.

Item 1B. Unresolved Staff Comments.

None.

Item 1C. Cybersecurity.

We have certain processes for assessing, identifying and managing cybersecurity risks, which are built into our overall risk management program/information technology function and are designed to help protect our information assets and operations from internal and external cyber threats, protect employee and clinical trial information from unauthorized access or attack, as well as secure our networks and systems. Such processes include physical, procedural and technical safeguards, response plans, regular tests on our systems, incident simulations and routine review of our policies and procedures to identify risks and enhance our practices. We engage certain external parties, including consultants, independent privacy assessors, computer security firms and risk management, peer companies, industry groups and governance experts, to enhance our cybersecurity oversight. We consider the internal risk oversight programs of third-party service providers before engaging them in order to help protect our company from any related vulnerabilities.

Based on an assessment using these processes, we do not believe that there are currently any risks from known cybersecurity threats that are reasonably likely to materially affect our company or our business strategy, results of operations or financial condition. For additional information, please see the risk factor titled "*Our internal computer systems, or those of our third-party vendors, collaborators or other contractors or consultants, may fail or suffer security breaches, which could result in a material disruption of our product development programs, compromise sensitive information related to our business or prevent us from accessing critical information, potentially exposing us to liability or otherwise adversely affecting our business."* under Item 1A., *Risk factors—General risk factors,* in this Annual Report on Form 10-K.

The Audit Committee of our board of directors provides direct oversight over cybersecurity risk, and provides updates to the board of directors regarding such oversight. The Audit Committee receives updates quarterly from management regarding cybersecurity matters, and is notified between such updates regarding significant new cybersecurity threats or incidents.

Our Vice President of Information Security, or the VP, IS, leads the operational oversight of company-wide cybersecurity strategy, policy, standards and processes and works across relevant departments to assess and help prepare us and our employees and third-party service providers to address cybersecurity risks. The VP, IS's cybersecurity training includes 25 years of experience building and maintaining cybersecurity programs, and he obtained his certified information systems security professional, or CISSP, certification from the International Information System Security Certification Consortium, or ISC2, and his Cybersecurity and Infrastructure Security Agency, or CISA, certification from ISACA.

In an effort to deter and detect cyber threats, we annually provide all employees, including part-time and temporary employees, with a data protection, cybersecurity and incident response and prevention training and compliance program, which covers timely and relevant topics. Past topics have included social engineering, phishing, password protection, confidential data protection, asset use and mobile security, and educate employees on the importance of reporting all incidents immediately. We also use technology-based tools to mitigate cybersecurity risks and to bolster our employee-based cybersecurity programs.

Item 2. Properties.

We have leased approximately 130,258 square feet of office and laboratory space in Cambridge, Massachusetts under a lease that expires in 2034. Additionally, we entered into a lease agreement with Alexandria Real Estate Equities, Inc. pursuant to which we built

a 100,000 square foot manufacturing facility in Research Triangle Park, North Carolina intended to support a broad range of clinical programs, which lease expires in 2037. We believe that our facilities are sufficient to meet our current needs and that suitable additional space will be available as and when needed.

Item 3. Legal Proceedings.

We are not currently a party to any material legal proceedings. From time to time, we may be subject to various legal proceedings and claims that arise in the ordinary course of our business activities.

Item 4. Mine Safety Disclosures.

Not Applicable.

PART II

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

Market information

Our common stock has been publicly traded on the Nasdaq Global Select Market under the symbol "BEAM" since February 6, 2020. Prior to that time, there was no public market for our common stock.

Holders

As of February 17, 2026, there were approximately 25 holders of record of our common stock. This number does not include beneficial owners whose shares are held by nominees in street name.

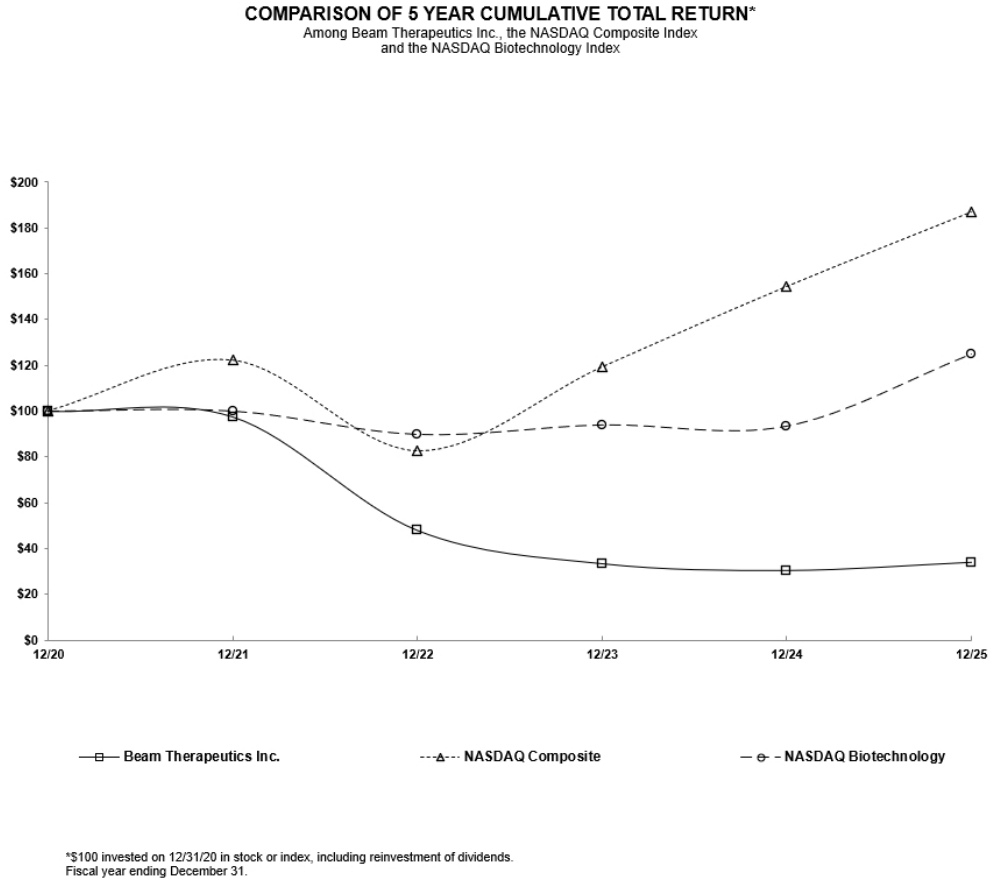
Dividends

We have not declared or paid any cash dividends on our capital stock since our inception. We intend to retain future earnings, if any, to finance the operation and expansion of our business and do not anticipate paying any cash dividends to holders of common stock in the foreseeable future. In addition, our financing agreement with Sixth Street Lending Partners contains restrictive covenants that prohibit us, subject to certain exceptions, from paying dividends on our common stock, and future debt securities or other financing arrangements could contain similar or more restrictive negative covenants.

Stock Performance Graph

The following performance graph and related information shall not be deemed to be "soliciting material" or to be "filed" with the Securities and Exchange Commission, or SEC, for purposes of Section 18 of the Securities Exchange Act of 1934, as amended, or the Exchange Act, nor shall such information be incorporated by reference into any future filing under the Exchange Act or Securities Act of 1933, as amended, or the Securities Act, except to the extent that we specifically incorporate it by reference into such filing.

The graph set forth below compares the cumulative total stockholder return on our shares of common stock between December 31, 2020 and December 31, 2025, with the cumulative total return of (a) the Nasdaq Biotechnology Index and (b) the Nasdaq Composite Index, over the same period. This graph assumes the investment of $100 on December 31, 2020 in our common stock, the Nasdaq Biotechnology Index and the Nasdaq Composite Index and assumes the reinvestment of dividends, if any. The comparisons shown in the graph below are based upon historical data. The stock price performance included in this graph is not necessarily indicative of future stock price performance.



Purchases of equity securities by the issuer or affiliated purchasers

Neither we nor any affiliated purchaser or anyone acting on our behalf or on behalf of an affiliated purchaser made any purchases of shares of our common stock during the fourth quarter of 2025.

Item 6. [Reserved]

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

The following discussion and analysis of our financial condition and results of operations should be read in conjunction with our consolidated financial statements and the related notes to those statements included elsewhere in this Annual Report on Form 10-K. In addition to historical financial information, the following discussion and analysis contains forward-looking statements that involve risks, uncertainties and assumptions. Some of the numbers included herein have been rounded for the convenience of presentation. Our actual results may differ materially from those anticipated in these forward-looking statements as a result of many factors, including those discussed under Item 1A, Risk factors, in this Annual Report on Form 10-K.

Information pertaining to fiscal year 2023 was included in our Annual Report on Form 10-K for the year ended December 31, 2024 on pages 111 through 123 under Part II, Item 7, "Management's Discussion and Analysis of Financial Position and Results of Operations," which was filed with the Securities and Exchange Commission (the "SEC") on February 25, 2025.

Overview

We are a biotechnology company committed to establishing the leading, fully integrated platform for precision genetic medicines. Our vision is to provide life-long cures to patients suffering from serious diseases. To achieve this vision, we have assembled a platform that includes a suite of gene editing and delivery technologies as well as internal manufacturing capabilities.

Our suite of gene editing technologies is anchored by our proprietary base editing technology, which potentially enables a differentiated class of precision genetic medicines that target a single base in the genome without making a double-stranded break in the DNA. This approach uses a chemical reaction designed to create precise, predictable and efficient genetic outcomes at the targeted sequence. Our proprietary base editors have two principal components: (i) a clustered regularly interspaced short palindromic repeats, or CRISPR, protein, bound to a guide RNA, that leverages the established DNA-targeting ability of CRISPR, but is modified to not cause a double-stranded break, and (ii) a base editing enzyme, such as a deaminase, which carries out the desired chemical modification of the target DNA base. We believe this design contributes to a more precise and efficient edit compared to traditional gene editing methods, with the potential to dramatically increase the impact of gene editing. We are also pursuing a suite of delivery modalities, including both *ex vivo* and *in vivo* approaches, depending on tissue type. The elegance of the base editing approach, combined with a tissue specific delivery modality, provides the basis for a targeted, efficient, precise, and highly versatile gene editing system that is designed to be capable of gene correction, gene silencing, gene activation, gene modification, and/or multiplex editing of several genes simultaneously.

Our goal is to advance a broad, diversified portfolio of base editing programs against distinct, genetically validated editing targets, as well as an innovative, platform business model that will expand the reach of our programs to more patients. Overall, we are seeking to build the leading integrated platform for precision genetic medicine, which may have broad therapeutic applicability and the potential to transform the field of precision genetic medicines.

Hematology

We are pursuing a long-term, staged development strategy for our base editing approach to treat hematological diseases, such as sickle cell disease and beta-thalassemia. Our initial wave consists of *ex vivo* programs in which hematopoietic stem cells, or HSCs, are collected from a patient, edited using electroporation, and then infused back into the patient following a conditioning regimen, such as treatment with busulfan, the standard of care in HSC transplantation, or HSCTs, today. Once reinfused, the HSCs begin repopulating a portion of the bone marrow in a process known as engraftment. The engrafted, edited HSCs give rise to progenitor cell types with the corrected gene sequences. We are deploying this *ex vivo* approach in our risto-cel program. We are also pursuing a next wave of *in vivo* base editing with delivery directly into HSCs of patients via LNPs. We believe this multi-wave strategy can maximize the potential applicability of our sickle cell disease programs to patients as well as create a platform for the treatment of many other severe genetic blood disorders.

Ex Vivo Base Editing via Autologous Transplant with risto-cel

We are using base editing to pursue the development of risto-cel for the treatment of sickle cell disease. Risto-cel is a patient-specific, autologous HSC investigational therapy designed to offer a potentially best-in-class profile, incorporating base edits that are intended to mimic single nucleotide polymorphisms seen in individuals with hereditary persistence of fetal hemoglobin, or HbF.

Risto-cel aims to alleviate the effects of sickle cell disease by increasing HbF, which is expected to increase functional hemoglobin production and, in the case of sickle cell disease, inhibit hemoglobins S, or HbS, polymerization.

We are conducting a Phase 1/2 clinical trial designed to assess the safety and efficacy of risto-cel for the treatment of sickle cell disease, which we refer to as our BEACON trial. The BEACON trial includes approximately 50 adults and adolescents with severe sickle cell disease who have received prior treatment with at least one disease-modifying agent with inadequate response or intolerance. Following mobilization, conditioning and treatment with risto-cel, patients are assessed for safety and tolerability, with safety endpoints including neutrophil and platelet engraftment. Patients are also assessed for efficacy, with efficacy endpoints including the change from baseline in severe vaso-occlusive events, transfusion requirements, HbF levels, and quality of life assessments. The adult and adolescent enrollment for BEACON is complete, and manufacturing of all doses was completed as of

December 2025. The U.S. Food and Drug Administration, or the FDA, has granted orphan drug designation and regenerative medicine advanced therapy designation to risto-cel. Risto-cel has also been accepted into the FDA's Chemistry, Manufacturing, and Controls Development and Readiness pilot program.

In December 2025, we presented updated data from the BEACON trial at the American Society of Hematology 2025 Annual Meeting, or ASH. The presentation contained preliminary data as of August 6, 2025, from 31 patients in the trial, with follow up ranging from 0.3 to 20.4 months. The presentation data included the following:

- Patients achieved mean HbF levels above 60% and a mean durable reduction in corresponding HbS below 40%. A pancellular distribution of HbF, reflecting expression across most of the circulating red blood cells, was observed, with mean per-cell HbF levels maintained above the sickling threshold throughout follow-up. Durable, high editing efficiency was observed in peripheral blood and bone marrow following treatment with risto-cel. Mean peripheral blood editing was 67.4% at Month 6 and 72.8% by Month 12
- Patients required a median of one (range: 1-5) stem cell collection cycle, comprising a median of three (range: 1–13) total collection days for the risto-cel manufacturing process and back-up cell collection. The median time to neutrophil engraftment was 17.5 days (range: 12-30), with a median duration of severe neutropenia of seven days (range: 1-17). The median time to platelet engraftment was 19 days (range: 11-53). In addition, 29% of patients did not require any platelet transfusions following risto-cel treatment.
- Total Hb levels increased rapidly with all patients experiencing resolution of anemia after elimination of the transfused blood. Key markers of hemolysis, including indirect bilirubin, haptoglobin, lactate dehydrogenase, and reticulocytes, normalized or improved in all patients following risto-cel treatment. Erythropoietin levels also trended toward normal, indicating significant improvement in oxygen delivery to tissues. Sickling parameters all decreased in the blood following risto-cel treatment to levels comparable to those seen in individuals with sickle cell trait.
- The initial safety profile of risto-cel was consistent with busulfan conditioning, autologous HSCT and underlying sickle cell disease. The most common treatment-emergent adverse events were consistent with busulfan conditioning, including febrile neutropenia, stomatitis and decreased appetite. As previously reported, one patient died four months after risto-cel infusion due to respiratory failure that was determined by the investigator to be likely related to busulfan conditioning and deemed unrelated to risto-cel. No patients experienced any investigator-reported severe vaso-occlusive crises post-engraftment.

We expect to submit a BLA for risto-cel as early as year-end 2026.

In Vivo B*ase Editing via HSC-targeted LNPs*

We continue to develop targeted LNPs for the *in vivo* delivery of gene editing payloads to HSCs. Based on recent advancements in this technology, we are now prioritizing *in vivo* delivery for our next wave approach to treating sickle cell disease. We have identified multiple targeted LNPs that have the potential for HSC delivery and are currently engaged in lead optimization. In parallel, we are also continuing development of our proprietary ESCAPE platform, which combines antibody-based conditioning with multiplex gene edited HSCs. ESCAPE has the potential to enable non-genotoxic treatment strategies that can be delivered either *ex vivo* or *in vivo*, including as part of any future *in vivo* program for sickle cell disease. We are conducting a Phase 1 healthy volunteer clinical trial of BEAM-103, an anti-CD117 monoclonal antibody that enables ESCAPE, and expect to complete dosing in the trial in the first half of 2026.

Genetic Diseases

BEAM-302: In Vivo LNP liver-targeting for AATD

BEAM-302 is a liver-targeting LNP formulation of base editing reagents designed to offer a one-time treatment to correct the E342K point mutation (PiZZ genotype) predominantly responsible for the severe form of alpha-1 antitrypsin deficiency, or AATD. AATD is an inherited genetic disorder that can cause early onset emphysema and liver disease. The most severe form of AATD arises when a patient has a point mutation in both copies of the SERPINA1 gene at amino acid 342 position (E342K, also known as the PiZ mutation or the "Z" allele). This point mutation causes Alpha-1 antitrypsin, or AAT, protein to misfold, accumulating inside liver cells rather than being secreted, resulting in very low levels (10%-15%) of circulating AAT. In addition to resulting in lower levels, the PiZ AAT protein variant is also less enzymatically effective compared to wildtype AAT protein. As a consequence, the lung is left unprotected from neutrophil elastase, resulting in progressive, destructive changes in the lung, such as emphysema, which can result in the need for lung transplants. The mutant AAT protein also accumulates in the liver, causing liver inflammation and cirrhosis, which can ultimately cause liver failure or cancer requiring patients to undergo a liver transplant. It is estimated that approximately 100,000 individuals in the United States have two copies of the Z allele. There are currently no curative treatments for patients with AATD.

We are conducting a Phase 1/2 open label, dose exploration and dose expansion clinical trial of BEAM-302 for the treatment of AATD. The trial will evaluate the safety, tolerability, pharmacodynamics, pharmacokinetics and efficacy of BEAM-302. Part A of the trial is designed to evaluate AATD patients with lung disease, and Part B will evaluate AATD patients with mild to moderate liver disease with or without lung disease.

Updated clinical data from the dose-escalation portions of Part A and Part B are expected to be shared in the first quarter of 2026, along with an updated clinical development plan for BEAM-302 in patients with AATD. We expect to finalize dose selection for registrational development based on the totality of data from the BEAM-302 trial. We have reached alignment with the FDA on a potential accelerated approval pathway for BEAM-302 based on AAT biomarkers evaluated over 12 months. To support a future BLA submission, we anticipate enrolling approximately 50 additional patients to be treated with the selected optimal biological dose of BEAM-302 in an expansion of the ongoing Phase 1/2 clinical trial.

BEAM-304: In vivo LNP liver-targeting for PKU

BEAM-304 is a newly announced liver-targeting LNP formulation of base editing reagents designed to correct disease-causing mutations responsible for phenylketonuria, or PKU. PKU is an autosomal recessive disorder caused by mutations in the phenylalanine hydroxylase, or PAH, gene that prevents the body from metabolizing the amino acid phenylalanine, or Phe. Elevated levels of Phe may result in severe neurological and neurocognitive impairments. Patients are generally identified via newborn screening, with the standard of care involving a Phe-restricted diet, as well as medicines that manage Phe levels. Initially, we plan to develop BEAM-304 for the treatment of the two most prevalent variants found in PKU patients in the United States, with ongoing research effort to address the majority of the remaining mutations. In preclinical studies, administration of BEAM-304 resulted in the normalization of Phe levels in mice at therapeutically relevant doses, even when consuming a standard diet. In 2026, we plan to submit a regulatory application for authorization to initiate an open-label, dose-ascending, Phase 1/2 trial of BEAM-304 in PKU patients with the R408W mutation. We believe that learnings from this trial have the potential to provide a predictable path to accelerated development of BEAM-304 for additional mutations, including as a result of novel FDA frameworks for platform medicines.

BEAM-301: In Vivo LNP liver-targeting for GSDIa

BEAM-301 is a liver-targeting LNP formulation of base editing reagents designed to correct the R83C mutation, the most prevalent disease-causing mutation for, and the mutation which results in the most severe form of, glycogen storage disease Ia, or GSDIa. GSDIa is an autosomal recessive disorder caused by mutations in the G6PC gene that disrupts a key enzyme, G6Pase, critical for maintaining glucose homeostasis. Inhibition of G6Pase activity results in low fasting blood glucose levels that can result in seizures and be fatal. Patients with this mutation typically require ongoing corn starch administration, without which they may enter into hypoglycemic shock within one to three hours.

We are conducting a Phase 1/2 clinical trial of BEAM-301 at a select number of sites in the United States. The trial is an open-label, multi-cohort, single-ascending dose evaluation of BEAM-301 for the treatment of GSDIa in patients with the R83C mutation. Key endpoints of the trial include safety and tolerability, time to hypoglycemia during fasting, and changes from baseline in corn starch supplementation. Dosing is complete in the first cohort, and enrollment has been initiated in the second cohort. We expect to report initial data from the trial in 2026.

Financing Agreement and Credit Facility

On February 24, 2026, or the Closing Date, we entered into a financing agreement, or the Financing Agreement, with certain of our subsidiaries as guarantors party thereto, the lenders party thereto, or the Lenders, and Sixth Street Lending Partners, as the administrative agent and collateral agent for the Lenders. The Financing Agreement provides for a senior secured term loan facility of up to $500 million, or the Credit Facility, consisting of (i) an initial draw of $100 million on the Closing Date, (ii) a potential additional $100 million draw upon the acceptance by the FDA of our BLA submission for risto-cel prior to a certain date, or the Delayed Draw A, (iii) a potential additional $100 million draw at the Company's option upon the FDA's approval of the risto-cel BLA prior to a certain date, or the Delayed Draw B, (iv) a potential additional $100 million draw at the Company's option upon achieving a revenue target from sales of risto-cel prior to a certain date and (v) a potential additional $100 million draw subject to agreement among us and the Lenders. The Credit Facility matures on February 24, 2033, or the Maturity Date, and bears interest at an annual rate equal to the 3-month Secured Overnight Financing Rate (SOFR) plus 6.50% (subject to a 1.00% floor) or permits interest on a base rate plus a margin. Certain additional commitment, administrative, undrawn amount and facility fees are also payable in connection with the Credit Facility.

The Credit Facility requires quarterly interest payments, but does not provide for scheduled amortization payments during the term. All principal will be due on the Maturity Date. We will have the right to prepay loans under the Credit Facility at any time. We are required to repay loans under the Credit Facility with proceeds from certain asset sales and licensing transactions, condemnation events and extraordinary receipts, subject, in some cases, to reinvestment rights. Repayments are subject, in some cases, to prepayment premiums.

All obligations under the Financing Agreement will be secured on a first-priority basis, subject to certain exceptions, by security interests in substantially all assets of us and our material subsidiaries, including our intellectual property, and will be guaranteed by our material subsidiaries, subject to certain exceptions.

The Financing Agreement contains customary covenants, including, without limitation, a financial covenant to maintain liquidity of at least $40 million (which shall increase to $80 million upon the draw of the Delayed Draw A and $125 million upon the draw of the Delayed Draw B) if our market capitalization is below $1.75 billion, a covenant to use commercially reasonable efforts to develop and commercialize risto-cel and negative covenants that, subject to certain exceptions, restrict our ability to incur additional indebtedness, grant liens, make investments (including acquisitions), effectuate mergers or consolidations, engage in asset sales and licensing transactions, pay dividends, modify material agreements, pay subordinated indebtedness, and undertake other matters customarily restricted in such agreements. Among other permissions, we are permitted, on terms and conditions set forth in the Financing Agreement, to have outstanding convertible unsecured notes in an amount not to exceed $400 million. We are subject to restrictions on sales and licensing transactions with respect to our core intellectual property, including risto-cel, subject to certain exceptions, including certain transactions related to areas outside the United States.

The Financing Agreement also contains certain events of default after which loans under the Credit Facility may be due and payable immediately, including payment defaults, material inaccuracy of representations and warranties, covenant defaults, bankruptcy and insolvency proceedings, cross-defaults to certain other agreements, judgments against us and our subsidiaries, and change of control.

Manufacturing

Due to the critical importance of high-quality manufacturing and control of production timing and know-how, we have established a 100,000 square foot manufacturing facility in Research Triangle Park, North Carolina intended to support a broad range of clinical programs. The cGMP facility is designed to support manufacturing for our *ex vivo* cell therapy programs in hematology and *in vivo* non-viral delivery programs for liver and liver-mediated diseases, with the capability to scale-up to support potential commercial supply. For our current clinical trials, we are relying primarily on our internal manufacturing capabilities, along with CMOs with relevant manufacturing experience in genetic medicines. We believe this investment will maximize the value of our portfolio and capabilities, the probability of technical success of our programs, and the speed at which we can provide potentially life-long cures to patients.

Financial operations overview

General

We were founded in January 2017 and began operations in July 2017. Since our inception, we have devoted substantially all of our resources to building our base editing platform and advancing development of our portfolio of programs, establishing and protecting our intellectual property, conducting research and development activities, organizing and staffing our company, conducting clinical trials, maintaining and expanding internal manufacturing capabilities, business planning, raising capital and providing general and administrative support for these operations. To date, we have financed our operations primarily through the sales of our redeemable convertible preferred stock, proceeds from offerings of our common stock, payments received under collaboration and license agreements, and our credit facility with Sixth Street Lending Partners.

We are an early-stage company, and our programs are at a preclinical or clinical stage of development. To date, we have not generated any revenue from product sales and do not expect to generate revenue from the sale of products in the near future. Our revenue to date has been primarily derived from license and collaboration agreements with partners. Since inception we have incurred significant operating losses. Our net losses for the years ended December 31, 2025, 2024 and 2023 were $80.0 million, $376.7 million and $132.5 million, respectively. As of December 31, 2025, we had an accumulated deficit of $1.6 billion. We expect to continue to incur significant expenses and increasing operating losses in connection with ongoing development activities related to our internal programs and collaborations as we continue our preclinical and clinical development of product candidates; advance additional product candidates toward clinical development; operate our cGMP facility in North Carolina; further develop our base editing platform; continue to make investments in delivery technology for our base editors; conduct research activities as we seek to discover and develop additional product candidates; maintain, expand, enforce, defend and protect our intellectual property portfolio; and continue to hire research and development, clinical, technical operations and commercial personnel. In addition, we expect to continue to incur the costs associated with operating as a public company.

As a result of these anticipated expenditures, we will need to raise additional capital to support our continuing operations and pursue our growth strategy. Until such time as we can generate significant revenue from product sales, if ever, we expect to finance our operations through a combination of equity offerings, debt financings, including our Credit Facility, collaborations, strategic alliances, and licensing arrangements. We may be unable to raise additional funds or enter into such other agreements when needed on favorable terms or at all. Our inability to raise capital as and when needed would have a negative impact on our financial condition and our ability to pursue our business strategy. We can give no assurance that we will be able to secure such additional sources of capital to support our operations, or, if such capital is available to us, that such additional capital will be sufficient to meet our needs for the short or long term.

Revenue recognition

In April 2019, we entered into a collaboration and license agreement, or the Verve Agreement, with Verve Therapeutics, Inc., or Verve, a company focused on gene editing for cardiovascular disease treatments. In June 2021, we entered into a research collaboration agreement, or the Apellis Agreement, with Apellis Pharmaceuticals, Inc., or Apellis, focused on the use of our base editing technology to discover new treatments for complement system-driven diseases. In December 2021, we entered into a four-year research collaboration agreement, or the Pfizer Agreement, with Pfizer Inc., or Pfizer, focused on *in vivo* base editing programs for three targets for rare genetic diseases of the liver, muscle and central nervous system. In September 2022, we entered into a License and Research Collaboration Agreement, or the Orbital Agreement, with Orbital Therapeutics, Inc., or Orbital, a newly formed entity focused on advancing non-viral delivery and RNA technologies. In October 2023, we entered into a Transfer and Delegation Agreement, or the Lilly Agreement, with Eli Lilly and Company, or Lilly, pursuant to which Lilly acquired certain assets and other rights under the Verve Agreement, including our opt-in rights to co-develop and co-commercialize Verve's base editing programs for cardiovascular disease.

We have not generated any revenue to date from product sales and do not expect to do so in the near future. During the years ended December 31, 2025, 2024, and 2023, we recognized $139.7 million, $63.5 million and $377.7 million respectively, of revenue from our license and collaboration agreements.

For additional information about our revenue recognition policy, see Note 2 and Note 10 of the notes to our audited consolidated financial statements included in this Annual Report on Form 10-K.

Research and development expenses

Research and development expenses consist of costs incurred in performing research and development activities, which include:

- expenses incurred in connection with our clinical trials, including contract research organization costs and costs related to study preparation;
- the cost of manufacturing materials for use in our preclinical studies, our IND enabling studies and clinical trials;
- expenses incurred in connection with investments in delivery technology for our base editors;
- expenses incurred in connection with the discovery and preclinical development of our research programs, including under agreements with third parties, such as consultants, contractors and contract research organizations;
- personnel-related expenses, including salaries, bonuses, benefits and stock-based compensation for employees engaged in research and development functions;
- the cost to obtain licenses to intellectual property, such as those with Harvard University, or Harvard, The Broad Institute, Inc., or Broad Institute, Editas Medicine, Inc., or Editas, and Bio Palette Co., Ltd., or Bio Palette, and related future payments should certain success, development and regulatory milestones be achieved;
- expenses incurred in connection with the building of our base editing platform;
- expenses to acquire in-process research and development with no alternative future use;
- expenses incurred in connection with regulatory filings;
- laboratory supplies and research materials; and
- facilities, depreciation and other expenses which include direct and allocated expenses.

Our external research and development expenses support our various preclinical and clinical programs. Our internal research and development expenses consist of employee-related expenses, facility-related expenses, and other indirect research and development expenses incurred in support of overall research and development. We expense research and development costs as incurred. Advance payments that we make for goods or services to be received in the future for use in research and development activities are recorded as prepaid expenses. The prepaid amounts are expensed as the benefits are consumed.

In the early phases of development, our research and development costs are often devoted to product platform and proof-of-concept preclinical studies that are not necessarily allocable to a specific target.

We expect that our research and development expenses will increase substantially as we advance our programs through their planned preclinical and clinical development.

General and administrative expenses

General and administrative expenses consist primarily of salaries and other related costs, including stock-based compensation, for personnel in our executive, intellectual property, business development, commercial readiness and administrative functions. General

and administrative expenses also include legal fees relating to intellectual property and corporate matters, professional fees for accounting, auditing, tax and consulting services, insurance costs, travel, and direct and allocated facility related expenses and other operating costs.

We anticipate that our general and administrative expenses will increase in the future to support our increased research and development and commercial readiness activities. We also expect to continue to incur costs associated with being a public company and maintaining controls over financial reporting, including costs of accounting, audit, legal, regulatory and tax-related services associated with maintaining compliance with Nasdaq and SEC requirements, director and officer insurance costs, and investor and public relations costs.

Other income and expenses

Other income and expenses consist of the following items:

- *Change in fair value of derivative liabilities* consists primarily of remeasurement gains or losses associated with changes in success payment liabilities associated with our license agreement with Harvard, dated as of June 27, 2017, as amended, or the Harvard License Agreement and the license agreement with The Broad Institute, as amended, dated as of May 9, 2018, or the Broad License Agreement.
- *Change in fair value of non-controlling equity investments* consists of changes in the fair value of our investments in equity securities.
- *Change in fair value of contingent consideration liabilities* consists of remeasurement of the fair value of the milestone payments associated with our contingent consideration liabilities from acquisitions.
- *Interest and other income (expense), net* consists primarily of interest income from our investments in fixed income securities as well as interest expense related to our equipment financings.

Results of operations

For discussion of 2024 results and comparison with 2023 results, refer to Part II, Item 7, "Management's Discussion and Analysis of Financial Condition and Results of Operations" in our Annual Report on Form 10-K for the fiscal year ended December 31, 2024.

Comparison of the years ended December 31, 2025 and 2024

The following table summarizes our results of operations (in thousands):

	Year Ended December 31,		
	2025	2024	Change
License and collaboration revenue	$ 139,743	$ 63,518	$ 76,225
Operating expenses:			
Research and development	409,618	367,561	42,057
General and administrative	113,818	111,525	2,293
Total operating expenses	523,436	479,086	44,350
Loss from operations	(383,693)	(415,568)	31,875
Other income (expense):			
Change in fair value of derivative liabilities	700	2,272	(1,572)
Change in fair value of non-controlling equity investments	3,942	(14,093)	18,035
Change in fair value of contingent consideration liabilities	180	1,592	(1,412)
Gain on sale of equity method investment	255,146	—	255,146
Interest and other income (expense), net	43,733	49,094	(5,361)
Total other income (expense)	303,701	38,865	264,836
Net loss before income taxes	(79,992)	(376,703)	296,711
Provision for income taxes	—	(39)	39
Net loss	$ (79,992)	$ (376,742)	$ 296,750

License and collaboration revenue

License and collaboration revenue was approximately $139.7 million for the year ended December 31, 2025, compared to approximately $63.5 million for the year ended December 31, 2024, an increase of $76.2 million. License and collaboration revenue recorded in 2025 represents revenue recorded under the Pfizer, Apellis and Orbital Agreements. License and collaboration revenue recorded in 2024 represents revenue recorded under the Pfizer, Apellis and Orbital Agreements, as well as $27.0 million of revenue recognized from development and regulatory milestones achieved under our agreements with Lilly and Sana Biotechnology, Inc, or Sana. The increase in revenue is driven primarily by $109.1 million of revenue recognized in the year ended December 31, 2025, due to the completion of our collaboration deal with Pfizer and the net change in activity amongst our other collaboration agreements.

Research and development expenses

Research and development expenses were $409.6 million and $367.6 million for the years ended December 31, 2025 and 2024, respectively. The following table summarizes our research and development expenses for the years ended December 31, 2025 and December 31, 2024, together with the changes in those items in dollars (in thousands):

	Year Ended December 31,		
	2025	2024	Change
External research and development expenses	$ 142,376	$ 114,473	$ 27,903
Employee related expenses	117,366	98,796	18,570
Facility and information technology related expenses	77,146	73,387	3,759
Stock-based compensation expenses	56,123	73,522	(17,399)
In-process research and development expenses	14,507	—	14,507
Other expenses	2,100	7,383	(5,283)
Total research and development expenses	$ 409,618	$ 367,561	$ 42,057

The increase of $42.1 million was primarily due to the following:

- An increase of $27.9 million in external research and development expenses driven by a $29.0 million increase in outsourced services, primarily due to increased clinical and manufacturing activities, slightly offset by a $1.1 million decrease in lab supply expenses due to the advancement of pipeline programs and a shift of programs beyond research activities;
- An increase of $18.6 million of employee related expenses due to the increase in the number of research and development employees between December 31, 2024 and December 31, 2025 and annual compensation increases;
- An increase of $14.5 million due to a one-time in-process research and development charge associated with assets acquired from our acquisition of an early-stage life sciences company during the year ended December 31, 2025 that were determined to have no alternative future use; and
- An increase of $3.8 million of facility and information technology, or IT, related costs, including depreciation and the expense allocated to research and development related to our leased facilities.

The increase was partially offset by the following:

- A decrease of $17.4 million in stock-based compensation driven by additional one-time stock awards granted to employees in 2024; and
- A decrease of $5.3 million of other expenses driven by milestone related expenses recognized in 2024 that were not incurred in 2025.

Research and development expenses are expected to continue to increase as we advance clinical trials for risto-cel, BEAM-302, and BEAM-304, continue our current research programs, initiate new research programs, continue the preclinical and clinical development of our product candidates, conduct any future preclinical studies, and enroll patients in and conduct clinical trials for any of our product candidates.

General and administrative expenses

General and administrative expenses were $113.8 million and $111.5 million for the years ended December 31, 2025 and 2024, respectively. The increase of $2.3 million was primarily due to the following:

- An increase of $8.6 million in personnel related costs due to an increase in the number of general and administrative employees between December 31, 2024 and December 31, 2025, and annual compensation increases; and
- An increase of $3.6 million in legal costs.

The increase was partially offset by the following:

- A decrease of $9.0 million in stock-based compensation driven by additional stock awards granted to employees in 2024; and
- A decrease of $0.9 million in other expenses.

Change in fair value of derivative liabilities

During the year ended December 31, 2025, we recorded $0.7 million of other income related to the change in fair value of derivative liabilities as compared to $2.3 million for the year ended December 31, 2024, driven primarily by a declining interest rate enviornment. There were no success payment obligations paid during the years ended December 31, 2025, 2024 or 2023. The success payment obligations are still outstanding as of December 31, 2025 and will continue to be revalued at each reporting period.

Change in fair value of non-controlling equity investments

During the years ended December 31, 2025 and 2024, we recorded other income of $3.9 million and other expense of $14.1 million, respectively, as a result of changes in the fair value of our investments in corporate equity securities.

Change in contingent consideration liabilities

During the years ended December 31, 2025 and 2024, we recorded $0.2 million and $1.6 million, respectively, of other income related to the change in fair value of the milestone payments related to our contingent consideration liabilities from acquisitions.

Interest and other income (expense), net

Interest and other income (expense), net was $43.7 million and $49.1 million of other income for the years ended December 31, 2025 and December 31, 2024, respectively. The decrease was primarily due to decreases in interest income offset slightly by the growth of our investment portfolio.

Gain on sale from equity method investment

On December 8, 2025, Bristol-Myers Squibb Company, or BMS, completed an acquisition of Orbital, or the Acquisition. At the closing of the Acquisition, we held 75 million shares of Orbital common stock, which were cancelled and converted into $255.1 million in closing cash consideration, plus the right to receive up to approximately $26.3 million in additional cash consideration upon the release, if any, of certain escrows.

Provision for income taxes

We did not record an income tax provision for the year ended December 31, 2025 and recorded an income tax provision of less than $0.1 million for the year ended December 31, 2024.

Liquidity and capital resources

Since our inception in January 2017, we have not generated any revenue from product sales, have generated only limited revenue from our license and collaboration agreements, 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 preclinical and the clinical development of our product candidates.

In February 2024, we filed a universal automatic shelf registration statement on Form S-3 with the SEC, to register for sale an indeterminate amount of our common stock, preferred stock, debt securities, warrants and/or units in one or more offerings, which became effective upon filing with the SEC (File No. 333-277427).

We have entered into an at the market sales agreement, or the Sales Agreement, with Jefferies LLC, or Jefferies, pursuant to which we are entitled to offer and sell, from time to time at prevailing market prices, shares of our common stock having aggregate gross proceeds of up to $1.1 billion. We agreed to pay Jefferies a commission of up to 3.0% of the aggregate gross sale proceeds of any shares sold by Jefferies under the Sales Agreement. There were no shares sold under the Sales Agreement during the year ended December 31, 2025. As of December 31, 2025, we have sold 13,769,001 shares of our common stock under the Sales Agreement at an average price of $62.75 per share for aggregate gross proceeds of $864.0 million, before deducting commissions and offering expenses payable by us.

In March 2025, we closed an underwritten public offering of 16,151,686 shares of common stock at a public offering price of $28.48 per share and pre-funded warrants to purchase 1,404,988 shares of common stock at a purchase price of $28.47 per pre-funded warrant for aggregate net proceeds of $470.5 million, after deducting underwriting discounts, commissions and approximately $0.8 million related to legal, accounting and other fees in connection with the offering. The pre-funded warrants have an exercise price equal to $0.01 per share and are immediately exercisable, subject to certain beneficial ownership restrictions. The pre-funded warrants do not expire.

On December 8, 2025, BMS completed the Acquisition. At the closing of the Acquisition, we held 75 million shares of Orbital common stock, which were cancelled and converted into $255.1 million in closing cash consideration, plus the right to receive up to approximately $26.3 million in additional cash consideration upon the release, if any, of certain escrows.

As of December 31, 2025, we had $1.2 billion in cash, cash equivalents, and marketable securities.

In February 2026, we entered into the Financing Agreement, which provides for the Credit Facility, consisting of an initial draw of $100.0 million on the closing date; up to $300 million available upon the achievement of certain clinical, regulatory and commercial milestones for risto-cel; and an additional $100 million available at our option, subject to mutual agreement between the parties, during the seven-year term of the agreement. The Credit Facility matures on February 24, 2033 and bears interest at an annual rate equal to the 3-month Secured Overnight Financing Rate (SOFR) plus 6.5% (subject to a 1.00% floor). Certain additional commitment, administrative, undrawn amount and facility fees are also payable in connection with the Credit Facility.

We are required to make success payments to Harvard and Broad Institute based on increases in the per share fair market value of our common stock. The amounts due may be settled in cash or shares of our common stock, at our discretion. We may owe Harvard and Broad Institute success payments of up to an additional $90.0 million each.

We have not yet commercialized any of our product candidates, and we do not expect to generate revenue from the sale of our product candidates in the near future. We anticipate that we may need to raise additional capital in order to continue to fund our research and development, including our planned preclinical studies and clinical trials, maintaining and operating our commercial-scale cGMP manufacturing facility, and new product development, as well as to fund our general operations. As necessary, we will seek to raise additional capital through various potential sources, such as equity and debt financings or through corporate collaboration and license agreements. We can give no assurances that we will be able to secure such additional sources of capital to support our operations, or, if such funds are available to us, that such additional financing will be sufficient to meet our needs.

Cash flows

The following table summarizes our sources and uses of cash (in thousands):

	Year Ended December 31,	
	2025	2024
Net cash provided by (used in) operating activities	$ (345,102)	$ (347,246)
Net cash provided by (used in) investing activities	(121,438)	185,007
Net cash provided by (used in) financing activities	478,049	7,736
Net change in cash, cash equivalents and restricted cash	$ 11,509	$ (154,503)

Operating activities

Net cash used in operating activities for the year ended December 31, 2025 was $345.1 million, including our net loss of $80.0 million, decreases in deferred revenue of $135.4 million, operating lease liabilities of $13.5 million and other long-term liabilities of $0.3 million. In addition, noncash items, including the gain on sale of equity method investment of $255.1 million, amortization of investment discounts and premiums of $16.0 million, an increase in the fair value of non-controlling equity investments of $3.9 million, a decrease in the fair value of derivative liabilities of $0.7 million and a change in the fair value of contingent consideration liabilities of $0.2 million, also contributed to net cash used in operating activities.

These uses of cash were partially offset by increases in other liabilities of $7.7 million and accounts payable of $6.0 million, a decrease in prepaid expenses and other current assets of $4.5 million, as well as noncash items, including stock-based compensation expense of $94.2 million, depreciation and amortization expense of $22.3 million, a decrease in operating lease right-of-use, or ROU, assets of $10.4 million and a realized loss of $0.4 million on our sale of marketable securities.

Net cash used in operating activities for the year ended December 31, 2024 was $347.2 million, including our net loss of $376.7 million, decreases in accrued expenses and other liabilities of $57.7 million, deferred revenue of $36.5 million and operating lease liabilities of $13.0 million, and an increase in prepaid expenses and other current assets of $5.4 million. In addition, noncash items, including the amortization of investment discounts and premiums of $22.8 million, decreases in the fair value of derivative liabilities of $2.3 million and a change in the fair value of contingent consideration liabilities of $1.6 million, also contributed to net cash used in operating activities.

These uses of cash were partially offset by increases in accounts payable of $1.8 million and other long-term liabilities of $0.5 million, as well as noncash items, including stock-based compensation expense of $120.7 million, depreciation and amortization expense of $21.9 million, a decrease in the fair value of non-controlling equity investments of $14.1 million and a decrease in operating lease right-of-use, or ROU, assets of $9.7 million.

Investing activities

For the year ended December 31, 2025, cash used in investing activities of $121.4 million was primarily driven by net purchases of marketable securities of $367.3 million, purchases of property and equipment of $15.0 million and the payment of $0.1 million of equity issuance costs associated with our acquisition of an early-stage life sciences company. These uses of cash were partially offset by proceeds from sale of our equity method investment in Orbital totaling $255.1 million and proceeds from sales of marketable securities of $5.7 million.

For the year ended December 31, 2024, cash provided by investing activities of $185.0 million was primarily the result of net maturities of marketable securities partially offset by purchases of marketable securities, in addition to purchases of property and equipment of $8.9 million.

Financing activities

Net cash provided by financing activities for the year ended December 31, 2025 of $478.0 million consisted of $470.5 million of proceeds from the March 2025 issuance of common stock and pre-funded warrants, $4.7 million of proceeds from the exercise of stock options and $2.8 million of proceeds from the issuance of common stock under our Employee Stock Purchase Plan, or ESPP.

Net cash provided by financing activities for the year ended December 31, 2024 of $7.7 million consisted of proceeds from the exercise of stock options of $5.6 million and $2.6 million of proceeds from the issuance of common stock under our ESPP, offset in part by net repayments of equipment financing liabilities of $0.5 million.

Funding requirements

Our operating expenses are expected to continue to increase substantially as we continue to advance our portfolio of programs.

Specifically, our expenses will increase if and as we:

- advance clinical trials of our product candidates;
- continue our research programs and our preclinical development of product candidates from our research programs;
- maintain and operate a commercial-scale cGMP manufacturing facility;
- seek to identify additional research programs and additional product candidates;
- initiate preclinical studies and clinical trials for additional product candidates we identify and develop;
- seek marketing approvals for any of our product candidates that successfully complete clinical trials;
- establish a sales, marketing, and distribution infrastructure to commercialize any medicines for which we may obtain marketing approval;
- maintain, expand, enforce, defend, and protect our intellectual property portfolio and provide reimbursement of third-party expenses related to our patent portfolio;
- further develop our base editing platform;
- continue to hire additional personnel including research and development, clinical and commercial personnel;
- add operational, financial, and management information systems and personnel, including personnel to support our product development; and
- acquire or in-license products, intellectual property, medicines and technologies.

We expect that our cash, cash equivalents, and marketable securities at December 31, 2025 will enable us to fund our current and planned operating expenses and capital expenditures for at least the next 12 months from the date of issuance of our accompanying consolidated financial statements. We have based these estimates on assumptions that may prove to be imprecise, and we may exhaust our available capital resources sooner that we currently expect. Because of the numerous risks and uncertainties associated with the development our programs, we are unable to estimate the amounts of increased capital outlays and operating expenses associated with completing the research and development of our product candidates.

Our future funding requirements will depend on many factors including:

- the cost of continuing to build our base editing platform;
- the costs of acquiring licenses for the delivery modalities that will be used with our product candidates;
- the scope, progress, results, and costs of discovery, preclinical development, laboratory testing, manufacturing and clinical trials for the product candidates we may develop;

- the costs of operating and expanding our manufacturing capacity;
- the costs of preparing, filing, and prosecuting patent applications, maintaining and enforcing our intellectual property and proprietary rights, and defending intellectual property-related claims;
- the costs, timing, and outcome of regulatory review of the product candidates we develop;
- the costs of future activities, including product sales, medical affairs, marketing, manufacturing, distribution, coverage and reimbursement for any product candidates for which we receive regulatory approval;
- the success of our license agreements and our collaborations;
- our ability to establish and maintain additional collaborations on favorable terms, if at all;
- the achievement of milestones or occurrence of other developments that trigger payments under any collaboration agreements we are a party to or may become a party to;
- the payment of success liabilities to Harvard and Broad Institute pursuant to the respective terms of the Harvard License Agreement and the Broad License Agreement, should we choose to pay in cash;
- the extent to which we acquire or in-license products, intellectual property, and technologies; and
- the impact on our business of macro-economic conditions, as well as the prevailing level of macro-economic, business, and operational uncertainty, including as a result of geopolitical events, the imposition of new or revised global trade tariffs or other global or regional events.

A change in the outcome of any of these or other variables with respect to the development of any of our product candidates could significantly change the costs and timing associated with the development of that product candidate. Further, our operating plans may change in the future, and we may need additional funds to meet operational needs and capital requirements associated with such operating plans.

Until such time, if ever, as we can generate substantial product revenues, we expect to finance our cash needs through a combination of equity offerings, debt financings, collaborations, strategic alliances, and licensing arrangements. Other than the Credit Facility, we do not have any committed external source of capital. We have historically relied on equity issuances and collaboration revenue to fund our capital needs. The Financing Agreement includes covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures, or declaring dividends, and future debt financings, if available, may include similar restrictions.

If we raise capital through additional collaborations, strategic alliances, or licensing arrangements with third parties, we may have to relinquish valuable rights to our technologies, future revenue streams, research programs, or product candidates, or we may have to grant licenses on terms that may not be favorable to us. If we are unable to raise additional capital through equity or debt financings when needed, we may be required to delay, limit, reduce, or terminate our product development or, if approved, future commercialization efforts or grant rights to develop and market product candidates that we would otherwise prefer to develop and market ourselves. We can give no assurance that we will be able to secure such additional sources of funds to support our operations, or, if such funds are available to us, that such additional funding will be sufficient to meet our needs.

Contractual obligations

We lease certain assets under noncancelable operating leases, which expire through 2037. The leases relate primarily to office space, laboratory and manufacturing space, and equipment. Aggregate future minimum commitments under these office and laboratory leases and equipment leases are $210.1 million as of December 31, 2025, excluding any related common area maintenance charges or real estate taxes.

Success payment obligations under the Harvard License Agreement and Broad License Agreement are still outstanding as of December 31, 2025. We may owe Harvard and Broad Institute success payments of up to an additional $90.0 million each, which is payable at our election in cash or shares of our common stock.

We are potentially obligated to pay certain milestone and success fees, non-royalty sublicense income fees, royalty fees, licensing maintenance fees, and reimbursement of patent maintenance costs under agreements to license intellectual property. These agreements include potential milestone payments that are dependent upon the development of products using the intellectual property licensed under the agreements and contingent upon the achievement of development or regulatory approval milestones, as well as commercial and success payment milestones. These amounts are contingent upon the occurrence of future events and the timing and likelihood of such potential obligations are not known with certainty.

In addition, we may owe up to an additional $89.0 million in development, clinical and commercial milestones to former stockholders of an early-stage life sciences company acquired during the year ended December 31, 2025. Milestone payments are payable at our sole discretion in cash or in shares of our common stock (valued using a volume-weighted average price). These payments are contingent upon the occurrence of future events and the timing and likelihood of such potential obligations are not known with certainty.

Additionally, we enter into contracts in the normal course of business with CROs, CMOs and other vendors to assist in the performance of our research and development activities and other services and products for operating purposes. These contracts generally provide for termination on notice, and therefore are cancelable contracts.

Critical accounting policies and significant judgments and estimates

Our management's discussion and analysis of our financial condition and results of operations is based on our consolidated financial statements, which we have prepared in accordance with U.S. generally accepted accounting principles. The preparation of these financial statements requires us to make estimates, judgments and assumptions that affect the reported amounts of assets, liabilities, and expenses and the disclosure of contingent assets and 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 consolidated financial statements in this Annual Report on Form 10-K, we believe that the following accounting policies are those most critical to the judgments and estimates used in the preparation of our financial statements.

Revenue recognition

At inception, we determine whether contracts are within the scope of ASC 606, *Revenue from Contracts with Customers*, or ASC 606, or other topics. For contracts that are determined to be within the scope of ASC 606, revenue is recognized when a customer obtains control of promised goods or services. The amount of revenue recognized reflects the consideration to which we expect to be entitled to receive in exchange for these goods and services. To achieve this core principle, we apply the following five steps (i) identify the contract with the customer; (ii) identify the performance obligations in the contract; (iii) determine the transaction price; (iv) allocate the transaction price to the performance obligations in the contract; and (v) recognize revenue when the performance obligation is satisfied. We only apply the five-step model to contracts when we determine that collection of substantially all consideration for goods and services that are transferred is probable based on the customer's intent and ability to pay the promised consideration.

Performance obligations promised in a contract are identified based on the goods and services that will be transferred to the customer that are both capable of being distinct and are distinct in the context of the contract. To the extent a contract includes multiple promised goods and services, we apply judgment to determine whether promised goods and services are both capable of being distinct and are distinct in the context of the contract. If these criteria are not met, the promised goods and services are accounted for as a combined performance obligation.

The transaction price is determined based on the consideration to which we will be entitled in exchange for transferring goods and services to the customer. To the extent the transaction price includes variable consideration, we estimate the amount of variable consideration that should be included in the transaction price utilizing either the expected value method or the most likely amount method, depending on the nature of the variable consideration. Variable consideration is included in the transaction price if, in management's judgment, it is probable that a significant future reversal of cumulative revenue under the contract will not occur. Any estimates, including the effect of the constraint on variable consideration, are evaluated at each reporting period for any changes. Determining the transaction price requires significant judgment and is discussed in further detail for each of our license and collaboration agreements in Note 10 to our consolidated financial statements in this Annual Report on Form 10-K.

If the contract contains a single performance obligation, the entire transaction price is allocated to the single performance obligation. Contracts that contain multiple performance obligations require an allocation of the transaction price to each performance obligation on a relative standalone selling price basis unless the transaction price is variable and meets the criteria to be allocated entirely to a performance obligation or to a distinct service that forms part of a single performance obligation. The consideration to be received is allocated among the separate performance obligations based on relative standalone selling prices. Determining the standalone selling price requires significant judgment and is discussed in further detail for each of our license and collaboration agreements in Note 10.

We satisfy performance obligations either over time or at a point in time. Revenue is recognized over time if either (i) the customer simultaneously receives and consumes the benefits provided by the entity's performance, (ii) the entity's performance creates or enhances an asset that the customer controls as the asset is created or enhanced, or (iii) the entity's performance does not create an asset with an alternative use to the entity and the entity has an enforceable right to payment for performance completed to date. If the entity does not satisfy a performance obligation over time, the related performance obligation is satisfied at a point in time by transferring the control of a promised good or service to a customer.

The timing of when services are performed and the occurrence of external costs associated with the programs under the collaboration agreements could impact how revenue is recognized in a certain period.

Licenses of intellectual property, or IP: If the license to our IP is determined to be distinct from the other performance obligations identified in the arrangement, we recognize revenues from consideration allocated to the license when the license is transferred to the customer and the customer can use and benefit from the licenses. For licenses that are combined with other promises, we utilize judgment to assess the nature of the combined performance obligation to determine whether the combined performance obligation is satisfied over time or at a point in time and, if over time, the appropriate method of measuring progress for purposes of recognizing revenue. We evaluate the measure of progress each reporting period and, if necessary, adjust the measure of performance and related revenue recognition. We generally recognize revenue using the cost incurred to date as compared to the total estimated cost. Changes in estimates of total internal and external costs expected to be incurred and timing of when those costs are expected to be incurred are recognized in the period of change as a cumulative catch-up adjustment. If estimates of the total estimated cost change, or if contract amendments change the scope of the performance obligations, the impacts could be material. Determining the revenue recognition of IP licenses requires significant judgment and is discussed in further detail for each of our license and collaboration agreements in Notes 9 and 10.

Milestone payments: At the inception of each arrangement that includes development or regulatory milestone payments, we evaluate the probability of reaching the milestones and estimate the amount to be included in the transaction price using the most likely amount method. If it is probable that a significant revenue reversal would not occur in the future, the associated milestone value is included in the transaction price. Milestone payments that are not within our control or the licensee's, such as regulatory approvals, are not considered probable of being achieved until those approvals are received and therefore revenue recognized is constrained as management is unable to assert that a reversal of revenue would not be possible. The transaction price is then allocated to each performance obligation on a relative standalone selling price basis, for which we recognize revenue as or when the performance obligations under the contract are satisfied. At the end of each subsequent reporting period, we re-evaluate the probability of achievement of such development milestones and any related constraint, and if necessary, adjusts its estimate of the overall transaction price. Any such adjustments are recorded on a cumulative catch-up basis, which would affect revenues and earnings in the period of adjustment. To date, we have not recognized any milestone revenue resulting from any of our agreements.

Commercial Milestone Payments and Royalties: For arrangements that include sales-based royalties, including milestone payments based on levels of sales, if the license is deemed to be the predominant item to which the royalties relate, we recognize revenue at the later of (i) when the related sales occur, or (ii) when the performance obligation to which some or all of the royalty has been allocated has been satisfied (or partially satisfied). To date, we have not recognized any royalty revenue resulting from any of our agreements.

When no performance obligations are required of us, or following the completion of the performance obligation period, amounts are recognized as revenue upon transfer of control of the goods or services to the customer. Generally, all amounts received or due other than sales-based milestones and royalties are classified as license and collaboration revenue. Sales-based milestones and royalties will be recognized as royalty revenue at the later of when the related sales occur or when the performance obligation to which some or all of the royalty has been allocated has been satisfied (or partially satisfied).

Deferred revenue expected to be recognized within the next twelve months is classified as a current liability.

Fair value measurements – Success payments

We are required to make success payments to Harvard and Broad Institute based on increases in the per share fair market value of our common stock. Any amounts due may be settled in cash or shares of our common stock, at our discretion. The success payments are accounted for as a derivative under Accounting Standards Codification 815, *Derivatives and Hedging* and were initially recorded at fair value with a corresponding charge to research and development expense. The liabilities are marked to market at each balance sheet date with all changes in value recognized in interest and other income (expense) in the consolidated statement of operations and other comprehensive loss. We will continue to adjust the liability for changes in fair value until the earlier of the achievement or expiration of the success payment obligation. To determine the estimated fair value of the success payments, we used a Monte Carlo simulation model, which models the value of the liability based on several key variables, including probability of event occurrence, timing of event occurrence, as well as the price per share at the time of success payment. A significant change in our stock price or volatility could have a significant impact on the value of the liability.

Accrued and prepaid research and development costs

As part of the process of preparing our financial statements, we are required to estimate our accrued expenses and prepaid research and development costs. This process involves reviewing open contracts and purchase orders, communicating with our personnel to identify services that have been performed on our behalf and 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 the actual cost. The majority of our service providers invoice us monthly in arrears for services performed or when contractual milestones are met. We make estimates of our accrued expenses as of each balance sheet date in our financial statements based on facts and circumstances known to us at that time. Examples of estimated accrued research and development expenses include fees paid to vendors in connection with preclinical development activities and vendors related to development, manufacturing and distribution of product candidate materials.

We base our expenses related to preclinical studies on our estimates of the services received and efforts expended pursuant to contracts with multiple vendors that conduct and manage preclinical studies on our behalf. 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 and adjust accordingly.

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

We are exposed to market risk related to changes in interest rates. As of December 31, 2025, we had cash, cash equivalents and marketable securities of $1.2 billion, which consisted of cash, money market funds, commercial paper, corporate notes and corporate equity securities. Our primary exposure to market risk is interest rate sensitivity, which is affected by changes in the general level of U.S. interest rates, particularly because our investments are in short-term marketable securities. Due to the short-term duration of our investment portfolio and the low risk profile of our investments, we believe an immediate 10% change in interest rates would not have a material effect on the fair market value of our investment portfolio. We have the ability to hold our investments until maturity, and therefore, we would not expect our operating results or cash flows to be affected to any significant degree by the effect of a change in market interest rates on our investment portfolio.

We are not currently exposed to significant market risk related to changes in foreign currency exchange rates; however, we do contract with vendors that are located outside of the United States and may be subject to fluctuations in foreign currency rates. We may enter into additional contracts with vendors located outside of the United States in the future, which may increase our foreign currency exchange risk.

Inflation generally affects us by increasing our cost of labor and research, manufacturing and development costs. We believe that inflation has not had a material effect on our financial statements included elsewhere in this Annual Report on Form 10-K. However, our operations may be adversely affected by inflation in the future.

Item 8. Financial Statements and Supplementary Data.

The financial statements required to be filed pursuant to this Item 8 are appended to this Annual Report on Form 10-K. An index of those financial statements is found in Item 15, *Exhibits and Financial Statement Schedules*, of this Annual Report on Form 10-K.

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 our principal financial officer, evaluated, as of December 31, 2025, the effectiveness of our disclosure controls and procedures as defined in Rules 13a-15(e) and 15d-15(e) under the Exchange Act. The term “disclosure controls and procedures,” as defined in Rules 13a-15(e) and 15d-15(e) under the Exchange Act, means controls and other procedures of a company that are designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is recorded, processed, summarized and reported, within the time periods specified in the SEC’s rules and forms. Disclosure controls and procedures include, without limitation, controls and procedures designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is accumulated and communicated to the company’s management, including its principal executive and principal financial officers, or persons performing similar functions, as appropriate to allow timely decisions regarding required disclosure. Management recognizes that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving their objectives and management necessarily applies its judgment in evaluating the cost-benefit relationship of possible controls and procedures.

Based on that evaluation of our disclosure controls and procedures as of December 31, 2025, our principal executive officer and principal financial officer concluded that our disclosure controls and procedures as of such date were effective at the 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. Internal control over financial reporting is defined in Rules 13a-15(f) and 15d-15(f) promulgated under the Exchange Act as a process designed by, or under the supervision of, our principal executive and principal financial officers, or persons performing similar functions, and effected by our board of directors, management and other personnel, 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 and includes policies and procedures that:

- pertain to the maintenance of records that in reasonable detail accurately and fairly reflect the transactions and dispositions of our assets;
- provide reasonable assurance that transactions are recorded as necessary to permit preparation of financial statements in accordance with generally accepted accounting principles, and that our receipts and expenditures are being made only in accordance with authorizations of our management and directors; and
- provide reasonable assurance regarding prevention or timely detection of unauthorized acquisition, use or disposition of our assets that could have a material effect on the consolidated financial statements.

Because of its inherent limitations, internal control over financial reporting may not prevent or detect misstatements. Projections of any evaluation of effectiveness to future periods are subject to the risk that controls may become inadequate because of changes in conditions, or that the degree of compliance with the policies or procedures may deteriorate. We continue to review our internal control over financial reporting and may from time to time make changes aimed at enhancing their effectiveness and to ensure that our systems evolve with our business.

Under the supervision and with the participation of our management, including our Chief Executive Officer and Chief Financial Officer, we conducted an evaluation of the effectiveness of our internal control over financial reporting based on the framework in "Internal Control — Integrated Framework (2013)" issued by the Committee of Sponsoring Organizations of the Treadway Commission. Based on this assessment, our senior management has concluded that the internal control over financial reporting was effective as of December 31, 2025.

Our independent registered public accounting firm, Deloitte & Touche LLP, issued an attestation report on our internal control over financial reporting. See below.

Changes in Internal Control over Financial Reporting

We continuously seek to improve the efficiency and effectiveness of our internal controls. This results in refinements to processes throughout our company. There were no changes in our internal control over financial reporting (as defined in Rules 13a-15(f) and 15d-15(f) under the Exchange Act) during the year ended December 31, 2025 that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

REPORT OF INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM

To the stockholders and the Board of Directors of Beam Therapeutics Inc.

Opinion on Internal Control over Financial Reporting

We have audited the internal control over financial reporting of Beam Therapeutics Inc. and subsidiaries (the "Company") as of December 31, 2025, based on criteria established in *Internal Control — Integrated Framework (2013)* issued by the Committee of Sponsoring Organizations of the Treadway Commission (COSO). In our opinion, the Company maintained, in all material respects, effective internal control over financial reporting as of December 31, 2025, based on criteria established in *Internal Control — Integrated Framework (2013)* issued by COSO.

We have also audited, in accordance with the standards of the Public Company Accounting Oversight Board (United States) (PCAOB), the consolidated financial statements as of and for the year ended December 31, 2025, of the Company and our report dated February 24, 2026, expressed an unqualified opinion on those financial statements.

Basis for Opinion

The Company's management is responsible for maintaining effective internal control over financial reporting and for its assessment of the effectiveness of internal control over financial reporting, included in the accompanying Management's Report on Internal Control over Financial Reporting. Our responsibility is to express an opinion on the Company's internal control over financial reporting based on our audit. 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 audit in accordance with the standards of the PCAOB. Those standards require that we plan and perform the audit to obtain reasonable assurance about whether effective internal control over financial reporting was maintained in all material respects. Our audit included obtaining an understanding of internal control over financial reporting, assessing the risk that a material weakness exists, testing and evaluating the design and operating effectiveness of internal control based on the assessed risk, and performing such other procedures as we considered necessary in the circumstances. We believe that our audit provides a reasonable basis for our opinion.

Definition and Limitations of Internal Control over Financial Reporting

A company's internal control over financial reporting is a process designed to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles. A company's internal control over financial reporting includes those policies and procedures that (1) pertain to the maintenance of records that, in reasonable detail, accurately and fairly reflect the transactions and dispositions of the assets of the company; (2) provide reasonable assurance that transactions are recorded as necessary to permit preparation of financial statements in accordance with generally accepted accounting principles, and that receipts and expenditures of the company are being made only in accordance with authorizations of management and directors of the company; and (3) provide reasonable assurance regarding prevention or timely detection of unauthorized acquisition, use, or disposition of the company's assets that could have a material effect on the financial statements.

Because of its inherent limitations, internal control over financial reporting may not prevent or detect misstatements. Also, projections of any evaluation of effectiveness to future periods are subject to the risk that controls may become inadequate because of changes in conditions, or that the degree of compliance with the policies or procedures may deteriorate.

/s/ Deloitte & Touche LLP

Boston, Massachusetts
February 24, 2026

Item 9B. Other Information.

Director and Officer Trading Arrangements

The following table describes for the quarterly period ended December 31, 2025 each trading arrangement for the sale or purchase of Company securities adopted or terminated by our directors and officers that is either (1) a contract, instruction or written plan intended to satisfy the affirmative defense conditions of Rule 10b5-1(c), or a "Rule 10b5-1 trading arrangement," or (2) a "non-Rule 10b5-1 trading arrangement" (as defined in Item 408(c) of Regulation S-K):

Name ***(Title)***	**Action Taken** ***(Date of Action)***	**Type of Trading Arrangement**	**Nature of Trading Arrangement**	**Duration of Trading Arrangement**	**Aggregate Number of Securities**
Sravan K. Emany *(Chief Financial Officer)*	Adoption *(August 11, 2025)*[(1)]	Durable Rule 10b5-1 trading arrangement for sell-to-cover transactions relating to all RSU equity awards that have been or may be granted	Sale	Until final settlement of any covered RSU	Indeterminable[(2)]

(1) Plan was adopted in the quarterly period ended September 30, 2025.

(2) The number of shares subject to covered restricted stock units, or RSUs, that will be sold to satisfy applicable tax withholding obligations upon vesting is unknown as the number will vary based on the extent to which vesting conditions are satisfied, the market price of the Company's common stock at the time of settlement and the potential future grant of additional RSUs subject to this arrangement. This trading arrangement, which applies to RSUs whether vesting is based on the passage of time and/or the achievement of performance goals, provides for the automatic sale of shares that would otherwise be issuable on each settlement date of a covered RSU in an amount sufficient to satisfy the applicable withholding obligation, with the proceeds of the sale delivered to the Company in satisfaction of the applicable withholding obligation.

Item 9C. Disclosure Regarding Foreign Jurisdictions That Prevent Inspections.

None.

PART III

Item 10. Directors, Executive Officers and Corporate Governance.

The information required by this Item 10 will be included in the sections captioned “Management and Corporate Governance” and “Executive Officers” in our definitive proxy statement to be filed with the Securities and Exchange Commission, or SEC, with respect to our 2026 Annual Meeting of Stockholders and is incorporated herein by reference.

We have adopted a written code of business conduct and ethics, or Code, that applies to all of our directors, officers and employees, including our principal executive officer, principal financial officer, principal accounting officer or controller, or persons performing similar functions. A current copy of the Code is available on the investor section of our website at investors.beamtx.com. We intend to disclose on our website any amendments to, or waivers from, our Code that are required to be disclosed pursuant to SEC rules.

Item 11. Executive Compensation.

The information required by this Item 11 will be included in the section captioned “Executive Compensation" and "Director Compensation” in our definitive proxy statement to be filed with the SEC with respect to our 2026 Annual Meeting of Stockholders 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 12 will be included in the sections captioned “Security Ownership of Certain Beneficial Owners and Management” and “Securities Authorized for Issuance under Equity Compensation Plans” in our definitive proxy statement to be filed with the SEC with respect to our 2026 Annual Meeting of Stockholders and is incorporated herein by reference.

Item 13. Certain Relationships and Related Transactions, and Director Independence.

The information required by this Item 13 will be included in the sections captioned “Certain Relationship and Related Person Transactions” and “Director Independence” in our definitive proxy statement to be filed with the SEC with respect to our 2026 Annual Meeting of Stockholders and is incorporated herein by reference.

Item 14. Principal Accounting Fees and Services.

The information required by this Item 14 will be included in the sections captioned “Principal Accountant Fees and Services” and “Audit Committee Pre-Approval Policy and Procedures” in our definitive proxy statement to be filed with the SEC with respect to our 2026 Annual Meeting of Stockholders and is incorporated herein by reference.

PART IV

Item 15. Exhibits, Financial Statement Schedules.

1. Financial Statements

For a list of the financial statements included herein, see *Index to the Consolidated Financial Statements* on page F-1 of this Annual Report on Form 10-K, incorporated into this Item by reference.

2. Financial Statement Schedules

Financial statement schedules have been omitted because they are either not required or not applicable or the information is included in the consolidated financial statements or the notes thereto.

3. Exhibits

Exhibit Number	Description of Exhibit	Form	File Number	Date of Filing	Exhibit Number	Filed Herewith
3.1	Fourth Amended Certificate of Incorporation of Beam Therapeutics Inc.	8-K	001-39208	02/11/2020	3.1	
3.2	Second Amended and Restated By-laws of Beam Therapeutics Inc.	10-K	001-39208	2/28/2023	3.2	
4.1	Specimen stock certificate evidencing shares of common stock	S-1	333-233985	09/27/2019	4.1	
4.2	Description of Registered Securities	S-3	333-254946	4/01/2021	4.11	
4.3	Form of Pre-Funded Warrant to Purchase Common Stock	8-K	001-39208	03/10/25	4.1	
10.1	Indenture of Lease, between Massachusetts Institute of Technology and Beam Therapeutics Inc., dated April 24, 2019	S-1	333-233985	09/27/2019	10.2	
10.2	Sales Agreement, dated April 1, 2021, by and between Beam Therapeutics Inc. and Jefferies LLC.	8-K	001-39208	04/01/2021	1.1	
10.3#	License Agreement, between the President and Fellows of Harvard College and Beam Therapeutics Inc., dated June 27, 2017	S-1	333-233985	09/27/2019	10.4	
10.4#	Amendment No. 1 to License Agreement, between the President and Fellows of Harvard College and Beam Therapeutics Inc., dated December 12, 2017	10-K	001-39208	03/15/2021	10.5	
10.5#	Amendment No. 2 to License Agreement, between the President and Fellows of Harvard College and Beam Therapeutics Inc., dated March 27, 2020	10-K	001-39208	03/15/21	10.6	
10.6#	License Agreement, between Editas Medicine, Inc. and Beam Therapeutics Inc., dated May 9, 2018	S-1	333-233985	09/27/2019	10.6	
10.7#	Letter Agreement, between Beam Therapeutics Inc., The Broad Institute, Inc., the President and Fellows of Harvard College, and Editas Medicine, Inc., dated September 26, 2018	10-K	001-39208	03/15/2021	10.10	
10.8	Letter Agreement, between the President and Fellows of Harvard College, The Broad Institute, Inc., and Beam Therapeutics Inc., dated January 7, 2021.	10-K	001-39208	03/15/22021	10.11	
10.9#	License Agreement, between Bio Palette Co., Ltd. and Beam Therapeutics Inc., dated March 27, 2019	S-1	333-233985	09/27/2019	10.7	
10.10+	Beam Therapeutics Inc. 2017 Stock Option and Grant Plan	S-1/A	333-233985	01/27/2020	10.8	
10.11+	Form of Incentive Stock Option Grant Notice under the Beam Therapeutics Inc. 2017 Stock Option and Grant Plan	S-1	333-233985	09/27/2019	10.10	
10.12+	Form of Non-Qualified Stock Option Grant Notice under the Beam Therapeutics Inc. 2017 Stock Option and Grant Plan	S-1	333-233985	09/27/2019	10.11	

10.13+	Form of Indemnification Agreement between Beam Therapeutics Inc. and its directors and officers	S-1	333-233985	09/27/2019	10.12
10.14+	Amended and Restated Letter Agreement between Beam Therapeutics Inc. and John Evans, dated June 9, 2021	10-Q	333-233985	08/10/2021	10.1
10.15+	Amended and Restated Employment Agreement between Beam Therapeutics Inc. and Giuseppe Ciaramella, dated January 24, 2020	S-1/A	333-233985	01/27/2020	10.14
10.16+	Letter Agreement between Beam Therapeutics Inc. and Sravan K. Emany, dated December 2, 2024	10-K	001-39208	02/25/2025	10.18
10.17+	Beam Therapeutics Inc. 2019 Equity Incentive Plan	S-1/A	333-233985	01/27/2020	10.16
10.18+	Form of Incentive Stock Option Agreement under the Beam Therapeutics Inc. 2019 Equity Incentive Plan	S-1/A	333-233985	01/27/2020	10.17
10.19+	Form of Non-Statutory Stock Option Agreement under the Beam Therapeutics Inc. 2019 Equity Incentive Plan	S-1/A	333-233985	01/27/2020	10.18
10.20+	Form of Non-Statutory Stock Option Agreement (Non-Employee Directors) under the Beam Therapeutics Inc. 2019 Equity Incentive Plan	S-1/A	333-233985	01/27/2020	10.19
10.21+	Form of Restricted Stock Unit Award Agreement under the Beam Therapeutics Inc. 2019 Equity Incentive Plan	10-K	001-39208	3/15/2021	10.25
10.22+	Form of Restricted Stock Award Agreement under the Beam Therapeutics Inc. 2019 Equity Incentive Plan	10-K	001-39208	03/15/2021	10.26
10.23+	Amended and Restated Beam Therapeutics Inc. 2019 Employee Stock Purchase Plan	10-K	001-39208	2/28/2022	10.27
10.24+	Beam Therapeutics Inc. 2019 Cash Incentive Plan	S-1/A	333-233985	01/27/2020	10.21
10.25+	Amended and Restated Beam Therapeutics Inc. Non-Employee Director Compensation Policy, dated June 2, 2025	10-Q	001-39208	08/05/2025	10.1
10.26	Lease Agreement between Beam Therapeutics Inc. and ARE-NC Region No. 14, LLC	10-Q	001-39208	08/12/2020	10.1
10.27	Amendment No. 1 to Indenture of Lease, between Massachusetts Institute of Technology and Beam Therapeutics Inc., dated April 14, 2020	10-K	001-39208	02/28/2022	10.31
10.28	Amendment No. 2 to Indenture of Lease, between Massachusetts Institute of Technology and Beam Therapeutics Inc., dated November 17, 2020	10-K	001-39208	02/28/2022	10.32
10.29	Amendment No. 3 to Indenture of Lease, between Massachusetts Institute of Technology and Beam Therapeutics Inc., dated August 24, 2021	10-K	001-39208	02/28/2022	10.33
10.30	Amendment No. 1 to Sales Agreement, dated July 7, 2021, by and between Beam Therapeutics Inc. and Jefferies LLC	8-K	001-39208	07/07/2021	1.1
10.31	Amendment No. 4 to Indenture of Lease, between Massachusetts Institute of Technology and Beam Therapeutics Inc., dated December 7, 2022	10-K	001-39208	02/28/2023	10.35
10.32+	Letter Agreement between Beam Therapeutics Inc. and Christine Bellon, dated January 24, 2020	10-Q	001-39208	5/10/2023	10.1
10.33+	Letter Agreement between Beam Therapeutics Inc. and Amy Simon, dated January 29, 2021	10-Q	001-39208	5/10/2023	10.2

10.34	Amendment No. 2 to Sales Agreement, dated May 10, 2023, by and between Beam Therapeutics Inc. and Jefferies LLC	8-K	001-39208	5/10/2023	1.1	
10.35	First Amendment to Lease, between Beam Therapeutics Inc. and ARE-NC Region No. 14, LLC, dated June 23, 2022	10-K	001-39208	02/27/2024	10.39	
10.36	Second Amendment to Lease, between Beam Therapeutics Inc. and ARE-NC Region No. 14, LLC, dated March 31, 2023	10-K	001-39208	02/27/2024	10.40	
10.37	Third Amendment to Lease, between Beam Therapeutics Inc. and ARE-NC Region No.14, LLC, dated January 1, 2024	10-K	001-39208	02/27/2024	10.41	
10.38+	Form of Addendum to Letter Agreement between Beam Therapeutics Inc. and each of John Evans, Giuseppe Ciaramella, Christine Bellon and Amy Simon	10-Q	001-39208	05/07/2024	10.2	
10.39	Amendment to License Agreement between The Broad Institute, Inc. and Beam Therapeutics Inc., dated May 31, 2024	10-Q	001-39208	05/07/2024	10.3	
10.40#	Amendment No. 4 to License Agreement, between the President and Fellows of Harvard College and Beam Therapeutics Inc., dated December 1, 2024	10-K	001-39208	02/25/2025	10.42	
10.41#	Standby License Agreement, between Beam Therapeutics Inc., Kobe University and Bio Palette Co., Ltd., dated February 9, 2026	8-K	001-39208	02/12/2026	10.1	
19.1	Insider Trading Policy	10-K	001-39208	02/25/2025	19.1	
21.1	List of Subsidiaries of Beam Therapeutics Inc.	10-K	001-39208	2/28/2022	21.1	
23.1	Consent of Deloitte & Touche LLP					X
31.1	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					X
31.2	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					X
32.1*	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					X
32.2*	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					X
97.1	Clawback Policy	10-K	001-39208	02/27/2024	97.1	
101.INS	Inline XBRL Instance Document - the instance document does not appear in the Interactive Data File as its XBRL tags are embedded within the Inline XBRL document					X
101.SCH	Inline XBRL Taxonomy Extension Schema with Embedded Linkbase Document					X
104	Cover Page Interactive Data File (embedded within the Inline XBRL document)					X

Portions of this exhibit have been omitted because the Registrant has determined they are not material and are they type that the Registrant treats as private or confidential.

+ Indicates management contract or compensatory plan.

* This certification will not be deemed "filed" for purposes of Section 18 of the Securities Exchange Act of 1934, as amended, or the Exchange Act, or otherwise subject to the liability of that section. Such certification will not be deemed to be incorporated by reference into any filing under the Securities Act of 1933, as amended, or the Exchange Act, except to the extent specifically incorporated by reference into such filing.

Item 16. Form 10-K Summary

None.

SIGNATURES

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

BEAM THERAPEUTICS INC.

Date: February 24, 2026

By: /s/ John Evans
John Evans
Chief Executive Officer

Pursuant to the requirements of the Securities Exchange Act of 1934, as amended, this Report has been signed below by the following persons on behalf of the Registrant in the capacities and on the dates indicated.

Name	Title	Date
/s/ John Evans **John Evans**	Chief Executive Officer and Director (Principal Executive Officer)	February 24, 2026
/s/ Sravan Emany **Sravan Emany**	Chief Financial Officer (Principal Financial Officer)	February 24, 2026
/s/ Bethany Cavanagh **Bethany Cavanagh**	Senior Vice President, Finance, and Treasurer (Principal Accounting Officer)	February 24, 2026
/s/ Graham Cooper **Graham Cooper**	Director	February 24, 2026
/s/ Mark Fishman **Mark Fishman, M.D.**	Director	February 24, 2026
/s/ Chirfi Guindo **Chirfi Guindo**	Director	February 24, 2026
/s/ Carole Ho **Carole Ho, M.D.**	Director	February 24, 2026
/s/ John Maraganore **John Maraganore, Ph.D.**	Director	February 24, 2026
/s/ Christi Shaw **Christi Shaw**	Director	February 24, 2026
/s/ Kathleen Walsh **Kathleen Walsh**	Director	February 24, 2026

INDEX TO CONSOLIDATED FINANCIAL STATEMENTS

REPORT OF INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM

To the stockholders and the Board of Directors of Beam Therapeutics Inc.

Opinion on the Financial Statements

We have audited the accompanying consolidated balance sheets of Beam Therapeutics Inc. and subsidiaries (the "Company") as of December 31, 2025 and 2024, the related consolidated statements of operations and other comprehensive loss, stockholders' equity, and cash flows, for each of the three years in the period ended December 31, 2025, and the related notes (collectively referred to as 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 each of the three years in the period ended December 31, 2025, in conformity with accounting principles generally accepted in the United States of America.

We have also audited, in accordance with the standards of the Public Company Accounting Oversight Board (United States) (PCAOB), the Company's internal control over financial reporting as of December 31, 2025, based on criteria established in *Internal Control — Integrated Framework (2013)* issued by the Committee of Sponsoring Organizations of the Treadway Commission and our report dated February 24, 2026, expressed an unqualified opinion on the Company's internal control over financial reporting.

Basis for Opinion

These financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on the Company's 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.

Critical Audit Matter

The critical audit matter communicated below is a matter arising from the current-period audit of the financial statements that was communicated or required to be communicated to the audit committee and that (1) relates to accounts or disclosures that are material to the financial statements and (2) involved our especially challenging, subjective, or complex judgments. The communication of critical audit matters does not alter in any way our opinion on the financial statements, taken as a whole, and we are not, by communicating the critical audit matter below, providing a separate opinion on the critical audit matter or on the accounts or disclosures to which it relates.

Research and Development Estimates – Refer to Notes 2 and 6 to the financial statements

Critical Audit Matter Description

During the year ended December 31, 2025, the Company incurred $409.6 million of research and development expenses, including accrued liabilities of $19.5 million as of December 31, 2025. As disclosed in Note 2 to the financial statements, the Company expenses research and development costs, which include external costs relating to preclinical, clinical, and process development and manufacturing activities, as incurred. Research and development activities performed by service providers are accrued and expensed based upon estimates of the proportion of work completed over the term of the individual arrangements. Estimates are determined by reviewing vendor agreements and purchase orders, and through discussions with internal personnel and external service providers as to the progress or stage of completion of services and the agreed-upon fee to be paid for such services. Amounts paid to these third parties in excess of recognized expenses are recorded as prepaid costs.

We identified auditing the estimates of the extent of work performed by research and development service providers as a critical audit matter because of the level of management judgment required and volume of such estimates made by management. This required a high degree of auditor judgment and an increased extent of effort when performing audit procedures to evaluate the reasonableness of management’s estimates.

How the Critical Audit Matter Was Addressed in the Audit

Our audit procedures related to external research and development expenses included the following, among others:

- We tested the design and effectiveness of controls over the estimation of accrued and prepaid research and development costs.
- We tested the accuracy and completeness of the underlying data used in the estimates.

- For a sample of service providers performing research and development activities, we performed the following:
  - Inspected the contracts and any amendments to the contracts with the service providers.
  - Evaluated the appropriateness of the method used by management to develop the estimates of the costs incurred to date.
  - Performed corroborating inquiries with the Company's research and development personnel and inspected information from the service providers, which may include the service providers' estimate of the costs incurred to date, to evaluate the progress of the activity.
  - Inspected the evidence of payments to the service providers related to the research activities performed.

/s/ Deloitte & Touche LLP

Boston, Massachusetts
February 24, 2026

We have served as the Company's auditor since 2018.

Beam Therapeutics Inc.
Consolidated Balance Sheets
(in thousands, except share and per share amounts)

	December 31, 2025	December 31, 2024
Assets		
Current assets:		
Cash and cash equivalents	$ 294,944	$ 281,967
Marketable securities	950,266	568,773
Prepaid expenses and other current assets	23,478	27,409
Total current assets	1,268,688	878,149
Property and equipment, net	104,500	111,412
Restricted cash	6,676	8,144
Operating lease right-of-use assets	100,679	104,865
Other assets	634	1,254
Total assets	$ 1,481,177	$ 1,103,824
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable	$ 10,231	$ 3,871
Accrued expenses and other current liabilities	55,267	47,468
Current portion of derivative liabilities	7,700	8,400
Current portion of deferred revenue	6,659	108,858
Current portion of lease liability	14,364	13,469
Current portion of contingent consideration liabilities	2,714	—
Total current liabilities	96,935	182,066
Long-term lease liability	139,759	147,956
Long-term portion of contingent consideration liabilities	5,952	1,131
Long-term portion of deferred revenue	—	33,218
Long-term portion of derivative liabilities	—	5,404
Other liabilities	173	504
Total liabilities	242,819	370,279
Commitments and contingencies (See Note 9, *License and other agreements* and Note 10, *Collaboration and license agreements*)		
Stockholders' equity:		
Preferred stock, $0.01 par value; 25,000,000 shares authorized, and no shares issued or outstanding at December 31, 2025 and December 31, 2024, respectively	—	—
Common stock, $0.01 par value; 250,000,000 shares authorized, 101,748,962 and 83,633,069 issued and outstanding at December 31, 2025 and December 31, 2024, respectively	1,017	836
Additional paid-in capital	2,877,449	2,298,661
Accumulated other comprehensive income	1,111	679
Accumulated deficit	(1,641,219)	(1,566,631)
Total stockholders' equity	1,238,358	733,545
Total liabilities and stockholders' equity	$ 1,481,177	$ 1,103,824

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

Beam Therapeutics Inc.
Consolidated Statements of Operations and Other Comprehensive Loss
(in thousands, except share and per share amounts)

	Year Ended December 31,		
	2025	2024	2023
License and collaboration revenue	$ 139,743	$ 63,518	$ 377,709
Operating expenses:			
Research and development	409,618	367,561	437,381
General and administrative	113,818	111,525	116,813
Total operating expenses	523,436	479,086	554,194
Loss from operations	(383,693)	(415,568)	(176,485)
Other income (expense):			
Change in fair value of derivative liabilities	700	2,272	7,500
Change in fair value of non-controlling equity investments	3,942	(14,093)	(18,592)
Change in fair value of contingent consideration liabilities	180	1,592	9,740
Gain on sale of equity method investment	255,146	—	—
Interest and other income (expense), net	43,733	49,094	46,676
Total other income (expense)	303,701	38,865	45,324
Net loss before income taxes	(79,992)	(376,703)	(131,161)
Provision for income taxes	—	(39)	(1,366)
Net loss	$ (79,992)	$ (376,742)	$ (132,527)
Unrealized gain on marketable securities	432	75	3,034
Comprehensive loss	$ (79,560)	$ (376,667)	$ (129,493)
Net loss per common share, basic and diluted	$ (0.81)	$ (4.58)	$ (1.72)
Weighted-average common shares outstanding, basic and diluted	98,905,577	82,313,008	77,151,771

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

Beam Therapeutics Inc.
Consolidated Statements of Stockholders' Equity
(in thousands, except share amounts)

	Common Stock		Additional Paid-in Capital	Accumulated Other Comprehensive Income (Loss)	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount				
Balance at December 31, 2022	71,277,339	$ 712	$ 1,792,554	$ (2,430)	$ (1,057,362)	$ 733,474
Purchase of common stock under ESPP	130,403	1	3,031	—	—	3,032
Issuance of common stock from At-the-Market offering, net of issuance costs of $6.1 million	6,952,703	70	235,868	—	—	235,938
Issuance of unregistered common shares in connection with license agreement	2,004,811	20	33,580	—	—	33,600
Vesting of restricted common stock	469,531	5	(5)	—	—	—
Stock-based compensation	—	—	98,647	—	—	98,647
Exercise of common stock options	797,709	8	6,123	—	—	6,131
Other comprehensive income (loss)	—	—	—	3,034	—	3,034
Net loss	—	—	—	—	(132,527)	(132,527)
Balance at December 31, 2023	81,632,496	$ 816	$ 2,169,798	$ 604	$ (1,189,889)	$ 981,329
Purchase of common stock under ESPP	135,187	1	2,620	—	—	2,621
Vesting of restricted common stock	1,302,037	13	(13)	—	—	—
Stock-based compensation	—	—	120,662	—	—	120,662
Exercise of common stock options	563,349	6	5,594	—	—	5,600
Other comprehensive income (loss)	—	—	—	75	—	75
Net loss	—	—	—	—	(376,742)	(376,742)
Balance at December 31, 2024	$ 83,633,069	$ 836	$ 2,298,661	$ 679	$ (1,566,631)	$ 733,545
Cumulative effect of adoption of ASU 2025-07	—	—	—	—	5,404	5,404
Issuance of common stock and pre-funded warrants, net of issuance costs of $30.8 million	16,151,686	162	470,312	—	—	470,474
Purchase of common stock under ESPP	174,708	2	2,762	—	—	2,764
Vesting of restricted common stock	1,048,601	10	(10)	—	—	—
Stock-based compensation	—	—	94,244	—	—	94,244
Issuance of common stock for acquisition	403,128	4	6,711	—	—	6,715
Exercise of common stock options	337,770	3	4,769	—	—	4,772
Other comprehensive income (loss)	—	—	—	432	—	432
Net loss	—	—	—	—	(79,992)	(79,992)
Balance at December 31, 2025	101,748,962	$ 1,017	$ 2,877,449	$ 1,111	$ (1,641,219)	$ 1,238,358

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

Beam Therapeutics Inc.
Consolidated Statements of Cash Flows
(in thousands)

	Year Ended December 31,		
	2025	2024	2023
Operating activities			
Net loss	$ (79,992)	$ (376,742)	$ (132,527)
Adjustments to reconcile net loss to net cash provided by (used in) operating activities:			
Depreciation and amortization	22,294	21,925	20,012
Amortization of investment discount (premiums)	(15,958)	(22,765)	(29,743)
In-process research and development charge	14,507	—	—
Stock-based compensation expense	94,244	120,662	98,647
Change in operating lease right-of-use assets	10,388	9,668	9,519
Change in fair value of derivative liabilities	(700)	(2,272)	(7,500)
Change in fair value of contingent consideration liabilities	(180)	(1,592)	(9,740)
Change in fair value of non-controlling equity investments	(3,942)	14,093	18,592
Realized loss (gain) on sale of marketable securities	399	—	—
Gain on sale of equity method investment	(255,146)	—	—
Changes in operating assets and liabilities:			
Prepaid expenses and other current assets	4,511	(5,349)	(6,619)
Accounts payable	6,040	1,807	(7,578)
Accrued expenses and other liabilities	7,685	(57,685)	67,663
Operating lease liabilities	(13,505)	(12,951)	(10,168)
Deferred revenue	(135,417)	(36,518)	(159,559)
Other long-term liabilities	(330)	473	(194)
Net cash provided by (used in) operating activities	(345,102)	(347,246)	(149,195)
Investing activities			
Purchases of property and equipment	(14,946)	(8,946)	(33,732)
Purchases of marketable securities	(1,209,792)	(486,439)	(984,338)
Maturities of marketable securities	842,489	680,392	1,089,910
Proceeds from sale of marketable securities	5,743	—	—
Cash paid for acquisition, net	(78)	—	—
Proceeds from sale of equity method investment	255,146	—	—
Net cash provided by (used in) investing activities	(121,438)	185,007	71,840
Financing activities			
Proceeds from issuance of common shares and pre-funded warrants, net of issuance costs	470,513	—	235,937
Proceeds from issuances of stock under ESPP	2,764	2,621	3,032
Equipment financings, net	—	(485)	(2,252)
Proceeds from exercise of stock options	4,772	5,600	6,131
Proceeds from private stock issuance	—	—	33,600
Net cash provided by (used in) financing activities	478,049	7,736	276,448
Net change in cash, cash equivalents and restricted cash	11,509	(154,503)	199,093
Cash, cash equivalents and restricted cash—beginning of year	290,111	444,614	245,521
Cash, cash equivalents and restricted cash—end of year	$ 301,620	$ 290,111	$ 444,614

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

Beam Therapeutics Inc.
Consolidated statements of cash flows (continued)
(in thousands)

	Year Ended December 31,		
	2025	2024	2023
Supplemental disclosure of cash flow information:			
Cash paid for interest	$ —	$ 32	$ 160
Supplemental disclosure of noncash investing and financing activities:			
Property and equipment additions in accounts payable and accrued expenses	$ 1,271	$ 834	$ 1,403
Operating lease liabilities arising from obtaining right-of-use assets	$ 6,203	$ (1,626)	$ 3,852
Contingent consideration liabilities assumed in acquisition	$ 7,715	$ —	$ —
Fair value of equity instruments issued in connection with acquisition	$ 6,715	$ —	$ —

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

1. Nature of the business and basis of presentation

Organization

Beam Therapeutics Inc., which we refer to herein as the "Company" or "Beam," is a biotechnology company committed to establishing the leading, fully integrated platform for precision genetic medicines. Beam's vision is to provide life-long cures to patients suffering from genetic diseases. The Company was incorporated on January 25, 2017 as a Delaware corporation and began operations in July 2017. Its principal offices are in Cambridge, Massachusetts.

Liquidity and capital resources

Since its inception, the Company has devoted substantially all of its resources to building its base editing platform and advancing development of its portfolio of programs, establishing and protecting its intellectual property, conducting research and development activities, continuing to invest in its internal manufacturing capabilities and making arrangements to conduct manufacturing activities with contract manufacturing organizations, research and development costs including preclinical studies and IND-enabling studies, organizing and staffing the Company, maintaining its facilities and new facility build-outs, business planning, raising capital and providing general and administrative support for these operations. The Company is subject to risks and uncertainties common to early-stage companies in the biotechnology industry including, but not limited to, technical risks associated with the successful research, development and manufacturing of product candidates, development by competitors of new technological innovations, dependence on key personnel, protection of proprietary technology, compliance with government regulations and the ability to secure additional capital to fund operations. Current and future programs will require significant research and development efforts, including extensive preclinical and clinical testing and regulatory approval prior to commercialization. These efforts require significant amounts of additional capital, adequate personnel and infrastructure. 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.

The Company has entered into an at the market sales agreement, or the Sales Agreement, with Jefferies LLC, or Jefferies, pursuant to which the Company is entitled to offer and sell, from time to time at prevailing market prices, shares of its common stock having aggregate gross proceeds of up to $1.1 billion. The Company agreed to pay Jefferies a commission of up to 3.0% of the aggregate gross sale proceeds of any shares sold by Jefferies under the Sales Agreement. There were no shares sold under the Sales Agreement during the year ended December 31, 2025. As of December 31, 2025, the Company has sold 13,769,001 shares of its common stock under the Sales Agreement at an average price of $62.75 per share for aggregate gross proceeds of $864.0 million, before deducting commissions and offering expenses payable by it.

In March 2025, the Company closed an underwritten public offering of 16,151,686 shares of common stock at a public offering price of $28.48 per share and pre-funded warrants to purchase 1,404,988 shares of common stock at a purchase price of $28.47 per pre-funded warrant for aggregate net proceeds of $470.5 million, after deducting underwriting discounts, commissions and approximately $0.8 million related to legal, accounting and other fees in connection with the offering.

Since its inception, the Company has incurred substantial losses and had an accumulated deficit of $1.6 billion as of December 31, 2025. The Company expects to generate operating losses and negative operating cash flows for the foreseeable future.

The Company expects that its cash, cash equivalents, and marketable securities as of December 31, 2025 of $1.2 billion will be sufficient to fund its operations for at least the next 12 months from the date of issuance of these financial statements. The Company will need additional financing to support its continuing operations and pursue its growth strategy. Until such time as the Company can generate significant revenue from product sales, if ever, it expects to finance its operations through a combination of equity offerings, debt financings, collaborations, strategic alliances and licensing arrangements. The Company may be unable to raise additional funds or enter into such other agreements when needed on favorable terms or at all. The inability to raise capital as and when needed would have a negative impact on the Company's financial condition and its ability to pursue its business strategy. The Company will need to generate significant revenue to achieve profitability, and it may never do so.

2. Summary of significant accounting policies

Basis of presentation

The accompanying consolidated financial statements have been prepared in accordance with United States generally accepted accounting principles, or GAAP. Any reference in these notes to applicable guidance is meant to refer to the authoritative GAAP as found in the Accounting Standards Codification, or ASC, of the Financial Accounting Standards Board, or the FASB.

Principles of consolidation

The accompanying consolidated financial statements include the results of operations of the Company and its wholly-owned subsidiaries. All intercompany transactions and balances have been eliminated in consolidation.

Use of estimates

The preparation of financial statements in conformity with GAAP requires management to make estimates and assumptions that affect the reported amounts of assets, liabilities, revenues and expenses, and the disclosure of contingent assets and liabilities as of and during the reporting period. The Company bases its estimates and assumptions on historical experience when available and on various factors that it believes to be reasonable under the circumstances. Significant estimates and assumptions reflected in these consolidated financial statements include, but are not limited to, incremental borrowing rate used in the calculation of lease liabilities, research and development expenses, stock-based compensation, contingent consideration liabilities, success payments and derivative liabilities, and certain judgments regarding revenue recognition. Actual results could differ from these estimates.

Cash, cash equivalents, and restricted cash

Cash and cash equivalents consist of checking accounts, money market accounts, and all highly liquid investments with a remaining maturity of three months or less at the date of purchase. Restricted cash represents collateral provided for letters of credit issued as security deposits in connection with the Company's leases of its facilities.

The following table reconciles cash, cash equivalents, and restricted cash reported within the Company's consolidated balance sheets to the total of the amounts shown in the consolidated statements of cash flows (in thousands):

	December 31,		
	2025	2024	2023
Cash and cash equivalents	$ 294,944	$ 281,967	$ 435,895
Restricted cash	6,676	8,144	8,719
Total cash, cash equivalents, and restricted cash	$ 301,620	$ 290,111	$ 444,614

Marketable debt securities

The Company classifies marketable debt securities as available-for-sale and includes such securities within marketable securities in the Company's consolidated balance sheets. Available-for-sale securities consist of commercial paper, high-grade corporate notes, U.S. Treasury securities and government securities. Available-for-sale securities are carried at fair value with the unrealized gains and losses included in accumulated other comprehensive income as a component of stockholders' equity until realized. Any premium or discount arising at purchase is amortized and/or accreted to interest income and/or amortized over the life of the instrument. Realized gains and losses are determined using the specific identification method and are included in interest and other income (expense), net.

Corporate equity securities

The Company classifies investments in equity securities that have a readily determinable fair value as marketable securities in the Company's consolidated balance sheets. The Company's marketable securities are stated at fair value. Typically, the fair value of these securities is based on a quoted price for an identical equity security. The Company records changes in the fair value of its equity securities in other income (expense), net in its consolidated statements of operations and other comprehensive loss.

Concentrations of credit risk

Financial instruments that are potentially subject to significant concentration of credit risk consist primarily of cash, cash equivalents, marketable securities, and restricted cash. The Company attempts to minimize the risk related to marketable securities by working with highly rated financial institutions that invest in a broad and diverse range of financial instruments. The Company has established guidelines relative to credit ratings and maturities intended to safeguard principal balances and maintain liquidity. The Company maintains its funds in accordance with its investment policy, which defines allowable investments, specifies credit quality standards and is designed to limit credit exposure to any single issuer.

Guarantees and indemnifications

As permitted under Delaware law, the Company indemnifies its officers, directors, consultants, and employees for certain events or occurrences that happen by reason of the relationship with, or position held at, the Company. For the years ended December 31, 2025, 2024 and 2023, the Company had not experienced any losses related to these indemnification obligations, and no claims were outstanding. The Company does not expect significant claims related to these indemnification obligations and, consequently, concluded that the fair value of these obligations is negligible, and no related reserves were established.

Equity issuance costs

The Company capitalizes incremental legal, professional, accounting and other third-party fees that were directly associated with its stock offerings as other non-current assets until the offerings are consummated. Upon consummation, these costs are recorded in stockholders' equity as a reduction of additional paid-in-capital generated as a result of the offerings.

Fair value of financial instruments

ASC Topic 820, *Fair Value Measurement*, or ASC 820, establishes a fair value hierarchy for instruments measured at fair value that distinguishes between assumptions based on market data (observable inputs) and the Company's own assumptions (unobservable inputs). Observable inputs are inputs that market participants would use in pricing the asset or liability based on market data obtained from independent sources. Unobservable inputs are inputs that reflect the Company's assumptions about the inputs that market participants would use in pricing the assets or liability and are developed based on the best information available in the circumstances. ASC 820 identifies fair value 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. As a basis for considering market participant assumptions in fair value measurements, ASC 820 establishes a three-tiered value hierarchy that distinguishes between the following:

Level 1—Quoted market prices in active markets for identical assets or liabilities.

Level 2—Inputs other than Level 1 inputs that are either directly or indirectly observable, such as quoted market prices, interest rates and yield curves.

Level 3—Unobservable inputs for the asset or liability (i.e., supported by little or no market activity). Level 3 inputs include management's own assumptions about the assumptions that market participants would use in pricing the asset or liability (including assumptions about risk).

To the extent the valuation is based on models or inputs that are less observable or unobservable in the market, the determination of fair values requires more judgment. Accordingly, the degree of judgment exercised by the Company in determining fair value is greatest for instruments categorized as Level 3. A financial instrument's level within the fair value hierarchy is based on the lowest level of any input that is significant to the fair value measurement.

There have been no changes to the valuation methods utilized during the years ended December 31, 2025 and 2024. The Company evaluates transfers between levels at the end of each reporting period.

Property and equipment

Property and equipment are stated at cost less accumulated depreciation. Depreciation expense is recognized using the straight-line method over the estimated useful life of each asset as follows:

Asset category	Estimated useful life
Computer equipment and software	3 years
Laboratory equipment and office furniture	5 years
Leasehold improvements	Shorter of useful life or remaining term

Upon retirement or sale, the cost of assets disposed of and the related accumulated depreciation are removed from the accounts and any resulting gain or loss is included in interest and other income (expense). Expenditures for repairs and maintenance are charged to expense as incurred.

Impairment of long-lived assets

The Company evaluates its long-lived assets, which consist primarily of property and equipment and operating lease right-of-use assets, for impairment whenever events or changes in circumstances indicate that the carrying amount of such assets (or asset groups) may not be recoverable. Recoverability of assets to be held and used is measured by a comparison of the carrying amount of an asset to the future undiscounted net cash flows expected to be generated by the asset. If such assets are considered to be impaired, the impairment to be recognized is measured by the amount by which the carrying amount of the asset exceeds the fair value of the asset. There were no impairment losses recognized during the years ended December 31, 2025, 2024 and 2023.

Freestanding financial instruments and derivatives

Pursuant to a license agreement between the President and Fellows of Harvard College, or Harvard, and the Company, or the Harvard License Agreement, and a license agreement with The Broad Institute, Inc., or Broad Institute, and the Company, or the Broad License Agreement, (see Note 9), the Company is required to make success payments to Harvard and Broad Institute based on the achievement of specified multiples of the initial weighted average value of the Company's redeemable convertible Series A-1 Preferred Stock and the Company's redeemable convertible Series A-2 Preferred Stock, or together the Series A Preferred, at specified valuation dates, payable in cash or Company common stock. Subsequent to the Company's initial public offering in February 2020, or the IPO, the amount of the success payments is based on the market value of Beam's common stock.

The success payments are accounted for as derivatives under ASC 815, *Derivatives and Hedging.* The liabilities are recorded at fair value at each balance sheet date with all changes in value recognized in other income (expense), in the consolidated statement of operations and other comprehensive loss. The Company will continue to adjust the liabilities for changes in fair value until the earlier of the achievement or expiration of the obligations. To determine the estimated fair value of the success payments, the Company used a Monte Carlo simulation model, which models the value of the liability based on several key variables, including probability of event occurrence, timing of event occurrence, as well as the value of the Series A Preferred, prior to the IPO, and the value of the Company's common stock, subsequent to the IPO.

Leases and rent expense

The Company accounts for leases using a right-of-use, or ROU, model, which recognizes that, at the date of commencement, a lessee has a financial obligation to make lease payments to the lessor for the right to use the underlying asset during the lease term.

At the inception of an arrangement, the Company determines whether the arrangement is or contains a lease based on the unique facts and circumstances present in the arrangement. Leases with a term greater than one year are recognized on the balance sheet as ROU assets and short-term and long-term lease liabilities, as applicable. ROU assets represent the Company's right to use an underlying asset for the lease term and lease liabilities represent its obligation to make lease payments arising from the lease. The Company typically only includes an initial lease term in its assessment of the term of the lease arrangement. It also considers termination options and factors those into the determination of lease payments. Options to renew a lease are not included in the lease term unless there is reasonable certainty that the Company will renew.

Operating lease liabilities and their corresponding ROU assets are recorded based on the present value of lease payments over the expected remaining lease term. Certain adjustments to the ROU asset may be required for items such as incentives received. The interest rate implicit in lease contracts is typically not readily determinable. As a result, the Company utilizes its incremental borrowing rate, which reflects the fixed rate at which it could borrow on a collateralized basis the amount of the lease payments in the same currency, for a similar term, in a similar economic environment. Lease expense for operating lease payments is recognized on a straight-line basis over the lease term.

The Company is required to pay fees for operating expenses in addition to monthly base rent for certain operating leases (non-lease components). The Company has elected the practical expedient which allows non-lease components to be combined with lease components for all current asset classes. Variable lease payments are not included within the lease right-of-use asset and lease liability on the consolidated balance sheet, and instead are reflected as expense in the period they are incurred.

Leasehold improvements are not unique and are retained by the lessor at the end of the lease. However, in the case of a space designed to be suitable for the Company's specific real estate needs and if the Company is responsible for cost overruns, the Company is the accounting owner of the leasehold improvements and costs associated are capitalized.

The Company's real estate operating leases provide for scheduled annual rent increases throughout the lease terms. The Company recognizes the effects of the scheduled rent increases on a straight-line basis over the full terms of the lease. Tenant improvement allowances, if any, provided by a landlord are recorded as a reduction of the ROU asset related to that lease at lease commencement.

Asset acquisitions

The Company measures and recognizes asset acquisitions that are not deemed to be business combinations based on the cost to acquire the assets, which includes transaction costs, and the consideration is allocated to the items acquired based on a relative fair value methodology. Goodwill is not recognized in asset acquisitions. In an asset acquisition, the cost allocated to acquire in-process research and development with no alternative future use is charged to research and development expense at the acquisition date.

At the time of acquisition, the Company determines if a transaction should be accounted for as a business combination or acquisition of assets.

Contingent consideration liabilities

The Company may be required or choose to make milestone payments to the former stockholders and optionholders of companies acquired, in the form of its common stock based on the achievement of certain development, clinical and commercial milestones. The payments are accounted for under ASC 480, *Distinguishing Liabilities from Equity*. These contingent consideration liabilities are carried at fair value which was estimated by applying a probability-based model, which utilized inputs primarily based upon the achievement and related timing of certain development, clinical and commercial milestones that were unobservable in the market. The estimated fair value of contingent consideration liabilities, initially measured and recorded on the acquisition date, are considered to be a Level 3 fair value measurement and are reviewed quarterly, or whenever events or circumstances occur that indicate a change in fair value. The contingent consideration liabilities are recorded at fair value at the end of each reporting period with changes in estimated fair values recorded in other income (expense) in the consolidated statements of operations and other comprehensive loss.

Significant changes in any of the probabilities of success or in the probabilities as to the periods in which milestones would be achieved could result in a significantly higher or lower fair value measurement. The Company will continue to adjust the liabilities for changes in fair value until the earlier of the achievement or expiration of the obligations.

Revenue recognition

At inception, the Company determines whether contracts are within the scope of ASC 606 or other topics. For contracts that are determined to be within the scope of ASC 606, revenue is recognized when a customer obtains control of promised goods or services. The amount of revenue recognized reflects the consideration to which the Company expects to be entitled to receive in exchange for these goods and services. To achieve this core principle, the Company applies the following five steps: (i) identify the contract with the customer; (ii) identify the performance obligations in the contract; (iii) determine the transaction price; (iv) allocate the transaction price to the performance obligations in the contract; and (v) recognize revenue when the performance obligation is satisfied. The Company only applies the five-step model to contracts when it determines that collection of substantially all consideration for goods and services that are transferred is probable based on the customer's intent and ability to pay the promised consideration.

Performance obligations promised in a contract are identified based on the goods and services that will be transferred to the customer that are both capable of being distinct and are distinct in the context of the contract. To the extent a contract includes multiple promised goods and services, the Company applies judgment to determine whether promised goods and services are both capable of being distinct and are distinct in the context of the contract. If these criteria are not met, the promised goods and services are accounted for as a combined performance obligation.

The transaction price is determined based on the consideration to which the Company will be entitled in exchange for transferring goods and services to the customer. To the extent the transaction price includes variable consideration, the Company estimates the amount of variable consideration that should be included in the transaction price utilizing either the expected value method or the most likely amount method, depending on the nature of the variable consideration. Variable consideration is included in the transaction price if, in management's judgment, it is probable that a significant future reversal of cumulative revenue under the contract will not occur. Any estimates, including the effect of the constraint on variable consideration, are evaluated at each reporting period for any changes. Determining the transaction price requires significant judgment and is discussed in further detail for each of the Company's license and collaboration agreements in Note 10.

If the contract contains a single performance obligation, the entire transaction price is allocated to the single performance obligation. Contracts that contain multiple performance obligations require an allocation of the transaction price to each performance obligation on a relative standalone selling price basis unless the transaction price is variable and meets the criteria to be allocated entirely to a performance obligation or to a distinct service that forms part of a single performance obligation. Determining the standalone selling price requires significant judgment and is discussed in further detail for each of the Company's license and collaboration agreements in Note 10.

The Company satisfies performance obligations either over time or at a point in time. Revenue is recognized over time if either (i) the customer simultaneously receives and consumes the benefits provided by the entity's performance, (ii) the entity's performance creates or enhances an asset that the customer controls as the asset is created or enhanced, or (iii) the entity's performance does not create an asset with an alternative use to the entity and the entity has an enforceable right to payment for performance completed to date. If the entity does not satisfy a performance obligation over time, the related performance obligation is satisfied at a point in time by transferring the control of a promised good or service to a customer.

Licenses of intellectual property, or IP: If the license to the Company's IP is determined to be distinct from the other performance obligations identified in the arrangement, the Company recognizes revenues from consideration allocated to the license when the license is transferred to the customer and the customer can use and benefit from the licenses. For licenses that are combined with other promises, the Company utilizes judgment to assess the nature of the combined performance obligation to determine whether the combined performance obligation is satisfied over time or at a point in time and, if over time, the appropriate method of measuring progress for purposes of recognizing revenue. The Company evaluates the measure of progress each reporting period and, if

necessary, adjusts the measure of performance and related revenue recognition. The Company generally recognizes revenue using the cost incurred to date as compared to the total estimated cost. The impact on revenue of changes in total estimated costs are recognized on a cumulative basis in the period that the change occurs. If estimates of the total cost change, or if contract amendments change the scope of the performance obligation, the required adjustments to revenue could be material. Determining the revenue recognition of IP licenses requires significant judgment and is discussed in further detail for each of the Company's license and collaboration agreements in Notes 9 and 10.

Milestone payments: At the inception of each arrangement that includes development or regulatory milestone payments, the Company evaluates the probability of reaching the milestones and estimates the amount to be included in the transaction price using the most likely amount method. If it is probable that a significant revenue reversal would not occur in the future, the associated milestone value is included in the transaction price. Milestone payments that are not within the Company's control or the licensee's, such as regulatory approvals, are not considered probable of being achieved until those approvals are received and therefore consideration included in the transaction price is constrained. The transaction price is then allocated to each performance obligation on a relative standalone selling price basis, for which the Company recognizes revenue as or when the performance obligations under the contract are satisfied. At the end of each subsequent reporting period, the Company re-evaluates the probability of achievement of such development milestones and any related constraint, and if necessary, adjusts its estimate of the overall transaction price. Any such adjustments are recorded on a cumulative catch-up basis, which would affect revenues and earnings in the period of adjustment.

Commercial Milestone Payments and Royalties: For arrangements that include sales-based royalties, including milestone payments based on levels of sales, if the license is deemed to be the predominant item to which the royalties relate, the Company recognizes revenue at the later of (i) when the related sales occur, or (ii) when the performance obligation to which some or all of the royalty has been allocated has been satisfied (or partially satisfied). To date, the Company has not recognized any royalty revenue resulting from any of its agreements.

When no remaining performance obligations are required of the Company, or following the completion of the performance obligation period, such amounts are recognized as revenue upon transfer of control of the goods or services to the customer. Generally, all amounts received or due other than sales-based milestones and royalties are classified as license and collaboration revenue. Sales-based milestones and royalties will be recognized as royalty revenue at the later of when the related sales occur or when the performance obligation to which some or all of the royalty has been allocated has been satisfied (or partially satisfied).

Deferred revenue expected to be recognized within the next twelve months is classified as a current liability.

Contract balances

The Company recognizes a contract asset when the Company transfers goods or services to a customer before the customer pays consideration or before payment is due, excluding any amounts presented as an account or other receivable. A contract asset is an entity's right to consideration in exchange for goods or services that the entity has transferred to a customer. The contract liabilities, or deferred revenue, primarily relate to contracts where the Company has received payment, but it has not yet satisfied or fully satisfied the related performance obligations. Upfront payments and fees are recorded as deferred revenue upon receipt or when due and may require deferral of revenue recognition to a future period until the Company satisfies its obligations under these arrangements. Upfront payment contract liabilities resulting from the Company's license agreements do not represent a financing component as the payment is not financing the transfer of goods or services, and the technology underlying the licenses granted reflects research and development expenses already incurred by the Company.

The changes in the total deferred revenue for the years ended December 31, 2025 and 2024 were as follows (in thousands):

	December 31, 2025	December 31, 2024
Beginning balance	$ 142,076	$ 178,594
Additions to deferred revenue from license agreements	—	—
Amounts recognized in revenue	(135,417)	(36,518)
Ending balance	$ 6,659	$ 142,076

Research and development costs

Research and development costs are charged to expense as incurred. Research and development costs consist of costs incurred in performing research and development activities, including salaries and bonuses, stock-based compensation, employee benefits, facilities costs, laboratory supplies, depreciation, manufacturing expenses, preclinical expenses, consulting, other contracted services, and up-front and continent payments dependent upon development activities. The cost of obtaining licenses for certain technology or IP is recorded to research and development expense when incurred if the licensed technology or IP has not yet reached technological feasibility and has no alternative future use. Costs for certain research and development activities are recognized based on the terms of the individual arrangements, which may differ from the pattern of costs incurred, and are reflected in the financial statements as prepaid or accrued research and development costs.

Stock-based compensation

The Company's stock-based compensation program allows for grants of stock options, restricted stock awards and restricted stock units. Grants are awarded to employees and non-employees, including directors.

The Company accounts for stock-based compensation in accordance with ASC Topic 718, *Compensation-Stock Compensation*, or ASC 718. ASC 718 requires all stock-based payments to employees, non-employees and directors to be recognized as expense in the consolidated statements of operations and other comprehensive loss based on their fair values. The Company estimates the fair value of options granted using the Black-Scholes option pricing model, or Black-Scholes, for stock option grants to both employees and non-employees. The fair value of the Company's common stock is used to determine the fair value of restricted stock awards and restricted stock units.

Stock-based compensation awards are subject to either service- or performance-based vesting conditions. Compensation expense related to awards to employees, directors and non-employees with service-based vesting conditions is recognized on a straight-line basis based on the grant date fair value over the associated service period of the award, which is generally the vesting term. Compensation expense related to awards to employees with performance-based vesting conditions is recognized based on grant date fair value over the requisite service period using the accelerated attribution method to the extent achievement of the of performance condition is probable.

The Black-Scholes option pricing model requires inputs based on certain subjective assumptions, including (i) the expected stock price volatility, (ii) the expected term of the award, (iii) the risk-free interest rate, and (iv) expected dividends. The Company bases its computation of expected volatility on the historical volatility of a representative group of public companies with similar characteristics to the Company, including stage of product development and life science industry focus, weighted with its own volatility for the period in which its stock has been publicly traded. The historical volatility is calculated based on a period of time commensurate with expected term assumption. The Company uses the simplified method as prescribed by the SEC Staff Accounting Bulletin No. 107, *Share-Based Payment*, to calculate the expected term for options granted to employees and non-employees, whereby the expected term equals the arithmetic average of the vesting term and the original contractual term of the options due to its lack of sufficient historical data. The risk-free interest rate is based on U.S. Treasury securities with a maturity date commensurate with the expected term of the associated award. The expected dividend yield is assumed to be zero as the Company has never paid dividends and has no current plans to pay any dividends on its common stock. The Company recognizes forfeitures as they occur.

Patent costs

All patent-related costs incurred in connection with filing and prosecuting patent applications are expensed as incurred. Due to the uncertainty about the recovery of the expenditure, amounts incurred are classified as general and administrative expenses in the accompanying consolidated statements of operations and other comprehensive loss.

Variable interest entities

The Company reviews each legal entity in which it has a financial interest to determine whether or not the entity is a variable interest entity, or VIE. If the entity is a VIE, the Company assesses whether or not it is the primary beneficiary of that VIE based on a number of factors, including (i) which party has the power to direct the activities that most significantly affect the VIE's economic performance, (ii) the parties' contractual rights and responsibilities pursuant to any contractual agreements and (iii) which party has the obligation to absorb losses or the right to receive benefits from the VIE. If the Company determines that it is the primary beneficiary of a VIE, it consolidates the financial statements of the VIE into its consolidated financial statements. If the Company determines that it is no longer the primary beneficiary of a consolidated VIE, or no longer has a variable interest in the VIE, the Company deconsolidates the VIE in the period that the determination is made.

Income taxes

The Company recognizes deferred tax assets and liabilities for the expected future tax consequences of events that have been included in the Company's financial statements and tax returns. Deferred tax assets and liabilities are determined based upon the differences between the financial statement carrying amounts and the tax bases of existing assets and liabilities and for loss and credit carryforwards, using enacted tax rates expected to be in effect in the year in which the differences are expected to reverse. Deferred tax assets are reduced by a valuation allowance if it is more likely than not that these assets may not be realized. The Company determines whether it is more likely than not that a tax position will be sustained upon examination. If it is not more likely than not that a position will be sustained, none of the benefit attributable to the position is recognized. The tax benefit to be recognized for any tax position that meets the more-likely-than-not recognition threshold is calculated as the largest amount that is more than 50% likely of being realized upon resolution of the contingency. The Company accounts for interest and penalties related to uncertain tax positions as part of its provision for income taxes.

Comprehensive loss

Comprehensive loss is defined as the change in stockholders' equity of a business enterprise during a period from transactions and other events and circumstances from non-owner sources. Comprehensive loss includes net loss as well as other changes in equity that are excluded from net loss. The Company's only element of other comprehensive loss is unrealized gains and losses on marketable securities.

Net loss per share

The Company follows the two-class method when computing net loss per share, as the Company has issued shares that meet the definition of participating securities. The two-class method determines net loss per 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 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.

Basic net loss per share attributable to common stockholders is computed by dividing the net loss attributable to common stockholders by the weighted average number of common shares outstanding for the period. Diluted net loss attributable to common stockholders is computed by adjusting net loss attributable to common stockholders to reallocate undistributed earnings based on the potential impact of dilutive securities. Diluted net loss per share attributable to common stockholders is computed by dividing the diluted net loss attributable to common stockholders by the weighted average number of common shares outstanding for the period, including potential dilutive common shares assuming the dilutive effect of common stock equivalents.

For purposes of the dilutive net loss per share calculation, stock options and stock units for which the performance and market vesting conditions have been deemed probable, potential dilutive securities, unvested restricted stock, and common stock options are considered to be common stock equivalents, while stock options and stock units with performance- or market-based vesting conditions that were not deemed probable of meeting the applicable vesting conditions are not considered to be common stock equivalents.

Accordingly, in periods in which the Company reported a net loss, such losses were not allocated to such participating securities. In periods in which the Company reported a net loss attributable to common stockholders, diluted net loss per share attributable to common stockholders was the same as basic net loss per share attributable to common stockholders, since dilutive common shares were not assumed to have been issued if their effect is anti-dilutive. The Company reported a net loss attributable to common stockholders for the years ended December 31, 2025, 2024 and 2023.

Equity method of accounting

In circumstances where the Company has the ability to exercise significant influence, but not control, over the operating and financial policies of an entity in which the Company has an investment in common stock or in-substance common stock, the Company utilizes the equity method of accounting for recording related investment activity. In assessing whether the Company exercises significant influence, the Company considers the nature and magnitude of the investment, participating rights the Company holds, and relevant factors such as the presence of a collaborative or other business relationship.

Under the equity method of accounting, the Company's investments are initially recorded at cost on the consolidated balance sheets. Upon recording an equity method investment, the Company evaluates whether there are basis differences between the carrying value and fair value of the Company's proportionate share of the investee's underlying net assets. Typically, the Company amortizes basis differences identified on a straight-line basis over the underlying asset's or liability's estimated useful lives when calculating the attributable earnings or losses, excluding the basis differences attributable to in-process research and development, or IPR&D, that has no alternative future use. To the extent a basis difference relates to IPR&D and the investee is not a business as defined in ASC 805, *Business Combinations,* or ASC 805, the Company immediately expenses such basis difference related to IPR&D. If the Company is unable to attribute all of the basis difference to specific assets or liabilities of the investee, the residual excess of the cost of the investment over the proportional fair value of the investee's assets and liabilities is considered to be Equity Method Goodwill and is recognized within the equity investment balance, which is tracked separately within the Company's memo accounts. The Company subsequently records in the consolidated statements of operations and comprehensive loss its share of income or loss of the other entity within the loss from equity method investment line item. If the share of losses exceeds the carrying value of the Company's investment, the Company will suspend recognizing additional losses and will continue to do so unless it commits to providing additional funding or commits to guarantee investee liabilities.

The Company evaluates its equity method investments for impairment whenever events or changes in circumstances indicate that the carrying amounts of such investments may be impaired and considers qualitative and quantitative factors including the investee's financial metrics, product and commercial outlook and cash usage. If a decline in the value of an equity method investment is determined to be other than temporary, a loss is recorded in earnings in the current period and the investment is written down to fair value.

Segment and geographic information

Operating segments are defined as components of an entity about which separate discrete information is available for evaluation by the chief operating decision maker, or CODM, or decision-making group, in deciding how to allocate resources and in assessing performance. The CODM is the Company's Chief Executive Officer. The Company views its operations as and manages its business in one operating segment operating exclusively in the United States.

Recently adopted accounting pronouncements

The Company early adopted ASU No. 2025-07, *Derivatives and Hedging (Topic 815) and Revenue from Contracts with Customers (Topic 606): Scope Refinements and Scope Clarification for Share-Based Noncash Consideration from a Customer in a Revenue Contract*, or ASU 2025-07, in the fourth quarter of 2025 using a modified retrospective approach. The new guidance modifies Accounting Standards Codification Topic 815, *Derivatives and Hedging*, or Topic 815, to add a scope exclusion for contracts that are not traded on an exchange if the underlying on which the settlement is based relates to operations or activities specific to one of the parties to the contract. As a result, an existing contract that includes a settlement feature based on the Company's operations or activities is now excluded from Topic 815 and will now be accounted for in accordance with ASC 450, *Contingencies*, or ASC 450, whereby any settlements will be recognized as such obligations become probable and estimable. The adoption of ASU 2025-07 using a modified retrospective approach requires the Company to adopt the standard as of January 1, 2025. Upon adoption, the Company recognized a cumulative-effect adjustment to remove the previously recognized derivative liability as of January 1, 2025, reducing the long-term portion of derivative liabilities by $5.4 million, with an offsetting adjustment to accumulated deficit. The elimination of this derivative liability reduces previously reported net loss by $0.9 million, $0.2 million, and $0.2 million for the quarters ended March 31, 2025, June 30, 2025, and September 30, 2025, respectively. The adjustment had no impact on previously reported cash flows from operating, investing, or financing activities within the Company's condensed consolidated statements of cash flows. In accordance with ASC 450, no liability has been recognized for the contingent payments under the contract as of January 1, 2025 and through December 31, 2025.

In December 2023, the FASB issued ASU 2023-09, *Income Taxes (Topic 740)*. The amendments in this update expand income tax disclosure requirements, including additional information pertaining to the rate reconciliation, income taxes paid, and other disclosures. This update is effective for annual periods beginning after December 15, 2024. The Company adopted this guidance for the year ended December 31, 2025 and has included the required disclosures in Note 14, *Income Taxes*. The adoption of this guidance did not have a material impact on the Company's consolidated financial statements.

Recently announced accounting pronouncements.

In September 2025, the FASB issued ASU 2025-06, *Targeted Improvements to the Accounting for Internal-Use Software*. This standard is intended to modernize the accounting for internal-use software. Under the new standard, the Company will capitalize eligible costs when (i) management has authorized and committed to funding the software project, and (ii) it is probable that the project will be completed and the software will be used to perform the function intended. The standard is effective for fiscal years, and interim periods within those fiscal years, beginning after December 15, 2027, with early adoption permitted as of the beginning of a fiscal year. The standard may be applied prospectively, retrospectively or using a modified transition approach. The Company is currently evaluating the impact that this standard will have on the Company's consolidated operating results, cash flows, financial condition and related disclosures.

In November 2024, the FASB issued ASU 2024-03, *Income Statement—Reporting Comprehensive Income—Expense Disaggregation Disclosures (Subtopic 220-40): Disaggregation of Income Statement Expenses*. The standard requires the Company to provide further disaggregated information of relevant expense captions within its consolidated statements of operations, including the purchases of inventory, employee compensation, depreciation and intangible asset amortization, as well as the inclusion of other specific expenses, gains and losses required by existing GAAP. The new standard also requires the Company to disclose its total selling expenses and, on an annual basis, provide a qualitative description of its selling expenses. The standard is effective for fiscal years beginning after December 15, 2026 and interim periods within fiscal years beginning after December 15, 2027, with early adoption permitted. The standard may be applied prospectively or retrospectively. The Company is currently evaluating the impact that this standard will have on the Company's consolidated operating results, cash flows, financial condition and related disclosures.

3. Property and equipment, net

Property and equipment consist of the following (in thousands):

	December 31, 2025	December 31, 2024
Leasehold improvements	$ 110,760	$ 101,195
Lab equipment	77,038	71,788
Furniture and fixtures	4,836	4,836
Computer equipment	3,170	3,170
Construction in process	2,823	2,530
Total property and equipment	198,627	183,519
Less accumulated depreciation	(94,127)	(72,107)
Property and equipment, net	$ 104,500	$ 111,412

The following table summarizes depreciation expense incurred (in thousands):

	Year Ended December 31, 2025	2024	2023
Depreciation expense	$ 22,294	$ 21,925	$ 20,012

4. Fair value of financial instruments

The Company's financial instruments that are measured at fair value on a recurring basis consist of cash equivalents, marketable securities, corporate equity securities, contingent consideration liabilities related to acquisitions, and success payment derivative liabilities pursuant to the Harvard and Broad License Agreements.

The following tables set forth the fair value of the Company's financial assets and liabilities by level within the fair value hierarchy at December 31, 2025 (in thousands):

	Carrying amount	Fair value	Level 1	Level 2	Level 3
Assets					
Cash equivalents:					
Money market funds	$ 274,944	$ 274,944	$ 274,944	$ —	$ —
U.S. Treasury securities backed repurchase agreements	20,000	20,000	—	20,000	—
Marketable securities:					
Commercial paper	304,959	304,959	—	304,959	—
Corporate notes	150,046	150,046	—	150,046	—
U.S. Treasury securities	462,984	462,984	—	462,984	—
U.S. Government securities	26,696	26,696	—	26,696	—
Corporate equity securities	5,581	5,581	5,581	—	—
Total assets	$ 1,245,210	$ 1,245,210	$ 280,525	$ 964,685	$ —
Liabilities					
Success payment liability – Harvard	$ 3,300	$ 3,300	$ —	$ —	$ 3,300
Success payment liability – Broad Institute	4,400	4,400	—	—	4,400
Contingent consideration liability milestones	8,666	8,666	—	—	8,666
Total liabilities	$ 16,366	$ 16,366	$ —	$ —	$ 16,366

The following tables set forth the fair value of the Company's financial assets and liabilities by level within the fair value hierarchy at December 31, 2024 (in thousands):

	Carrying amount	Fair value	Level 1	Level 2	Level 3
Assets					
Cash equivalents:					
Money market funds	$ 281,786	281,786	$ 281,786	$ —	$ —
Marketable securities:					
Commercial paper	181,296	181,296	—	181,296	—
Corporate notes	100,165	100,165	—	100,165	—
U.S. Treasury securities	164,770	164,770	—	164,770	—
U.S. Government securities	114,761	114,761	—	114,761	—
Corporate equity securities	7,781	7,781	7,781	—	—
Total assets	$ 850,559	$ 850,559	$ 289,567	$ 560,992	$ —
Liabilities					
Success payment liability – Harvard	$ 3,900	$ 3,900	$ —	$ —	$ 3,900
Success payment liability – Broad Institute	4,500	4,500	—	—	4,500
Derivative settlement liability	5,404	5,404	—	—	5,404
Contingent consideration liability – Technology	496	496	—	—	496
Contingent consideration liability – Product	635	635	—	—	635
Total liabilities	$ 14,935	$ 14,935	$ —	$ —	$ 14,935

Cash equivalents – Money market funds included within cash equivalents are classified within Level 1 of the fair value hierarchy because they are valued using quoted market prices in active markets and repurchase agreements backed by U.S. Treasury securities that are classified within Level 2 of the fair value hierarchy because pricing inputs are other than quoted prices in active markets, which are either directly or indirectly observable as of the reporting date, and fair value is determined through using models or other valuation methodologies.

Marketable securities – Marketable securities, excluding corporate equity securities, are classified within Level 2 of the fair value hierarchy because pricing inputs are other than quoted prices in active markets, which are either directly or indirectly observable as of the reporting date, and fair value is determined using models or other valuation methodologies.

During the year ended December 31, 2025, Eli Lilly and Company, or Lilly, completed its acquisition of Verve Therapeutics, Inc., or Verve. As a result of the acquisition, the Company received total proceeds of $5.7 million as consideration for its 546,970 shares of Verve's common stock and recorded a realized loss of $0.4 million during the year ended December 31, 2025, presented in "Interest and other income (expense), net" in the consolidated statements of operations and other comprehensive loss. The shares were previously classified as marketable securities on the Company's consolidated balance sheet and measured at fair value. There were no other realized gains or losses from the sale of marketable securities during the year ended December 31, 2025. Proceeds from the sale are reflected in investing activities on the consolidated statements of cash flows. The Company recognized $3.1 million of other income during the year ended December 31, 2025 and $4.5 million and $3.0 million of other expense during the years ended December 31, 2024 and 2023, respectively, associated with changes in the fair value of Verve's common stock.

As of December 31, 2025 and 2024, the Company owned 1,608,337 shares of Prime's common stock valued at $5.6 million and $4.7 million, respectively. The Company recognized $0.9 million of other income and $9.6 million of other expense during the years ended December 31, 2025 and 2024, respectively, associated with changes in the fair value of Prime's common stock.

The following table summarizes other income (expense) incurred due to changes in the fair value of corporate equity securities held (in thousands):

	Year Ended December 31, 2025	2024	2023
Other income (expense)	$ 3,942	$ (14,093)	$ (18,592)

Success Payment Liability – As discussed further in Note 9, the Company is required to make payments to Harvard and Broad Institute based upon the achievement of specified multiples of the initial weighted average value of the Company's Series A Preferred or, subsequent to the IPO, the market value of the Company's common stock, at specified valuation dates. The Company's liability for the share-based success payments under the Harvard and Broad License Agreements are carried at fair value. To determine the estimated fair value of the success payment liability, the Company uses a Monte Carlo simulation methodology, which models the future movement of stock prices based on several key variables.

The following variables were incorporated in the calculation of the estimated fair value of the Harvard and Broad Institute success payment liabilities:

	Harvard		Broad Institute	
	December 31, 2025	December 31, 2024	December 31, 2025	December 31, 2024
Fair value of common stock (per share)	$ 27.72	$ 24.80	$ 27.72	$ 24.80
Expected volatility	71%	78%	75%	81%
Expected term (years)	0.01-3.49	0.03-4.49	0.01-4.36	0.03-5.36

The computation of expected volatility was estimated using the Company's historical volatility along with available information about the historical volatility of stocks of similar publicly traded companies for a period matching the expected term assumption. In addition, the Company incorporated the estimated number, timing, and probability of valuation measurement dates in the calculation of the success payment liability.

The following table reconciles the change in the fair value of success payment liabilities based on Level 3 inputs (in thousands):

	Year Ended December 31, 2025		
	Harvard	Broad Institute	Total
Balance at December 31, 2023	$ 5,200	$ 5,600	$ 10,800
Change in fair value	(1,300)	(1,100)	(2,400)
Balance at December 31, 2024	$ 3,900	$ 4,500	$ 8,400
Change in fair value	(600)	(100)	(700)
Balance at December 31, 2025	$ 3,300	$ 4,400	$ 7,700

Contingent consideration liabilities – On July 1, 2025, the Company acquired an early-stage life sciences company, which was consolidated by the Company under ASC 810, *Consolidation*, as the Company determined that the acquiree is a variable interest entity and that the Company is the primary beneficiary through its 100% ownership interest. The total consideration paid was $14.5 million, which is comprised of an upfront payment of 403,128 shares of the Company's common stock valued at $6.7 million, contingent consideration payments based on the achievement of certain development, clinical and commercial milestones valued at $7.7 million and $0.1 million of seller transaction expenses. The maximum amount of the milestone payments is $89.0 million. The primary asset acquired included in-process research and development valued at $14.5 million upon acquisition. As no alternative future use was identified for the acquired in-process research and development, the Company expensed the full fair value of the asset as research and development expense upon acquisition.

Milestone payments are payable at the Company's sole discretion in cash or in shares of the Company's common stock (valued using a volume-weighted average price). As these milestones are payable with a variable number of shares of the Company's common stock, the milestone payments result in liability classification under ASC 480, *Distinguishing Liabilities from Equity*. These contingent consideration liabilities are carried at fair value which was estimated by applying a probability-based model, which utilized inputs based on timing of achievement that were unobservable in the market. These contingent consideration liabilities are classified within Level 3 of the fair value hierarchy and had a fair value of $7.7 million as of the acquisition date.

The following variables were incorporated in the calculation of the estimated fair value of the contingent consideration liabilities:

	Contingent consideration liability milestones
	December 31, 2025
Discount rate	8.00%
Probability of achievement	2-32%
Projected year of achievement	2026-2037

The following table reconciles the change in fair value of the contingent consideration liabilities based on level 3 inputs (in thousands):

	Year Ended December 31, 2025
	Contingent consideration liability milestones
Balance at July 1, 2025 (inception)	$ 7,715
Change in fair value	951
Balance at December 31, 2025	$ 8,666

Under the Agreement and Plan of Merger, dated February 23, 2021, between the Company and Guide Therapeutics, Inc., or Guide, Guide's former stockholders and optionholders were eligible to receive up to an additional $100.0 million in technology milestone

payments and $220.0 million in product milestone payments, payable in the Company's common stock valued using the volume-weighted average price of the Company's stock over the ten-day trading period ending two trading days prior to the date on which the applicable milestone is achieved. During the year ended December 31, 2025, the Company assessed the fair value of the contingent consideration liabilities related to potential technology and product milestone payments from its prior acquisition of Guide and determined that the fair value of the underlying technology and product milestones was zero. The milestones were removed from the Company's balance sheet as of December 31, 2025. The fair value of the contingent consideration liabilities related to Guide as of December 31, 2024 was $1.1 million and was classified within Level 3 of the fair value hierarchy as of December 31, 2024. During the years ended December 31, 2025, 2024 and 2023, the Company recognized $1.1 million, $1.6 million and $9.7 million of other income, respectively, related to the change in fair value of the Guide technology and product contingent consideration liabilities.

5. Marketable securities

The following table summarizes the Company's marketable securities held at December 31, 2025 (in thousands):

	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Value
Commercial paper	$ 304,861	$ 162	$ (64)	$ 304,959
Corporate notes	149,928	128	(10)	150,046
U.S. Treasury securities	462,112	872	—	462,984
U.S. Government securities	26,673	23	—	26,696
Corporate equity securities	5,581	—	—	5,581
Total	$ 949,155	$ 1,185	$ (74)	$ 950,266

The following table summarizes the Company's marketable securities held at December 31, 2024 (in thousands):

	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Value
Commercial paper	$ 181,095	$ 210	$ (9)	$ 181,296
Corporate notes	100,175	103	(113)	100,165
U.S. Treasury securities	164,491	289	(10)	164,770
U.S. Government securities	114,552	235	(26)	114,761
Corporate equity securities	7,781	—	—	7,781
Total	$ 568,094	$ 837	$ (158)	$ 568,773

The amortized cost of marketable securities is adjusted for amortization of premiums and accretion of discounts to maturity. At December 31, 2025 and 2024, the balance in accumulated other comprehensive (loss) income was comprised solely of activity related to marketable debt securities. During the year ended December 31, 2025, Lilly completed its acquisition of Verve and as a result, the Company received total proceeds of $5.7 million as consideration for its 546,970 shares of Verve's common stock. The sale resulted in a realized loss of $0.4 million, recorded in "Interest and other income (expense), net" in the Company's statements of operations and other comprehensive loss. There were no other realized gains or losses from the sale of marketable securities for the years ended December 31, 2025, 2024 and 2023.

The Company holds debt securities of companies with high credit quality and has determined that there was no material change in the credit risk of any of its debt securities. The contractual maturity dates of all the investments are less than one year.

6. Accrued expenses and other current liabilities

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

	December 31, 2025	December 31, 2024
Employee compensation and related benefits	29,342	21,572
Research costs	15,836	11,179
Professional fees	4,458	3,381
Process development and manufacturing costs	3,646	3,525
Other	1,985	7,811
Total	$ 55,267	$ 47,468

7. Leases

Operating leases

The Company's operating leases are as follows:

- A February 2018 lease for office and laboratory space as amended, which commenced in March 2018 and terminates in September 2029. The lease is subject to fixed-rate rent escalations and provided for $6.1 million in tenant improvements allowances and a term extension option.
- An October 2018 lease for laboratory space as amended, which commenced in April 2019 and terminates in December 2028. The amended lease is subject to fixed-rate rent escalations and provides an option to extend the lease for two additional two-year periods through December 31, 2031.
- An April 2019 lease for office and laboratory space that was built over the course of 2020 and 2021. Pursuant to the terms of the original lease agreement, the first phase of the lease commenced in October 2020 (rent payments for the first phase began in August 2021) and the second phase of the lease commenced in January 2021 (rent payments for the second phase began in February 2022). The lease is subject to fixed-rate rent escalations and provides for $23.4 million in tenant improvements and the option to extend the lease for two terms of five years each. The Company determined that it is the accounting owner of all tenant improvements. In August 2021, the Company executed an amendment to this lease to occupy additional space. The term of this lease runs concurrent with the term of the April 2019 lease through February 2034.
- An August 2020 lease for a 100,000 square foot manufacturing facility in Research Triangle Park, North Carolina. Construction of the manufacturing facility began in 2020 and the Company began making rent payments in the fourth quarter of 2022. The lease will terminate 15 years from the rent commencement date, December 2022. The lease is subject to fixed-rate rent escalations and provides for $20.0 million in tenant improvements and the option to extend the lease for two terms of five years each, which were not reasonably certain of exercise as of December 31, 2025.

The following table summarizes operating lease costs as well as sublease income (in thousands):

	Year Ended December 31,		
	2025	2024	2023
Operating lease costs	$ 21,311	$ 21,649	$ 22,063
Variable lease costs	6,795	6,295	5,777
Short-term lease costs	4,893	9,000	9,000
Sublease income	(487)	—	—
Total	$ 32,512	$ 36,944	$ 36,840

The following table summarizes the lease term and discount rate for operating leases:

	December 31, 2025	December 31, 2024
Weighted-average remaining lease term (years)	8.5	9.5
Weighted-average discount rate	7.3%	7.3%

The following table summarizes the lease costs included in the measurement of lease liabilities (in thousands):

	Year Ended December 31,		
	2025	2024	2023
Operating cash flows used for operating leases	$ 24,428	$ 24,981	$ 22,603
Operating lease liabilities arising from obtaining ROU assets	6,203	(1,626)	3,852

At December 31, 2025, the future maturity of the Company's operating leases for each of the next five years and total thereafter were as follows (in thousands):

2026	$ 24,727
2027	25,436
2028	25,664
2029	22,147
2030	21,009
Thereafter	91,158
Undiscounted lease payments	210,141
Less: imputed interest	(56,018)
Total operating lease liabilities	$ 154,123

8. Strategic restructuring

In October 2023, the Company announced updated portfolio priorities and strategic plans to restructure the Company to streamline its business operations. In connection with this portfolio prioritization and strategic restructuring, the Company reduced its employee headcount by approximately 100 positions, or about 20% of its workforce. During the year ended December 31, 2023, the Company recognized $6.7 million of restructuring charges in the consolidated statement of operations related to these actions. These charges included $6.5 million of one-time termination benefits and contractual termination benefits for severance, healthcare, and related benefits and $0.2 million of non-cash stock-based compensation expense. The workforce reductions were substantially completed as of December 31, 2023 and all severance related costs were paid as of December 31, 2024. There were no restructuring costs incurred during the year ended December 31, 2025.

9. License and other agreements

The Company has various license agreements related to technology used in its research and development activities. The license agreements may include up-front payments, option fees, ongoing maintenance fees, sublicense fees, royalty-based payments, milestone payments, success-based payments, and other payments. Option fees, when applicable, are recognized when exercised, maintenance fees, sublicense fees, and other payments are recorded as incurred based on the estimated amounts due or that will ultimately be paid. Contingent payments that are not required to be accounted for as a derivative are recognized as incurred. As the success-based payments due under the Company's license arrangements are derivatives, the change in the fair value of the success-based payments are recognized in a separate line item in the statement of operations and comprehensive loss, as discussed further below. The total contingent obligations and non-royalty sublicense fees included in research and development expenses in the statement of operations and comprehensive loss were $0.5 million, $3.2 million, and $43.5 million for the years ended December 31, 2025, 2024, and 2023, respectively.

The value attributable to sublicenses and the related sublicense fees due under the Company's license agreements may require estimates and other judgments related to contractual requirements, which creates uncertainty over the ultimate amount that would be paid under these arrangements. Contractual amounts due are accrued and if a contingency exists related to the interpretation of the amounts due under the license agreement, the Company recognizes a liability for the amount that is probable and estimable. When no amount within the range of potential payments is a better estimate than any other amount, however, the minimum amount in the range is accrued.

Harvard license agreement

In June 2017, the Company entered into the Harvard License Agreement for certain base editing technology pursuant to which the Company received an exclusive, worldwide, sublicensable, royalty-bearing license under specified patent rights to develop and commercialize licensed products and a nonexclusive, worldwide, sublicensable, royalty-bearing license under certain patent rights to research and develop licensed products. The Company agreed to use commercially reasonable efforts to develop licensed products in accordance with the development plan, to introduce any licensed products that gain regulatory approval into the commercial market, to market licensed products that have gained regulatory approval following such introduction into the market, and to make licensed products that have gained regulatory approval reasonably available to the public. The license term extends until the later of the expiration of (i) the last to expire licensed patent covering a licensed product, (ii) the period of exclusivity associated with a licensed product or (iii) a certain period after the first commercial sale of a licensed product, unless terminated earlier by either party under certain provisions.

Partial consideration for the rights granted under the Harvard License Agreement include success payments, which are further described below.

Success Payments – Under the Harvard License Agreement, Harvard is entitled to receive success payments, in cash or shares of Company stock, determined based upon the achievement of specified multiples of the initial weighted average value of the Company's Series A Preferred at specified valuation dates. The success payments range from $5.0 million to a maximum of $105.0 million and have valuation multiples that range from 5 times to 40 times the initial weighted average value of the Series A Preferred. Subsequent to the IPO, the amount of success payments is based on the market value of the Company's common stock.

The Company is required to make success payments to Harvard during a period of time, or the Harvard Success Payment Period, which has been determined to be the later of (1) the ninth anniversary of the Harvard License Agreement or (2) the earlier of (a) the twelfth anniversary of the Harvard License Agreement and (b) the third anniversary of the first date on which a licensed product receives regulatory approval in the United States. During the Harvard Success Payment Period, the Company will perform a calculation of any amounts owed to Harvard on each rolling 90-day period, commencing one year after the IPO.

In May 2021, the first success payment measurement occurred and amounts due to Harvard were calculated to be $15.0 million. The Company elected to make the payment in shares of the Company's common stock and issued 174,825 shares of the Company's common stock to settle this liability on June 10, 2021. The Company may owe Harvard success payments of up to an additional $90.0 million. As of December 31, 2025 and 2024, no success payments were due to Harvard.

The following table summarizes the Company's success payment liability for Harvard (in thousands):

	December 31, 2025	December 31, 2024
Harvard success payment liability	$ 3,300	$ 3,900

The following table summarizes the expense (income) resulting from the change in the fair value of the success payment liability for Harvard (in thousands):

	Year Ended December 31,		
	2025	2024	2023
Change in fair value of Harvard success payment liability	$ (600)	$ (1,300)	$ (3,800)

Other Payments – The Company agreed to pay Harvard an annual license maintenance fee ranging from low-to-mid five figures to low six figures, depending on the calendar year. The Company is responsible for the payment of certain patent prosecution and maintenance costs incurred by Harvard related to licensed patents. To the extent achieved, the Company is obligated to pay up to an aggregate of $75.9 million in product development and regulatory approval milestones, or Harvard Product Milestones. If the Company completes a change of control during the term of the Harvard License Agreement, then certain of the milestone payments would be increased. To the extent there are sales of a licensed product, the Company is required to pay low single digit royalties on net sales. The Company is entitled to certain reductions and offsets on these royalties with respect to a licensed product in a given country. If the Company sublicenses its rights to develop or commercialize a licensed product under the Harvard License Agreement to a third party and the Company receives non-royalty sublicense income, then Harvard is entitled to a percentage of such consideration, ranging from the high single digits to low double digits depending on the date in which such sublicense agreement is executed and the stage of development of the Company's licensed products at such time.

The annual maintenance fees are recorded as an expense on an annual basis based on the stated amount for the applicable year. Annual patent costs are expensed as incurred. Upon determination that a Harvard Product Milestone is probable to occur, the amount due will be recorded as research and development expense. The Company will monitor the Harvard Product Milestone payments for this arrangement on an ongoing basis.

To the extent products are commercialized under the Harvard License Agreement, the Company will accrue royalty expense and sublicense nonroyalty payments, as applicable, for the amount it is obligated to pay, with adjustments as sales are made.

Broad license agreement

In May 2018, the Broad License Agreement was entered into with Broad Institute for certain gene editing technology. Under the Broad License Agreement, Broad Institute granted exclusive and non-exclusive worldwide, sublicensable, royalty-bearing licenses under specified patent rights to develop and commercialize licensed product and a nonexclusive, worldwide, sublicensable, royalty-bearing license under certain patent rights to research and develop licensed products. Under the agreement the Company shall use commercially reasonable efforts to develop licensed products in accordance with the development plan, to introduce any licensed products that gain regulatory approval into the commercial market, to market licensed products that have gained regulatory approval following such introduction into the market, and to make licensed products that have gained regulatory approval reasonably available to the public. The license term extends until the later of the expiration of (i) the last to expire licensed patent covering a licensed product, (ii) the period of regulatory exclusivity associated with a licensed product or (iii) a certain period after the first commercial sale of a licensed product unless terminated earlier by either party under certain provisions.

Additional consideration under the Broad License Agreement included Success Payments, which are further described below.

Success Payments – Under the Broad License Agreement, Broad Institute is entitled to receive success payments, in cash or shares of Company common stock, determined based upon the achievement of specified multiples of the initial weighted average value of the Series A Preferred at specified valuation dates. The success payments range from $5.0 million to a maximum of $105.0 million and have valuation multiples that range from 5 times to 40 times the initial weighted average value of the Series A Preferred. Subsequent to the IPO, the amount of success payments is based on the market value of the Company's common stock. The Company is required to make success payments to Broad Institute during a period of time, or the Broad Success Payment Period, which has been determined to be the earliest of (1) the twelfth anniversary of the Broad License Agreement or (2) the third anniversary of the first date on which a licensed product receives regulatory approval in the United States. During the Broad Success Payment Period, the Company will perform a calculation of any amounts owed to Broad Institute on each rolling 90-day period, commencing one year after the IPO.

In May 2021, the first success payment measurement occurred and amounts due to Broad Institute were calculated to be $15.0 million. The Company elected to make the payment in shares of the Company's common stock and issued 174,825 shares of the Company's common stock to settle this liability on June 10, 2021. The Company may owe Broad Institute success payments of up to an additional $90.0 million. As of December 31, 2025 and 2024, no success payments were due to Broad Institute.

The following table summarizes the Company's success payment liability for Broad Institute (in thousands):

	December 31, 2025	December 31, 2024
Broad Institute success payment liability	$ 4,400	$ 4,500

The following table summarizes the expense (income) resulting from the change in the fair value of the success payment liability for Broad Institute (in thousands):

	Year Ended December 31,		
	2025	2024	2023
Change in fair value of Broad Institute success payment liability	$ (100)	$ (1,100)	$ (3,700)

Other Payments – The Company agreed to pay Broad Institute an annual license maintenance fee ranging from low-to-mid five figures to low six figures, depending on the particular calendar year. The Company is responsible for the payment of certain patent prosecution and maintenance costs incurred by Broad Institute related to licensed patents. To the extent achieved, the Company is obligated to pay up to an aggregate of $75.9 million in product development and regulatory approval milestones, or Broad Product Milestones. If the Company completes a change of control during the term of the Broad License Agreement, then certain of the milestone payments would be increased. To the extent there are commercial sales of a licensed product, the Company is required to pay low single digit royalties on net sales. The Company is entitled to certain reductions and offsets on these royalties with respect to a licensed product in a given country. If the Company sublicenses its rights to develop or commercialize a licensed product under the Broad License Agreement to a third party and the Company receives non-royalty sublicense income, then Broad Institute is entitled to a percentage of such consideration, ranging from the high single digits to low double digits depending on the date in which such sublicense agreement is executed and the stage of development of the Company's licensed products at such time.

The annual maintenance fees are recorded as an expense on an annual basis based on the stated amount for the applicable year. Annual patent costs will be expensed as incurred. Upon determination that a Broad Product Milestone is probable to occur, the amount due will be recorded as research and development expense. The Company monitors the Broad Product Milestone payments for this arrangement on an ongoing basis.

To the extent products are commercialized under the Broad License Agreement, the Company will accrue royalty expense and sublicense nonroyalty payments, as applicable, for the amount it is obligated to pay, with adjustments as sales are made.

Editas license agreement

In May 2018, the Company entered into a license agreement, or the Editas License Agreement, with Editas Medicine, Inc., or Editas. Pursuant to the Editas License Agreement, Editas granted to the Company licenses and options to acquire licenses to certain intellectual property rights owned or controlled by Editas, for specified uses. More specifically, Editas granted to the Company a worldwide, exclusive, sublicensable, license (subject to certain exceptions and conditions) under certain intellectual property controlled by Editas for the use of base editing therapies for the treatment of any field of human diseases and conditions, subject to certain exceptions, or the Beam Field, and the licenses granted or to be granted under the Editas License Agreement, or the Editas Development and Commercialization License. Additionally, Editas granted to the Company a royalty-free, non-exclusive license under certain intellectual property owned or controlled by Editas to perform research activities in the Beam Field, or the Editas Research License. Editas provided the Company with an exclusive option to obtain an Editas Development and Commercialization License to three additional groups of intellectual property owned or controlled by Editas, on a group by group basis, during the specified option period, subject to certain exceptions. Pursuant to the Editas License Agreement, the Company will use commercially reasonable efforts to develop a product that includes the rights licensed to the Company within a specified period of time and to commercialize any such products that have received regulatory approval in certain specified countries.

Additional consideration will be due to Editas if the Company elects to exercise its option to obtain an Editas Development and Commercialization License to any of the three categories of intellectual property underlying the Editas Research License, for a fee ranging from a mid-teen million dollar amount to a low to mid-eight digit dollar amount per group, depending on the timing of the option exercise. Additionally, the Company is required to reimburse Editas for certain payments Editas may be obligated to make under existing Editas license agreements related to the intellectual property being licensed to the Company, including (i) development, regulatory and commercial milestone payments and certain sublicense income payments due as a result of the Editas License Agreement and (ii) a percentage of the annual maintenance fees and patent fees due to certain of the Editas' licensors. In addition, to the extent any products are commercialized under an Editas Development and Commercialization License, the Company would be required to make royalty payments equivalent to the royalties that would be due from Editas to any applicable licensors of Editas related to the sales of such licensed products, plus an additional tiered low- to mid-single digit royalty, depending on whether such licensed product is covered by an Editas-owned patent.

The license rights and option rights granted by Editas to the Company are subject to the terms and conditions of the underlying license agreements that Editas is a party to and under which Editas licensed rights or option rights to the Company and the termination of such in-licenses, as applicable. Unless earlier terminated by either party pursuant to the terms of the agreement, the Editas License Agreement will continue in full force and effect and will expire on a licensed product-by licensed product and country-by-country basis upon the later of (i) the last-to-expire royalty term under any applicable institutional license to Editas and (ii) the date at which such product is no longer covered by a valid claim of a licensed Editas-owned patent in such country. The Company has the right, at its sole discretion, at any time to terminate the Editas License Agreement in its entirety or on a group-by-group of intellectual property basis, upon ninety days written notice to Editas. Upon termination of the Editas License Agreement, all rights and licenses granted by Editas to the Company (including the rights to exercise options and obtain such licenses) will immediately terminate and patents within a group of patents will no longer be deemed licensed patents. Expiration or termination of the Editas License Agreement for any reason does not release either party of any obligation or liability which had accrued, or which is attributable to a period prior to such expiration or termination.

The option exercise fees under the agreement will be recorded as research and development expense, if and when the Company exercises such options. To date, no options have been exercised. The annual maintenance fees are recorded as an expense on an annual basis based on the stated amount for the applicable year. Annual patent costs are expensed as incurred. In addition, the Company is required to make certain development, regulatory and commercial milestone payments to Editas upon the achievement of specified milestones. To the extent applicable, sublicense income payments will be accrued for the amount the Company is obligated to pay under each applicable in-license as amounts are due to Editas. Lastly, to the extent products are commercialized under the Editas License agreement, the Company will accrue royalty expense for the amount it is obligated to pay, with adjustments as sales are made.

Settlement agreement

On July 19, 2024, the Company entered into a settlement agreement with a research institution pursuant to which, in exchange for a release of claims in its favor, the Company agreed, among other things, to pay the research institution an upfront payment of $15.0 million and to make additional payments contingent upon the development and commercialization of BEAM-102 and BEAM-302. These contingent payments consist of certain development, regulatory, and sales-based milestone payments, as well as 1% royalty through 2038. Any amounts due must be settled in cash. The maximum amount of development and regulatory milestone payments under the settlement agreement is $15.0 million, and the maximum amount of sales milestone payments is $35.0 million, per program. The Company paid the $15.0 million upfront payment during the year ended December 31, 2024. Following the adoption of ASU 2025-07 discussed in Note 2, *Accounting Policies*, the Company reassessed its accounting for the settlement agreement and accounts for the settlement liability in accordance with ASC 450. The Company determined that the recognition criteria under ASC 450 was not met as the likelihood of a loss is not considered probable and estimable as of December 31, 2025 and therefore no related liability is recorded as of December 31, 2025.

10. Collaboration and license agreements

Eli Lilly and Company

In October 2023, the Company entered into a Transfer and Delegation Agreement, or the Lilly Agreement, with Eli Lilly and Company, or Lilly, pursuant to which Lilly acquired certain assets and other rights under the Company's amended collaboration and license agreement, or the Verve Agreement, with Verve Therapeutics, Inc., or Verve, including the Company's opt-in rights to co-develop and co-commercialize Verve's base editing programs for cardiovascular disease (see discussion below related to the Verve Agreement). The Company granted Lilly an exclusive sublicense to the Verve technology originally licensed to the Company under the Verve Agreement. Lilly also acquired the right to receive any future milestone or royalty payments payable by Verve under the Verve Agreement and the rights and obligations to designate representatives and participate on the joint steering committee with Verve. The Company received a $200.0 million nonrefundable upfront payment and is eligible to receive up to $350.0 million in potential future development-stage payments upon the completion of certain clinical, regulatory and alliance events. If Lilly does not opt-in to co-develop and co-commercialize a licensed product, Lilly is obligated to pay the Company a percentage of any royalties received from Verve for sales of such product, subject to certain caps on a licensed product-by-licensed product basis.

For a period of six years from the effective date of the Lilly Agreement, Lilly has the right to request the Company to perform any critical research and development services, if Lilly reasonably determines that the Company is uniquely able to provide such services and other conditions are met, including that no other third parties can provide such services. The parties will negotiate an agreement governing the Company's performance of such activity, if any, and the Company will be compensated for any services at approximately cost plus a margin. The Company has not been requested to perform any services and believes it is remote that Beam would be requested to provide any services.

In connection with the Lilly Agreement, the Company and Lilly entered into a Stock Purchase Agreement providing for the sale and issuance of 2,004,811 shares of the Company's common stock to Lilly for an aggregate purchase price of $50.0 million.

The Company received the consideration under the Stock Purchase Agreement of $50.0 million in October 2023 and the upfront payment of $200.0 million in November 2023.

The Lilly Agreement and Stock Purchase Agreement were negotiated at the same time as a package and have been accounted for as one combined contract. The Company accounts for the component of the arrangement to transfer common stock to Lilly under ASC 505, *Equity*, or ASC 505, and the revenue component under ASC 606, as it includes a customer-vendor relationship as defined under ASC 606 and meets the criteria to be considered a contract. The Company first applied the guidance in ASC 505 to measure the fair value of the common stock issued and allocated the remaining consideration to the ASC 606 component of the arrangement.

The overall ASC 606 transaction price as of the inception of the contract was determined to be $216.4 million, which is comprised of the upfront payment of $200.0 million and the residual value of the proceeds received in excess of the fair value of the common stock sold to Lilly of $16.4 million. The fair value of the common stock issued to Lilly was $33.6 million, as determined by management with the assistance of a third-party valuation specialist. There is no variable consideration included in the transaction price at inception. The Company will re-evaluate the transaction price at each reporting period.

The Company concluded that the collaboration rights and licenses to intellectual property have the same pattern and timing of transfer and are transferred as of the effective date of the Lilly Agreement. Lilly's right to request research and development services represents an optional purchase in the agreement that does not constitute a material right. All other items promised to Lilly are immaterial in the context of the Lilly Agreement.

The Company recognized revenue for the performance obligation at a point-in-time in October 2023 as all requirements related to the performance obligation have been completed. Any consideration received related to Lilly's optional purchase of the Company's research and development services will be accounted for as a separate contract if and when the option is exercised in accordance with ASC 606. The Company did not recognize any revenue related to the Lilly Agreement during the year ended December 31, 2025. During the year ended December 31, 2024 the Company recognized $25.0 million in revenue related to the achievement of certain clinical, regulatory and alliance events and during the year ended December 31, 2023 the Company recognized $216.4 million of revenue related to the Lilly Agreement. As of December 31, 2025, there was no deferred revenue remaining related to the Lilly Agreement.

Orbital and Bristol Myers Squibb Company

In September 2022, the Company entered into a License and Research Collaboration Agreement, or the Orbital Agreement, with Orbital Therapeutics, Inc., or Orbital. Under the terms of the Orbital Agreement, the Company agreed to collaborate with Orbital to advance nonviral delivery and ribonucleic acid, or RNA, technology by providing Orbital with certain proprietary materials, a non-exclusive research license to certain RNA technology and nonviral delivery technology controlled by the Company, and by performing research and development support services as outlined in a research plan. The Company also granted Orbital an exploitation license to certain RNA technology and nonviral delivery technology controlled by the Company. The collaboration is managed on an overall basis by a Joint Steering Committee, or JSC, comprised of an equal number of representatives from the Company and Orbital.

In exchange for the licenses and services provided by the Company under the Orbital Agreement, the Company received a non-exclusive research license to certain RNA technology and nonviral delivery technology controlled by Orbital, and research and development support services as outlined in a research plan. Orbital also granted the Company an exploitation license to certain RNA technology and nonviral delivery technology controlled by Orbital. The Company also received 75 million shares of Orbital's common stock at closing.

The research plan had a term of three years and could have been extended for unspecified periods upon mutual agreement between the Company and Orbital. The exploitation licenses were exclusive for an initial research term of three years, which could have been extended for up to two successive one-year periods by mutual agreement between the Company and Orbital. As of December 31, 2025 the initial research term was not extended. Either party may terminate the licenses granted to it under the Orbital Agreement for convenience on a product-by-product basis at any time by providing 90 days' prior written notice.

The Company accounts for the consideration received under the Orbital Agreement under ASC 606, as it includes a customer-vendor relationship as defined under ASC 606 and meets the criteria to be considered a contract. The overall transaction price as of the

inception of the contract was determined to be $25.5 million, which represents the fair value of the Company's equity interest in Orbital's common stock at inception. There is no variable consideration included in the transaction price.

The Company concluded that the research and exploitation licenses are not distinct from the other promises in the Orbital Agreement, and as such the Company determined that the licenses combined with the research and development services, know-how transfers, committee participation and materials transfer represent a performance obligation. The Company recognized revenue associated with the Orbital performance obligation over time as it was satisfied during the term of the Orbital Agreement, which was three years. The Company recognized $6.4 million of revenue during the year ended December 31, 2025 and $8.5 million of revenue during each of the years ended December 31, 2024 and 2023, respectively. As of December 31, 2025, there was no deferred revenue remaining related to the Orbital Agreement.

On December 8, 2025, Bristol-Myers Squibb Company, or BMS, completed an acquisition of Orbital, or the Acquisition. At the closing of the Acquisition, the Company held 75 million shares of Orbital common stock, which were cancelled and converted into $255.1 million in closing cash consideration, plus the right to receive up to approximately $26.3 million in additional cash consideration upon the release, if any, of certain escrows.

Pfizer

In December 2021, the Company entered into a research collaboration agreement, or the Pfizer Agreement, with Pfizer Inc., or Pfizer, focused on the use of certain of the Company's base editing technology to develop *in vivo* therapies for rare genetic diseases. Under the terms of the Pfizer Agreement, the Company conducted all research activities through development candidate selection for three base editing programs that target specific genes corresponding to specific diseases that were the subject of such programs. Pfizer had exclusive rights to license each of the three programs at no additional cost, each an Opt-In Right, and would assume responsibility for subsequent development, manufacturing and commercialization. In December 2025, at the completion of the research term, Pfizer exercised its Opt-In Right to an exclusive, worldwide license for a liver-targeted development candidate in the collaboration.

At the end of the Phase 1/2 clinical trials, the Company may elect to enter into a global co-development and co-commercialization agreement with Pfizer with respect the development candidate licensed under the collaboration for an option exercise fee equal to a percentage of the applicable development costs incurred by Pfizer, or the Participation Election. In the event the Company elects to exercise its Participation Election, upon the payment of its option exercise fee, Pfizer and the Company would share net profits as well as development and commercialization costs in a 65%/35% (Pfizer/Company) split for such program. The research collaboration was managed on an overall basis by a Joint Research Committee, or JRC, formed by an equal number of representatives from the Company and Pfizer.

At the inception of the Pfizer Agreement, the Company was entitled to receive a nonrefundable upfront payment of $300.0 million in consideration for the rights granted to Pfizer under the collaboration. As a result of Pfizer's exercise of its Opt-In Right for a liver-targeted development candidate, the Company is eligible to receive development, regulatory, and commercial milestones of up to $350.0 million for the licensed program, plus royalty payments on global net sales of the program, if any. As Pfizer did not exercise its Opt-In Right for the remaining two programs, the Company's rights in such programs reverted to the Company and the Company will be required to pay Pfizer earn-out payments equal to a low single digit percentage of net sales earned on each such program for a ten-year period, if any.

The collaboration had an initial term of four years and could have been extended for an additional year on a program-by-program basis. During the collaboration term, Pfizer had a one-time option to substitute a disease that is the subject of a specific program with one pre-defined substitute disease. Pursuant to the terms of the collaboration agreement, the Company's obligation to provide services under the collaboration agreement concluded during the 2025 fiscal year as Pfizer did not elect to extend the agreement beyond the initial term.

The Company accounts for the Pfizer Agreement under ASC 606, as it includes a customer-vendor relationship as defined under ASC 606 and meets the criteria to be considered a contract.

The overall transaction price as of the inception of the contract was determined to be $300.0 million, which is comprised entirely of the nonrefundable upfront payment. There is no variable consideration included in the transaction price at inception as the future milestone payments are fully constrained and the Company is not required to estimate variable consideration for the royalty payments at contract inception. The Company re-evaluated the transaction price in each reporting period.

The Company has concluded that the licenses to its base editing technology, including the exclusive development and commercialization rights, are not capable of being distinct from the other performance obligations, and as such the Company has determined that the licenses combined with the other research and development services represent performance obligations and no up-front revenue was recognized for the licenses.

The selling price of each performance obligation was determined based on the Company's estimated standalone selling price, or the ESSP. The Company developed the ESSP for all of the performance obligations included in the Pfizer Agreement by determining the total estimated costs to fulfill each performance obligation identified with the objective of determining the price at which it would sell

such an item if it were to be sold regularly on a standalone basis. The Company allocated the stand-alone selling price to the performance obligations based on the relative standalone selling price method.

The Company recognized revenue for each performance obligation as it was satisfied during the term of the Pfizer Agreement using an input method. The Company allocated the transaction price of $300.0 million to each of the three performance obligations, which included each of the three base editing programs combined with the research and development services, licenses, and exclusive development and commercialization rights. Revenue was recognized using an input method based on the actual costs incurred as a percentage of total estimated costs towards satisfying the performance obligation as this method provided the most faithful depiction of the entity's performance in transferring control of the goods and services promised to Pfizer and represented the Company's best estimate of the period of the obligation. The impact on revenue of changes in total estimated costs were recognized on a cumulative basis in the period that the change occurred. If estimates of the total cost change, the required adjustments to revenue could be material. As a result of Pfizer's decision to not extend the research period, the Company recognized the remaining deferred revenue balance during the year ended December 31, 2025. The Company recognized $109.1 million, $8.4 million and $134.3 million of revenue under the Pfizer Agreement, during the years ended December 31, 2025, 2024 and 2023, respectively. There was no remaining deferred revenue related to the Pfizer Agreement as of December 31, 2025.

Apellis Pharmaceuticals

In June 2021, the Company entered into a research collaboration agreement, or the Apellis Agreement, with Apellis Pharmaceuticals, Inc., or Apellis, focused on the use of certain of the Company's base editing technology to discover new treatments for complement system-driven diseases. Under the terms of the Apellis Agreement, the Company will conduct preclinical research on six base editing programs that target specific genes within the complement system in various organs, including the eye, liver, and brain. Apellis has an exclusive option to license any or all of the six programs, or in each case, an Opt-In Right, and collectively, the Opt-In Rights, and will assume responsibility for subsequent development. As of September 30, 2025, Apellis notified the Company of its decision to opt-in to one of the six base editing programs. As a result of Apellis' decision to opt-in to the program, the Company received a cash opt-in fee of $3.8 million which was recognized as revenue during the year ended December 31, 2025. The Company may elect to enter into a 50-50 U.S. co-development and co-commercialization agreement with Apellis with respect to one program licensed under the collaboration. The collaboration is managed on an overall basis by an alliance steering committee formed by an equal number of representatives from the Company and Apellis.

As part of the collaboration, the Company received a total of $75.0 million in upfront and near-term milestones from Apellis, which was comprised of $50.0 million received upon signing and an additional $25.0 million payment on June 30, 2022, the one-year anniversary of the effective date of the Apellis Agreement, or the First Anniversary Payment. Following any exercise of an Opt-In Right for any of the six programs, the Company will be eligible to receive development, regulatory, and sales milestones from Apellis, as well as royalty payments on sales. The collaboration has an initial term of five years and may be extended up to two years on a per year and program-by-program basis. During the collaboration term, Apellis may, subject to certain limitations, substitute a specific complement gene and/or organ for any of the initial base editing programs. Apellis may terminate the Apellis Agreement for convenience on any or all of the programs by providing prior written notice.

The Company accounts for the Apellis Agreement under ASC 606 as it includes a customer-vendor relationship as defined under ASC 606 and meets the criteria to be considered a contract.

The overall transaction price as of the inception of the contract was determined to be $75.0 million, which is composed of the upfront payment of $50.0 million and the First Anniversary Payment of $25.0 million. The Company re-evaluates the transaction price in each reporting period.

The Company concluded that each of the six base editing programs combined with the research and development service, licenses, substitution rights and governance participation were material promises that were both capable of being distinct and were distinct within the context of the Apellis Agreement and represented separate performance obligations. The Company further concluded that the Opt-In Rights and option to extend the collaboration term did not grant Apellis a material right. The Company determined that the term of the contract is five years, as this is the period during which both parties have enforceable rights.

The selling price of each performance obligation was determined based on the Company's ESSP. The Company developed the ESSP for all of the performance obligations included in the Apellis Agreement by determining the total estimated costs to fulfill each performance obligation identified with the objective of determining the price at which it would sell such an item if it were to be sold regularly on a standalone basis. The Company allocated the stand-alone selling price to the performance obligations based on the relative standalone selling price method.

The Company recognizes revenue for each performance obligation as it is satisfied over the five-year term using an input method. The Company allocated the transaction price of $75.0 million to each of the six performance obligations, which includes each of the six base editing programs combined with the research and development service, licenses, substitution rights and governance participation, and is being recognized using an input method based on the actual costs incurred as a percentage of total estimated costs towards satisfying the performance obligation as this method provides the most faithful depiction of the entity's performance in transferring control of the goods and services promised to Apellis and represents the Company's best estimate of the period of the obligation. For

the years ended December 31, 2025, 2024, and 2023, the Company recognized $24.2 million, $19.7 million and $16.4 million of revenue, respectively, related to the Apellis Agreement. As of December 31, 2025, there is $6.7 million of current deferred revenue remaining related to the Apellis Agreement.

Verve

In April 2019, the Company entered into a collaboration and license agreement with Verve, or the Verve Agreement, to investigate gene editing strategies to modify genes associated with an increased risk of coronary diseases and in July 2022, the Company and Verve amended the Verve Agreement. Under the terms of the Verve Agreement, as amended, the Company granted Verve an exclusive license to certain base editor technology and improvements and Verve granted the Company a non-exclusive license under certain know-how and patents controlled by Verve, an interest in joint collaboration technology and a non-exclusive license under certain delivery technology.

In October 2023, the Company, Verve, and Lilly entered into a Side Letter Agreement whereby the Company agreed to delegate to Lilly certain rights and obligations existing under the Verve Agreement, including the Company's rights and obligations to co-develop and co-commercialize opt-in products, the financial rights to milestone and royalty payments, and the right to participate on certain joint decision-making committees. The Company determined that the transfer and delegation of rights represents a modification of the Verve Agreement to be accounted for as if it were part of the existing contract in accordance with ASC 606. Prior to the modification, the Company determined that its performance obligations associated with the Verve Agreement at contract inception and subsequent modifications were not distinct and represented a single performance obligation, and that the obligation would be completed over the performance period of the agreement. Accordingly, the upfront payment was being recognized as revenue using a time-based proportional performance model over the contract term (April 2019 through 2038) of the collaboration, as license revenue. The milestone payments and royalties were considered variable consideration and were constrained up until the modification date as no milestones or royalties were achieved. Subsequent to the modification, management concluded that the Company does not have material ongoing obligations to Verve in the arrangement and that the remaining revenue under the agreement should be recognized at a point-in-time upon modification. Therefore, the Company recognized the remaining $0.3 of unrecognized revenue during the year ended December 31, 2023.

The Company recognized $0.4 million of license revenue during the year ended December 31, 2023 and did not recognize any revenue under the Verve Agreement during the year ended December 31, 2025 or December 31, 2024. As of December 31, 2025 there is no deferred revenue remaining related to the Verve Agreement.

During the year ended December 31, 2025, Lilly, completed its acquisition of Verve and as a result, the Company received total proceeds of $5.7 million as consideration for its 546,970 shares of Verve's common stock. The sale resulted in a realized loss of $0.4 million, recorded in "Interest and other income (expense), net" in the Company's statements of operations and other comprehensive loss. There were no other realized gains or losses from the sale of marketable securities for the years ended December 31, 2025, 2024 and 2023.

11. Common stock

The Company has entered into the Sales Agreement with Jefferies pursuant to which the Company is entitled to offer and sell, from time to time at prevailing market prices, shares of its common stock having aggregate gross proceeds of up to $1.1 billion. The Company agreed to pay Jefferies a commission of up to 3.0% of the aggregate gross sale proceeds of any shares sold by Jefferies under the Sales Agreement. As of December 31, 2025, the Company has sold 13,769,001 shares of its common stock under the Sales Agreement at an average price of $62.75 per share for aggregate gross proceeds of $864.0 million, before deducting commissions and offering expenses payable by it. There were no shares sold under the Sales Agreement during the year ended December 31, 2025.

In March 2025, the Company closed an underwritten public offering of 16,151,686 shares of common stock at a public offering price of $28.48 per share and pre-funded warrants to purchase 1,404,988 shares of common stock at a purchase price of $28.47 per pre-funded warrant for aggregate net proceeds of $470.5 million, after deducting underwriting discounts, commissions and approximately $0.8 million related to legal, accounting and other fees in connection with the offering.

The holders of the Company's common stock are entitled to one vote for each share of common stock. The holders of the Company's common stock shall be entitled to receive ratably dividends out of funds legally available. In the event of any voluntary or involuntary liquidation, dissolution, or winding up of the Company, after the payment or provision for payment of all debts and liabilities of the Company, the holders of common stock shall be entitled to share ratably in the remaining assets of the Company available for distribution.

12. Stock option and grant plan

2017 stock option and grant plan

In June 2017, the Company's board of directors adopted the Beam Therapeutics Inc. 2017 Stock Option and Grant Plan, or the 2017 Plan, which provided for the grant of qualified incentive stock options and nonqualified stock options, restricted stock or other awards to the Company's employees, officers, directors, advisors, and outside consultants for the issuance or purchase of shares of the Company's common stock.

The 2017 Plan is administered by the board of directors. Stock options awarded under the 2017 Plan expire 10 years after the grant date. Certain options provide for accelerated vesting if there is a change in control, as defined in the 2017 Plan.

2019 incentive plan

In October 2019, the Company's board of directors adopted the Beam Therapeutics Inc. 2019 Equity Incentive Plan, or the 2019 Plan, and, following the IPO, all equity-based awards are granted under the 2019 Plan. The 2019 Plan provides for the grant of qualified and nonqualified stock options, stock appreciation rights, restricted and unrestricted stock and stock units, performance awards, and other share-based awards to the Company's employees, officers, directors, advisors, and outside consultants.

The maximum number of shares of the Company's common stock that may be issued under the 2019 Plan was initially 3,700,000 shares, or the Share Pool, plus the number of shares of the Company's common stock underlying awards under the 2017 Plan, not to exceed 5,639,818 shares, that become available again for grant under the 2017 Plan in accordance with its terms. The Share Pool will automatically increase on January 1st of each year from 2021 to 2029 by the lesser of (i) four percent of the number of shares of the Company's common stock outstanding as of the close of business on the immediately preceding December 31st and (ii) the number of shares determined by the Company's board of directors on or prior to such date for such year.

As of December 31, 2025, the Company had 15,721,419 shares reserved and 1,888,002 shares available for future issuance under the 2019 Plan.

Stock-based compensation expense recorded as research and development and general and administrative expenses in the consolidated statements of operations and other comprehensive loss is as follows (in thousands):

	Year Ended December 31,		
	2025	2024	2023
Research and development	$ 56,123	$ 73,523	$ 57,812
General and administrative	38,121	47,139	40,835
Total stock-based compensation expense	$ 94,244	$ 120,662	$ 98,647

Stock options

The assumptions used in the Black-Scholes option-pricing model for stock options granted were:

	Years Ended December 31,		
	2025	2024	2023
Expected volatility	79.7%-80.9%	76.2%-78.5%	76.3-78.8%
Weighted-average risk-free interest rate	4.31%	4.02%	3.73%
Expected dividend yield	0.00%	0.00%	0.00%
Expected term (in years)	5.99	6.02	6.03

The following table provides a summary of option activity under the Company's equity award plans:

	Number of options	Weighted average exercise price	Weighted average remaining contractual life (years)	Aggregate intrinsic value (1) (in thousands)
Outstanding at December 31, 2024	9,605,542	$ 38.62	7.1	$ 37,182
Granted	2,723,284	24.51		
Exercised	(337,770)	14.13		
Forfeited	(674,507)	43.30		
Outstanding at December 31, 2025	11,316,549	35.68	6.8	53,845
Exercisable as of December 31, 2025	7,377,984	$ 40.45	5.8	$ 42,179

(1) The aggregate intrinsic value is calculated as the difference between the exercise price of the underlying options and the closing price of the common stock for the options that were in the money as of December 31, 2025 and 2024.

The weighted-average grant date fair value per share of stock options granted during the years ended December 31, 2025, 2024 and 2023, was $17.53, $17.13 and $27.88, respectively. The aggregate intrinsic value of stock options exercised during the years ended December 31, 2025, 2024 and 2023 was $3.4 million, $10.7 million and $20.0 million, respectively. The weighted-average exercise price of stock options exercised for the years ended December 31, 2025, 2024 and 2023 was $14.13, $9.94 and $7.69, respectively.

As of December 31, 2025, there was 70.8 million of unrecognized compensation cost related to unvested stock options, which is expected to be recognized over a weighted-average period of approximately 2.3 years.

Restricted stock

The Company issued shares of restricted common stock during the years ended December 31, 2025, 2024 and 2023, which consisted only of restricted stock units. Restricted common stock issued generally vests over a period of two to four years.

Generally, if the holders of restricted stock units cease to have a business relationship with the Company, any unvested restricted stock units will be cancelled.

The following summarizes the Company's restricted stock activity:

	Shares	Weighted-average grant date fair value
Unvested as of December 31, 2024	2,576,855	$ 36.98
Issued	1,380,227	19.88
Vested	(1,048,601)	42.19
Forfeited	(364,629)	25.79
Unvested as of December 31, 2025	2,543,852	$ 27.16

The aggregate fair value of restricted stock that vested during the years ended December 31, 2025, 2024 and 2023 was $22.9 million, $35.6 million and $13.8 million, respectively.

At December 31, 2025, there was approximately $49.7 million of unrecognized stock-based compensation expense related to restricted stock that is expected to vest. These costs are expected to be recognized over a weighted-average remaining vesting period of approximately 2.5 years.

2019 Employee Stock Purchase Plan

In February 2020, the Company's board of directors adopted the Beam Therapeutics Inc. 2019 Employee Stock Purchase Plan, or ESPP, which was approved by the Company's stockholders. Pursuant to the ESPP, certain employees of the Company, excluding consultants and non-employee directors, are eligible to purchase common stock of the Company at a reduced rate during offering periods. The ESPP permits participants to purchase common stock using funds contributed through payroll deductions, subject to a calendar year limit of $25,000 and at a purchase price of 85% of the lower of the fair market value of the Company's common stock on the first trading day of the offering period or on the applicable purchase date, which will be the final trading day of the applicable purchase period.

The Company used the Black-Scholes option valuation model to estimate the fair value of the purchase right under the ESPP on the date of grant. The expected volatility is based on the historical volatility of the Company's common stock for a period of months corresponding with the expected life of the option. The risk-free interest rate is based on the U.S. Treasury yield curve at the time of grant for securities with a maturity period similar to the expected life of the option. The expected life is based on the term of the purchase period for the grants made under the ESPP.

The Company uses the straight-line attribution approach to record the expense over the offering period. Stock-based compensation expense related to the ESPP for the years ended December 31, 2025, 2024 and 2023 was $1.6 million, $1.3 million and $1.3 million, respectively.

The Company issued 174,708, 135,187 and 130,403 shares under the ESPP during the years ended December 31, 2025, 2024 and 2023, respectively. As of December 31, 2025, the Company had 3,590,328 shares available for issuance under the ESPP.

13. Net loss per share attributable to common stockholders

As noted above, for periods in which the Company reports a net loss attributable to common stockholders, potentially dilutive securities have been excluded from the computation of diluted net loss per share as their effects would be anti-dilutive. Therefore, the weighted average number of common shares outstanding used to calculate both basic and diluted net loss per share attributable to common stockholders is the same. The Company excluded the following potential common shares, presented based on amounts outstanding at period end, from the computation of diluted net loss per share attributable to common stockholders because including them would have had an anti-dilutive effect:

	As of December 31,		
	2025	2024	2023
Unvested restricted stock	2,543,852	2,576,855	2,927,152
Outstanding options to purchase common stock	11,316,549	9,605,542	8,276,033
ESPP	100,118	73,399	73,415
Total	13,960,519	12,255,796	11,276,600

The following table summarizes the computation of basic and diluted net loss per share attributable to common stockholders of the Company (in thousands, except share and per share amounts):

	Year Ended December 31,		
	2025	2024	2023
Numerator:			
Net loss	$ (79,992)	$ (376,742)	$ (132,527)
Denominator:			
Weighted average common shares outstanding, basic and diluted	98,905,577	82,313,008	77,151,771
Net loss per common share, basic and diluted	$ (0.81)	$ (4.58)	$ (1.72)

14. Income taxes

The Company's operations are all domestic. As further described in Note 2, Summary of significant accounting policies, the Company has elected to prospectively adopt the guidance in ASU No. 2023-09, Income Taxes (Topic 740): *Improvements to Income Tax Disclosures* or ASU 2023-09. The following table is a reconciliation of the U.S. federal statutory rate of 21% to the Company's effective tax rate for the year ended December 31, 2025 in accordance with the guidance in ASU No. 2023-09 (in thousands):

	For the Year Ended December 31, 2025	
Provision for income taxes at U.S. federal statutory rate	$ (16,799)	21.0%
Tax Credits	(10,168)	12.7%
Changes in valuation allowances	11,750	-14.7%
Non-taxable or non-deductible items:		
Stock-based compensation	10,450	-13.1%
In-process research and development expense	3,046	-3.8%
Nondeductible compensation	1,701	-2.1%
Other	20	0.0%
Effective tax rate	$ —	0.0%

The following table is a reconciliation of the U.S. federal statutory rate of 21% to the Company's effective rate for the years ended December 31, 2024 and 2023 in accordance with the guidance prior to the adoption of ASU 2023-09:

	As of December 31,	
	2024	2023
Federal statutory rate	21.0%	21.0%
State income taxes, net of federal benefit	7.0	13.3
Research and development tax credits	4.7	18.0
Nondeductible/ nontaxable permanent items	(1.6)	(2.5)
Change in valuation allowance	(31.1)	(50.9)
Total	0.0%	-1.1%

The components of the Company’s deferred taxes are as follows (in thousands):

	December 31,	
	2025	2024
Deferred tax assets:		
Net operating loss carryforwards	$ 141,126	$ 48,954
Research and development tax credits	100,617	87,554
Accrued expenses and other	7,850	12,868
Deferred revenue	1,819	38,815
Derivative liabilities	2,104	3,771
Equity compensation	26,256	21,218
Property and equipment	58	—
Amortization	28,412	30,508
Capitalized research	133,984	180,954
Lease liability	42,106	44,096
Total deferred tax assets	484,332	468,738
ROU asset	(27,506)	(28,649)
Property and equipment	—	(267)
Other	(1,497)	(1,563)
Less: valuation allowance	(455,329)	(438,259)
Total	$ —	$ —

During the year ended December 31, 2025, the Company paid no income taxes. For the year ended December 31, 2025, the Company did not record a current tax provision and recorded a current tax provision of less than $0.1 million and $1.4 million of income tax expense for the years ended December 31, 2024 and 2023, respectively, which reflects that Company generated taxable income that was not fully offset by the use of net operating loss carryforwards and tax credits. Management has evaluated the positive and negative evidence bearing upon the realizability of the Company's deferred tax assets and has determined that it is not more likely than not that the Company will recognize the benefits of the deferred tax assets. As a result, the Company has recorded a full valuation allowance at December 31, 2025 and 2024. The valuation allowance increased by $17.1 million and $108.9 million for the years ended December 31, 2025 and 2024, respectively.

Additionally, Sections 382 and 383 of the Internal Revenue Code of 1986, as amended, limit a corporation's ability to utilize tax attributes to the extent the corporation experiences an "ownership change," generally defined as a greater than 50 percentage point change in ownership, measured by value, among 5% or greater shareholders over a rolling three-year testing period. To the extent a corporation experiences an ownership change, utilization of pre-ownership change tax attributes (e.g., net operating losses and general business tax credits) to offset post-ownership change taxable income or taxes, is subject to an annual limitation, generally calculated as the pre-ownership change equity value of the corporation, subject to certain prescribed adjustments, multiplied by the long-term tax exempt rate published monthly by the Internal Revenue Service. The Company completed a Section 382 study as of December 31, 2024, and determined that no historical ownership changes occurred since December 2021. The Company has not completed an analysis through December 31, 2025. To the extent there was a change in control during 2025, the Company's attributes could be subject to limitation. The Company may experience ownership changes in the future as a result of shifts in stock ownership.

As of December 31, 2025 and 2024, the Company had $511.7 million of federal net operating loss carryforwards compared to $181.5 million as of December 31, 2024. Additionally, as of December 31, 2025, the Company had $71.9 million of federal and $36.4 million of Massachusetts tax credits that expire starting in 2043 and 2038, respectively. As of December 31, 2024, the Company had $61.7 million of federal and $32.7 million of Massachusetts tax credits that expire starting in 2043 and 2038.

As of December 31, 2025, and 2024, the Company had no uncertain tax positions. The Company recognizes both interest and penalties associated with unrecognized tax benefits as a component of income tax expense. The Company has not recorded any interest or penalties for unrecognized tax benefits since its inception.

The Company filed income tax returns in the United States and the Commonwealth of Massachusetts in all tax years since inception. Tax years beginning in 2022 remain open to examination in these jurisdictions, as carryforward attributes generated in past years may be adjusted in a future period.

15. Employee benefits

In 2018, the Company established a defined-contribution plan under Section 401(k) of the Internal Revenue Code, or the 401(k) Plan. The 401(k) Plan covers all employees who meet defined minimum age and service requirements and allows participants to defer a portion of their annual compensation on a pre-tax basis. Beginning January 1, 2020, the Company made matching contributions equal to 50% of the employee's contributions, subject to a maximum of 6% of eligible compensation. The Company made matching contributions of $2.6 million, $2.3 million, and $2.5 million for the years ended December 31, 2025, 2024, and 2023 respectively.

16. Segment Data

The Company defines its segments on the basis of the way in which internally reported financial information is regularly reviewed by the chief operating decision maker, or CODM, to analyze financial performance, make decisions, and allocate resources. The Company's CODM is John Evans, its Chief Executive Officer. The Company manages its operations as a single operating and reportable segment and the measure of segment profit or loss is consolidated net income (loss). The CODM uses net income (loss) in the budget and forecasting process and considers budget-to-actual variances on a quarterly basis when making decisions about the allocation of operating and capital resources.

The internal reporting of significant segment expenses is based on the functional classification. External expenses include costs from external manufacturing, clinical and research organizations, supply chain and logistics costs, consultants, and other vendors. Employee related expenses include, employee salaries and benefits costs, employee meal, travel and entertainment spend, along with payroll related taxes and other similar items. These functional costs exclude stock-based compensation, facility and information technology costs, depreciation and amortization, and other segment items.

The table below provides information about the Company's segment, including significant expenses, other segment items, certain other segment expenses, and a reconciliation to net income (loss):

	Year Ended December 31,		
	2025	2024	2023
License and collaboration revenue	$ 139,743	$ 63,518	$ 377,709
Research and development expenses			
External research and development expenses*	142,695	115,189	155,063
Employee related expenses*	117,366	98,796	109,878
General and administrative expenses			
External general and administrative expenses*	28,319	25,115	40,016
Employee related expenses*	43,124	34,822	31,305
Facility and information technology related expenses*	59,093	55,911	53,804
Depreciation and amortization	22,294	21,925	20,012
Stock-based compensation	94,244	120,662	98,647
Interest and other income	(43,733)	(49,094)	(46,676)
Income tax expense	—	39	1,366
Other segment items	(243,667)	16,895	46,821
Net income (loss)	$ (79,992)	$ (376,742)	$ (132,527)

* Denotes significant segment expense

Other Segment Items includes:

- Change in fair value of derivative liabilities
- Change in fair value of non-controlling equity investments
- Change in fair value of contingent consideration liabilities
- Gain on sale of equity method investment
- Milestone expense
- License and Sublicenses fees
- In-process research and development charges

17. Subsequent Events

On February 24, 2026, or the Closing Date, the Company entered into a financing agreement, or the Financing Agreement, with certain of its subsidiaries as guarantors party thereto, the lenders party thereto, or the Lenders, and Sixth Street Lending Partners, as the administrative agent and collateral agent for the Lenders. The Financing Agreement provides for a senior secured term loan facility of up to $500 million, or the Credit Facility, consisting of (i) an initial draw of $100 million on the Closing Date, (ii) a potential additional $100 million draw upon the acceptance by the FDA of our BLA submission for risto-cel prior to a certain date, or the Delayed Draw A, (iii) a potential additional $100 million draw at the Company's option upon the FDA's approval of the risto-cel BLA prior to a certain date, or the Delayed Draw B, (iv) a potential additional $100 million draw at the Company's option upon achieving a revenue target from sales of risto-cel prior to a certain date and (v) a potential additional $100 million draw subject to agreement among the Company and the Lenders. The Credit Facility matures on February 24, 2033, or the Maturity Date, and bears interest at an annual rate equal to the 3-month Secured Overnight Financing Rate (SOFR) plus 6.50% (subject to a 1.00% floor) or permits interest

on a base rate plus a margin. Certain additional commitment, administrative, undrawn amount and facility fees are also payable in connection with the Credit Facility.

The Credit Facility requires quarterly interest payments, but does not provide for scheduled amortization payments during the term. All principal will be due on the Maturity Date. The Company will have the right to prepay loans under the Credit Facility at any time. The Company is required to repay loans under the Credit Facility with proceeds from certain asset sales and licensing transactions, condemnation events and extraordinary receipts, subject, in some cases, to reinvestment rights. Repayments are subject, in some cases, to prepayment premiums.

All obligations under the Financing Agreement will be secured on a first-priority basis, subject to certain exceptions, by security interests in substantially all assets of the Company and its material subsidiaries, including its intellectual property, and will be guaranteed by the Company's material subsidiaries, subject to certain exceptions.

The Financing Agreement contains customary covenants, including, without limitation, a financial covenant to maintain liquidity of at least $40 million (which shall increase to $80 million upon the draw of the Delayed Draw A and $125 million upon the draw of the Delayed Draw B) if the Company's market capitalization is below $1.75 billion, a covenant to use commercially reasonable efforts to develop and commercialize risto-cel and negative covenants that, subject to certain exceptions, restrict the Company's ability to incur additional indebtedness, grant liens, make investments (including acquisitions), effectuate mergers or consolidations, engage in asset sales and licensing transactions, pay dividends, modify material agreements, pay subordinated indebtedness, and undertake other matters customarily restricted in such agreements. Among other permissions, the Company is permitted, on terms and conditions set forth in the Financing Agreement, to have outstanding convertible unsecured notes in an amount not to exceed $400 million. The Company is subject to restrictions on sales and licensing transactions with respect to its core intellectual property, including risto-cel, subject to certain exceptions, including certain transactions related to areas outside the United States.

The Financing Agreement also contains certain events of default after which loans under the Credit Facility may be due and payable immediately, including payment defaults, material inaccuracy of representations and warranties, covenant defaults, bankruptcy and insolvency proceedings, cross-defaults to certain other agreements, judgments against the Company and its subsidiaries, and change of control.

Exhibit 23.1

CONSENT OF INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM

We consent to the incorporation by reference in Registration Statement No. 333-277427 on Form S-3 and Registration Statement Nos. 333-236582, 333-254378, 333-263067, 333-270079, 333-277382 and No. 333-285182 on Form S-8 of our reports dated February 24, 2026, relating to the financial statements of Beam Therapeutics Inc., and the effectiveness of Beam Therapeutics Inc.'s internal control over financial reporting appearing in this Annual Report on Form 10-K for the year ended December 31, 2025.

/s/ Deloitte & Touche LLP

Boston, Massachusetts
February 24, 2026

Exhibit 31.1

CERTIFICATION 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

I, John Evans, certify that:

1. I have reviewed this Annual Report on Form 10-K of Beam 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(s) 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(s) 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.

Date: February 24, 2026

By: /s/ John Evans

John Evans
Chief Executive Officer
(Principal executive officer)

Exhibit 31.2

CERTIFICATION 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

I, Sravan Emany, certify that:

1. I have reviewed this Annual Report on Form 10-K of Beam 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(s) 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(s) 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.

Date: February 24, 2026

By: /s/ Sravan Emany

Sravan Emany
Chief Financial Officer
(Principal financial officer)

Exhibit 32.1

CERTIFICATION PURSUANT TO

18 U.S.C. SECTION 1350, AS ADOPTED PURSUANT TO

SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002

In connection with this Annual Report of Beam 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. § 1350, as adopted pursuant to § 906 of the Sarbanes-Oxley Act of 2002, that:

(1) The Report fully complies with the requirements of section 13(a) or 15(d) of the Securities Exchange Act of 1934; and

(2) The information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

Date: February 24, 2026

By: /s/ John Evans

John Evans
Chief Executive Officer
(Principal executive officer)

Exhibit 32.2

CERTIFICATION PURSUANT TO
18 U.S.C. SECTION 1350, AS ADOPTED PURSUANT TO
SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002

In connection with this Annual Report of Beam 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. § 1350, as adopted pursuant to § 906 of the Sarbanes-Oxley Act of 2002, that:

(1) The Report fully complies with the requirements of section 13(a) or 15(d) of the Securities Exchange Act of 1934; and

(2) The information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

Date: February 24, 2026

By: /s/ Sravan Emany
Sravan Emany
Chief Financial Officer
(Principal financial officer)