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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 001-37687

EDITAS MEDICINE, INC.

(Exact name of registrant as specified in its charter)

Delaware **(State or other jurisdiction of incorporation or organization)**	**46-4097528** **(I.R.S. Employer Identification No.)**
11 Hurley Street **Cambridge, Massachusetts** **(Address of principal executive offices)**	**02141** **(Zip Code)**

(617) 401-9000
(Registrant's telephone number, including area code)
Securities registered pursuant to Section 12(b) of the Act:

Title of each class	**Trading Symbol(s)**	**Name of each exchange on which registered**
Common Stock, $0.0001 par value per share	**EDIT**	**The Nasdaq Stock Market LLC**

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

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

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

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

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

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, a smaller reporting company, or an emerging growth company. See 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 Act). Yes ☐ No ☒

As of June 30, 2025, the last business day of the registrant's most recently completed second fiscal quarter, the aggregate market value of the registrant's Common Stock held by non-affiliates of the registrant was approximately $191,377,146 based upon the closing price of the registrant's Common Stock on June 30, 2025.

The number of shares of the registrant's Common Stock outstanding as of February 27, 2026 was 97,871,999.

DOCUMENTS INCORPORATED BY REFERENCE

Portions of the registrant's definitive proxy statement for its 2026 Annual Meeting of Stockholders to be filed pursuant to Regulation 14A within 120 days of the end of the registrant's fiscal year ended December 31, 2025 are incorporated by reference into Part III of this Annual Report on Form 10-K to the extent stated herein.

Editas Medicine, Inc.
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References to Editas

Throughout this Annual Report on Form 10-K, the "Company," "Editas," "Editas Medicine," "we," "us," and "our," except where the context requires otherwise, refer to Editas Medicine, Inc. and its consolidated subsidiary, and "our Board of Directors" refers to the board of directors of Editas Medicine, Inc.

Special Note Regarding Forward-Looking Statements and Industry Data

This Annual Report on Form 10-K contains forward-looking statements regarding, among other things, the initiation, timing, progress and results of our preclinical activities and our research and development programs, the timing for our receipt and presentation of data from our preclinical studies and planned clinical trials, potential of, and expectations for, our product candidates, the timing or likelihood of regulatory filings and approvals, our expectations regarding cash runway, and our business strategy, and other objectives for our operations. The words "anticipate," "believe," "estimate," "expect," "intend," "may," "plan," "predict," "project," "would" and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. There are a number of important risks and uncertainties that could cause our actual results to differ materially from those indicated by forward-looking statements. We may not actually achieve the plans, intentions or expectations disclosed in our forward-looking statements, and you should not place undue reliance on our forward-looking statements. Actual results or events could differ materially from the plans, intentions and expectations disclosed in the forward-looking statements we make. We have included important factors in the cautionary statements included in this Annual Report on Form 10-K, particularly in the section entitled "Risk Factors" in Part I that could cause actual results or events to differ materially from the forward-looking statements that we make. Our forward-looking statements do not reflect the potential impact of any future acquisitions, mergers, dispositions, joint ventures or investments that we may make.

You should read this Annual Report on Form 10-K and the documents that we have filed as exhibits to this Annual Report on Form 10-K completely and with the understanding that our actual future results may be materially different from what we expect. The forward-looking statements contained in this Annual Report on Form 10-K are made as of the date of this Annual Report on Form 10-K, and we do not assume any obligation to update any forward-looking statements, whether as a result of new information, future events or otherwise, except as required by applicable law.

This Annual Report on Form 10-K includes statistical and other industry and market data, which we obtained from our own internal estimates and research, as well as from industry and general publications and research, surveys, and studies conducted by third parties. Industry publications, studies, and surveys generally state that they have been obtained from sources believed to be reliable, although they do not guarantee the accuracy or completeness of such information. While we believe that each of these studies and publications is reliable, we have not independently verified market and industry data from third-party sources. While we believe our internal company research is reliable and the market definitions are appropriate, neither such research nor these definitions have been verified by any independent source.

Risk Factor Summary

- *We are dependent on the success of our lead product candidate, EDIT-401, which is in preclinical development. Development of product candidates may not be successful. If we are unable to commence and complete the clinical development of, obtain marketing approval for, or successfully commercialize EDIT-401, either alone or with a collaborator, or if we experience significant delays in doing so, our business would be substantially harmed.*
- *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 commercialization efforts.*
- *We have never generated revenue from product sales and may never be profitable.*
- *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.*
- *The gene editing field is relatively new and is evolving rapidly. We are focusing our research and development efforts on CRISPR gene editing technology using Cas9 and Cas12a enzymes, but other gene editing technologies may be discovered that provide significant advantages over CRISPR/Cas9 or CRISPR/Cas12a, which could materially harm our business.*
- *All of our ongoing product development programs are at the preclinical or research stage. Preclinical testing and clinical trials of product candidates, including EDIT-401, may not be successful. If we are unable to commercialize any product candidates we develop or experience significant delays in doing so, our business will be materially harmed.*
- *If serious adverse events, undesirable side effects, or unexpected characteristics are identified during the development of any product candidates we may develop, we may need to abandon or limit our further clinical development of those product candidates, and it may delay or prevent their regulatory approval, limit the commercial potential, or result in significant negative consequences following any potential marketing approval.*
- *We have not tested any of our proposed delivery modes, combined with our product candidates, in clinical trials and have not begun clinical trials in any of our current development programs.*
- *Adverse public perception of genomic medicines, and gene editing in particular, may negatively impact regulatory approval of, or demand for, our potential products.*
- *We face significant competition in an environment of rapid technological change, and our competitors may achieve regulatory approval before us or develop therapies that are safer or more advanced or effective than ours.*
- *Due to the novel nature of our technology and the potential for some of our product candidates 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.*
- *Genomic medicines are novel, and our product candidates can be complex and difficult to manufacture. We could experience production problems that result in delays in our development or commercialization programs, limit the supply of our products, or otherwise harm our business.*
- *We expect to depend on collaborations with third parties for the research, development, and commercialization of certain of the product candidates we develop or for development of certain of our research programs, and to conduct our future clinical trials and some aspects of our research and preclinical testing.*

- *If we are unable to obtain and maintain patent protection for any products we develop and for our technology, or if the scope of the patent protection obtained is not sufficiently broad, our competitors could develop and commercialize products and technology similar or identical to ours.*
- *Our rights to develop and commercialize our technology and product candidates are subject, in part, to the terms and conditions of licenses granted to us by others.*
- *Some of our in-licensed patents are subject to priority and validity disputes. Our owned and in-licensed patents, patent applications and other intellectual property may be subject to further priority and validity disputes, and other similar intellectual property proceedings including inventorship disputes.*
- *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 of our product candidates.*
- *Our future success depends on our ability to attract and retain key executives and to attract, retain, and motivate qualified personnel.*
- *The market price of our common stock has been, and is likely to remain volatile.*

PART I

Item 1. Business

We are a pioneering gene editing company dedicated to developing potentially transformative genomic medicines to treat a broad range of serious diseases. The promise of genomic medicines is supported by the advancing knowledge of the human genome and by harnessing the progress in technologies for cell therapy, gene therapy, and, most recently, gene editing. We believe this progress sets the stage for us to create medicines with the potential to have a durable benefit for patients. Our core capability in gene editing uses the technology known as CRISPR (clustered, regularly interspaced, short palindromic repeats) to allow us to create molecules that efficiently and specifically edit DNA. Our mission is to translate the promise of gene editing into a broad class of differentiated, transformational medicines for previously untreatable diseases.

We have developed a proprietary gene editing platform based on CRISPR technology and we continue to expand its capabilities. CRISPR uses a protein-RNA complex composed of an enzyme, including either Cas9 (CRISPR associated protein 9) or Cas12a (CRISPR from *Prevotella* and *Francisella* 1, also known as Cpf1), bound to a guide RNA molecule designed to recognize a particular DNA sequence. Once the complex binds to the DNA sequence it is designed to recognize, the complex makes a specific cut in the DNA. We believe we are the only human gene editing company with a platform that includes CRISPR/Cas9, CRISPR/Cas12a, engineered forms of both of these CRISPR systems, and foundational intellectual property for both of these CRISPR systems. Because of the broad nature of this platform, we believe we can create gene editing molecules for more than 95% of the human genome.

Our Strategy

We seek to be a leader in *in vivo* gene editing, leveraging cutting edge gene editing technology to deliver transformative therapies that simplify the usability for patients, minimize the burdens to patients and healthcare systems, and are meaningfully differentiated from the current standards of care, while also allowing a simple, scalable manufacturing process to reduce costs to manufacture and administer the therapies.

Our approach is focused on the *in vivo* use of functional upregulation, which aims to increase the expression of a normal gene copy and its normal protein function to treat diseases caused by genetic mutations that eliminate or disrupt normal function. This approach edits non-coding regulatory sequences upstream and downstream of a gene and does not alter the sequence of the encoded protein, unlike other approaches, including gene knockdown, which aims to reduce the expression of one or more targeted genes by editing coding regions to knockdown a disease-causing protein, and gene correction, which aims to edit coding regions to correct a disease-causing protein. We believe this approach will allow us to address those diseases where the gene knockdown approach has limited effectiveness and to create treatments designed for most or all patients with the same disease, regardless of the specific genetic mutation causing the disease.

We believe the ability to provide *in vivo* gene editing, in which the medicine is injected or infused into the patient to edit the cells inside their body, and functionally upregulate normal gene expression and normal protein function in the target tissues holds the potential to significantly expand the addressable therapeutic possibilities of CRISPR-based gene editing. To that end, our preclinical efforts are also focused on the creation of a “plug ‘n play” lipid nanoparticle (“LNP”) platform to enable targeted delivery of *in vivo* gene editing medicines to multiple cells and tissues, including the liver, hematopoietic stem cells (“HSCs”), and other cells and tissues.

In September 2025, we announced the nomination of our lead *in vivo* development candidate, EDIT-401, an experimental, potential best-in-class, one-time therapy to significantly reduce LDL-cholesterol (“LDL-C”) through upregulation of the LDL receptor (“LDLR”). EDIT-401 is designed to treat elevated levels of LDL-C, or hyperlipidemia, by directly editing the noncoding region of the LDLR gene to increase LDLR protein expression and reduce LDL-C levels. This targeted approach has demonstrated an approximately 90% mean reduction of LDL-C in non-human primates (“NHPs”) in our preclinical studies with favorable tolerability data and supports the potential of EDIT-401 to deliver meaningful clinical outcomes for patients underserved by current lipid-lowering therapies. We are on track to submit an investigational new drug application (“IND”) or foreign equivalent to conduct a clinical trial of EDIT-401 in patients with heterozygous familial hypercholesteremia (“HeFH”), a common inherited genetic disorder causing extremely high LDL-C, by mid-2026 with the expectation of achieving early human proof-of-concept data for EDIT-401 by the end of 2026. We plan to complete enrolling the dose-finding portion of the first-in-human clinical trial with topline data results available in 2027. We expect to present additional preclinical data for EDIT-401 by mid-2026.

We also previously demonstrated proof-of-concept of our functional upregulation strategy in our former clinical trials of renizgamglogene autogedtemcel ("reni-cel"), an experimental *ex vivo* gene-edited medicine to treat sickle cell disease ("SCD"), a severe inherited blood disease that causes vaso-occlusive events and target organ damage, and often leads to premature death, and transfusion-dependent beta thalassemia ("TDT"), the most severe form of beta-thalassemia, an inherited blood disorder characterized by severe anemia. Despite the robust and clinically meaningful improvements observed in these trials, we determined in December 2024 not to pursue commercialization for reni-cel in order to optimize our cost structure and accelerate our intent to achieve human proof of concept of our *in vivo* gene editing medicines. Leveraging our differentiated approach from our former reni-cel program, we are developing an experimental *in vivo* gene-editing medicine to edit HSCs through targeted delivery of our AsCas12a enzyme to our clinically validated HBG1 and HBG2 promoter site.

Our *in vivo* discovery and development efforts further include other cells and tissues, with the goal of selecting therapeutic targets we believe have a significant probability of technical, clinical, regulatory, and commercial success. We previously announced *in vivo* delivery to two additional cell types in humanized mice using our proprietary LNP targeting platform, demonstrating the "plug 'n play" potential of our proprietary extrahepatic LNP platform.

Licensing and Business Development

We are pursuing the right combination of gene editing and targeted delivery tools through internal development and the in-licensing of complementary technologies to build our preclinical pipeline, including our "plug 'n play" *in vivo* gene editing LNP delivery platform, and accelerate the achievement of our goal of delivering lifesaving medicines to patients with previously untreatable diseases. For example, we are party to a collaboration and license agreement accessing LNPs targeting the liver in support of our strategy to upregulate certain liver targets, including LDLR for our EDIT-401 program.

We are also leveraging our gene editing technology to drive the development of CRISPR-based medicines in therapeutic areas outside of our core focus through partnerships and collaborations. For example, our collaboration with Bristol Myers Squibb Company ("BMS") through its wholly owned subsidiary, Juno Therapeutics, Inc. ("Juno Therapeutics") is advancing alpha-beta T-cell experimental medicines for the treatment of solid tumors, liquid tumors, and autoimmune disease in collaboration. This collaboration, which leverages our Cas9 and AsCas12a platform technologies, has resulted in 14 total programs, including BMS' CD19 HD Allo CAR T program for the treatment of autoimmune disease currently in Phase I clinical development. We are also party to a non-exclusive collaboration and licensing agreement with Immatics to combine gamma-delta T cell adoptive cell therapies and gene editing for the treatment of cancer.

In addition, we are leveraging our intellectual property portfolio to drive potential out-licensing opportunities that can provide non-dilutive capital. In December 2023, we and Vertex Pharmaceuticals Incorporated ("Vertex") entered into a license agreement (the "Vertex License Agreement"), under which Vertex obtained a non-exclusive license for our Cas9 gene editing technology for *ex vivo* gene editing medicines targeting the BCL11A gene in the fields of SCD and TDT, including Vertex's CASGEVY™ (exagamglogene autotemcel). We received a $50.0 million upfront cash payment in the fourth quarter of 2023 and the 2024 annual license fee of $10.0 million in the first quarter of 2024. The Vertex License Agreement further provides for the payment by Vertex of a potential additional $50.0 million contingent upfront payment and further future fixed and sales-based annual license fees, ranging from $5.0 million to $40.0 million annually, inclusive of certain sales-based annual license fee increases, through 2034. We are required to pay The Broad Institute, Inc. ("Broad") and the President and Fellows of Harvard College ("Harvard") a mid-double-digit percentage of amounts payable to us from Vertex under the Vertex License Agreement as it relates to Cas9 technology licensed by us from Broad and Harvard. In October 2024, we entered into an agreement (the "DRI Agreement") with a wholly owned subsidiary of DRI Healthcare Trust ("DRI") providing for an upfront cash payment by DRI to us of $57.0 million. Under the DRI Agreement, DRI is purchasing up to 100% of certain future fixed and sales-based annual license fees that the Company is entitled to receive under the Vertex License Agreement, which fees range from $5.0 million to $40.0 million per year, including increases based on sales. In addition, DRI is purchasing a mid-double-digit percentage of a $50.0 million contingent upfront payment that the Company may receive under the Vertex License Agreement. All amounts above will be adjusted to exclude payments that the Company owes to Broad and Harvard. The Company has retained rights to certain portions of certain other sales-based annual license fees and the contingent upfront payment that may become due under the Vertex License Agreement, and the amounts that correspond to our licensor obligations.

Our Core Capability — Gene Editing

Gene editing is the process of revising, removing, or repairing defective DNA *in situ*. In general, gene editing repairs the defective DNA in its native genomic location, and consequently the repaired genetic region retains the cell's normal control and feedback mechanisms. Gene editing typically takes advantage of naturally occurring DNA repair mechanisms, including non-homologous end joining ("NHEJ") and homology directed repair ("HDR"), to achieve its desired therapeutic outcome. Edits that are repaired by NHEJ typically result in an insertion or deletion (an "indel") that can disrupt a regulatory sequence to functionally upregulate normal gene expression and normal protein function. Edits that are repaired by HDR, including targeted insertion, aim to correct or replace aberrant DNA sequences. The diversity of genetic drivers of disease demands a variety of solutions. Gene editing has the potential to deliver a variety of types of genome modification to address a broad range of diseases.

CRISPR technology uses a protein-RNA complex composed of a type of enzyme, referred to as a DNA endonuclease, bound to an RNA molecule, referred to as a guide RNA, that has been designed to recognize a particular DNA sequence. A DNA endonuclease is an enzyme that cleaves DNA. This combination of a DNA endonuclease and a guide RNA only bind and cut DNA when two criteria are met: first, the protein recognizes a short DNA specific to the enzyme called the protospacer adjacent motif ("PAM"), and second, the appropriate portion of the guide RNA matches the adjacent DNA sequence. The PAM sequence that is recognized by the DNA endonuclease creates a second layer of recognition in addition to the guide RNA. We believe that CRISPR technology has three principal advantages for gene editing:

- *Rapid, comprehensive, and systematic identification of product candidates.* The key targeting mechanism for the endonuclease, whether it is Cas9 or Cas12a, is a guide RNA, which can be rapidly replaced with a different guide RNA or optimized by changes as small as a single nucleotide. This allows for the flexible design, synthesis, and testing of hundreds of guide RNA/endonuclease combinations for each genetic target in order to find those that cut the DNA target with the optimal efficiency and specificity. In contrast, other commonly used DNA nucleases for gene editing have inherently limited flexibility. For example, zinc finger nucleases, engineered meganucleases, and transcription activator-like effector nucleases use proteins for DNA sequence recognition to bring the endonuclease to the site of the genome where cleavage is desired, requiring the creation of an entirely new protein for each target site.

- *Simultaneous and efficient targeting of multiple sites.* In CRISPR technology, multiple guide RNAs can be provided along with the same endonuclease, enabling the simultaneous and efficient targeting of multiple sites. This ability to target multiple DNA sequences expands the applicability of CRISPR technology and also creates the potential for self-regulating systems that control exposure to the editing machinery. To address more than one target, other gene editing technologies require the engineering, characterization, manufacture, and delivery of distinct nuclease proteins for each target.

- *Ability to achieve a range of different types of edits.* The inherent differences in Cas9 and Cas12a and the availability of different engineered variants of both enzymes allow for different types of cuts for gene editing. We are able to make a blunt cut, cut either strand of the DNA, or create overhangs of differing length. This may be a critical component of improved HDR-driven approaches because the type of DNA cut can influence the type of repair mechanism used by a cell in response to that cut. We believe the ability to modify CRISPR technology to allow for different types of cuts will expand the potential of our gene editing platform.

Our Gene Editing Platform

We have developed a proprietary gene editing platform that includes different natural and engineered variants of Cas9 and Cas12a. We have characterized different Cas9 and Cas12a enzymes for several reasons. Firstly, a lower molecular weight enzyme will have advantages for delivering the endonuclease using a viral vector due to the inherent size limitations of most such delivery systems. For example, the Cas9 enzyme from *Staphylococcus aureus* Cas9 is significantly smaller than that from *Streptococcus pyogenes* ("*S. pyogenes*" or "SpCas9") (3,159 vs. 4,104 base pairs), and this decreased size is important when working with adeno-associated viral vectors ("AAV") as a delivery vector, which has an effective packaging limit of approximately 4,700 base pairs. Secondly, we have gained access to modified versions of Cas12a and Cas12a guide RNAs that increase Cas12a activity. This increased activity may allow us to use Cas12a editing in more indications where editing at a Cas12a susceptible site is desirable from a biological perspective but technically difficult with the wild-type Cas12a editing system. Thirdly, identifying Cas9 and Cas12a enzymes with different editing properties will expand the number of potential editing sites in the human genome. The range of natural and engineered

variants of Cas9 and Cas12a have significantly expanded the number of sites in the human genome that we can potentially target. As compared to the most commonly used, naturally occurring version of Cas9, from the bacterial species *S. pyogenes*, the range of endonucleases in our platform can target approximately ten times as many genomic sites. Thus, while the *S. pyogenes* Cas9 can target approximately 1 in 10 bases in the human genome, we have the potential to hit over 95% of all bases due to the wide range of endonucleases at our disposal.

The guide RNA molecule is another component of our gene editing platform. We seek to use reprogrammable guide RNA in which changing the targeting region of the guide RNA can create a new product for a new disease target, thus enabling the rapid development of new medicines. We have made substantial advances in the design, synthesis, modification, analysis, and characterization of guide RNAs. For example, in order to accelerate and standardize the selection of guide RNAs, we have created proprietary analytical software that supports guide RNA design through single nucleotide polymorphism analysis, specificity prediction, and assessment of relative importance of potential off target sites. Of critical importance in determining the activity and specificity of an endonuclease-guide RNA complex is understanding the quality and composition of the guide RNA. The ability to understand the quality and composition of the guide RNA is an essential component to developing product candidates that have the potential to be safe and efficacious medicines. In order to understand the absolute composition of our guide RNAs, we utilize state-of-the-art mass spectrometry and sequencing methodologies.

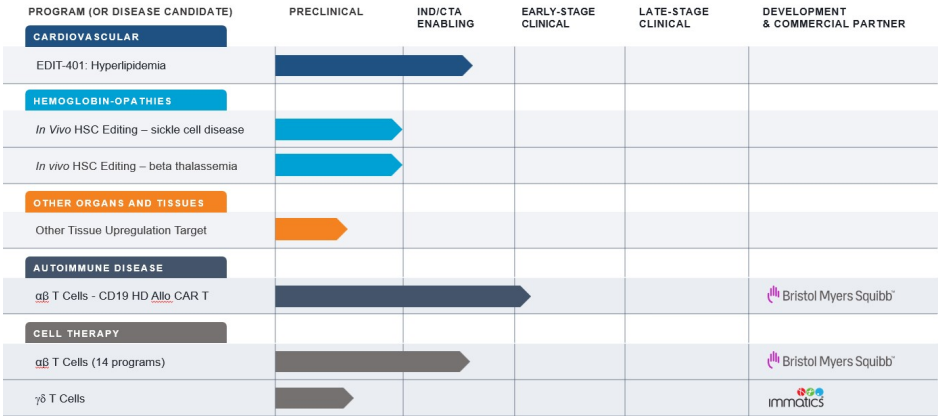
Our gene editing platform includes multiple modular delivery modes that can be efficiently adapted to deliver different CRISPR gene editing components to address the specific needs of each disease targeted. Our strategy is to leverage existing delivery technologies to target cell types of interest while developing next generation capabilities as warranted. We have made substantial advances in the *in vivo* delivery of CRISPR systems to a number of cell types. Our “plug ‘n play” LNP platform is designed to enable targeted delivery of *in vivo* gene editing medicines to multiple cells and tissues, including the liver, HSCs, and other cells and tissues. Our ability to deliver to the liver using this LNP platform enables us to develop new therapies by only reprogramming guide RNAs for specific liver targets. For extrahepatic delivery, our LNP platform enables us to target multiple tissues through the substitution of different targeting ligands conjugated to the LNP. For example, using the LNP platform for delivery to extrahepatic tissues, we achieved *in vivo* preclinical proof-of-concept editing of HSCs in NHPs and to date have also achieved in vivo delivery to two extrahepatic, non-HSC cells in humanized mice.

To optimize the specificity of our product candidates, there are a number of different aspects of the product configuration that we customize in addition to the sequence and quality of the guide RNA, including the length of the guide RNA, the type of Cas9 or Cas12a enzyme, including engineered forms, the delivery vector, including targeting moiety, and the duration of exposure, all of which contribute to overall specificity.

Our Gene Editing Medicine Programs

Our research and development efforts are focused on next generation *in vivo* medicines, including *in vivo* editing of liver cells, HSCs and other tissues. Our product development strategy is to target diseases where gene editing can be used to enable or enhance therapeutic outcomes for patients, while maximizing probability of technical, clinical, regulatory and commercial success. We believe the therapeutic programs and delivery technologies we have chosen to pursue to date and those that are currently under development will demonstrate the depth and breadth of our ability to deploy our gene

editing platform to develop differentiated, transformational medicines for previously untreatable diseases. The following summarizes our research programs and disease areas:



Cardiovascular

Our lead program, EDIT-401, is an experimental, potential best-in-class, one-time therapy designed to significantly reduce LDL-cholesterol ("LDL-C") through the upregulation of the LDL receptor ("LDLR").

Hyperlipidemia, or elevated levels of LDL-C, affects an estimated 70 million people in the United States, including approximately 1.2 million people who have HeFH. Hyperlipidemia is a major causal factor for atherosclerotic cardiovascular disease ("ASCVD"), a serious condition driven by cholesterol-rich plaque accumulation in the arteries that can lead to severe events like heart attack and stroke. ASCVD is the leading cause of death worldwide, and in the United States alone is projected to result in over $300 billion in related expenditures by 2035. We estimate that approximately 15 million people in the United States suffer from ASCVD. Lower LDL-C levels is well correlated to reduced risk of cardiovascular event. The current standard of care for hyperlipidemia includes the use of statin therapy with additional treatment modalities to achieve specified targets for LDL-C reduction based on patient risk level, such as the inclusion of PCSK9 inhibitors. This current standard of care has demonstrated a mean LDL-C reduction ranging from 40% to 60%, but can require multiple therapies and life-long administration, frequently resulting in non-adherence. Non-adherence and treatment underutilization has resulted in approximately 75% of patients with established cardiovascular disease failing to achieve LDL-C targets, creating a significant unmet need across multiple at-risk segments of patients with hyperlipidemia.

EDIT-401 uses a differentiated upregulation strategy designed to significantly reduce LDL-C and treat hyperlipidemia. Using our Cas9 gene editing nuclease and dual guide RNAs, EDIT-401 disrupts DNA sequences that inhibit expression of the LDLR gene, thereby increasing LDLR protein expression to reduce LDL-C levels. In preclinical studies in NHPs, EDIT-401 achieved LDL-C reductions equal to or exceeding 90% within 48 hours of a single dose of EDIT-401. EDIT-401 also achieved LDL-C reductions equal to or exceeding 90% in mice with high baseline LDL-C and reduced LDLR function. The EDIT-401 LDL-C reductions were achieved with an at least six-fold mean increase in LDLR protein in the NHP liver, requiring only approximately 10-40% functional editing of LDLR alleles. LDL-C reduction was maintained in mouse models in a three-month study, demonstrating the durability of effect. EDIT-401 was well tolerated across all doses administered and no adverse clinical observations were noted. While there were some transient increases in liver enzymes, these resolved within a week. We are on track to submit an IND or foreign equivalent to conduct a clinical trial of EDIT-401 in patients with HeFH by mid-2026 with the expectation of achieving early human proof-of-concept data

for EDIT-401 by the end of 2026. We plan to complete enrolling the dose-finding portion of the first-in-human clinical trial with topline data results available in 2027. We expect to present additional preclinical data for EDIT-401 by mid-2026.

Owing to the physiological relationship between LDL-C and Lp(a), and the observation that Lp(a) levels have been reduced with PCSK9 inhibitors, we are exploring the effects of EDIT-401, our LDLR upregulation program, on Lp(a) levels in pre-clinical species.

Hemoglobinopathies

We are developing an approach for *in vivo* gene editing in HSCs to support the advancement of research programs to treat non-malignant hematological diseases. Our initial focus is on the development of therapies to treat SCD and TDT.

Sickle cell disease is an inherited life-threatening hematological disorder, which starts to cause serious complications in early childhood. It affects millions of people worldwide, including approximately 100,000 people in the United States. Patients suffering from SCD can experience severe anemia and sickling-induced blood vessel blockages resulting in unpredictable and severe attacks of acute pain, stroke, acute chest syndrome, liver disease, renal failure, and a shortened life span. It is estimated that approximately 50% of patients with the most severe form of SCD die before 45 years of age. Advances in supportive care and disease modifying therapies have improved outcomes for patients with SCD, but curative therapies have been limited to allogeneic HSC transplantation. However, less than 20% of patients can find matched donors for this procedure and there is a risk of serious complications. Transfusion-dependent beta thalassemia is also an inherited hematological disorder that often appears in infancy. It is relatively rare in the United States, affecting approximately 1,000 people, but is one of the most common autosomal recessive disorders in the world, found most often among individuals of Mediterranean, Middle Eastern, and South Asian descent. TDT is characterized by severe anemia, hemolysis, and severe ineffective erythropoiesis, often requiring patients to undergo regular, lifelong blood transfusions for survival.

We are pursuing a distinct gene editing approach to treating these hemoglobinopathies. We aim to use our AsCas12a gene editing nuclease to target the clinically validated *HBG1/2* promoter site of the gamma-globin gene in human CD34+ cells and disrupt the binding site of the BCL11A protein, consistent with observed naturally occurring human mutations. These mutations mimic the asymptomatic condition of hereditary persistence of fetal hemoglobin with high levels of fetal hemoglobin ("HbF") in red blood cells. HbF levels greater than 30% are associated with a significant reduction in, or the absence of, SCD symptoms, particularly vaso-occlusive events ("VOEs"). By editing the *HBG1/2* promoter in the gamma-globin gene, we seek to generate protective changes that increase HbF production in a manner that is independent of erythropoietic stress, resulting in reduced sickling and VOEs in SCD patients, and resolving anemia and transfusion dependence in TDT patients. Building on our experience with reni-cel, we have achieved *in vivo* preclinical proof-of-concept data of HSC editing in NHPs. We intend to continue optimizing candidates for our HSC program, but plan to focus our resources on the advancement of our lead EDIT-401 program to human proof-of-concept.

Other Cells/Tissues

We seek to additionally develop *in vivo* gene editing medicines targeted to other cells and tissues. We believe our proprietary targeting LNP platform can enable specific targeting of different extrahepatic cell types by substitution of different targeting ligands conjugated to the LNP. We previously announced *in vivo* delivery to two additional cell types in humanized mice using our proprietary LNP targeting platform, demonstrating *in vivo* proof of concept for our "plug n' play" LNP delivery platform.

Licensing and Business Development

We are focused on driving solutions for people living with serious, previously untreatable diseases by leveraging our gene editing platform. Through in-licensing of complementary technologies, we can expand our existing gene editing platform and further drive the development of our *in vivo* pipeline. This was demonstrated with our entry into a collaboration and license agreement to access LNPs targeting the liver. Using this in-licensed LNP, we achieved *in vivo* editing of hepatocytes in NHPs, furthering our strategy to upregulate certain liver targets, including LDLR for EDIT-401.

Further, as the exclusive licensee of Broad and Harvard's Cas9 patent estates and Broad's Cas12a patent estate for human medicines, we actively seek opportunities to out-license and partner our robust intellectual property portfolio to drive the development of CRISPR-based medicines in therapeutic areas outside of our core focus and to provide non-dilutive capital. Examples of these licensing efforts include our non-exclusive license to Vertex for our Cas9 gene editing

technology for *ex vivo* gene editing medicines targeting the BCL11A gene in the fields of SCD and TDT, including Vertex's CASGEVY™ (exagamglogene autotemcel); our non-exclusive license to Immatics of our AsCas12a technology to advance gamma-delta T cell therapies for the treatment of cancer; and our collaboration with BMS. Certain of our current collaborators and partners are expected to achieve clinical milestones in the next 12 to 18 months, which would result in milestone payments to us.

Our collaboration with BMS is advancing engineered alpha-beta T cell therapies to treat solid tumors, liquid tumors, and autoimmune disease leveraging our platform technologies, including Cas9 and AsCas12a. For example, engineered T cells, including alpha-beta T cells, have shown encouraging clinical activity against multiple cancers, culminating in recent approvals of such therapies in the United States. Because of these promising results, there is significant interest in the medical community in expanding the application of this technology across a broader range of cancers and patients. We believe that our gene editing technology has the potential to improve multiple properties of these alpha-beta T cell therapies. Alpha-beta cells are part of the adaptive immune system and recognize tumors with endogenous alpha-beta T cell receptors or CARs or engineered T cell receptors ("eTCRs"). If we are successful, genome-edited engineered alpha-beta T cells have the potential to significantly expand the types of cancers treatable by CAR/ eTCR alpha-beta T cells and to improve the outcomes of these therapies. Through our collaboration with BMS, we have applied our Cas9 and AsCas12a platform technologies to multiple gene targets in order to improve the efficacy and safety of CAR/eTCR alpha-beta T cells directed against a range of tumor types. In addition, we have optimized gene editing components and delivery methods compatible with engineered alpha-beta T cell manufacturing methods developed by BMS. To date, this collaboration has resulted in 14 total programs, including BMS' CD19 HD Allo CAR T program for the treatment of autoimmune disease currently in Phase I clinical development.

Our Collaborations and Licensing Strategy

BMS Collaboration and License Agreement

In May 2015, we entered into a collaboration and license agreement with Juno Therapeutics, a subsidiary of BMS, for the research and development of engineered T cells with CARs and eTCRs that have been genetically modified to recognize and kill other cells. We and BMS amended and restated this agreement in November 2019 and further amended it in March 2024 (such agreement, as amended and restated to date, the "BMS Collaboration Agreement"). In connection with the amendment and restatement in November 2019, we entered into a license agreement with BMS (such agreement, the "BMS License Agreement," and together with the BMS Collaboration Agreement, the "BMS Agreements"). Under the terms of the BMS Collaboration Agreement, we received an upfront payment of $25.0 million, amendment fees totaling $75.0 million and have received milestone payments totaling $42.5 million, in addition to certain opt-in fees.

The BMS Agreements relate to technology used to edit or modify the genome of a cell in connection with the research, development, manufacture, commercialization or other exploitation of T cells that express or have ever expressed T cell receptor dimers consisting of an alpha (α) chain and a beta (β) chain (such cells, "Alpha-beta T Cells"), and T cells derived from pluripotent stem cells or any other precursor cell (such cells, "Other Derived T Cells"), subject to certain exclusions for certain of our existing obligations. The exploitation of Alpha-beta T Cells and Other Derived T Cells specifically excludes the exploitation of T Cells that express a T cell receptor dimer consisting of a gamma (γ) chain and a delta (δ) chain, which we refer to as gamma-delta T Cells.

During the research term under the BMS Collaboration Agreement, we may research ribonucleoprotein complexes comprising an RNA-guided engineered nuclease paired with an oligonucleotide ("RNP Complexes") that recognize or modulate the expression of up to twenty gene targets selected by BMS (each, a "Research Program") for the purpose of identifying the RNP Complexes that may be used in the creation of potential drug development candidates. Pursuant to the March 2024 amendment, we provided BMS the ability to select up to three additional gene targets for research subject to the payment of a low single digit million-dollar payment for each new target selected. The research term, as amended, extends to November 2026, with options to extend the collaboration for up to an additional two years under certain circumstances, including the payment of a high single digit million-dollar payment.

Under the BMS Collaboration Agreement, if BMS elects to opt-in with respect to a Research Program, it shall make a mid-six digit dollar payment to us and we shall amend the BMS License Agreement to include such Research Program by executing a licensed program addendum for such Research Program. Following BMS' opt-in for each program we shall grant to BMS an exclusive (even as to us), royalty-bearing worldwide right and license under specified intellectual property rights to research, develop, manufacture commercialize or otherwise exploit the RNP Complexes in such Research

Program to create products containing, incorporating, comprising or containing Alpha-beta T Cells and/or Other Derived T Cells, in each case modified using the RNP Complexes in such Research Program (each, a "BMS Licensed Product").

We are entitled to receive high single-digit to low double-digit percentage royalties on net sales made by BMS, its affiliates and sublicensees of any BMS Licensed Products, subject to reductions in certain circumstances. We are also entitled to receive development milestones totaling up to $135.0 million in the aggregate upon achievement of certain clinical milestones and specified regulatory approvals and commercial milestone payments totaling up to $60.0 million in the aggregate for each of the first two BMS Licensed Products to achieve specified net sales milestones.

We have agreed during the term of the BMS Collaboration Agreement not to use (directly or indirectly), or license others to use, gene editing technology in connection with any research, development, manufacture, commercialization or other exploitation of any Alpha-beta T Cells or Other Derived T Cells. Our exclusivity obligation will not apply to activities related to (i) any identified RNP Complexes in a program for which BMS elects not to exercise its opt-in right, (ii) certain of our existing obligations to third parties, and (iii) certain existing programs of an acquiror of our company in a change of control.

We have agreed during the term of any licensed program addendum under the BMS License Agreement not to use (directly or indirectly), or license others to use, any gene editing technology that modulates or recognizes a gene target covered by such licensed program addendum for the conduct of any research, development, manufacture, commercialization or other exploitation with respect to any product that constitutes, incorporates, comprises or contains any Alpha-beta T Cell or Other Derived T Cells.

The BMS Collaboration Agreement continues in effect until the later of expiration of the research term or expiration of the last to expire of BMS' right to opt-in with respect to any Research Program. BMS may terminate the BMS Collaboration Agreement in its discretion upon six months' prior written notice to us. Either party may terminate the BMS Collaboration Agreement for uncured material breach of the other party, provided that the breaching party has had sixty days to cure such breach, or in the event of insolvency or bankruptcy of the other party.

The BMS License Agreement continues in effect on a BMS Licensed Product-by-BMS Licensed Product and country-by-country basis until the expiration of the royalty term with respect to such licensed product in such country and in its entirety upon the expiration of all royalty terms with respect to all BMS Licensed Products in all countries. BMS may terminate the BMS License Agreement in its entirety or on a BMS Licensed Product-by-BMS Licensed Product basis in its discretion upon ninety days' prior written notice to us. Either party may terminate the BMS License Agreement on a BMS Licensed Product-by-BMS Licensed Product basis in the event of an uncured material breach of the other party, provided that the breaching party has had sixty days to cure such breach, or in the event of insolvency or bankruptcy of the other party. We have the right to terminate the BMS License Agreement on a program-by-program basis in the event that BMS fails to make any undisputed payment to us and has not cured such payment breach within the cure period. Other than BMS' right to wind-down its operations with respect to BMS Licensed Products during the twelve months following the date of effectiveness of termination, all licenses and other exclusive rights granted under the BMS License Agreement shall terminate.

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 gene 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.

The Broad Institute and President and Fellows of Harvard College License Agreement

In October 2014, we entered into a license agreement with Broad and Harvard for specified patent rights. In December 2016, we amended and restated this license agreement and further amended the agreement in March 2017 and February 2024 (as amended, the "Cas9-I License Agreement"). Among other things, the Cas9-I License Agreement amended the original license agreement by excluding additional fields from the scope of the exclusive license granted to us; converting the exclusive license to three specified targets to a non-exclusive license, subject to specified limitations; revising certain provisions relating to the rights of Harvard and Broad to grant further licenses under specified circumstances to third parties that wish to develop and commercialize products that target a particular gene and that

otherwise would fall within the scope of our exclusive license; and providing Harvard and Broad with certain rights to designate, and reserve all rights to, gene targets for which the designating institution has an interest in researching and developing products that would otherwise be covered by rights licensed to us. The licenses granted to us under the Cas9-I License Agreement include rights to certain patents solely owned by Harvard (the "Harvard Cas9-I Patent Rights"), certain patents co-owned by the Massachusetts Institute of Technology ("MIT") and Broad, certain patents co-owned by MIT, The Rockefeller University ("Rockefeller"), and Broad, and certain patents co-owned by MIT, Broad and Harvard. We refer to all the patents and patent applications licensed to us under the Cas9-I License Agreement as the Harvard/Broad Cas9-I Patent Rights.

Certain patent applications in the Harvard/Broad Cas9-I Patent Rights are jointly owned by Rockefeller. In February 2017, Broad and Rockefeller entered into an inter-institutional agreement pursuant to which Rockefeller authorized Broad to act as its sole and exclusive agent for the purposes of licensing Rockefeller's rights in such Harvard/Broad Cas9-I Patent Rights and any additional related patents or patent applications that Rockefeller may jointly own with Broad. The March 2017 amendment to the Cas9-I License Agreement included a license to Rockefeller's rights in such patents and patent applications.

The Harvard/Broad Cas9-I Patent Rights are directed, in part, to certain CRISPR/Cas9 compositions of matter and their use for gene editing and to certain CRISPR/Cas9 related delivery technologies. Pursuant to the Cas9-I License Agreement, and as of December 31, 2025, we have certain rights under 85 U.S. patents, 57 pending U.S. patent applications, 37 European patents and related validations, 32 pending European patent applications, and other related patent applications in jurisdictions outside of the United States and Europe.

Pursuant to the Cas9-I License Agreement, Harvard and Broad granted us an exclusive, worldwide, royalty-bearing, sublicensable license to the Harvard/Broad Cas9-I Patent Rights to make, have made, use, sell, offer for sale, have sold, import, and export products and services in the field of the prevention and treatment of human disease, subject to certain limitations and retained rights. The exclusive license granted by Broad and Harvard excludes certain fields, including the modification of animals or animal cells for the creation and sale of organs suitable for xenotransplantation into humans; the research, development and commercialization of products or services in the field of livestock applications; plant-based agricultural products; and, subject to certain limitations, products providing nutritional benefits. Moreover, the license granted by Broad is non-exclusive with respect to the treatment of medullary cystic kidney disease 1 and three other specified targets, subject to the limitation that for such three targets, each of Broad and Harvard is only permitted to grant a non-exclusive license to one third party at a time with respect to each such target within the field of exclusive license granted to us. Harvard and Broad also granted us a non-exclusive, worldwide, royalty-bearing, sublicensable license to the Harvard/Broad Cas9-I Patent Rights for all purposes, with the exception that the non-exclusive license to certain Harvard Cas9-I Patent Rights excludes the modification of animals or animal cells for the creation and sale of organs suitable for xenotransplantation into humans and the development and commercialization of products or services in the field of livestock applications. In addition to the exclusions described above, the following are excluded from the scope of both the exclusive and non-exclusive licenses granted to us under the Cas9-I License Agreement: human germline modification; the stimulation of biased inheritance of particular genes or traits within a population of plants or animals; the research, development, manufacturing, or commercialization of sterile seeds; and the modification of the tobacco plant with specified exceptions.

We are obligated to use commercially reasonable efforts to research, develop, and commercialize products for the prevention or treatment of human disease under the Cas9-I License Agreement. Also, we are required to achieve certain development milestones within specified time periods for products incorporating the technologies covered by the Harvard/Broad Cas9-I Patent Rights. Harvard and Broad have the right to terminate our license with respect to the Harvard/Broad Cas9-I Patent Rights covering the technology or technologies with respect to which we fail to achieve these development milestones.

The licenses granted by Broad and Harvard to us under the Cas9-I License Agreement are subject to retained rights of the U.S. government in the Harvard/Broad Cas9-I Patent Rights and the rights retained by Broad, Harvard, MIT, and Rockefeller on behalf of themselves and other academic, government and non-profit entities, to practice the Harvard/Broad Cas9-I Patent Rights for research, educational, or teaching purposes. In addition, certain rights granted to us under the Cas9-I License Agreement are further subject to a non-exclusive license to the Howard Hughes Medical Institute for research purposes. Our exclusive license rights also are subject to rights retained by Broad, Harvard, MIT, and Rockefeller for any third party to research, develop, make, have made, use, offer for sale, sell, have sold, import or otherwise exploit the Harvard/Broad Cas9-I Patent Rights and licensed products as research products or research tools, or for research purposes.

We have the right to sublicense our licensed rights provided that the sublicense agreement must be in compliance and consistent with the terms of the Cas9-I License Agreement. Any sublicense agreement cannot include the right to grant further sublicenses without the written consent of Broad and Harvard. In addition, any sublicense agreements must contain certain terms, including a provision requiring the sublicensee to indemnify Harvard, Broad, MIT, and Howard Hughes Medical Institute according to the same terms as are provided in the Cas9-I License Agreement and a statement that Broad, Harvard, MIT, and Howard Hughes Medical Institute are intended third party beneficiaries of the sublicense agreement for certain purposes.

Under the Cas9-I License Agreement, Harvard and Broad also retained rights to grant further licenses under specified circumstances to third parties, other than specified entities, that wish to develop and commercialize products that target a particular gene and that otherwise would fall within the scope of our exclusive license from Harvard and Broad. If a third party requests a license under the Harvard/Broad Cas9-I Patent Rights for the development and commercialization of a product that would be subject to our exclusive license grant from Harvard and Broad under the Cas9-I License Agreement, Harvard and Broad may notify us of the request (the "Cas9-I Third Party Proposed Product Requests"). Our process to address Cas9-I Third Party Proposed Product Requests has been conformed to the same process established in our Cpf1 license agreement described below.

The Cas9-I License Agreement also provides Broad with the right, after a specified period of time and subject to certain limitations, to designate gene targets for which Broad, whether alone or together with an affiliate or third party, has an interest in researching and developing products that would otherwise be covered by rights licensed to us under the Cas9-I License Agreement. Broad may not so designate any gene target for which we, directly or through any of our affiliates, sublicensees, or collaborators, are researching, developing, or commercializing a product, or for which we can demonstrate to Broad's reasonable satisfaction that we are interested in researching, developing, and commercializing a product, that we have a commercially reasonable research, development, and commercialization plan to do so, and we commence and continue reasonable commercial efforts under such plan. If we directly or through any of our affiliates, sublicensees, or collaborators, are not researching, developing, or commercializing a product directed toward the gene target designated by Broad and are not able to develop and implement a plan reasonably satisfactory to Broad, Broad is entitled to reserve all rights under the Cas9-I License Agreement, including the right to grant exclusive or non-exclusive licenses to third parties, to develop and commercialize products directed to such gene target and our license granted with respect to such gene target will terminate, and we will not be entitled under the Cas9-I License Agreement to develop and commercialize products directed to that gene target.

Under the Cas9-I License Agreement, we paid Broad and Harvard an upfront license fee in the low six figures and issued a single-digit percentage of shares of our common stock to Broad (with Broad holding a right to request re-issuance to its designees, including MIT or MIT's designee) and Harvard. We also must pay an annual license maintenance fee ranging from the low- to mid-five figures to the low-six figures, depending on the calendar year. This annual license maintenance fee is creditable against royalties owed on licensed products and services in the same year as the maintenance fee is paid. We are obligated to reimburse Broad and Harvard for expenses associated with the prosecution and maintenance of the Harvard/Broad Cas9-I Patent Rights, including expenses associated with any interference proceedings in the U.S. Patent and Trademark Office ("USPTO"), any opposition proceedings in the European Patent Organization, or any other *inter partes* or other post grant proceedings in these or other jurisdictions where we are seeking patent protection. Therefore, we are obligated to reimburse Broad and/or Harvard for expenses associated with the interference and opposition proceedings involving patents licensed to us under this agreement (described in more detail under Part I, Item 1A "Risk Factors—Risks Related to Our Intellectual Property—Some of Our In-Licensed Patents are Subject to Priority and Validity Disputes" of this Annual Report on Form 10-K).

Broad and Harvard are collectively entitled to receive clinical and regulatory milestone payments totaling up to $14.8 million in the aggregate per licensed product approved in the United States, the European Union (the "EU") and Japan for the prevention or treatment of a human disease that afflicts at least a specified number of patients in the aggregate in the United States. If we undergo a change of control during the term of the Cas9-I License Agreement, these clinical and regulatory milestone payments will be increased by a certain percentage in the mid double-digits. We are also obligated to make additional payments to Broad and Harvard, collectively, of up to an aggregate of $54.0 million upon the occurrence of certain sales milestones per licensed product for the prevention or treatment of a human disease that afflicts at least a specified number of patients in the aggregate in the United States. Broad and Harvard are collectively entitled to receive clinical and regulatory milestone payments totaling up to $4.1 million in the aggregate per licensed product approved in the United States and at least one jurisdiction outside the United States for the prevention or treatment of a human disease that afflicts fewer than a specified number of patients in the aggregate in the United States or a specified number of patients per year in the United States, which we refer to as an ultra-orphan disease. We are also obligated to make additional payments

to Broad and Harvard, collectively, of up to an aggregate of $36.0 million upon the occurrence of certain sales milestones per licensed product for the prevention or treatment of an ultra-orphan disease.

Broad and Harvard, collectively, are entitled to receive mid single-digit percentage royalties on net sales of licensed products for the prevention or treatment of human disease, and ranging from low single-digit to high single-digit percentage royalties on net sales of other licensed products and services, made by us, our affiliates, or our sublicensees. The royalty percentage depends on the licensed product and licensed service, and whether such licensed product or licensed service is covered by a valid claim within the Harvard/Broad Cas9-I Patent Rights. If we are legally required to pay royalties to a third party on net sales of our licensed products because such third party holds patent rights that cover such licensed product, then we can credit up to a mid double-digit percentage of the amount paid to such third party against the royalties due to Harvard and Broad in the same period. Our obligation to pay royalties will expire on a product-by-product and country-by-country basis upon the later of the expiration of the last to expire valid claim of the Harvard/Broad Cas9-I Patent Rights that cover the composition, manufacture, or use of each covered product or service in each country or the tenth anniversary of the date of the first commercial sale of the licensed product or licensed service. If we sublicense any of the Harvard/Broad Cas9-I Patent Rights to a third party pursuant to our exclusive license under the Cas9-I License Agreement, Broad and Harvard, collectively, had the right to receive a low double-digit percentage of the sublicense income, which percentage decreased in 2018 and may still decrease to a high single-digit percentage for licensed products for the prevention or treatment of human disease under sublicenses executed after we meet a certain clinical milestone.

Broad and Harvard retain control of the prosecution of their respective patent rights. If an interference is declared or a derivation proceeding is initiated, with respect to any Harvard/Broad Cas9-I Patent Rights, then our prosecution related rights, including our right to receive correspondence from a patent office, will be suspended with respect to the patent rights involved in the interference or derivation proceeding until, under some circumstances, we enter into a common interest agreement with that institution. Nevertheless, we remain responsible for the cost of such interference or derivation proceeding. We are responsible for the cost of the interference proceeding and appeal with respect to the subject patents. Broad and Harvard are required to maintain any application or patent within the Harvard/Broad Cas9-I Patent Rights so long as we meet our obligation to reimburse Broad and Harvard for expenses related to prosecution and there is a good faith basis for doing so. If we cease payment for the prosecution of any Harvard/Broad Cas9-I Patent Right, then any license granted to us with respect to such Harvard/Broad Cas9-I Patent Right will terminate.

We have the first right, but not the obligation, to enforce the Harvard/Broad Cas9-I Patent Rights with respect to our licensed products so long as certain conditions are met, such as providing Broad and Harvard with evidence demonstrating a good faith basis for bringing suit against a third party. We are solely responsible for the costs of any lawsuits we elect to initiate and cannot enter into a settlement without the prior written consent of Broad and Harvard (and MIT and Rockefeller, if applicable). Any sums recovered in such lawsuits will be shared between us, Broad, and Harvard.

Unless terminated earlier, the term of the Cas9-I License Agreement will expire on a country-by-country basis, upon the expiration of the last to expire valid claim of the Harvard/Broad Cas9-I Patent Rights in such country. However, our royalty obligations, discussed above, may survive expiration or termination. We have the right to terminate the agreement at will upon four months' written notice to Broad and Harvard. Broad and Harvard may terminate the agreement upon a specified period of notice in the event of our uncured material breach, such notice period varying depending on the nature of the breach. Both Broad and Harvard may terminate the Cas9-I License Agreement immediately if we challenge the enforceability, validity, or scope of any Harvard/Broad Cas9-I Patent Right or assist a third party to do so, or in the event of our bankruptcy or insolvency. Neither Broad nor Harvard acting alone has the right to terminate the Cas9-I License Agreement. However, Broad and Harvard may separately terminate the licenses granted to us with respect to their respective patent rights upon the occurrence of the same events that would give rise to the right of both institutions acting collectively to terminate the Cas9-I License Agreement.

The Broad Institute—Cpf1 License Agreement

In December 2016, we entered into a license agreement with Broad, for specified patent rights ("Cpf1 Patent Rights") related primarily to Cas12a compositions of matter and their use for gene editing, which was amended in January 2021 and February 2024 (as amended, the "Cpf1 License Agreement"). Pursuant to the Cpf1 License Agreement, Broad, on behalf of itself, Harvard, MIT, Wageningen University ("Wageningen"), and the University of Tokyo ("Tokyo" and collectively with the other institutions, the "Cpf1 Institutions") granted us an exclusive, worldwide, royalty-bearing, sublicensable license to the Cpf1 Patent Rights, to make, have made, use, have used, sell, offer for sale, have sold, export and import products solely in the field of the prevention or treatment of human disease using gene therapy, editing of genetic material, or targeting of genetic material, subject to certain limitations and retained rights (collectively, the

"Exclusive Cpf1 Field"), as well as a non-exclusive, worldwide, royalty-bearing, sublicensable license to the Cpf1 Patent Rights for all other purposes, subject to certain limitations and retained rights. The licenses granted to us under the Cpf1 License Agreement exclude certain fields, including human germline modification; the stimulation of biased inheritance of particular genes or traits within a population of plants or animals; the research, development, manufacturing, or commercialization of sterile seeds; and the modification of the tobacco plant with specified exceptions.

Tokyo and the U.S. National Institutes of Health ("NIH") are joint owners on certain Cpf1 Patent Rights. Broad has only granted a license to us with respect to its interests and to Tokyo's interests in these U.S. patent applications but not to any foreign equivalents thereof. Broad does not, and does not purport to, grant any rights in NIH's interest in these U.S. patent applications under our agreement. As a result, we may not have exclusive rights under any U.S. patents that issue from these U.S. patent applications and we may not have any rights under any foreign patents that issue from any foreign equivalents thereof.

Pursuant to the Cpf1 License Agreement, and as of December 31, 2025, we have certain rights under 18 U.S. patents, eight pending U.S. patent applications, six European patents and related validations, six pending European patent applications, and other related patents and patent applications in jurisdictions outside of the United States and Europe.

We are obligated to use commercially reasonable efforts to research, develop, and commercialize licensed products in the Exclusive Cpf1 Field. We are also required to achieve certain development milestones within specified time periods for products covered by the Cpf1 Patent Rights, with Broad having the right to terminate the Cpf1 License Agreement if we fail to achieve these milestones within the required time periods. We have the right to sublicense our licensed rights provided that the sublicense agreement must be in compliance and consistent with the terms of the Cpf1 License Agreement. Any sublicense agreement cannot include the right to grant further sublicenses without the written consent of Broad. In addition, any sublicense agreements must contain certain terms, including a provision requiring the sublicensee to indemnify the Cpf1 Institutions according to the same terms as are provided in the Cpf1 License Agreement and a statement that the Cpf1 Institutions are intended third party beneficiaries of the sublicense agreement for certain purposes.

The licenses granted to us under the Cpf1 License Agreement are subject to retained rights of the U.S. government in the Cpf1 Patent Rights and rights retained by the Cpf1 Institutions on behalf of themselves and other academic, government and non-profit entities, to practice the Cpf1 Patent Rights for research, teaching, or educational purposes. Our exclusive license rights also are subject to rights retained by the Cpf1 Institutions for themselves and any third party to research, develop, make, have made, use, offer for sale, sell, have sold, import or otherwise exploit the Cpf1 Patent Rights and licensed products as research products or research tools, or for research purposes.

Under the Cpf1 License Agreement, Broad also retained rights to grant further licenses under specified circumstances to third parties that wish to develop and commercialize products that target a particular gene and that otherwise would fall within the scope of our exclusive license from Broad. If a third party requests a license under the Cpf1 Patent Rights for the development and commercialization of a product that would be subject to our exclusive license grant from Broad (a "Cpf1 Third Party Proposed Product Request"), Broad may notify us of such request. A Cpf1 Third Party Proposed Product Request must be accompanied by a research, development and commercialization plan reasonably satisfactory to Broad, including evidence that the third party has, or reasonably expects to have, access to any necessary intellectual property and funding. Broad may not grant a Cpf1 Third Party Proposed Product Request (i) if we, directly or through any of our affiliates, sublicensees, or collaborators are researching, developing, or commercializing a product directed to the same gene target that is the subject of the Cpf1 Third Party Proposed Product Request ("Cpf1 Licensee Product") and we can demonstrate such ongoing efforts to Broad's reasonable satisfaction, or (ii) if we, directly or through any of our affiliates or sublicensees, wish to do so either alone or with a collaboration partner, and we can demonstrate to Broad's reasonable satisfaction that we are interested in researching, developing, and commercializing a Cpf1 Licensee Product, that we have a commercially reasonable research, development, and commercialization plan to do so, and we commence and continue reasonable commercial efforts under such plan. If we, directly or through any of our affiliates, sublicensees, or collaborators, are not researching, developing, or commercializing a Cpf1 Licensee Product nor able to develop and implement a plan reasonably satisfactory to Broad, Broad may grant an exclusive or non-exclusive license to the third party on a gene target-by-gene target basis.

The Cpf1 License Agreement also provides Broad with the right, subject to certain limitations, to designate gene targets for which Broad, whether alone or together with a Cpf1 Institution, affiliate or third party, has an interest in researching and developing products that would otherwise be covered by rights licensed to us under the Cpf1 License Agreement. Broad may not so designate any gene target for which we, directly or through any of our affiliates,

sublicensees, or collaborators, are researching, developing, or commercializing a product, or for which we can demonstrate to Broad's reasonable satisfaction that we are interested in researching, developing, and commercializing a product, that we have a commercially reasonable research, development, and commercialization plan to do so, and we commence and continue reasonable commercial efforts under such plan. If we, directly or through any of our affiliates, sublicensees, or collaborators, are not researching, developing, or commercializing a product directed toward the gene target designated by Broad and are not able to develop and implement a plan reasonably satisfactory to Broad, Broad is entitled to reserve all rights under the Cpf1 License Agreement, including the right to grant exclusive or non-exclusive licenses to third parties, to develop and commercialize products directed to such gene target, our license with respect to such gene target will terminate, and we will not be entitled under the Cpf1 License Agreement to develop and commercialize products directed to such gene target.

Under the Cpf1 License Agreement, Broad and Wageningen are collectively entitled to receive clinical and regulatory milestone payments totaling up to $20.0 million in the aggregate per licensed product approved in the United States, the EU and Japan for the prevention or treatment of a human disease that afflicts at least a specified number of patients in the aggregate in the United States. If we undergo a change of control during the term of the Cpf1 License Agreement, certain of these clinical and regulatory milestone payments will be increased by a certain percentage in the mid double-digits. We are also obligated to make additional payments to Broad and Wageningen, collectively, of up to an aggregate of $54.0 million upon the occurrence of certain sales milestones per licensed product for the prevention or treatment of a human disease that afflicts at least a specified number of patients in the aggregate in the United States. Broad and Wageningen are collectively entitled to receive clinical and regulatory milestone payments totaling up to $6.0 million in the aggregate per licensed product approved in the United States, the EU and Japan for the prevention or treatment of an ultra-orphan disease. We are also obligated to make additional payments to Broad and Wageningen, collectively, of up to an aggregate of $36.0 million upon the occurrence of certain sales milestones per licensed product for the prevention or treatment of an ultra-orphan disease.

Broad and Wageningen, collectively, are entitled to receive mid single-digit percentage royalties on net sales of products for the prevention or treatment of human disease, and ranging from sub single-digit to high single-digit percentage royalties on net sales of other products and services, made by us, our affiliates, or our sublicensees. The royalty percentage depends on the product and service, and whether such licensed product or licensed service is covered by a valid claim within the Cpf1 Patent Rights. If we are legally required to pay royalties to a third party on net sales of our products because such third party holds patent rights that cover such licensed product, then we can credit up to a mid double-digit percentage of the amount paid to such third party against the royalties due to Broad and Wageningen in the same period. Our obligation to pay royalties will expire on a product-by-product and country-by-country basis upon the later of the expiration of the last to expire valid claim of the Cpf1 Patent Rights that covers each licensed product or licensed service in each country or the tenth anniversary of the date of the first commercial sale of the product or service. If we sublicense any of the Cpf1 Patent Rights to a third party, Broad and Wageningen, collectively, had the right to receive high single-digit to low double-digit percentages of the sublicense income, which percentage decreased to a high single-digit percentage in 2022 for sublicenses executed thereafter.

Under the Cpf1 License Agreement, Broad and Wageningen are also entitled, collectively, to receive success payments in the event our market capitalization reaches specified thresholds ascending from a high nine digit dollar amount to $10.0 billion ("Market Cap Success Payments") or sale of our company for consideration in excess of those thresholds, ("Company Sale Success Payments," which with the Market Cap Success Payments, the "Success Payments"). Market Cap Success Payments are payable by us in cash, in shares of our common stock, with such shares being valued for such purpose at the closing price of our common stock as reported the Nasdaq Stock Market for the trading day immediately preceding the date of such payment if our common stock was then listed on the Nasdaq Stock Market, or in the form of promissory notes (the "Promissory Notes"). The Promissory Notes bear interest at 4.8% per annum. Principal and interest on the Promissory Notes are payable on, subject to certain exceptions, 150 days following issuance (or if earlier, a specified period of time following a sale of our company). We could elect to make any payment of amounts outstanding under the Promissory Notes either in the form of cash or, subject to certain conditions, in shares of our common stock of equal value, with such shares being valued for such purpose at the closing price of our common stock as reported the Nasdaq Stock Market for the trading day immediately preceding the date of such payment if our common stock was then listed on the Nasdaq Stock Market. In the event of a change of control of our company or a sale of our company, we are required to pay all remaining principal and accrued interest on the Promissory Notes in cash within a specified period following such event. Following a change in control of our company, Market Cap Success Payments are required to be made in cash. Company Sale Success Payments are payable solely in cash. In 2017, two Market Cap Success Payments of $5.0 million each became due and payable and we issued Promissory Notes in such amounts, which we fully settled by issuing shares of our common stock in 2017 and 2018. In December 2020, an additional Market Cap Success Payment of $15.0 million

became due and payable, which we settled through the issuance of shares of our common stock in January 2021. The remaining Success Payments that may be paid to Broad and Wageningen range from a low-eight digit dollar amount to a mid-eight digit dollar amount, and collectively will not exceed, in aggregate, $100.0 million, which maximum would be payable only if we achieve a market capitalization threshold of $10.0 billion and have at least one product candidate covered by a claim of a patent right licensed to us under either the Cpf1 License Agreement or the Cas9-I License Agreement that is or was the subject of a clinical trial pursuant to development efforts by us or any of our affiliates or sublicensees.

In addition, in the event that a sale of our company or change of control has occurred and the maximum amount of potential Success Payments under the Cpf1 License Agreement has not been paid to Broad and Wageningen, Broad and Wageningen are entitled to receive, upon the subsequent achievement of specified regulatory milestones, percentages ranging from high single digits to mid-to-low double digits of the remaining unpaid maximum amount of Success Payments. Broad and Wageningen are further entitled to receive up to the full remaining unpaid maximum amount of Success Payments upon the subsequent achievement of specified sales milestones. All such post-sale or post-change of control milestone payments are required to be made in cash.

Broad retains control of the prosecution and maintenance of the Cpf1 Patent Rights. We have the right to provide input in the prosecution of the Cpf1 Patent Rights, including to direct Broad to file and prosecute patents in certain countries. We are also obligated to reimburse Broad and Wageningen for all unreimbursed expenses incurred by them in connection with the prosecution and maintenance of the Cpf1 Patent Rights prior to the date of the Cpf1 License Agreement, and to reimburse Broad for expenses associated with the prosecution and maintenance of the Cpf1 Patent Rights following the date of the Cpf1 License Agreement.

We have the first right, but not the obligation, to enforce the Cpf1 Patent Rights with respect to our licensed products in the Exclusive Cpf1 Field so long as certain conditions are met, such as providing Broad and the applicable Cpf1 Institutions with evidence demonstrating a good faith basis for bringing suit against a third party. We are solely responsible for the costs of any lawsuits we elect to initiate and cannot enter into a settlement without the prior written consent of Broad. Any sums recovered in such lawsuits will be shared between Broad, Wageningen, and us.

Unless terminated earlier, the term of the Cpf1 License Agreement will expire on a country-by-country basis, upon the expiration of the last to expire valid claim of the Cpf1 Patent Rights in such country. However, our royalty obligations, discussed above, may survive expiration or termination. We have the right to terminate the Cpf1 License Agreement at will upon four months' written notice to Broad. Either party may terminate the Cpf1 License Agreement upon a specified period of notice in the event of the other party's uncured material breach of a material obligation, such notice period varying depending on the nature of the breach. Broad may terminate the Cpf1 License Agreement immediately if we challenge the enforceability, validity, or scope of any Cpf1 Patent Right or assist a third party to do so, or in the event of our bankruptcy or insolvency.

Other Broad Agreements

In addition to the Cas9-I License Agreement and the Cpf1 License Agreement, in December 2016, we entered into a license agreement with Broad for certain patent rights covering Cas9 compositions of matter and their use for gene editing, which was amended in January 2021 and February 2024 (as amended, the "Cas9-II Agreement"), and, in June 2018, we entered into a Sponsored Research Agreement with Broad providing for Broad to conduct research useful or relevant to gene editing in the field of genomic medicines for the prevention of treatment of human diseases with funding from us, which was amended in January 2021 (as amended, the "Sponsored Research Agreement"). Under the Cas9-II Agreement and the Sponsored Research Agreement, we have potential obligations with respect to success payments, which are described in Note 8 to the Notes to Consolidated Financial Statements included in Part II, Item 8 "Financial Statements and Supplementary Data—Commitments and Contingencies" of this Annual Report on Form 10-K.

Intellectual Property

Our success depends in part on our ability to obtain and maintain proprietary protection for our platform technology, 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 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. 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.

Our in-licensed patents and patent applications cover various aspects of our gene editing platform technology, including CRISPR systems that employ Cas9 including *S. aureus* Cas9, high-fidelity Cas9 nucleases and Cas9 PAM variants, self-inactivating forms of Cas9, Cas9 nickases, CRISPR systems that employ Cas12a including Cas12a nickases and other variants and self-inactivating forms of Cas12a, and also CRISPR systems that employ viral vectors for delivery, single guide RNAs, or modified guide RNAs, including guide nucleic acids containing both DNA and RNA components. We also have filed patent applications and have in-licensed rights to filed patent applications directed to each of the four components of our gene editing platform technology. We intend 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. We also intend to obtain rights to existing delivery technologies through one or more licenses from third parties.

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. Moreover, trade secrets can be difficult to protect. While we have confidence in the measures we take 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 Part I, Item 1A “Risk Factors—Risks Related to Our Intellectual Property” of 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. In the United States, a patent’s term may, in certain cases, be lengthened by patent term adjustment, which compensates a patentee for administrative delays by the USPTO in examining and granting a patent, or may be shortened if a patent is terminally disclaimed over a commonly owned patent or a patent naming a common inventor and having an earlier expiration date. The Drug Price Competition and Patent Term Restoration Act of 1984 extension cannot extend the remaining term of a patent beyond a total of 14 years from the date of product approval, only one patent applicable to each regulatory review period may be extended and only those claims covering the approved drug or a method for using it may be extended.

CRISPR

As of December 31, 2025, we owned 50 U.S. patents, 57 pending U.S. non-provisional patent applications, 23 European patents and related validations, 59 pending European patent applications, 14 pending U.S. provisional patent applications, eight pending Patent Cooperation Treaty (“PCT”) patent applications, and other related patents and patent applications in jurisdictions outside the United States and Europe that are related to our CRISPR technology and which include claims directed to our gene editing platform, including our directed editing component, as well as composition of matter and method of use claims for our therapeutic programs. Four of these U.S. patents, one of these European patents and their U.S., European and foreign counterpart applications are co-owned with Broad and Iowa and we have obtained an exclusive license to such co-ownership rights from these third parties in the field of prevention or treatment of human disease using gene therapy or gene editing. In addition, seven of these issued U.S. patents and seven of these pending U.S. non-provisional patent applications are co-owned with certain of our collaborators because they encompass inventions developed under our collaborations. Our current issued U.S. patents, if the appropriate maintenance fees are paid, are expected to expire between 2034 and 2039, excluding any additional term for patent term adjustments or patent term extensions. If issued as U.S. patents, and if the appropriate maintenance fees are paid, the U.S. patent applications would be expected to expire between 2034 and 2046, excluding any additional term for patent term adjustments or patent term extensions.

As of December 31, 2025, we in-licensed 153 U.S. patents, 68 European patents and related validations, and approximately 350 pending patent applications, including 85 pending U.S. non-provisional patent applications, 55 pending

European patent applications, and other related patents and patent applications in jurisdictions outside the United States and Europe that are related to our CRISPR technology collectively from various universities and institutions. The patents and patent applications outside of the United States and Europe are held primarily in Canada, China, Japan, and Australia, although some of our in-licensed patent families were filed in a larger number of countries. The claims from our in-licensed portfolio include claims to compositions of matter, methods of use, and certain processes.

These include claims directed to CRISPR systems that employ Cas9 including Cas9 nickases, *S. aureus* Cas9, high-fidelity Cas9 nucleases, Cas9 PAM variants and self-inactivating forms of Cas9, CRISPR systems that employ Cas12a including Cas12a nickases and other variants and self-inactivating forms of Cas12a, and also CRISPR systems that employ viral vectors for delivery, single guide RNAs, or modified guide RNAs. Our current U.S. patents in-licensed from the various universities and institutions, if the appropriate maintenance fees are paid, are expected to expire between 2033 and 2037, excluding any additional term for patent term adjustments or patent term extensions. If issued as U.S. patents, and if the appropriate maintenance fees are paid, the U.S. patent applications would be expected to expire between 2033 and 2037, excluding any additional term for patent term adjustments or patent term extensions.

Our in-licensed patents and patent applications claim the inventions of investigators at various universities and institutions and the majority of these licensed patents and patent applications are licensed on an exclusive basis. The exclusive licenses are, in some cases, limited to certain technical fields. Certain U.S. patent applications licensed to us by Broad include Tokyo and NIH as joint applicants. Broad has only granted a license to us with respect to its interests and to Tokyo's interests in these U.S. patent applications but not to any foreign equivalents thereof. Broad does not and does not purport to grant any rights in NIH's interest in these U.S. patent applications under our agreement. As a result, we may not have exclusive rights under any U.S. patents that issue from these U.S. patent applications and we may not have any rights under any foreign patents that issue from any foreign equivalents thereof. For more information regarding these license agreements, please see the section titled "Business —Intellectual Property Licenses" of Part I, Item 1 of this Annual Report on Form 10-K.

Trademarks

As of December 31, 2025, our registered trademark portfolio consisted of registrations in the United States for EDITAS, EDITAS in Stylized Letters, the Infinity Logo, UDITAS, SLEEK, the Double Helix Design, and PIONEERING THE POSSIBLE, registrations in Australia, China, the EU, Japan, Switzerland and the United Kingdom (the "UK") for EDITAS, registrations in Australia, China, the EU, Japan, Switzerland and the UK for the Infinity Logo, registrations in the EU and the UK for UDITAS, registrations in Australia, China, the EU, Japan, Switzerland and the UK for SLEEK, and registrations in Australia, China, the EU and the UK for the Double Helix Design.

Competition

The biotechnology and pharmaceutical industries, including in the gene therapy, gene editing and cell therapy fields, are characterized by rapidly advancing technologies, intense competition, and a strong emphasis on intellectual property and proprietary products. While we believe that our technology, development experience, and scientific knowledge provide us with competitive advantages, we face potential competition from many different sources, including major pharmaceutical, specialty pharmaceutical, biotechnology companies, governmental agencies, and public and private research institutions. Any product candidates that we successfully develop and commercialize may compete with existing therapies and new therapies may become available in the future.

We compete in the segments of the pharmaceutical, biotechnology, and other related markets that utilize technologies encompassing genomic medicines to create therapies, including gene editing and gene therapy. There are additional companies that are working to develop therapies in areas related to our research programs. For hyperlipidemia, these companies include AccurEdit Therapeutics, CRISPR Therapeutics, Eli Lilly , EmendoBio, nChroma Bio, Scribe Therapeutics, Tune Therapeutics, and Yoltech Therapeutics, For hemoglobinopathies, these companies include Beam Therapeutics, BRL Medicine, CRISPR Therapeutics, Ensoma, Genetix Biotherapeutics (formerly bluebird bio), Kamau Therapeutics, Orna Therapeutics, Reforgene Medicine, Sanofi, Scribe Therapeutics, Tessera Therapeutics, Vertex, and Yoltech Therapeutics.

Our platform and product focus is the development of therapies using CRISPR technology specifically for gene editing. Other companies developing CRISPR Cas9 or Cas12a technology or therapies using CRISPR Cas9 or Cas12a technology include AccurEdit Therapeutics, Arsenal Biosciences, AvenCell Therapeutics, Caribou Biosciences, Cellistic, Century Therapeutics, CRISPR Therapeutics, EdiGene, eGenesis, ERS Genomics, Excision Biotherapeutics, Fate

Therapeutics, Inscripta, Intellia Therapeutics, Kamau Therapeutics, Sarepta Therapeutics, Sigma-Aldrich, ToolGen, Vittoria Biotherapeutics, and Yoltech Therapeutics.

In addition, there have been and may continue to be discoveries of new CRISPR-based gene editing technologies. There are additional companies developing therapies using related CRISPR gene editing technologies, including other CRISPR nucleases, base editing, prime editing and gene writing. These companies include Amber Bio, Arbor Biotechnologies, Aurora Therapeutics, Beam Therapeutics, Eligo Biosciences, Eli Lilly, Emendo Biotherapeutics, Ensoma, Epicrispr Biotechnologies, Integra Therapeutics, KSQ Therapeutics, Locus Biosciences, Mammoth Biosciences, Metagenomi, Modalis Therapeutics, nChroma Bio, Prime Medicine, Profluent Bio, Roche, Scribe Therapeutics, Tessera Therapeutics, and Tune Therapeutics.

There are also companies developing therapies using transcription activator-like effector nucleases, meganucleases, Mega-TALs and zinc finger nucleases. These companies include Allogene Therapeutics, BMS, Cellectis, Genetix Biotherapeutics, Precision Biosciences, and Sangamo Therapeutics.

In addition to competition from other gene editing therapies, gene therapies or cell medicine therapies, any products that we may develop may also face competition from other types of therapies, such as small molecule, antibody, protein, oligonucleotide, or ribonucleic acid therapies. For hyperlipidemia, these companies include Amgen, Arrowhead Therapeutics, LIB Therapeutics, Merck, NewAmsterdam Pharma, Novartis, Regeneron Pharmaceuticals, and Wave Life Sciences. In addition, statin medications are widely available from numerous pharmaceutical manufacturers and are offered in both branded and generic forms.

In addition, many of our current or potential competitors, either alone or with their collaboration partners, may have greater financial resources and expertise in research and development, manufacturing, preclinical testing, conducting clinical trials, commercialization, 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 other early-stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies. These competitors also compete with us in recruiting and retaining qualified scientific and management personnel and establishing clinical trial sites and patient registration for clinical trials, as well as in acquiring technologies complementary to, or necessary for, our programs. Our commercial opportunity could be reduced or eliminated if our competitors develop and commercialize products that are safer, more effective, have fewer or less severe side effects, are more convenient or are less expensive than any products that we may develop. Our competitors also may obtain U.S. Food and Drug Administration (“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.

If our current programs are approved for the indications for which we are currently planning clinical trials, they may compete with other products currently under development, including gene editing and gene therapy products. Competition with other related products currently under development may include competition for clinical trial sites, patient recruitment, and product sales.

Manufacturing

We contract with third parties for the manufacturing of materials for preclinical studies and our planned clinical trials. We have no manufacturing operations and do not own or operate any manufacturing facilities for large-scale production of our program materials. At the appropriate time in the product development of each product candidate, we will determine whether to invest in internal manufacturing capabilities or rely on third parties to manufacture late-stage clinical or commercial quantities of any products that we may successfully develop and have approved. The use of contracted manufacturing and reliance on collaboration partners is relatively cost-efficient and has eliminated the need for direct investment in manufacturing facilities and additional staff. Although we rely on contract manufacturers, we have personnel with manufacturing experience to oversee our contract manufacturers. We expect third-party manufacturers to be capable of providing sufficient quantities of our program materials to meet anticipated needs for preclinical studies and clinical trials. We believe that there are alternate sources of supply that can satisfy our preclinical, clinical, and commercial requirements, although we cannot be certain that identifying and establishing relationships with such sources, if necessary, would not result in significant delay or material additional costs.

Commercialization

We will evaluate whether to build the commercial infrastructure in the United States necessary to effectively support the commercialization of our programs, if and when we first believe a regulatory approval of a product candidate under one of our programs in a particular geographic market appears probable. In Europe and elsewhere outside of the United States, where appropriate, we may elect in the future to utilize strategic partners, distributors, or contract sales forces to assist in the commercialization of our products. In certain instances, we may consider building our own commercial infrastructure.

As product candidates advance through our pipeline, our commercial plans may change. In particular, some of our research programs target potentially larger indications. Data, the size of the development programs, the size of the target market, the size of a commercial infrastructure, and manufacturing needs may all influence our strategies in the United States, Europe, and the rest of the world.

Government Regulation and Licensure of Products

Government authorities in the United States, at the federal, state and local level, and in other countries and jurisdictions, including the EU, extensively regulate, among other things, the research, development, testing, manufacture, pricing, reimbursement, sales, quality control, approval, packaging, storage, recordkeeping, labeling, advertising, promotion, distribution, marketing, post-approval monitoring and reporting, and import and export of pharmaceutical products, including biological products. The processes for obtaining marketing approvals in the United States and in foreign countries and jurisdictions, along with subsequent compliance with applicable statutes and regulations and other regulatory authorities, require the expenditure of substantial time and financial resources. 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.

Licensure and Regulation of Biologics in the United States

In the United States, our candidate products would be regulated as biological products, or biologics, under the Public Health Service Act (the "PHSA") and the Federal Food, Drug and Cosmetic Act (the "FDCA") and its implementing regulations and guidances. A company, institution, or organization which takes responsibility for the initiation and management of a clinical development program for such products, and their approval by regulatory authorities, is generally referred to as a sponsor. The failure to comply with the applicable U.S. requirements at any time during the product development process, including non-clinical testing, clinical testing, the approval process or post-approval process, may subject a sponsor to delays in the conduct of the study, regulatory review and approval, and/or administrative or judicial sanctions.

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 ("GLP") regulations and standards;
- completion of the manufacture, under current Good Manufacturing Practices ("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 application ("IND") for human clinical testing, which must become effective before human clinical trials may begin;
- approval by an independent institutional review board ("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 ("GCP");

- preparation and submission to the FDA of a Biologic License Application ("BLA") for a biologic product requesting marketing for one or more proposed indications, including submission of detailed information on the manufacture and composition of the product in clinical development and proposed labelling;
- review of the product by an FDA advisory committee, where appropriate or if 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 and 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 ("GTP") 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 GCPs and the integrity of clinical data in support of the BLA;
- payment of application and program fees pursuant to the Prescription Drug User Free Act ("PDUFA") securing FDA approval of the BLA and licensure of the new biologic product; and
- compliance with any post-approval requirements, including the potential requirement to implement a Risk Evaluation and Mitigation Strategy ("REMS") and any post-approval studies required by the FDA.

Preclinical Studies and Investigational New Drug Application

Before testing any biologic product candidate in humans, including a gene therapy 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. With passage of the FDA's Modernization Act 2.0 in December 2022, Congress eliminated provisions in both the FDCA and the 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, such as organ-on-a-chip systems and computational modeling, which are referred to as in silica models, as well as advanced in vitro assays. 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 product candidate to be shipped in interstate commerce for use in an investigational clinical trial and a request for FDA authorization to administer such investigational product to humans. The 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. In that case, the IND sponsor and the FDA must resolve any outstanding FDA concerns before the clinical trials can begin or recommence.

As a result, submission of the IND may result in the FDA not allowing the trials to commence or allowing the trial to commence on the terms originally specified by the sponsor in the IND. If the FDA raises concerns or questions either during this initial 30-day period, or at any time following the allowance of the IND, it may choose to impose a partial or complete clinical hold. Clinical holds are imposed by the FDA whenever there is concern for patient safety and may be a result of new data, findings, or developments in clinical, nonclinical, and/or chemistry, manufacturing, and controls ("CMC"). This order issued by the FDA would delay either a proposed clinical study or cause suspension of an ongoing study, until all outstanding concerns have been adequately addressed and the FDA has notified the company that investigations may proceed. This could cause significant delays or difficulties in completing planned clinical studies in a timely manner.

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. The rules and regulations related to expanded access are intended to improve

access to investigational products for patients who may benefit from investigational therapies. 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.

When considering an IND application for expanded access to an investigational product with the purpose of treating a patient or a group of patients, the sponsor and treating physicians or investigators will determine suitability 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 initiation, conduct, or completion of clinical investigations that could support marketing approval of the product or otherwise compromise the potential development of the product.

There is no obligation for a sponsor to make its drug products available for expanded access; however, as required by the 21st Century Cures Act (the "Cures Act"), passed in 2016, if a sponsor has a policy regarding how it responds to expanded access requests, it must make that policy publicly available. Although these requirements were rolled out over time, they have now come into full effect. 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 October 2025, the FDA issued final guidance further clarifying the statutory and regulatory requirements governing expanded access.

In addition, on May 30, 2018, the Right to Try Act, was signed into law. The law, among other things, provides a federal framework for certain patients to access certain investigational products that have completed a Phase I clinical trial and that are undergoing investigation for FDA approval. Under certain circumstances, eligible patients can seek treatment without enrolling in clinical trials and without obtaining FDA permission under the FDA expanded access program. There is no obligation for a manufacturer to make its investigational products available to eligible patients as a result of 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 a qualified principal investigator in accordance with GCP requirements. 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.

Further, 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. 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 or the subjects or patients are being exposed to an unacceptable health risk. Clinical testing also must satisfy extensive GCP rules and the requirements for informed consent.

Progress reports detailing the results of the clinical trials must be submitted at least annually to the FDA and more frequently if serious adverse events occur. These reports must include a development safety update report ("DSUR"). In addition, IND safety reports must be submitted to the FDA for any of the following: serious and unexpected suspected adverse reactions; findings from other studies or animal or in vitro testing that suggest a significant risk in humans exposed to the drug; and any clinically important increase in the case of a serious suspected adverse reaction over that listed in the protocol or investigator brochure. The FDA will typically inspect one or more clinical sites to assure compliance with GCP and the integrity of the clinical data submitted. In December 2025, the FDA released final guidance outlining its processes and practices applicable to bioresearch monitoring inspections.

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 ("DMC"). This group may recommend continuation of the

study as planned, changes in study conduct, or cessation of the study at designated check points based on certain available data from the study to which only the DMC has access. 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 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 patients, such as patients suffering from sickle cell disease or cancer.

- *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 may 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. Phase 3 clinical trials are undertaken within an expanded patient population to further evaluate dosage, provide substantial evidence of clinical efficacy, and further test for safety in an expanded and diverse patient population at multiple, geographically dispersed clinical trial sites. 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."

In February 2026, the Commissioner of FDA and the Director of Center for Biologics Evaluation and Research published an editorial in the New England Journal of Medicine in which they declared that, in most cases, the new default requirement for FDA approval of a new product will be one adequate and well-controlled pivotal clinical trial plus confirmatory evidence, rather than two pivotal clinical trials. In determining whether to rely on one trial, the FDA will focus on the single trial's quality, including magnitude of effect, appropriateness of control arms, endpoint selection, statistical power, blinding, handling of missing data, biological plausibility and alignment with intermediate biomarkers. The FDA has long had authority to approve new products on the basis of one trial plus confirmatory evidence and, in recent years, the agency has exercised that authority with respect to certain types of products. The FDA now takes the position that this will be the new official default standard for most product candidates. At this point, it is unclear how this new policy will be implemented by the FDA and how, if at all, it will affect our clinical development programs.

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 and effectiveness after approval. Such post-approval trials are typically referred to as Phase 4 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. If the FDA approves a product while a company has ongoing clinical trials that were not necessary for approval, a company may be able to use the data from these clinical trials to meet all or part of any Phase 4 clinical trial requirement or to request a change in the product labeling. Failure to exhibit due diligence with regard to conducting Phase 4 clinical trials could result in withdrawal of approval for products.

In December 2022, with the passage of Food and Drug Omnibus Reform Act ("FDORA"), Congress required sponsors to develop and submit a Diversity Action Plan ("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. On January 27, 2025, in response to an executive order issued by President Trump on January 21, 2025, relating to Diversity, Equity and Inclusion programs, the FDA removed the draft DAP 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 diversity action plans 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 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 September 2024, 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 ("COAs") in clinical trials, and the role that COAs may play in evaluating the clinical benefit of a medical product.

Sponsors of clinical trials are required to register and disclose certain clinical trial information on a public registry (clinicaltrials.gov) maintained by the NIH. The NIH's Final Rule on registration and reporting requirements for clinical trials became effective in 2017, and both NIH and the FDA have signaled the government's willingness to begin enforcing those requirements against non-compliant clinical trial sponsors. Although the FDA has historically not enforced these reporting requirements due to Department of Health and Human Services' ("HHS") long delay in issuing final implementing regulations, FDA has as of January 2026 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 of non-compliance 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.

Clinical Trials Outside the United States in Support of FDA Approval

In connection with our clinical development program, we may have trial sites outside the United States from time to time. 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 ("IEC") and seeking and receiving informed consent from subjects. GCP requirements encompass both ethical and data integrity standards for clinical studies. The FDA's regulations are intended to help ensure the protection of human subjects enrolled in non-IND foreign clinical 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 United States, 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 December 2025, in the context of negotiations involving reauthorization of PDUFA, the FDA proposed cutting fees for companies conducting clinical development programs in the United States, rather than abroad. It is unclear whether and how this proposal will be adopted and finalized.

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.

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-new drug application ("NDA") meetings as well as end of phase meetings such as EOP2 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. 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.

Pediatric Studies

Under the Pediatric Research Equity Act of 2003 ("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 also submit pediatric study plans prior to the assessment data. Those plans must contain an outline of the proposed pediatric study or studies the sponsor plans to conduct, including study objectives and design, any deferral or waiver requests, and other information required by regulation. The sponsor, the FDA, and the FDA's internal review committee must then review the information submitted, consult with each other, and agree upon a final plan. The FDA or the sponsor may request an amendment to the plan at any time.

For 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, the FDA will meet early in the development process to discuss pediatric study plans with sponsors and the 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 the 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. 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 FDA is required 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. Unless otherwise required by regulation, the pediatric data requirements do not apply to products with orphan designation, although the FDA has 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. 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 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 transferred to cells *ex vivo* prior to administration to the recipient.

Within the FDA, the Center for Biologics Evaluation and Research ("CBER") regulates gene therapy products. Within the CBER, the review of gene therapy and related products is consolidated in the Office of Cellular Therapeutic Products, and the FDA has established the Cellular, Tissue and Gene Therapies Advisory Committee to advise CBER on its reviews. The NIH, including its Novel and Exceptional Technology Research Advisory Committee ("NExTRAC"), also

advises the FDA on gene therapy issues and other issues related to emerging biotechnologies. The FDA and the NIH have published guidance documents with respect to the development and submission of gene therapy protocols.

The FDA has issued numerous guidance documents regarding gene therapies. Although the FDA has indicated that these and other guidance documents it previously issued are not legally binding, compliance with them is likely necessary to gain approval for any gene therapy product candidate. The guidance documents provide additional factors that the FDA will consider at each of the above stages of development and relate to, among other things, the proper preclinical assessment of gene therapies; the chemistry, manufacturing, and control 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; and measures to observe delayed adverse effects in subjects who have been exposed to investigational gene therapies when the risk of such effects is high. Further, the FDA usually recommends that sponsors observe subjects for potential gene therapy-related delayed adverse events for a 15-year period, including a minimum of five years of annual examinations followed by 10 years of annual queries, either in person or by questionnaire.

Compliance with cGMP and GTP Requirements

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.

For a gene therapy product, the FDA also will not approve the product if the manufacturer is not in compliance with GTP. These standards are found in FDA regulations and guidances 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 ("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. Establishments may be subject to periodic unannounced inspections by government authorities to ensure compliance with cGMPs and other laws. 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.

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. 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 and Filing of a BLA

The results of product candidate development, preclinical testing, and clinical trials, including negative or ambiguous results as well as positive findings, are submitted to the FDA as part of a BLA requesting license to market the product. The BLA must contain extensive manufacturing information and detailed information on the composition of the product and proposed labeling as well as payment of a user fee. Under federal law, the submission of most BLAs is subject

to an application user fee, which for federal fiscal year 2026 is approximately $4.7 million for an application requiring clinical data. The sponsor of a licensed BLA is also subject to an annual program fee, which for fiscal year 2026 is $442,213. Certain exceptions and waivers are available for some of these fees, such as an exception from the application fee for products with orphan designation and a waiver for certain small businesses.

The FDA conducts a preliminary review of all applications within 60 days of receipt and must inform the sponsor at that time or before whether an application is sufficiently complete to permit substantive review. In pertinent part, the FDA's regulations 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 ("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 the subject of an RTF. The internal guidance also provides that the agency 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.

On the other hand, once the submission has been accepted for filing, the FDA begins an in-depth review of the application. Under the goals and policies agreed to by the FDA under the PDUFA, the FDA has ten months in which to complete its initial review of a standard application and respond to the sponsor, and six months for a priority review of the application. The FDA does not always meet its PDUFA goal dates for standard and priority BLAs. The review process may often be significantly extended by FDA requests for additional information or clarification. The review process and the PDUFA goal date may be extended by three months if the FDA requests or if the sponsor otherwise provides additional information or clarification regarding information already provided in the submission within the last three months before the PDUFA goal date.

Before approving an application, the FDA typically will inspect the facility or facilities where the product is or will be manufactured. These pre-approval inspections may cover all facilities associated with a BLA submission, including component manufacturing (e.g., active pharmaceutical ingredients), finished product manufacturing, and control testing laboratories. The FDA will not approve an application unless it determines that the manufacturing processes and facilities are in compliance with cGMP requirements and adequate to assure consistent production of the product within required specifications. Additionally, before approving a BLA, the FDA will typically inspect one or more clinical sites to assure compliance with GCP. 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 the 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. That 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 and efficacy of the investigational product.

In connection with its review of a BLA, the FDA may refer the application to an advisory committee for review, evaluation, and recommendation as to whether the application should be approved. In particular, the FDA may refer applications for novel biologic products or biologic products that present difficult questions of safety or efficacy to an advisory committee. Typically, an advisory committee is a panel of independent experts, including clinicians and other scientific experts, that reviews, evaluates, and provides a recommendation as to whether the application should be approved and under what conditions. The FDA is not bound by the recommendations of an advisory committee, but it considers such recommendations carefully when making decisions.

The FDA's Decision on a BLA

Under the PHSA, the FDA may approve a BLA if it determines that the product is safe, pure, and potent and the facility where the product will be manufactured meets standards designed to ensure that it continues to be safe, pure, and potent. The potency of a product is required to be supported by substantial evidence. The FDA has previously interpreted this evidentiary standard to require at least two adequate and well-controlled clinical investigations to establish

effectiveness of a new product. In December 2025, however, the FDA signaled that it is considering only requiring one clinical study for approval of certain 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 issued such guidance.

On the basis of the FDA's evaluation of the application and accompanying information, including the results of the inspection of the manufacturing facilities and any FDA audits of non-clinical and clinical trial sites to assure compliance with GCPs, the FDA may issue an approval letter or a complete response letter ("CRL"). To reach this determination, the FDA must determine that the investigational product is effective and that its expected benefits 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.

An approval letter authorizes commercial marketing of the product with specific prescribing information for specific indications. If the application is not approved, the FDA will issue a CRL, which will contain the conditions that must be met in order to secure final approval of the application, and when possible will outline recommended actions the sponsor might take to obtain approval of the application. Sponsors that receive a CRL may submit to the FDA information that represents a complete response to the issues identified by the FDA. Such resubmissions are classified under PDUFA as either Class 1 or Class 2. The classification of a resubmission is based on the information submitted by a sponsor in response to an action letter. Under the goals and policies agreed to by the FDA under PDUFA, the FDA has two months to review a Class 1 resubmission and six months to review a Class 2 resubmission. The FDA will not approve an application until issues identified in the CRL have been addressed. While CRLs were previously treated by the FDA as confidential and were only disclosed in action packages for approved products, the agency 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.

If the FDA approves a new product, it may limit the approved indications for use of the product. It may also require that contraindications, warnings or precautions be included in the product labeling. In addition, the FDA may call for post-approval studies, including Phase 4 clinical trials, to further assess the product's safety after approval. The agency may also require testing and surveillance programs to monitor the product after commercialization, or impose other conditions, including distribution restrictions or other risk management mechanisms, including REMS, to help ensure that the benefits of the product outweigh the potential risks. REMS can include medication guides, communication plans for healthcare professionals, and elements to assure safe use ("ETASU"). ETASU can include, but are not limited to, special training or certification for prescribing or dispensing, dispensing only under certain circumstances, special monitoring, and the use of patent registries. The FDA may prevent or limit further marketing of a product based on the results of post-market studies or surveillance programs. After approval, many types of changes to the approved product, such as adding new indications, manufacturing changes and additional labeling claims, are subject to further testing requirements and FDA review and approval.

Expedited Review Programs

The FDA is authorized to expedite the review of applications in several ways. None of these expedited programs, however, changes the standards for approval but they may help expedite the development or approval process of product candidates.

- *Fast Track designation.* Candidate products are eligible for Fast Track designation if they are intended to treat a serious or life-threatening condition and demonstrate the potential to address unmet medical needs for the condition. Fast Track designation applies to the combination of the product candidate and the specific indication for which it is being studied. In addition to other benefits, such as the ability to have greater interactions with the FDA, the FDA may initiate review of sections of a Fast Track application before the application is complete, a process known as rolling review.

- *Breakthrough Therapy designation.* To qualify for the Breakthrough Therapy program, product candidates must be intended to treat a serious or life-threatening condition and preliminary clinical evidence must indicate that such product candidates may demonstrate substantial improvement on one or more clinically significant endpoints over existing therapies. The FDA will seek to ensure the sponsor of a Breakthrough Therapy product candidate

receives intensive guidance on an efficient development program, intensive involvement of senior managers and experienced staff on a proactive, collaborative and cross-disciplinary review and rolling review.

- *Priority review.* A product candidate is eligible for priority review if it treats a serious condition and, if approved, it would be a significant improvement in the safety or effectiveness of the treatment, diagnosis or prevention compared to marketed products. The FDA aims to complete its review of priority review applications within six months as opposed to 10 months for standard review.

- *Regenerative advanced therapy.* With passage of the Cures Act in December 2016, Congress authorized the FDA to accelerate review and approval of products designated as regenerative advanced therapies. A product is eligible for this designation if it is a regenerative medicine therapy that is intended to treat, modify, reverse or cure a serious or life-threatening disease or condition and preliminary clinical evidence indicates that the product candidate has the potential to address unmet medical needs for such disease or condition. The benefits of a regenerative advanced therapy designation include early interactions with the FDA to expedite development and review, benefits available to Breakthrough Therapies, potential eligibility for priority review and accelerated approval based on surrogate or intermediate endpoints.

- *Commissioner's National Priority Voucher Program*. 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 ("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.

- *Rare Disease Evidence Principles*. In September 2025, the FDA introduced a framework intended to streamline the approval of new therapies for ultrarare diseases. The Rare Disease Evidence Principles ("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

Drug or biologic products studied for their safety and effectiveness in treating serious or life-threatening conditions and that provide meaningful therapeutic benefit over existing treatments may receive accelerated approval. Accelerated approval means that a product candidate may be approved on the basis of adequate and well controlled clinical trials establishing that the product candidate has an effect on a surrogate endpoint that is reasonably likely to predict a clinical benefit, or on the basis of an effect on a clinical endpoint other than survival or irreversible morbidity or mortality or other clinical benefit, taking into account the severity, rarity and prevalence of the condition and the availability or lack of alternative treatments. As a condition of approval, the FDA may require that a sponsor of a drug or biologic product candidate receiving accelerated approval perform adequate and well controlled post-marketing clinical trials. In addition, the FDA currently requires as a condition for accelerated approval pre-approval of promotional materials.

With passage of 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 a sponsor to have its confirmatory clinical trial underway before accelerated approval is awarded, require a sponsor of a product granted accelerated approval to submit progress reports on its post-approval studies to the FDA every six months (until the study is completed); and 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 agency 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. 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 have 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 and efficacy 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;
- 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 seizure or detention, or refusal to permit the import or export of products; or
- injunctions or the imposition of civil or criminal penalties.

The FDA strictly regulates the marketing, labeling, advertising and promotion of prescription drug products placed on the market. 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. After approval, a drug product generally may not be promoted for uses that are not approved by the FDA, as reflected in the product's prescribing information. 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 or biologic.

On September 9, 2025, President Trump issued a Memorandum directing HHS to "ensure transparency and accuracy in direct-to-consumer ("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 agency 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."

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. Moreover, with passage of the Pre-Approval Information Exchange 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. In addition, in January 2025, the FDA published final guidance outlining the agency's 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 adverse public relations and administrative and judicial enforcement by the FDA, the Department of Justice, or the Office of the Inspector General of the 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.

Orphan Drug Designation and Exclusivity

Orphan drug designation in the United States is designed to encourage sponsors to develop products intended for 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 available the biologic for the disease or condition will be recovered from sales of the product in the United States.

Orphan drug designation qualifies a company for tax credits and market exclusivity for seven years following the date of the product's marketing approval if granted by the FDA. An application for designation as an orphan product can be made any time prior to the filing of an application for approval to market the product. A product becomes an orphan when it receives orphan drug designation from the Office of Orphan Products Development at the FDA based on acceptable confidential requests made under the regulatory provisions. The product must then go through the review and approval process like any other product.

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.

If a product with orphan designation receives the first FDA approval for the disease or condition for which it has such designation or for a select indication or use within the rare disease or condition for which it was designated, the product generally will receive orphan drug exclusivity. Orphan drug exclusivity means that the FDA may not approve another sponsor's marketing application for the same product for the same indication for seven years, except in certain limited circumstances. In particular, the concept of what constitutes the "same drug" for purposes of orphan drug

exclusivity remains in flux in the context of gene therapies, and the FDA has recently issued guidance indicating it would consider two gene therapy products for the same indication to be different, thus each eligible for orphan drug exclusivity, if they express different transgenes or have or use different vectors, so long as those differences are not "minor." 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. If a product designated as an orphan drug ultimately receives marketing approval for an indication broader than what was designated in its orphan drug application, it may not be entitled to exclusivity.

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. The FDA cannot, however, 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.

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 ("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 marketing exclusivity in the United States and, if granted, provides for the attachment of an additional six months of marketing protection to the term of any existing regulatory exclusivity, including orphan exclusivity. 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. The data do not need to show the product to be effective in the pediatric population studied; rather, if the clinical trial is deemed to fairly respond to the FDA's request, the additional protection is granted. If reports of requested pediatric studies are submitted to and accepted by the FDA within the statutory time limits, whatever statutory or regulatory periods of exclusivity or patent protection cover the product are extended by six months. This is not a patent term extension, but it effectively extends the regulatory period during which the FDA cannot approve another application.

Biosimilars and Exclusivity

The 2010 Patient Protection and Affordable Care Act, which was signed into law in March 2010, included a subtitle called the Biologics Price Competition and Innovation Act of 2009 ("BPCIA"). The BPCIA established a regulatory scheme authorizing the FDA to approve biosimilars and interchangeable biosimilars. A biosimilar is a biological product that is highly similar to an existing FDA-licensed "reference product." To date, the FDA has approved both biosimilar and interchangeable biosimilar products.

Under the BPCIA, a manufacturer may submit an application for licensure of a biologic 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 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.

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 approved. Even if a product is considered to be a reference product eligible for exclusivity, another company could market a competing version of that product if the FDA approves a full BLA for such product containing the sponsor's own preclinical data and data from adequate and well-controlled clinical trials to demonstrate the safety, purity, and potency of their product. The BPCIA also created certain exclusivity periods for biosimilars approved as 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.

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.

Patent Term Restoration and Extension

A patent claiming a new biologic product, its method of use or its method of manufacture 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 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 of the IND and the submission date of an application, plus the time between the submission date of an application and the ultimate approval date, less any time the applicant failed to act with 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, 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.

The 2014 guidance also explains that a companion diagnostic device used to make treatment decisions in clinical trials of a biologic product candidate generally will be considered an investigational device, unless it is employed for an intended use for which the device is already approved or cleared. If used to make critical treatment decisions, such as patient selection, the diagnostic device generally will be considered a significant risk device under the FDA's Investigational Device Exemption ("IDE") regulations. Thus, the sponsor of the diagnostic device will be required to comply with the IDE regulations. According to the guidance, if a diagnostic device and a product are to be studied together to support their respective approvals, both products can be studied in the same investigational study, if the study meets both the requirements of the IDE regulations and the IND regulations. The guidance provides that depending on the details of the study plan and subjects, a sponsor may seek to submit an IND alone, or both an IND and an IDE.

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 ("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. For federal fiscal year 2026, the standard fee is $579,272 and the small business fee is $144,818.

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.

Regulation and Procedures Governing Approval of Medicinal Products in the European Union

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 and efficacy 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 regulatory authorities of foreign countries or economic areas, such as the 27-member EU, before it can commence clinical trials or marketing of the product in those countries or jurisdictions. 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 and efficacy of the product for each proposed indication. It also requires the submission to the relevant competent authorities of a marketing authorization application ("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 ("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 ("CTR") became effective in the EU 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 European Union ("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 ("EMA") and available to clinical trial sponsors, competent authorities of the EU Member States and the public. As of January 31, 2025, all clinical trials in the EU (including those which are ongoing) are subject to the CTR.

Beyond streamlining the process, the new regulation 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, which we refer to as 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 concerned EU Member State. However, overall related timelines will be defined by the CTR.

The new regulation 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 study site after the applicable ethics committee has issued a favorable opinion.

Parties conducting certain clinical studies must, as in the U.S., post clinical trial information in the EU at the EudraCT website: https://eudract.ema.europa.eu.

PRIME Designation

In March 2016, the EMA launched an initiative to facilitate development of product candidates in indications, often rare, for which few or no therapies currently exist. The PRIority Medicines ("PRIME") scheme 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. Importantly, a dedicated EMA contact and rapporteur from the Committee for Human Medicinal Products ("CHMP") or Committee for Advanced Therapies are appointed early in the PRIME scheme facilitating increased understanding of the product at the EMA's Committee level. A kick-off meeting initiates these relationships and includes a team of multidisciplinary experts at the EMA to provide guidance on the overall development and regulatory strategies.

Pediatric Studies

Sponsors developing a new medicinal product must agree upon a Pediatric Investigation Plan ("PIP") with the EMA's pediatric committee ("PDCO"), and must conduct pediatric clinical trials in accordance with that PIP, unless a waiver applies (e.g., because the relevant disease or condition occurs only in adults). The PIP sets out the timing and measures proposed to generate data to support a pediatric indication of the drug for which marketing authorization is being sought. The marketing authorization application for the product must include the results of pediatric clinical trials conducted in accordance with the PIP, unless a waiver applies, or a deferral has been granted by the PDCO of the obligation to implement some or all of the measures of the PIP until there are sufficient data to demonstrate the efficacy and safety of the product in adults, in which case the pediatric clinical trials must be completed at a later date.

Marketing Authorization

To obtain a marketing authorization for a product under the EU regulatory system, a sponsor must submit an MAA, either under a centralized procedure administered by the EMA or one of the procedures administered by competent authorities in EU Member States (decentralized procedure, national procedure, or mutual recognition procedure). A marketing authorization may be granted only to a sponsor established in the EU. Regulation (EC) No 1901/2006 provides that prior to obtaining a marketing authorization in the EU, a sponsor 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, class waiver, or a deferral for one or more of the measures included in the PIP.

The centralized procedure provides for the grant of a single marketing authorization by the European Commission that is valid for all EU Member States. Pursuant to Regulation (EC) No. 726/2004, the centralized procedure is compulsory for specific products, including for medicines produced by certain biotechnological processes, products designated as orphan medicinal products, advanced therapy products and products with a new active substance indicated for the treatment

of certain diseases, including products for the treatment of cancer. For products with a new active substance indicated for the treatment of other diseases and products that are highly innovative or for which a centralized process is in the interest of patients, the centralized procedure may be optional. Manufacturers must demonstrate the quality, safety, and efficacy of their products to the EMA, which provides an opinion regarding the MAA. The European Commission grants or refuses marketing authorization in light of the opinion delivered by the EMA.

Specifically, the grant of marketing authorization 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 EMA which provides an opinion regarding the application for marketing authorization. The European Commission grants or refuses marketing authorization in light of the opinion delivered by EMA.

Under the centralized procedure, the CHMP established at the EMA is responsible for conducting an initial assessment of a product. Under the centralized procedure in the EU, the maximum timeframe for the evaluation of an MAA 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 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, EU 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 marketing authorization 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

A marketing authorization 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 marketing authorization is close to the conditional marketing authorization 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 marketing authorization. However, unlike the conditional marketing authorization, the applicant does not, and will not in the future, have to provide the missing data. Although the marketing authorization “under exceptional circumstances” is granted definitively, the risk-benefit balance of the medicinal product is reviewed annually and the marketing authorization is withdrawn in case the risk-benefit ratio is no longer favorable. Under these procedures, before granting the marketing authorization, 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 marketing authorizations, marketing authorizations 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.

Specialized Procedures for Gene Therapies

The grant of marketing authorization in the EU for gene therapy 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 includes specific rules concerning the authorization, supervision, and pharmacovigilance of gene therapy medicinal products. Manufacturers of advanced therapy medicinal products must demonstrate the quality, safety, and efficacy of their products to the EMA, which provides an opinion regarding the MAA. The European Commission grants or refuses marketing authorization in light of the opinion delivered by the EMA.

Regulatory Data Protection in the European Union

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. 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. Even if a compound is considered to be a new chemical 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 December 11. 2025, the European Parliament and 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 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. If the legislation is finalized in line with the provisional political agreement it will have a profound impact on the pharmaceutical industry.

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 and efficacy, 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, for which a separate manufacturer's license is mandatory, 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. Finally, 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.

Reimbursement and Pricing of Prescription Pharmaceuticals

The EU/European Economic Area ("EEA") applies harmonized regulatory rules for medicinal products, for the approval process and requirements governing the conduct of clinical trials, and for the regulatory approval of medicinal products. However, pricing and reimbursement for medicinal products varies greatly between countries and jurisdictions and can involve additional testing for health technology assessments.

In the EU, similar political, economic and regulatory developments to those in the United States may affect our ability to profitably commercialize our product candidates, if approved. In markets outside of the U.S. 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 many countries, including those of the EU, the pricing of prescription pharmaceuticals is subject to governmental control and access. 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, pharmaceutical firms may be required to conduct a clinical trial that compares the cost-effectiveness of the product to other available therapies.

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.

An orphan drug designation provides a number of benefits, including fee reductions, regulatory assistance, and the possibility to apply for a centralized European Union marketing authorization. Marketing authorization for an orphan drug leads to a ten-year period of market exclusivity. During this market exclusivity period, neither the EMA nor the European Commission or the member states can accept an application or grant a marketing authorization for a "similar medicinal product." A "similar medicinal product" is defined as a medicinal product containing a similar active substance or substances as contained in an authorized orphan medicinal product, and which is intended for the same therapeutic indication. The market exclusivity period for the authorized therapeutic indication may, however, be reduced to six years if, at the end of the fifth year, it is established that the product no longer meets the criteria for orphan drug designation because, for example, the product is sufficiently profitable not to justify market exclusivity.

Pediatric Exclusivity

Products that are granted a marketing authorization with the results of the pediatric clinical trials conducted in accordance with the PIP are eligible for a six-month extension of the protection under a supplementary protection certificate (if any is in effect at the time of approval) even where the trial results are negative. In the case of orphan medicinal products, a two-year extension of the orphan market exclusivity may be available. This pediatric reward is subject to specific conditions and is not automatically available when data in compliance with the PIP are developed and submitted.

Patent Term Extensions in the European Union and Other Jurisdictions

The EU also provides for patent term extension through Supplementary Protection Certificates ("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. These periods can 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.

Brexit and the Regulatory Framework in the United Kingdom

As of January 1, 2025, the Medicines and Healthcare Products Regulatory Agency (the "MHRA"), is responsible for approving all medicinal products destined for the UK market (Great Britain and Northern Ireland), and the EMA will no longer have any role in approving medicinal products destined for Northern Ireland. The MHRA relies on the Human Medicines Regulations 2012 (SI 2012/1916) (as amended) (the "HMR"), as the basis for regulating medicines. The HMR has incorporated into domestic law the body of EU law instruments governing medicinal products that pre-existed prior to the United Kingdom's withdrawal from the EU. On April 28, 2025,the UK Parliament adopted amendments to improve and strengthen the UK's clinical trials regulatory regime; they will take effect on April 28, 2026. These changes were needed since the current UK 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 UK left the EU prior to the date on which the EU CTR took effect, the UK legal framework did not benefit from the same revisions as occurred at EU level.

As of January 1, 2024, a new international recognition procedure ("IRP") applies which intends to facilitate approval of pharmaceutical products in the UK. 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 ("RRs"). The RRs notably include 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 U.S.). 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 authorisation for the purposes of IRP.

General Data Protection Regulation

Many countries outside of the United States maintain rigorous laws governing the privacy and security of personal information. 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 subject to the General Data Protection Regulation ("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 United States, 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.

Following the July 2020 Court of Justice of the European Union judgment invalidating the so-called EU-U.S. Privacy Shield, the European Commission adopted an adequacy decision for the EU-U.S. Data Privacy Framework in July 2023. This adequacy decision permits U.S. companies who self-certify under the EU-U.S. Data Privacy Framework to rely on it as a valid data transfer mechanism for data transfers from the European Union to the United States. However, some privacy advocacy groups have already suggested that they will be challenging the EU-U.S. Data Privacy Framework, and there is currently one pending litigation against the EU-U.S. Data Privacy Framework before the Court of Justice of the European Union (the "CJEU"), C-703/25 P – Latombe v Commission. 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 so-called standard contractual clauses and other data transfer mechanisms.

The European Commission decided in June 2021 that the level of data protection in the UK is "essentially adequate" for purposes of data transfer from the EU to the UK. On December 19, 2025, the European Commission renewed this decision until December 27, 2031. The UK and the U.S. have also agreed to a U.S.- UK "Data Bridge," which functions similarly to the EU-U.S. Data Privacy Framework and provides an additional legal mechanism for companies to transfer personal data from the UK to the U.S. Switzerland has also taken 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.-UK Data Bridge in relation to data transfers from Switzerland to the U.S.).

Additionally, in October 2022, President Biden signed an executive order to implement the EU-U.S. Data Privacy Framework, which would serve as a replacement to the EU-U.S. Privacy Shield. The EU initiated the process to adopt an adequacy decision for the EU-U.S. Data Privacy Framework in December 2022 and the European Commission adopted the adequacy decision on July 10, 2023. The adequacy decision will permit 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.

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. Even if any product candidates we may develop are approved, sales of such product candidates will depend, in part, on the extent to which third-party payors, including government health programs in the United States such as Medicare and Medicaid, commercial health insurers, and managed care organizations, provide coverage, and establish adequate reimbursement levels for, such product candidates. The process for determining whether a payor will provide coverage for a product may be separate from the process for setting the price or reimbursement rate that the payor will pay for the product once coverage is approved. Third-party payors are increasingly challenging the prices charged, examining the medical necessity, and reviewing the cost-effectiveness of medical products and services and imposing controls to manage costs. Third-party payors may limit coverage to specific products on an approved list, also known as a formulary, which might not include all of the approved products for a particular indication.

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

The containment of healthcare costs also has become a priority of federal, state and foreign governments and the prices of pharmaceuticals have been a focus in this effort. Governments have shown significant interest in implementing cost-containment programs, including price controls, restrictions on reimbursement, and requirements for substitution of generic products. Adoption of price controls and cost-containment measures, and adoption of more restrictive policies in jurisdictions with existing controls and measures, could further limit a company's revenue generated from the sale of any approved products. Coverage policies and third-party reimbursement rates may change at any time. Even if favorable coverage and reimbursement status is attained for one or more products for which a company or its collaborators receive marketing approval, less favorable coverage policies and reimbursement rates may be implemented in the future.

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. Some countries provide that products may be marketed only after a reimbursement price has been agreed. Some countries may require the completion of additional studies that compare the cost-effectiveness of a particular product candidate to currently available therapies (so called health technology assessments) in order to obtain reimbursement or pricing approval. For example, the EU provides options for its member states to restrict the range of products for which their national health insurance systems provide reimbursement and to control the prices of medicinal products for human use. EU Member States may approve a specific price for a product or it may instead adopt a system of direct or indirect controls on the profitability of the company placing the product on the market. Other member states allow companies to fix their own prices for products, but monitor and control prescription volumes and issue guidance to physicians to limit prescriptions. Recently, many countries in the EU have increased the amount of discounts required on pharmaceuticals and these efforts could continue as countries attempt to manage healthcare expenditures, especially in light of the severe fiscal and debt crises experienced by many countries in the EU. The downward pressure on 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 physicians and teaching physicians and patient privacy laws and regulations and other healthcare laws and regulations that may constrain our business and/or financial arrangements. Restrictions under applicable federal and state healthcare laws and regulations, include the following:

- the U.S. federal Anti-Kickback Statute, which prohibits, among other things, persons and entities from knowingly and willfully soliciting, offering, paying, receiving, or providing remuneration, directly or indirectly, in cash or in kind, to induce or reward either the referral of an individual for, or the purchase, order or recommendation of, any good or service, for which payment may be made, in whole or in part, under a federal healthcare program such as Medicare and Medicaid;

- the federal civil and criminal false claims laws, including the civil False Claims Act, and civil monetary penalties laws, which prohibit individuals or entities from, among other things, knowingly presenting, or causing to be presented, to the federal government, claims for payment that are false, fictitious, or fraudulent or knowingly making, using, or causing to made or used a false record or statement to avoid, decrease, or conceal an obligation to pay money to the federal government;

- the federal civil monetary penalty and false statement laws and regulations relating to pricing and submission of pricing information for government programs, including penalties for knowingly and intentionally overcharging 340b eligible entities and the submission of false or fraudulent pricing information to government entities;

- the federal Health Insurance Portability and Accountability Act of 1996 (“HIPAA”), which created additional federal criminal laws that prohibit, among other things, knowingly and willfully executing, or attempting to execute, a scheme to defraud any healthcare benefit program or making false statements relating to healthcare matters;

- HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act, and their respective implementing regulations, including the Final Omnibus Rule published in January 2013, which impose obligations, including mandatory contractual terms, with respect to safeguarding the privacy, security, and transmission of individually identifiable health information;

- the federal false statements statute, which prohibits knowingly and willfully falsifying, concealing or covering up a material fact or making any materially false statement in connection with the delivery of or payment for health care benefits, items or services;

- the Foreign Corrupt Practices Act, which prohibits companies and their intermediaries from making, or offering or promising to make improper payments to non-U.S. officials for the purpose of obtaining or retaining business or otherwise seeking favorable treatment;

- the federal transparency requirements known as the federal Physician Payments Sunshine Act, under the Patient Protection and Affordable Care Act ("ACA"), as amended by the Health Care Education Reconciliation Act, which requires certain manufacturers of drugs, devices, biologics and medical supplies to report annually to the Centers for Medicare & Medicaid Services ("CMS") within the HHS, information related to payments and other transfers of value made by that entity to physicians, other healthcare providers and teaching hospitals, as well as ownership and investment interests held by physicians and their immediate family members; and

- analogous state and foreign laws and regulations, such as state 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.

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

Healthcare 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.

By way of example, the United States and state governments continue to propose and pass legislation designed to reduce the cost of healthcare. In March 2010, the United States Congress enacted the ACA, which, among other things, includes changes to the coverage and payment for products under government health care programs. In addition, other legislative changes have been proposed and adopted since the ACA 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 pursuant to the Coronavirus Aid, Relief and Economic Security Act.

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. Under current legislation, the actual reductions in Medicare payments may vary up to 4%. The Consolidated Appropriations Act (the "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 ("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 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 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. In

June 2021, the U.S. Supreme Court dismissed an action seeking to strike down the ACA after finding that the plaintiffs do not have standing to challenge the constitutionality of the ACA. Litigation and legislation over the ACA 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 October 2020, the HHS and the FDA published a final rule allowing states and other entities to develop a Section 804 Importation Program ("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 ("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 the 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 agency 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 agency and ultimately shortening the review timeline.

On August 16, 2022, the Inflation Reduction Act of 2022 ("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 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. The 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 nine 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 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 will become effective January 1, 2026. On January 17, 2025, the 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, the 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 the 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., Inc., 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 U.S. Constitution. Subsequently, other parties, including the U.S. Chamber of Commerce, or Chamber of Commerce, Bristol Myers Squibb Company, the PhRMA, Astellas Pharma US, Inc., Novo Nordisk Inc., Janssen Pharmaceuticals, Inc., Novartis Pharmaceutical Corporation, AstraZeneca L.P. and Boehringer Ingelheim Pharmaceuticals, Inc. 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 would pursue other actions, including proposing a rulemaking that imposes Most-Favored Nation ("MFN") pricing in the United States. Thereafter, on July 31, 2025, the President 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 patients in the U.S. and provide for direct purchasing at MFN pricing. Since that time, virtually all of these pharmaceutical companies have entered into agreements with the 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. A number of states, for example, require drug manufacturers and other entities in the drug supply chain, including health carriers, pharmacy benefit managers, wholesale distributors, to disclose information about pricing of pharmaceuticals. 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 pharmaceutical 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.

Federal and State Data Privacy 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 HIPAA, the HHS has issued regulations to protect the privacy and security of protected health information ("PHI") used or disclosed by covered entities including certain healthcare providers, health plans and healthcare clearinghouses. HIPAA also regulates standardization of data content, codes and formats used in healthcare transactions and standardization of identifiers for health plans and providers. 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.

Our clinical trials will be regulated by 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. New laws and regulations governing privacy and security may be adopted in the future as well.

In 2018, California passed into law the California Consumer Privacy Act (the "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 GDPR, 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 (the "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 – whose sole responsibility is to enforce the CPRA, which will further increase compliance risk. The provisions in the CPRA may apply to some of our business activities.

In addition to California, several other states have passed comprehensive privacy laws similar to the CCPA and CPRA that 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 will go into effect that could go into effect in 2026 and beyond. Other states will be considering similar laws in the future, 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. Because of the breadth of these laws and the narrowness of the statutory exceptions and regulatory safe harbors available under such laws, it is possible that some of our current or future business activities, including certain clinical research, sales and marketing practices and the provision of certain items and services to our customers, could be subject to challenge under one or more of such privacy and data security laws. The heightening compliance environment and the need to build and maintain robust and secure systems to comply with different privacy compliance and/or reporting requirements in multiple jurisdictions could increase the possibility that a healthcare company may fail to comply fully with one or more of these requirements. If our operations are found to be in violation of any of the privacy or data security laws or regulations described above that are applicable to us, or any other laws that apply to us, we may be subject to penalties, including potentially significant criminal, civil and administrative penalties, damages, fines, contractual damages, reputational harm, diminished profits and future earnings, additional reporting requirements and/or oversight if we become subject to a consent decree or similar agreement to resolve allegations of non-compliance with these laws, and the curtailment or restructuring of our operations, any of which could adversely affect our ability to operate our business and our results of operations. To the extent that any product candidates we may develop, once approved, are sold in a foreign country, we may be subject to similar foreign laws.

Additional Regulations

In addition to the foregoing, state, and federal laws regarding environmental protection and hazardous substances, including the Occupational Safety and Health Act, the Resource Conservation and Recovery Act, and the Toxic Substances Control Act, affect our business. These and other laws govern the use, handling, and disposal of various biologic, chemical, and radioactive substances used in, and wastes generated by, operations. If our operations result in contamination of the environment or expose individuals to hazardous substances, we could be liable for damages and governmental fines. Equivalent laws have been adopted in third countries that impose similar obligations.

Human Capital

As of February 1, 2026, we had 87 full-time employees, including 26 employees with M.D. or Ph.D. degrees. Approximately 66 of our full-time employees are primarily engaged in research and development activities. None of our employees are represented by a labor union or covered by a collective bargaining agreement.

At Editas, we seek to translate the power and potential of CRISPR gene editing into a robust pipeline of medicines for people living with serious diseases around the world. We recognize that our success is driven by our courageous, collaborative, and passionate employees, who celebrate diversity and together foster a workplace that values inclusivity, where all voices are heard and respected. We seek to be an employer of choice that empowers our employees to drive innovation to develop transformative medicines for people living with serious diseases around the world.

Our Core Values

We work together with integrity, guided by our distinct culture. At the center of our culture are our core values, which guide and define the behaviors that make our culture unique and enable us to bring our best selves forward to achieve our mission of translating the promise of gene editing into a broad class of differentiated, transformational medicines for previously untreatable diseases:

- Engagement – We are active within our teams, Editas, and the broader community.
- Teamwork – We succeed together through collaboration, communication, and mutual respect.
- Drive – We are focused to urgently deliver transformative medicines to patients.
- Resilience – We adapt and learn from setbacks and proactively prepare for future challenges.
- Accountability – We hold ourselves, our teams, and Editas responsible for both our successes and failures.

Our Commitment to Diversity, Equity and Inclusion

We strongly believe that our greatest strength comes from the people who make up our team. Each employee brings diverse perspectives, backgrounds, and thinking styles, and when we champion inclusion, we cultivate a culture where every individual feels valued and can thrive. Our commitment to embracing and celebrating our differences fuels our drive to excel in scientific innovation, allowing us to harness the power of gene editing to develop groundbreaking and life-changing therapies for people living with serious diseases with the greatest unmet needs.

We have cultivated a diverse and inclusive workforce, including in our senior management team, to ensure an environment where employees feel empowered to achieve their fullest potential. As of December 31, 2025, 48% of our full-time employees were women and 37% of our senior management (director level and above) were women. As of December 31, 2025, 44% of our full-time employees identify as racially/ethnically diverse and 37% of our senior management identify as racially/ethnically diverse.

Recruitment, Retention and Development

Successful execution of our strategy is dependent on attracting, retaining and motivating a diverse team of highly skilled employees at all levels. We believe a key component of recruiting, retaining and motivating our employees is our total compensation package. For this reason, we provide employees with competitive compensation, including market-competitive salary and equity awards, along with competitive benefits packages, including medical, dental, vision and life insurance, flexible spending accounts, short- and long-term disability and matching contributions to a 401(k) tax-deferred savings plan. We also provide annual cash incentive bonus opportunities that are tied to both company performance and

individual performance to foster a pay-for-performance culture. We regularly benchmark these total rewards against our industry peers to ensure we remain competitive and attractive to potential new hires.

We believe that continued learning and development, training and other resources are also an essential part of retaining our employees and creating a culture of learning and leadership. We encourage our employees to participate and take advantage of a variety of learning and development resources, including online skills courses, professional development events, and external training programs based on individual needs. We have also implemented formal coaching and mentoring programs, which enable employees to connect with, and learn and develop from, individuals across our company.

Communication and Engagement

We recognize that our employees perform best when they know how their work contributes to our overall strategy. To achieve this, we emphasize open and direct communication through the use of a variety of channels, including quarterly all-company business updates from the senior management team, fireside chats with new members of the Board of Directors and our executive management team, open forums and company-wide written communications, and postings on our company intranet.

In addition, we periodically conduct employee surveys to gauge employee engagement and solicit feedback, and enhance our understanding of the views of our employees, work environment and culture. The results from engagement surveys are used to implement programs and processes designed to enhance employee engagement and improve the employee experience.

Health, Wellness and Safety

Employee safety and well-being is of paramount importance to us. In addition to traditional benefits such as healthcare, flexible time off, paid parental leave, and retirement benefits, we offer a variety of benefits and resources to support employees' physical and mental health, including a lifestyle spending allowance that employees may allocate to certain wellness programs and a third-party employee assistance program, which help us both attract talent and help to realize a healthier workforce.

Our Corporate Information

We were incorporated under the name Gengine, Inc. as a Delaware corporation in September 2013, and we changed our name to Editas Medicine, Inc. in November 2013. Our principal executive offices are located at 11 Hurley St., Cambridge, Massachusetts, 02141, and our telephone number is (617) 401-9000.

Available Information

We maintain an internet website at www.editasmedicine.com and make available free of charge through our website our Annual Reports on Form 10-K, Quarterly Reports on Form 10-Q, Current Reports on Form 8-K and amendments to those reports filed or furnished pursuant to Sections 13(a) and 15(d) of the Securities Exchange Act of 1934 (the "Exchange Act"). We make these reports available through our website as soon as reasonably practicable after we electronically file such reports with, or furnish such reports to, the U.S. Securities and Exchange Commission (the "SEC").

You can review our electronically filed reports and other information that we file with the SEC on the SEC's web site at http://www.sec.gov. We also make available, free of charge on our website, the reports filed with the SEC by our executive officers, directors and 10% stockholders pursuant to Section 16 under the Exchange Act as soon as reasonably practicable after copies of those filings are provided to us by those persons. In addition, we regularly use our website to post information regarding our business, product development programs and governance, and we encourage investors to use our website, particularly the information in the section entitled "Investors & Media," as a source of information about us.

The information on our website is not incorporated by reference into this Annual Report on Form 10-K and should not be considered to be a part of this Annual Report on Form 10-K. Our website address is included in this Annual Report on Form 10-K as an inactive technical reference only.

Item 1A. Risk Factors

Our business is subject to numerous risks. The following important factors, among others, could cause our actual results to differ materially from those expressed in forward-looking statements made by us or on our behalf in this Annual Report on Form 10-K and other filings with the U.S. Securities and Exchange Commission (the "SEC"), press releases, communications with investors, and oral statements. Actual future results may differ materially from those anticipated in our forward-looking statements. We undertake no obligation to update any forward-looking statements, whether as a result of new information, future events, or otherwise.

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 losses were $160.1 million, $237.1 million, and $153.2 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 public offerings of our common stock, our research collaboration with Bristol Myers Squibb Company ("BMS") through its wholly owned subsidiary, Juno Therapeutics, Inc., payments under our former strategic alliance with Allergan Pharmaceuticals International Limited (together with its affiliates, "Allergan"), which was terminated in August 2020, payments received under our purchase and sale agreement with DRI Healthcare Acquisitions LP, and payments under our license agreement with Vertex Pharmaceuticals, Incorporated ("Vertex"). 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:

- continue to support preclinical studies and prepare for the clinical development of EDIT-401;
- initiate and conduct clinical trials of EDIT-401;
- continue our current research programs and our preclinical development of product candidates from our current research programs;
- seek to identify additional product candidates and research programs;
- initiate preclinical testing and clinical trials for other product candidates we identify and develop;
- maintain, expand, 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 gene editing platform;
- hire additional clinical, quality control, and scientific personnel;
- add operational, financial, and management information systems and personnel, including personnel to support our product development;
- acquire or in-license other medicines and technologies; and
- establish, expand or contract for commercial-scale current Good Manufacturing Practices ("cGMP") manufacturing capabilities.

We expect that it will be many years, if ever, before we have a product candidate ready for commercialization. To become and remain profitable, we must develop and 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 testing 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. We are currently only in the preclinical testing stages for our most advanced research programs. 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. A decline in the value of our company could cause our stockholders to lose all or part of their investments in us.

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 commercialization efforts.

We expect our expenses to increase in connection with our ongoing activities, particularly as we identify, continue the research and development of, initiate preclinical studies and clinical trials of, and seek marketing approval for, product candidates, including EDIT-401. In addition, if we obtain marketing approval for any product candidates we 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. We may also need to raise additional funds sooner if we choose to pursue additional indications or geographies for our product candidates or otherwise expand more rapidly than we presently anticipate. Accordingly, we will need to obtain substantial additional funding in connection with our continuing operations. If we are unable to raise capital when needed or on attractive terms, we would be forced to delay, reduce, or eliminate our research and product development programs or future commercialization efforts.

We expect that our existing cash and cash equivalents on December 31, 2025 will fund our operating expenses and capital expenditure requirements into the third quarter of 2027. As of December 31, 2025, our right to contingent payments under our collaboration agreements with BMS, as well as the retained portions of the contingent upfront payment and other amounts under our license agreement with Vertex, are our only significant committed potential external source of funds. Our future capital requirements will depend on many factors, including:

- the costs of progressing the preclinical and clinical development of EDIT-401;
- the scope, progress, results, and costs of drug discovery, preclinical development, laboratory testing, and clinical or natural history study 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 establishing and maintaining a supply chain for the development and manufacture of our product candidates;
- the costs of future activities, including product sales, medical affairs, marketing, manufacturing, and distribution, for any product candidates for which we receive regulatory approval;
- the success of our collaboration with BMS, including whether BMS exercises any of its options to extend the research program term and/or to additional research programs under our collaboration;
- our ability to establish and maintain additional collaborations on favorable terms, if at all;
- the extent to which we acquire or in-license other medicines and technologies;
- the costs of reimbursing our licensors for the prosecution and maintenance of the patent rights in-licensed by us; and

- our ability to establish and maintain healthcare coverage and adequate reimbursement for any product candidates for which we receive regulatory approval.

Identifying potential product candidates and conducting preclinical testing 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 are approved, we will require significant additional funding in order to launch and commercialize our product candidates and 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 at all. 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.

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

Until such time, if ever, as we can generate substantial product revenues, we expect to finance our cash needs through a combination of public or private equity offerings, debt financings, collaborations, strategic alliances, and licensing arrangements. We have limited significant committed potential external source of funds. To the extent that we raise additional capital through the sale of equity or convertible debt securities, the ownership interests of our stockholders may be materially diluted, and the terms of these securities may include liquidation or other preferences that adversely affect the rights of our stockholders. Debt financing, if available, would result in increased fixed payment obligations and may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures, or declaring dividends.

If we raise 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, or we may have to grant licenses on terms that may not be favorable to us.

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

We are currently a pre-clinical stage company and have limited experience conducting clinical trials. We were founded and commenced operations in the second half of 2013. Our operations to date have consisted of organizing and staffing our company, business planning, raising capital, acquiring and developing our technology, identifying potential product candidates, undertaking preclinical studies and initiating and conducting clinical trials. All of our ongoing research programs are in the preclinical or research stage of development, and the risk of failure of all of our research programs is high. We have not yet demonstrated an ability to successfully complete any clinical trials, including large-scale, 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. In addition, we may encounter unforeseen expenses, difficulties, complications, delays, and other known and unknown factors.

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

We expect that our financial condition and operating results will continue to fluctuate significantly from quarter-to-quarter and year-to-year due to a variety of factors, many of which are beyond our control. Accordingly, our stockholders should not rely upon the results of any quarterly or annual periods as indications of future operating performance.

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

Our ability to generate revenue from product sales and achieve profitability depends on our ability, alone or with collaboration 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 years, if ever.

Even if one or more of the product candidates we develop is 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 U.S. Food and Drug Administration (the "FDA"), the European Medicines Agency (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.

Unfavorable national or global economic conditions or political developments could adversely affect our business, financial condition or results of operations.

Our results of operations could be adversely affected by general conditions in the national or global economy and financial markets. For example, governmental statements, actions or policies, political unrest and global financial crises can cause extreme volatility and disruptions in the capital and credit markets. A severe or prolonged economic downturn, political unrest or additional global financial crises, could result in a variety of risks to our business, including weakened demand for our products, if approved, or our ability to raise additional capital when needed on acceptable terms, if at all. A weak or declining economy or unfavorable political developments could also strain our suppliers, possibly resulting in supply disruption. Any of the foregoing could harm our business and we cannot anticipate all of the ways in which the current economic climate, further political developments and financial market conditions could adversely impact our business.

Risks Related to Discovery, Development, and Commercialization

We are dependent on the success of our lead product candidate, EDIT-401, which is in preclinical development. Development of product candidates may not be successful. If we are unable to commence and complete the clinical development of, obtain marketing approval for, or successfully commercialize EDIT-401, either alone or with a collaborator, or if we experience significant delays in doing so, our business would be substantially harmed.

We currently have no products approved for sale and are investing a significant portion of our efforts and financial resources in the development of EDIT-401, which is currently in preclinical development.

Our prospects are substantially dependent on our ability, or that of any future collaborator, to develop, obtain marketing approval for and successfully commercialize EDIT-401. Because our business is significantly dependent upon this one product candidate, any setback in the preclinical or clinical development or the obtaining of regulatory approval for EDIT-401, or any delays in doing so, would have a material adverse effect on our business and prospects.

The success of our EDIT-401 program will depend on several factors, including the following:

- obtaining required regulatory approvals to commence clinical trials of EDIT-401, and the successful enrollment and completion of any such clinical trials;
- safety, tolerability and efficacy profiles that are satisfactory to the FDA, or any comparable foreign regulatory authority for marketing approval;
- timely receipt of marketing approvals from applicable regulatory authorities;
- the performance of our future collaborators, if any;
- the extent of any required post-marketing approval commitments to applicable regulatory authorities;
- establishment and maintenance of supply arrangements with third-party raw materials suppliers and manufacturers for clinical development and, if approved, commercialization of our product candidates;
- establishment and maintenance of arrangements with third-party manufacturers to obtain finished drug products that are appropriately packaged for sale;
- successful development of internal manufacturing processes and transfer to larger-scale facilities operated by either a contract manufacturing organization or by us;

- obtaining and maintaining patent, trade secret protection and regulatory exclusivity, both in the United States and internationally;
- protection of our rights in our intellectual property portfolio;
- successful launch of commercial sales following any marketing approval;
- a continued acceptable safety profile following any marketing approval;
- commercial acceptance by patients, the medical community and third-party payors following any marketing approval; and
- our ability to compete with other therapies.

Many of these factors are beyond our control, including the outcome of clinical development, the regulatory submission process, potential threats to our intellectual property rights, and the manufacturing, marketing and sales efforts of any future commercial partner. If we are unable to develop, receive marketing approval for and successfully commercialize EDIT-401, on our own or with any future collaborator, or experience delays as a result of any of these or other factors, our business would be substantially harmed.

We intend to identify and develop product candidates based on a relatively novel gene editing technology, which makes it difficult to predict the time and cost of product candidate development. Only one therapeutic product that utilizes gene editing technology has been approved in the United States or in Europe.

We have concentrated our research and development efforts on our gene editing platform, which uses CRISPR technology. Our future success depends on the successful development of this relatively novel gene editing therapeutic approach. In 2023, the first and, to date, only, approved therapeutic product that utilizes CRISPR-based gene editing, which is an *ex vivo* therapeutic, was approved in the United States and Europe. To date, no *in vivo* CRISPR-based gene editing therapeutic has been approved in the United States or Europe. It is difficult for us to predict the time and cost of product candidate development, and we cannot predict whether the application of our gene editing platform, or any similar or competitive gene editing platforms, will result in the identification, development, and regulatory approval of any medicines. There can be no assurance that any development problems we experience in the future related to our gene editing platform or any of our research programs will not cause significant delays or unanticipated costs, or that such development problems can be solved. We may also experience delays in developing a sustainable, reproducible, and scalable manufacturing process or transferring that process to commercial partners. Any of these factors may prevent us from completing our preclinical studies or any clinical trials that we may initiate or commercializing any product candidates we develop on a timely or profitable basis, if at all.

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 Therapeutic Products ("OTP") to oversee the review of genetic medicines and related products. It has also established the Cellular, Tissue and Gene Therapies Advisory Committee to advise the Center for Biologics Evaluation and Research on its review of gene therapy products. The FDA has also issued guidance documents, including a January 2024 final guidance entitled "Human Gene Therapy Products Incorporating Human gene editing" outlining the agency's current recommendations regarding information that should be provided in an Investigational New Drug application ("IND") in order to assess the safety and quality of the investigational product. The FDA's regulatory requirements governing genetic and cellular medicines continue to evolve and we will need to monitor and adapt to these requirements as they develop.

The same is true for activities in the European Union (the "EU"). The EMA's Committee for Advanced Therapies ("CAT") is responsible for assessing the quality, safety and efficacy 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 Committee for Human Medicinal Products ("CHMP") before it 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 guidance 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 Recombinant DNA Advisory Committee ("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 a U.S. National Institutes of Health ("NIH")-funded institution to conduct a clinical trial, that institution's institutional biosafety committee as well as its institutional review board ("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.

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 and efficacy of a product candidate vary substantially according to the type, complexity, novelty and intended use and market of the potential products. The regulatory approval process for 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 may 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.

We may not be successful in our efforts to identify, develop, or commercialize potential product candidates.

The success of our business depends primarily upon our ability to identify, develop, and commercialize products based on our gene editing platform. All of our ongoing product development programs are in the preclinical or research stage of development. Our research programs, including those subject to our collaboration with BMS, may fail to identify potential product candidates for clinical development for a number of reasons. Our research methodology may be unsuccessful in identifying potential product candidates, or our potential product candidates may be shown to have smaller patient populations than initially estimated or may have harmful side effects or may have other characteristics or unforeseeable consequences that may make the products impractical to manufacture or commercialize, or unlikely to receive marketing approval.

The occurrence of these events may force us to abandon our development efforts for a program or programs, which could have a material adverse effect on our business, financial condition, results of operations, and prospects. Research programs to identify new product candidates require substantial technical, financial, and human resources. We may focus our efforts and resources on potential programs or product candidates that ultimately prove to be unsuccessful or are otherwise discontinued due to unforeseen circumstances. For example, in December 2024 we announced the discontinuation of our clinical trials of reni-cel. Though reni-cel had demonstrated a favorable safety profile and promising preliminary efficacy, we determined not to pursue commercialization for reni-cel in order to optimize our cost structure and accelerate our *in vivo* pipeline development.

The gene editing field is relatively new and is evolving rapidly. We are focusing our research and development efforts on CRISPR gene editing technology using Cas9 and Cas12a enzymes, but other gene editing technologies may be discovered that provide significant advantages over CRISPR/Cas9 or CRISPR/Cas12a, which could materially harm our business.

To date, we have focused our efforts on gene editing technologies using CRISPR and the Cas9 and Cas12a enzymes. Other companies have previously undertaken research and development of gene editing technologies using zinc finger nucleases, engineered meganucleases, and transcription activator-like effector nucleases, but to date none has obtained marketing approval for a product candidate. There can be no certainty that these other gene editing technologies will not be considered better or more attractive for the development of medicines. Similarly, a new gene editing technology that has not been discovered yet may be determined to be more attractive than CRISPR. Moreover, if we decide to develop genome technologies other than CRISPR technology using a Cas9 or Cas12a enzyme, we cannot be certain we will be able to obtain rights to such technologies. Any of these factors could reduce or eliminate our commercial opportunity, and could have a material adverse effect on our business, financial condition, results of operations, and prospects.

All of our product development programs are at the preclinical or research stage. Preclinical testing and clinical trials of product candidates, including EDIT-401, may not be successful. If we are unable to commercialize any product candidates we develop or experience significant delays in doing so, our business will be materially harmed.

Our ability to generate product revenues, which we do not expect will occur for many years, if ever, will depend heavily on our successful development and eventual commercialization of product candidates that we have identified or may identify in the future. The success of product candidates we may identify and develop will depend on many factors, including the following:

- sufficiency of our financial and other resources to complete the necessary preclinical studies and clinical trials;
- successful completion of preclinical studies and IND-enabling studies;
- successful enrollment in, and completion of, clinical trials;
- timely receipt of marketing approvals from applicable regulatory authorities;

- the performance of collaborators, if any;
- the extent of any required post-marketing approval commitments to applicable regulatory authorities;
- establishment and maintenance of supply arrangements with third-party raw materials suppliers and manufacturers for clinical development and, if approved, commercialization of our product candidates;
- establishment and maintenance of arrangements with third-party manufacturers to obtain finished drug products that are appropriately packaged for sale;
- successful development of internal manufacturing processes and transfer to larger-scale facilities operated by either a contract manufacturing organization ("CMO") or by us;
- obtaining and maintaining patent, trade secret protection and regulatory exclusivity, both in the United States and internationally;
- protection of our rights in our intellectual property portfolio;
- successful launch of commercial sales following any marketing approval;
- a continued acceptable safety profile following any marketing approval;
- commercial acceptance by patients, the medical community and third-party payors following any marketing approval; and
- our ability to compete with other therapies.

If we do not achieve one or more of these factors in a timely manner or at all, we could experience significant delays or an inability to successfully commercialize any product candidates we may identify and develop, which would materially harm our business. We currently generate no revenue from sales of any product and we may never be able to develop or commercialize a marketable product.

In addition, if any product candidates encounter safety or efficacy problems, development delays, regulatory issues or other problems, our development plans and business could be significantly harmed. For the reasons described above, among others, regulatory authorities, particularly the FDA, have requested, and may request in the future, additional preclinical studies for gene editing products, such as additional studies related to toxicology, biodistribution or reproductive health, and/or preclinical studies earlier in clinical development compared to other therapeutic modalities. It is possible that the FDA may impose requirements that result in a delay of any of our programs or their regulatory approval of our product candidates. If we are unable to complete any required studies satisfactorily, the FDA or other regulatory authorities could require that we exclude certain patient populations from clinical studies, place our clinical studies on hold, or require us to cease further clinical studies or deny approval of such product candidates. Further, competitors that are developing products with similar technology may experience problems with their product candidates or programs that could in turn cause us to identify problems with our product candidates and programs, or cause the FDA or other regulatory authorities to impose additional requirements, that could cause us to delay or pause development of our product candidates. Any of these occurrences may harm our ability to identify and develop product candidates, and may harm our business, financial condition, results of operations and prospects significantly. We cannot guarantee that the FDA or other regulatory authorities will not change their requirements in the future or agree with our proposed regulatory strategies and filings.

If serious adverse events, undesirable side effects, or unexpected characteristics are identified during the development of any product candidates we may develop, we may need to abandon or limit our further clinical development of those product candidates.

We have limited experience in evaluating product candidates in human clinical trials, having dosed our first patient in a clinical trial in 2020, and our proposed delivery modes, combined with CRISPR technology, have a limited history of being tested clinically. It is impossible to predict when or if any product candidates we develop will ultimately prove safe in humans. In the genomic medicine field, there have been several significant adverse events from gene therapy treatments in the past, including reported cases of leukemia and death. There can be no assurance that gene editing technologies will not cause severe or undesirable side effects.

A significant risk in any gene editing product is that the edit will be "off-target" and cause serious adverse events, undesirable side effects, or unexpected characteristics. For example, off-target cuts could lead to disruption of a gene or a genetic regulatory sequence at an unintended site in the DNA. We cannot be certain that off-target editing will not occur in any of our future clinical studies. There is also the potential risk of delayed adverse events following exposure to gene editing therapy due to the potential for persistent biological activity of the genetic material or other components of products used to carry the genetic material.

If any product candidates we develop are associated with serious adverse events, or undesirable side effects, or have characteristics that are unexpected, 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.

If any of the product candidates we develop or the delivery modes we rely on cause undesirable side effects, it could delay or prevent their regulatory approval, limit the commercial potential, or result in significant negative consequences following any potential marketing approval.

Product candidates we develop may be associated with off-target editing or other serious adverse events, undesirable side effects, or unexpected characteristics. In addition to serious adverse events or side effects caused by any product candidate we develop and test, the administration process or related procedures also can cause undesirable side effects. If any such events occur, our clinical trials could be suspended or terminated. If we are unable to demonstrate that such 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 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, the FDA could require us to adopt a Risk Evaluation and Mitigation Strategy ("REMS") to ensure that the benefits of treatment with such product candidate outweighs 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. Furthermore, if we or others later identify undesirable side effects caused by any of our product candidates, 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;
- we may be required to change the way a product candidate is administered or 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, results of operations, and prospects.

We have not tested any of our proposed delivery modes, combined with our product candidates, in clinical trials and have not begun clinical trials in any of our current development programs.

Our proposed delivery modes, combined with our product candidates, have a limited history of being evaluated in human clinical trials. Any of our product candidates may fail to show the desired safety and efficacy in later stages of clinical development despite having successfully advanced through initial clinical trials.

There is a high failure rate for drugs and biologics proceeding through clinical trials. A number of companies in the pharmaceutical and biotechnology industries have suffered significant setbacks in later stage clinical trials even after achieving promising results in earlier stage clinical trials. Data obtained from preclinical and clinical activities are subject to varying interpretations, which may delay, limit, or prevent regulatory approval. In addition, 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 planned clinical trials, any of which would have a material adverse effect on our business, financial condition, results of operations, and prospects.

Because we are developing product candidates for the treatment of diseases in which there is little clinical experience using new technologies, there is increased risk that the FDA, the EMA, or other regulatory authorities may not consider the endpoints of our future clinical trials to provide clinically meaningful results and that these results may be difficult to analyze.

During the regulatory review process, we will need to identify success criteria and endpoints such that the FDA, the EMA, or other regulatory authorities will be able to determine the clinical efficacy and safety profile of our product candidates. As we are seeking to identify and develop product candidates to treat diseases in which there is little clinical experience using new technologies, there is heightened risk that the FDA, the EMA, or other regulatory authorities may not consider the clinical trial endpoints that we propose to provide clinically meaningful results. Even if a regulatory authority does find our success criteria to be sufficiently validated and clinically meaningful, we may not achieve the pre-specified endpoints to a degree of statistical significance. This may be a particularly significant risk for genetically defined diseases with small patient populations, where designing and executing a rigorous clinical trial with appropriate statistical power is more difficult than with diseases that have larger patient populations. Regulatory authorities weigh the benefits of a product against its risks, and may view the efficacy results in the context of safety as not being supportive of regulatory approval. Any product candidates we 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. Only one gene editing therapeutic product has been approved in the United States or in Europe.

We currently plan to conduct and may in the future conduct clinical trials for our product candidates outside the U.S., and the FDA and comparable foreign regulatory authorities may not accept data from such trials.

We currently plan to conduct and may in the future conduct clinical trials for our product candidates outside the United States. The acceptance of data from clinical trials conducted outside the U.S. or another jurisdiction by the FDA or comparable foreign regulatory authority 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 United States, 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. 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 product candidates that we may develop not receiving approval for commercialization in the applicable jurisdiction.

If clinical trials of any product candidates we identify and develop fail to demonstrate safety and efficacy 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 of our product candidates, we must complete preclinical development and then conduct extensive clinical trials to demonstrate the safety and efficacy in humans of any such product candidates. 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 testing 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.

We or 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 may identify and develop, including:

- delays in reaching a consensus with regulators on trial design;
- regulators, IRBs or independent ethics committees ("IECs") not authorizing 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 contract research organizations ("CROs") and clinical trial sites;
- clinical trials of any product candidates we develop producing negative or inconclusive results, and us deciding, or regulators requiring us, to conduct additional clinical trials or abandon product development or research programs;
- the number of patients required for clinical trials of any product candidates we develop may be larger than we anticipate; the number of subjects willing to enroll may be smaller than required; enrollment of suitable participants in these clinical trials 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 failing to comply with regulatory requirements or meet their contractual obligations to us in a timely manner, or at all;
- regulators, IRBs, or IECs requiring 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 supply or quality of any product candidates we develop or other materials necessary to conduct clinical trials of any product candidates we develop being insufficient or inadequate, including as a result of delays in the testing, validation, manufacturing, and delivery of any product candidates we develop to the clinical sites by us or by third parties with whom we have contracted to perform certain of those functions;
- occurrence of serious adverse events associated with any product candidates we develop that are viewed to outweigh their potential benefits; and
- changes in regulatory requirements and guidance that require amending or submitting new clinical protocols.

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 tests of any product candidates we develop, or if the results of these trials or tests are not positive or 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 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 modified risk evaluation and mitigation strategy;
- be sued; or
- experience damage to our reputation.

Product development costs will also increase if we or our collaborators experience delays in testing or marketing approvals. We do not know whether 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 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 develop, any of which may harm our business, financial condition, results of operations, and prospects.

If we experience delays or difficulties in the enrollment of patients in clinical trials, our ability to conduct clinical trials on a timely basis or at all and 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 of our product candidates if we are unable to locate and enroll a sufficient number of eligible patients to participate in these trials as required by the FDA or analogous regulatory authorities outside the United States, or as needed to provide appropriate statistical power for a given trial. In addition, if patients are unwilling to participate in our gene 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 develop may be delayed. Moreover, some of our competitors may have approved products or ongoing clinical trials for product candidates that would treat the same indications as any product candidates we develop, and patients who would otherwise be eligible for our clinical trials may instead select the approved product or enroll in clinical trials of our competitors' product candidates.

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 gene 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;
- proximity and availability of clinical trial sites for prospective patients; and
- negative publicity resulting from medicines approved by our competitors.

The eligibility criteria of our clinical trials may limit the pool of available trial participants. Additionally, the process of finding and diagnosing patients may prove costly. Future pandemics or other global health crises may impact our ability to timely enroll trial participants and conduct our studies.

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 CROs and physicians;
- different standards for the conduct of clinical trials;
- different standard-of-care for patients with a particular disease;
- inability to locate 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.

Enrollment delays in our clinical trials may result in increased development costs for any of our product candidates, 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 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.

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 and managerial resources, we focus on research programs and our product candidates for specific indications among many potential options. As a result, we may forgo 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 medicines 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. Any such event could have a material adverse effect on our business, financial condition, results of operations, and prospects.

If we are unable to successfully identify patients who are likely to benefit from therapy with any medicines 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 of our medicines, which may require 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;
- any product candidates we develop may not receive marketing approval if safe and effective use of such product candidates depends on an in vitro diagnostic; 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.

As a result, 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.

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, and any such approval may be for a more narrow 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 develop meet their safety and efficacy 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 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. These regulatory authorities may require 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 develop. Any of the foregoing scenarios could materially harm the commercial prospects for any product candidates we develop and materially adversely affect our business, financial condition, results of operations, and prospects.

Even if any product candidates we 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 will depend upon its degree of market acceptance by physicians, patients, third-party payors, and others in the medical community. The degree of market acceptance of any of our product candidates, 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 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 European Commission, or other regulatory agencies;
- public attitudes regarding genomic medicine generally and gene editing technologies specifically;

- the willingness of the target patient population to try new 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 genome;
- product labeling or product insert requirements of the FDA, 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.

If any of our product candidates do not achieve an adequate level of acceptance, we may not generate significant product revenues, and we may not become profitable.

Adverse public perception of genomic medicines, and gene editing in particular, may negatively impact regulatory approval of, 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. To date, only one gene editing therapy has been approved for sale by the FDA. Public attitudes may be influenced by claims that gene editing is unsafe, unethical, or immoral, and, consequently, our products may not gain the acceptance of the public or the medical community. Adverse public attitudes may adversely impact our ability to enroll eligible patients in 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 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. For example, academic scientists in several countries, including the United States, have reported on their attempts to edit the genome of human embryos as part of basic research and, in November 2018, Dr. Jiankui He, a Chinese biophysics researcher who was an associate professor in the Department of Biology of the Southern University of Science and Technology in Shenzhen, China, announced he had created the first human genetically edited babies, twin girls and helped create a second gene-edited pregnancy. The announcement was negatively received by the public, in particular by those in the scientific community. In the United States, germline editing for clinical application has been expressly prohibited since enactment of a December 2015 U.S. FDA ban on such activity. Prohibitions are also in place in the United Kingdom (the "UK"), across most of Europe, in China, and many other countries around the world. In the United States, the NIH has announced that it would not fund any use of gene editing technologies in human embryos, noting that there are multiple existing legislative and regulatory prohibitions against such work, including the Dickey-Wicker Amendment, which prohibits the use of appropriated funds for the creation of human embryos for research purposes or for research in which human embryos are destroyed. Laws in the UK prohibit genetically modified embryos from being implanted into women, but embryos can be altered in research labs under license from the Human Fertilisation and Embryology Authority. Basic research on embryos is more tightly controlled in many other European countries.

Although we do not use our technologies to edit human embryos or the human germline, such public debate about the use of gene editing technologies in human embryos 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 products we may develop.

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 of our product candidates, we may not be successful in commercializing those product candidates if and when they are approved.

We do not have a sales or marketing infrastructure and have no experience in the sale, marketing, or distribution of pharmaceutical 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 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 medicines on our own include:

- our inability to recruit and retain adequate numbers of effective sales and marketing personnel;
- the inability of sales personnel to obtain access to physicians or persuade adequate numbers of physicians to prescribe any future medicines;
- 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 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 face significant competition in an environment of rapid technological change, and our competitors may achieve regulatory approval before us or develop therapies that are safer or more advanced or effective than ours.

The development and commercialization of new drug products is highly competitive. Moreover, the biotechnology and pharmaceutical industries, including in the gene therapy, gene editing and cell therapy fields, are characterized by rapidly advancing technologies, intense competition, and a strong emphasis on intellectual property and proprietary products. We will face competition with respect to any of our product candidates now and 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. For hyperlipidemia, these companies include AccurEdit Therapeutics, CRISPR Therapeutics, Eli Lilly, EmendoBio, nChroma Bio, Scribe Therapeutics, Tune Therapeutics, and Yoltech Therapeutics. For hemoglobinopathies, these companies include Beam Therapeutics, BRL Medicine, CRISPR Therapeutics, Ensoma, Genetix Biotherapeutics (formerly bluebird bio), Kamau Therapeutics, Orna Therapeutics, Reforgene Medicine, Sanofi, Scribe Therapeutics, Tessera Therapeutics, Vertex, and Yoltech Therapeutics.

Our platform and product focus is the development of therapies using CRISPR technology specifically for gene editing. Other companies developing CRISPR Cas9 or Cas12a technology or therapies using CRISPR Cas9 or Cas12a technology include AccurEdit Therapeutics, Arsenal Biosciences, AvenCell Therapeutics, Caribou Biosciences, Cellistic, Century Therapeutics, CRISPR Therapeutics, EdiGene, eGenesis, ERS Genomics, Excision Biotherapeutics, Fate Therapeutics Inscripta, Intellia Therapeutics, Kamau Therapeutics, Sarepta Therapeutics, Sigma-Aldrich, ToolGen, Vittoria Biotherapeutics, and Yoltech Therapeutics.

In addition, there have been and may continue to be discoveries of new CRISPR-based gene editing technologies. There are additional companies developing therapies using related CRISPR gene editing technologies, including other CRISPR nucleases, base editing, prime editing and gene writing. These companies include Amber Bio, Arbor Biotechnologies, Aurora Therapeutics, Beam Therapeutics, Eli Lilly, Eligo Biosciences, Emendo Biotherapeutics, Ensoma, Epicrispr Biotechnologies, Integra Therapeutics, KSQ Therapeutics, Locus Biosciences, Mammoth Biosciences, Metagenomi, Modalis Therapeutics, nChroma Bio, Prime Medicine, Profluent Bio, Roche, Scribe Therapeutics, Tessera Therapeutics, and Tune Therapeutics.

There are also companies developing therapies using transcription activator-like effector nucleases, meganucleases, Mega-TALs and zinc finger nucleases. These companies include Allogene Therapeutics, BMS, Cellectis, Genetix Biotherapeutics, Precision Biosciences, and Sangamo Therapeutics. In addition to competition from other gene editing therapies, gene therapies or cell medicine therapies, any products that we may develop may also face competition from other types of therapies, such as small molecule, antibody, protein, oligonucleotide, or ribonucleic acid therapies. For hyperlipidemia, these companies include Amgen, Arrowhead Therapeutics, LIB Therapeutics, Merck, NewAmsterdam Pharma, Novartis, Regeneron Pharmaceuticals, and Wave Life Sciences. In addition, statin medications are widely available from numerous pharmaceutical manufacturers and are offered in both branded and generic forms.

Many of our competitors 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 other early-stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies. These competitors also compete with us in recruiting and retaining qualified scientific and management personnel and establishing clinical trial sites and patient registration for clinical trials, as well as in acquiring technologies complementary to, or necessary for, our programs. Our commercial opportunity could be reduced or eliminated if our competitors develop and commercialize products that are safer, more effective, have fewer or less severe side effects, are more convenient, or are less expensive than any products that we may develop or that would render any products that we may develop obsolete or non-competitive. 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. 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 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 products that we may develop and commercialize.

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 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, even if any of our product candidates 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 health administration authorities, private health insurers, and other organizations. Government authorities and third-party payors, such as private health insurers and health maintenance organizations, decide which medications they will pay for and establish reimbursement levels. 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 (the "IRA") includes several measures intended to lower the cost of prescription drugs and related healthcare reforms, including limits on price increases and subjecting an escalating number of drugs to annual price negotiations with The Centers for Medicare & Medicaid Services ("CMS"). We cannot be sure whether additional legislation or rulemaking related to the IRA will be issued or enacted, or what impact, if any, such changes will have on the profitability of any of our product candidates, if approved for commercial use, in the future. Increasingly, third-party payors are requiring that drug companies provide them with predetermined discounts from list prices and are challenging the prices charged for medical products. We cannot be sure that reimbursement will be available for any medicine that we commercialize and, if reimbursement is available, the level of reimbursement. 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 or similar 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. Third-party payors often rely upon Medicare coverage policy and payment limitations in setting their own reimbursement policies. 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 some of our product candidates 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.

The pricing and reimbursement of any of our product candidates, 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 of our product candidates, e.g., for administration of our product to patients, is also important. Inadequate reimbursement for such services may lead to physician resistance and adversely affect our ability to market or sell our products. In addition, it may be necessary for us 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.

The cost of a single administration of our genomic medicine products may 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 develop will be paid by health maintenance, managed care, pharmacy benefit, and similar healthcare management organizations, or will be reimbursed by government authorities, private health coverage insurers, and other third-party payors. 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 of our product candidates. Even if coverage is provided, the approved reimbursement amount may not be adequate to realize a sufficient return on our investment. If we are unable to obtain adequate levels of reimbursement, our ability to successfully market and sell any product candidates we develop will be harmed.

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 of our product candidates 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 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.

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

We and any contract manufacturers and suppliers we engage are subject to numerous federal, state, and local environmental, health, and safety laws, regulations, and permitting requirements, including those governing laboratory procedures; the generation, handling, use, storage, treatment, and disposal of hazardous and regulated materials and wastes; the emission and discharge of hazardous materials into the ground, air, and water; and employee health and safety. Our operations involve the use of hazardous and flammable materials, including chemicals and 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. We do not carry specific biological or hazardous waste insurance coverage, and our commercial general liability and umbrella liability 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.

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.

Genomic medicines are novel, and our product candidates can be complex and difficult to manufacture. We could experience production problems that result in delays in our development or commercialization programs, limit the supply of our products, or otherwise harm our business.

Our product candidates can require processing steps that are more complex than those required for most chemical pharmaceuticals. Moreover, unlike chemical pharmaceuticals, the physical and chemical properties of a biologic such as our product candidates generally cannot be fully characterized. As a result, assays of the finished product may not be sufficient to ensure that the product will perform in the intended manner. Problems with the manufacturing process, even minor deviations from the normal process, could result in development delays, product defects or manufacturing failures that result in lot failures, product recalls, product liability claims, or insufficient inventory. 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, 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.

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 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 are planning to conduct and meet market demand for any products we commercialize.

Risks Related to Our Dependence on Third Parties

We expect to depend on collaborations with third parties for the research, development, and commercialization of certain of the product candidates we develop or for development of certain of our research programs. If any such collaborations are not successful, we may not be able to capitalize on the market potential of those product candidates or research programs.

We anticipate seeking third-party collaborators for the research, development, and commercialization of certain of the product candidates we develop or for development of certain of our research programs. Our likely collaborators include large and mid-size pharmaceutical companies, regional and national pharmaceutical companies, and biotechnology companies. If we enter into any such arrangements with any third parties, we will likely have limited control over the amount and timing of resources that our collaborators dedicate to the development or commercialization of any product candidates we may seek to develop with them and, if applicable, whether they exercise any additional options to commercialize a product. Our ability to generate revenues from these arrangements would 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. Further, if we seek a collaboration partner for a program or product candidate, we may be unable to identify or enter into a collaboration with respect to such program or product candidate on a timely basis, on favorable terms or at all. For example, despite a favorable safety profile and promising preliminary efficacy data, we had difficulties identifying a collaboration partner for our former lead candidate, reni-cel.

Collaborations involving our research programs or any of our product candidates and alliance arrangements we may enter into under which our research programs or product candidates may be involved pose the following risks to us:

- Collaborators may 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 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.

- 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 products, or if one of our collaborators terminates its agreement with us, we may not receive any milestone or royalty payments under such collaborations. If we do not receive the funding we expect under these agreements, 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 any collaborators.

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

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 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 or allies. For example, under our amended and restated collaboration with BMS, we may not use directly or indirectly, or license others to use, gene editing technology in connection with any research, development, manufacture, commercialization or other exploration of certain T cells, subject to certain exceptions, as more fully described in Part I, Item 1 "Business—Our Collaborations and Licensing Strategy" of this Annual Report on Form 10-K. Collaborations are also 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, delay its potential commercialization or reduce the scope of any sales or marketing activities, or increase our expenditures and undertake development or commercialization activities at our own expense. If we elect to increase our expenditures to fund development or commercialization activities on our own, we may need to obtain additional capital, which may not be available to us on acceptable terms or at all. If we do not have sufficient funds, we may not be able to further develop product candidates or bring them to market and generate product revenue.

We expect to rely on third parties to conduct our future 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 currently rely and expect to continue to rely on third parties to conduct some aspects of our research and preclinical testing. We previously relied and expect to rely in the future on third parties, such as CROs, clinical data management organizations, medical institutions, and clinical investigators, to conduct any future clinical trials. Any of these third parties may terminate their engagements with us at any time. If we need to enter into alternative arrangements, our product development activities would be delayed.

Our reliance on these third parties for research and development activities will reduce our control over these activities but will not relieve us of our responsibilities. For example, we will remain responsible for ensuring that each of our clinical trials is conducted in accordance with the general investigational plan and protocols for the trial. Moreover, the FDA requires us to comply with 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. 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.

Furthermore, these third parties may also have relationships with other entities, some of which may be our competitors. If these third parties do not successfully carry out their contractual duties, meet expected deadlines, or conduct our clinical trials in accordance with regulatory requirements or our stated protocols, we will not be able to obtain, or may be delayed in obtaining, marketing approvals for any product candidates we develop and will not be able to, or may be delayed in our efforts to, successfully commercialize our medicines.

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

We contract with third parties for the manufacture of materials for our research programs and preclinical studies and expect to do so in the future for clinical trials and for commercialization of any product candidates that we 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 do not have manufacturing facilities and currently rely on third-party CMOs for the manufacture of our materials for preclinical studies and expect to continue to do so for future clinical trials and for commercial supply of any product candidates that we develop and for which we or our collaborators obtain marketing approval. If we were to experience an unexpected loss or interruption of supply for any of our product candidates, whether as a result of manufacturing, supply or storage issues or otherwise, we could experience delays, disruptions, suspensions or terminations of, or be required to restart or repeat, any pending or ongoing clinical trials. Further, our product candidates may be composed of multiple components and require specialized formulations for which scale-up and manufacturing could be difficult. We will depend on a limited number of third parties for any such scale-up and manufacturing, who may not be able to deliver in a timely manner, or at all. In order to develop products, apply for regulatory approvals, and commercialize our products, we will need to develop, contract for, or otherwise arrange for the necessary manufacturing capabilities.

Additionally, our product candidates have not yet been manufactured for commercial use. If any of our product candidates become approved for commercial sale, we will need to establish either internal or third-party manufacturing capacity. Manufacturing partner requirements may require us to fund capital improvements, perhaps on behalf of third parties, to support the scale-up of manufacturing and related activities. We may not be able to establish scaled manufacturing capacity for an approved product in a timely or economic manner, if at all. If we or our third-party manufacturers are unable to provide commercial quantities of such an approved product, we will have to successfully transfer manufacturing technology to a different manufacturer. Engaging a new manufacturer for such an approved product could require us to conduct comparative studies or utilize other means to determine bioequivalence of the new and prior manufacturers' products, which could delay or prevent our ability to commercialize such an approved product. If we or any of these manufacturers is unable or unwilling to increase its manufacturing capacity or if we are unable to establish

alternative arrangements on a timely basis or on acceptable terms, the development and commercialization of such an approved product may be delayed or there may be a shortage in supply. Any inability to manufacture our product candidates or future approved drugs in sufficient quantities when needed would seriously harm our business. While we are exploring alternative suppliers for certain critical materials, there can be no assurance that our efforts will be successful.

Third-party manufacturers may not be able to comply with cGMP regulations or similar regulatory requirements outside the United States. Our failure, or the failure of our third-party manufacturers, 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. There are a limited number of manufacturers that operate under cGMP regulations and that might be capable of manufacturing for us.

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

Risks Related to Our Intellectual Property

If we are unable to obtain and maintain patent protection for any products we develop and for our technology, or if the scope of the patent 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 of our product candidates, and our technology may be adversely affected.

Our success depends in large part on our ability to obtain and maintain patent protection in the United States and other countries with respect to our CRISPR platform technology and any proprietary product candidates and technology we develop. 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 technologies and product candidates that are important to our business. If we or our licensors and/or collaborators are unable to obtain or maintain patent protection with respect to our CRISPR platform technology and any proprietary products and technology we develop, our business, financial condition, results of operations, and prospects could be materially harmed.

No consistent policy regarding the scope of claims allowable in the field of gene editing, including CRISPR 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, and enforce our intellectual property rights and, more generally, could affect the value of our intellectual property or narrow the scope of our owned and licensed patents. With respect to both in-licensed and owned intellectual property, we cannot predict whether the patent applications we and our licensors are currently pursuing will issue as patents in any particular jurisdiction or whether the claims of any issued patents will provide sufficient protection from competitors.

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. 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.

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 issue as patents, they may not issue in a form that will provide us with any meaningful protection, prevent competitors or other third parties from competing with us, or otherwise provide us with any competitive advantage. Any patents that we hold or in-license may be challenged, narrowed, circumvented, or invalidated by third parties. Consequently, we do not know whether any of our platform advances and product candidates 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. For example, we are aware that third parties have suggested the use of the CRISPR

technology in conjunction with a protein other than Cas9 or Cas12a. Our owned and in-licensed patents may not cover CRISPR technology in conjunction with a protein other than Cas9 or Cas12a. If our competitors commercialize the CRISPR technology in conjunction with a protein other than Cas9 or Cas12a, our business, financial condition, results of operations, and prospects could be materially adversely affected.

The issuance of a patent is not conclusive as to its inventorship, scope, validity, or enforceability, and our patents may be challenged in the courts or patent offices in the United States and abroad. Our licensors are currently, and we or our licensors may in the future become, subject to a third-party pre-issuance submission of prior art to the United States Patent and Trademark Office (the "USPTO") or opposition, derivation, revocation, re-examination, post-grant and *inter partes* review, or interference proceedings and other similar proceedings challenging our patent rights or the patent rights of others. An adverse determination in any such submission, proceeding or litigation could reduce the scope of, or invalidate, our patent rights, allow third parties to commercialize our technology or products and compete directly with us, without payment to us, or result in our inability to manufacture or commercialize products without infringing third-party patent rights. 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 priority of invention or other features of patentability. 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. Such proceedings also may result in substantial cost and require significant time from our scientists and management, even if the eventual outcome is favorable to us. As discussed below, some of our in-licensed patents are subject to interference, opposition and *ex parte* re-examination proceedings and therefore subject to these risks.

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 with third parties. 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 or our licensors may need the cooperation of any such co-owners of our owned and in-licensed patents in order to enforce such patents against third parties, and such cooperation may not be provided to us or our licensors. Any of the foregoing could have a material adverse effect on our competitive position, business, financial conditions, results of operations, and prospects.

Furthermore, our owned and in-licensed patents and patent applications may be subject to a reservation of rights by one or more third parties. For example, the research resulting in certain of our owned and in-licensed patent rights and technology was funded in part by the U.S. government. As a result, the U.S. government has certain rights to such patent rights and technology. These rights may permit the U.S. government to disclose our confidential information to third parties and to exercise march-in rights to use or allow third parties to use our licensed technology. Any exercise by the government of any of the foregoing rights could harm our competitive position, business, financial condition, results of operations, and prospects.

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

We are heavily reliant upon licenses to certain patent rights and proprietary technology from third parties that are important or necessary to the development of our gene editing technology, including our CRISPR technology, and product candidates. 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 technology and products in the future. As a result, we may not be able to prevent competitors from developing and commercializing competitive products in territories included in all of our licenses. For example, pursuant to our license agreements with The Broad Institute, Inc. ("Broad"), and Broad and the President and Fellows of Harvard College ("Harvard"), the licensors may, under certain circumstances, grant a license to the patents that are the subject of such license agreements to a third party. Such third party would have full rights to the patent rights that are the subject of such licenses, which could impact our competitive position and enable a third party to commercialize products similar to our future product candidates and technology. The terms of these license agreements are described more fully under Part I, Item 1 "Business—Our Collaborations and Licensing Strategy" in this Annual Report on Form 10-K.

In addition, we may not have the right to control the preparation, filing, prosecution, maintenance, enforcement, and defense of patents and patent applications covering the technology that we license from third parties. For example, pursuant to each of our intellectual property licenses with Broad and Harvard, our licensors retain control of preparation, filing, prosecution, and maintenance, and, in certain circumstances, enforcement and defense of their patents and patent applications. Therefore, 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, and our right to develop and commercialize any of our products that are the subject of such licensed rights could be adversely affected. Additionally, we are required to reimburse our licensors for all of their expenses related to the prosecution, maintenance, enforcement and defense of patents and patent applications that we in-license from them, and we anticipate that our obligation to reimburse our licensors for expenses related to these matters will continue to be substantial.

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 and patent applications we in-license. For example, certain patent applications licensed to us by Broad are co-owned with NIH. Broad does not and does not purport to grant any rights in NIH's interest in these patent applications under our agreement. If other third parties have ownership rights to our in-licensed patents and patent applications, they may be able to license such patents and patent applications to our competitors, and our competitors could market competing products and technology. This could have a material adverse effect on our competitive position, business, financial conditions, results of operations, and prospects.

Some of our in-licensed patents are subject to priority and validity disputes. In addition, our owned and in-licensed patents, patent applications and other intellectual property may be subject to further priority and validity disputes, and other similar intellectual property proceedings including inventorship disputes. 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 develop, which could have a material adverse impact on our business.

Certain U.S. patents and a U.S. patent application directed to CRISPR/Cas9 that are co-owned by the Broad Institute and the Massachusetts Institute of Technology ("MIT"), and in some cases Harvard (collectively referred to as "Broad"), and in-licensed by us were involved in a first interference with a U.S. patent application that is co-owned by the University of California, the University of Vienna, and Emmanuelle Charpentier (collectively referred to "CVC"). An interference is a proceeding in USPTO before the Patent Trial and Appeal Board of the USPTO ("PTAB") to determine priority of invention of the subject matter of patent claims filed by different parties. In this first interference, the PTAB made a judgment of no interference-in-fact in favor of the Broad, which was upheld on appeal. This decision was final and bars any further interference between the same parties for claims to the same invention that was considered in the interference. As a result of this decision, the U.S. patents and application that we in-license from the Broad and others were not modified or revoked.

On June 24, 2019, the PTAB declared a second interference between certain pending U.S. patent applications that are co-owned by CVC and certain U.S. patents and a U.S. patent application that are co-owned by Broad and in-licensed by us. Most of the Broad U.S. patents and the patent application that are involved in the second interference were also part of the first interference. The invention that was considered in the first interference related to a method involving contacting a target DNA in a eukaryotic cell with certain defined CRISPR/Cas9 components for the purpose of cleaving or editing that target DNA molecule or modulating transcription of at least one gene encoded thereon. The second interference is directed to a different invention, namely a eukaryotic cell comprising a target DNA and certain defined CRISPR/Cas9 components including a single molecule guide RNA that are capable of cleaving or editing the target DNA molecule.

On September 10, 2020, the PTAB granted Broad's motion for priority benefit while denying CVC priority benefit to their two earliest provisional patent applications. As a result, Broad entered the priority phase of the interference as "Senior Party" while CVC remained the "Junior Party" for purposes of determining which entity was the first to invent the inventions at issue. On February 28, 2022, the PTAB issued a decision regarding the priority phase of the interference determining that Broad was the first entity to invent the claims at issue. This decision was appealed by CVC and the Broad cross-appealed. On May 12, 2025, the U.S. Court of Appeals for the Federal Circuit ("CAFC") affirmed-in-part and vacated-in-part the PTAB's previous decision and remanded it back to the PTAB for further review. This decision does not impact our ability to grant licenses or change existing licenses we have to certain issued U.S. patents and a U.S. patent application that are co-owned by Broad and in-licensed by us. It is uncertain when or in what manner the PTAB will ultimately render a decision.

On December 14, 2020, the PTAB, declared two new interferences involving a pending U.S. patent application that is owned by ToolGen, Inc. (the "ToolGen application"). One of the two interferences is between the ToolGen application and certain U.S. patents and U.S. patent applications that are co-owned by Broad and in-licensed by us. Most of the Broad U.S. patents and patent applications that are involved in the interference with ToolGen are also part of the second interference with CVC. The other ToolGen interference is between the same ToolGen application and the U.S. patent applications that are co-owned by CVC and involved in the second interference with Broad. The claims in ToolGen's patent application relate to a mammalian cell with a CRISPR/Cas system comprising a codon optimized nucleic acid encoding a Cas9 polypeptide with a nuclear localization signal and a single-molecule guide RNA that, together, are capable of forming a Cas9/RNA complex that mediates double stranded cleavage of a target nucleic acid sequence. On September 28, 2022, the PTAB suspended both of these interferences. On January 13, 2026, the PTAB indicated that once a decision on remand and judgment have been issued by the PTAB in the second interference between Broad and CVC, the suspension in the interference between ToolGen and the prevailing party of the second interference will be lifted. The other ToolGen interference will remain suspended.

On June 21, 2021, the PTAB declared two new interferences involving a pending U.S. patent application owned by Sigma-Aldrich (the "Sigma-Aldrich application"). One of the two new interferences is between the Sigma-Aldrich application and certain U.S. patents and U.S. patent applications that are co-owned by Broad and in-licensed by us. The other Sigma interference is between the same Sigma-Aldrich application and the U.S. patent applications that are co-owned by CVC. Most of the Broad U.S. patents and patent applications that are involved in the interference with Sigma-Aldrich are also part of the concurrent interferences with CVC and ToolGen. The claims in Sigma-Aldrich's application relate to a method for modifying a chromosomal sequence in a eukaryotic cell by integrating a donor sequence into that chromosomal sequence. These methods use a CRISPR/Cas9 system comprising a Cas9 polypeptide with a nuclear localization signal, a guide RNA, and a donor sequence that, together, are capable of mediating double stranded cleavage and repair of a target nucleic acid sequence leading to integration of the donor sequence into the chromosomal sequence. On December 14, 2022, the PTAB suspended both of these interferences. It is uncertain when these suspensions will be lifted.

As a result of these declarations of interference, five parallel adversarial proceedings in the USPTO before the PTAB have been initiated – the patent interferences between Broad and CVC, Broad and ToolGen, CVC and ToolGen, Broad and Sigma-Aldrich, and CVC and Sigma-Aldrich. We cannot predict with any certainty how long each interference proceeding will take. It is also possible that other third parties may seek to become a party to these interferences.

Our owned and in-licensed patents and patent applications are, or may in the future become, subject to validity disputes in the USPTO and other foreign patent offices. For example, a request for ex parte re-examination was filed with the USPTO on February 16, 2016 against a U.S. patent that we have in-licensed from Broad, which is involved in certain of the interferences. The request for ex parte re-examination was granted on May 9, 2016 thereby initiating a re-examination procedure between the USPTO and Broad, acting on behalf of itself and MIT. The PTAB has suspended the re-examination noting that it has jurisdiction over any file that involves a patent involved in an interference. It is uncertain when the PTAB will lift the suspension. If Broad is unsuccessful during the re-examination, the patent in question may be revoked or narrowed, which could have a material adverse effect on the scope of our rights under such patent.

We or our licensors may also be subject to claims that former employees, collaborators, or other third parties have an interest in our owned or in-licensed patents or patent applications, or other intellectual property rights 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, patent applications or other intellectual property rights, 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, including any patents that issue from patent applications, against third parties, and such cooperation may not be provided to us. Any of the foregoing could have a material adverse effect on the conduct of our business, financial condition, results of operations, and prospects.

We or our licensors are subject to and may in the future become a party to similar proceedings or priority disputes in Europe or other foreign jurisdictions. For example, certain European patents that we have in-licensed from Broad have been revoked in their entirety by the European Patent Office Opposition Division (the "Opposition Division"). Certain other European patents that we have in-licensed from Broad were maintained with amended patent claims. Certain of these decisions have been appealed by both Broad and the opposing party(s), and it is uncertain when or in what manner the Boards of Appeal will act on these appeals. The Opposition Division has also initiated opposition proceedings against certain other European patents that we have in-licensed from Broad. The European Patent Office opposition proceedings may involve issues including, but not limited to, procedural formalities related to filing the European patent application, priority, and the patentability of the involved claims. In view of certain arguments made by the third parties against the

revoked patents and similar arguments made by the third parties against other in-licensed European patents under opposition, the opposition proceedings may lead to the revocation of certain additional in-licensed European patents. The loss of priority for, or the loss of, these European patents could have a material adverse effect on the conduct of our business. One or more of the third parties that have filed oppositions against these European patents or other third parties may file future oppositions against other European patents that we in-license or own. There may be other oppositions against these European patents that have not yet been filed or that have not yet been made available to the public.

If we or our licensors are unsuccessful in any patent related disputes, including interference proceedings, patent oppositions, re-examinations, or other priority, inventorship, or validity disputes to which we or they are subject (including any of the proceedings discussed above), we may lose valuable intellectual property rights through the loss of one or more patents owned or licensed or our owned or licensed patent claims may be narrowed, invalidated, or held unenforceable. In addition, if we or our licensors are unsuccessful in any inventorship disputes to which we or they are subject, we may lose valuable intellectual property rights, such as exclusive ownership of, or the exclusive right to use, our owned or in-licensed patents and patent applications. If we or our licensors are unsuccessful in any interference proceeding or other priority or inventorship dispute, 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 may be non-exclusive or may not be available at all. 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 develop. The loss of exclusivity or the narrowing of our owned and in-licensed patent claims could limit our ability to stop others from using or commercializing similar or identical technology and products. Any of the foregoing could result in a material adverse effect on our business, financial condition, results of operations, or prospects. Even if we are successful in any interference proceeding or other priority, inventorship, or validity disputes, it could result in substantial costs and be a distraction to our management and other employees.

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

Filing, prosecuting, and defending patents on product candidates in all countries throughout the world would be prohibitively expensive, and the laws of foreign countries may not protect our rights to the same extent as the laws of the United States. In addition, our intellectual property license agreements may not always include worldwide rights. For example, certain U.S. patent applications licensed to us by Broad include The University of Tokyo ("Tokyo") and NIH as joint applicants. Broad has only granted a license to us with respect to its interests and to Tokyo's interests in these U.S. patent applications but not to any foreign equivalents thereof. 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 or licenses but enforcement is not as strong as that in the United States. These products may compete with our products, and our patents or other intellectual property rights may not be effective or sufficient to prevent them from competing.

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

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 will be due to be paid to the USPTO and various government 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 various non-U.S. government 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. In some cases, 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 in abandonment or lapse of the patent or patent application, resulting in a partial or complete loss of patent rights in the relevant jurisdiction. In such an event, potential 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.

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 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 that 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 CRISPR gene editing technology, manufacturing methods, product candidates, or future methods or products resulting in either an injunction prohibiting our manufacture or sales, or, with respect to our 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, and we expect in our future agreements, we are 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. Disputes may arise regarding intellectual property subject to a licensing agreement, including:

- the scope of rights granted under the license agreement and other interpretation-related issues;
- the extent to which our technology and processes infringe on intellectual property of the licensor that is not subject to the licensing agreement;
- the sublicensing of patent and other rights under our collaborative development relationships;
- our diligence obligations under the license agreement and what activities satisfy those diligence obligations;
- the inventorship and ownership of inventions and know-how resulting from the joint creation or use of intellectual property by our licensors and us and our partners; 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 increase what we believe to be our financial or other obligations under the relevant agreement, including the amount, if any, that may become due and payable to our licensors in connection with sublicense income. If these events were to occur, they 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, which could have a material adverse effect on our business, financial conditions, results of operations, and prospects.

We may not be successful in obtaining necessary rights to any product candidates we develop through acquisitions and in-licenses.

We currently have rights to intellectual property, through licenses from third parties, to identify and develop product candidates. Many pharmaceutical companies, biotechnology companies, and academic institutions are competing with us in the field of gene editing technology and filing patent applications potentially relevant to our business. For example, we are aware of third party patents and patent applications that may be construed to cover our CRISPR technology and product candidates. In order to avoid infringing these third party patents, or patents that issue from these third party patent applications, we may find it necessary or prudent to obtain licenses from such third party intellectual property holders. We may also require licenses from third parties for certain non-CRISPR technologies including certain delivery methods that we are evaluating for use with product candidates we develop. In addition, with respect to any patents we co-own with third parties, we may require licenses to such co-owners' interest in such patents. 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 our CRISPR technology and product candidates we develop. For example, certain methods for editing cells, guide RNA modifications and delivery modes that we are evaluating for use are covered by patents held by third parties. If we are unable to successfully obtain rights to required third party intellectual 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.

Issued patents covering our technology and product candidates could be found invalid or unenforceable if challenged in court or before administrative bodies in the United States or abroad.

If we or one of our licensors or our collaborators were to initiate legal proceedings against a third party to enforce a patent covering a product candidate we develop or our technology, including CRISPR gene editing technology, the defendant could counterclaim that such patent is invalid or unenforceable. Third parties have raised challenges to the validity of certain of our in-licensed patent claims and may in the future raise similar claims before administrative bodies in the United States or abroad, even outside the context of litigation. These and other proceedings could result in the revocation or cancellation of, or amendment to our patents in such a way that they no longer cover our technology or platform, or any product candidates that we develop. The outcome following legal assertions of invalidity and unenforceability is unpredictable. If a third party were to prevail on a legal assertion of invalidity or unenforceability, we would lose at least part, and perhaps all, of the patent protection on our technology or platform, or any product candidates that we develop. Such a loss of patent protection would have a material adverse impact on our business, financial condition, results of operations, and prospects.

The intellectual property landscape around gene editing technology, including CRISPR, 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 could have a material adverse effect on the success of our business.

The field of gene editing, especially in the area of CRISPR technology, is still in its infancy, and only one CRISPR-based product has 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, the intellectual property landscape is 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 to develop, manufacture, market, and sell any product candidates that we develop and use our proprietary technologies without infringing, misappropriating, or otherwise violating the intellectual property and proprietary rights of third parties. We are 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 technology and any product candidates we develop, including interference, re-examination, post-grant review, inter partes review, and derivation proceedings before the USPTO and similar proceedings in foreign jurisdictions such as oppositions before the European Patent Office. Third parties may assert infringement claims

against us based on existing patents or patents that may be granted in the future, regardless of their merit. We are aware of certain third party patents and patent applications in this landscape that may be asserted to encompass our CRISPR/Cas9 technology. In particular, we are aware of several separate families of U.S. patents and/or U.S. patent applications and foreign counterparts which relate to CRISPR/Cas9 technology, where the earliest priority dates of each family pre-date the priority dates of our in-licensed patents and patent applications, including patent families filed by Vilnius University, by the University of California, the University of Vienna, and Emmanuelle Charpentier, by ToolGen, and by Sigma-Aldrich. Each of these patent families are owned by a different third party and contain claims that may be construed to cover components and uses of CRISPR/Cas9 technology. If we are not able to obtain or maintain a license on commercially reasonable terms to any third-party patents that cover our product candidates or activities, such third parties could potentially assert infringement claims against us, which could have a material adverse effect on the conduct of our business.

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 develop and any other product candidates or technologies covered by the asserted third party patents. 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 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. 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.

If we do not obtain patent term extension and data exclusivity for any product candidates we may develop, it could have a material adverse effect on our business.

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 patent term extension under the Drug Price Competition and Patent Term Restoration Action of 1984 (the "Hatch-Waxman Amendments"). The Hatch-Waxman Amendments permit a patent extension term of up to five years as compensation for patent term lost during the FDA regulatory review process. A patent term extension cannot extend the remaining term of a patent beyond a total of 14 years from the date of product approval, only one patent may be extended and only those claims covering the approved drug, a method for using it, or a method for manufacturing it may be extended. However, an extension may not be granted because of, for example, failure to exercise due diligence during the testing phase or regulatory review process, failure to apply within applicable deadlines, failure to apply prior to expiration of relevant patents, or otherwise failing to satisfy applicable requirements. Further, the applicable time period or the scope of patent protection afforded could be less than we request. If we are unable to obtain patent term extension or if the term of any such extension is less than we request, we will be unable to rely on our patent position to forestall the marketing of competing products and/or competitors may obtain approval of competing products following our patent expirations, and it could have a materially adverse effect on our business, financial condition, results of operations, and prospects.

We may be subject to claims that our employees, consultants, or advisors have wrongfully used or disclosed alleged trade secrets of their current or former employers or claims asserting ownership of what we regard as our own intellectual property.

Many of our employees, consultants, and advisors are currently or were previously employed at universities or other biotechnology or pharmaceutical companies, including our competitors or potential competitors. Although we try to ensure that our employees, consultants, and advisors do not use the proprietary information or know-how of others in their work for us, we may be subject to claims that we or these individuals have used or disclosed intellectual property, including trade secrets or other proprietary information, of any such individual's current or former employer. Litigation may be necessary to defend against these claims. If we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights or personnel. Even if we are successful in defending against such claims, litigation could result in substantial costs and be a distraction to management.

In addition, while it is our policy to require our employees and contractors who may be involved in the conception or development of intellectual property to execute agreements assigning such intellectual property to us, we may be unsuccessful in executing such an agreement with each party who, in fact, conceives or develops intellectual property that we regard as our own. The assignment agreements may be breached, and we may be forced to bring claims against third parties, or defend claims that they may bring against us, to determine the ownership of what we regard as our intellectual property. Such claims could have a material adverse effect on our business, financial condition, results of operations, and prospects.

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 some of our technology and product candidates, we also rely on trade secrets and confidentiality agreements to protect our unpatented know-how, technology, and other proprietary information and to maintain our competitive position. With respect to our technology platform, we consider trade secrets and know-how to be one of our primary sources of intellectual property. Trade secrets and know-how can be difficult to protect. In particular, we anticipate that with respect to our technology platform, these trade secrets and know-how will over time be disseminated within the industry through independent development, the publication of journal articles describing the methodology, and the movement of personnel from academic to industry scientific positions.

We seek to protect these trade secrets and other proprietary technology, in part, by entering into non-disclosure and confidentiality agreements with parties who have access to them, such as our employees, collaborators, CROs, contract manufacturers, consultants, advisors, and other third parties. We also enter into confidentiality and invention or patent assignment agreements with our employees and consultants. 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. Despite these efforts, 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, some courts inside and outside the United States are less willing or unwilling to protect trade secrets. If any of our trade secrets were to be lawfully obtained or independently developed by a competitor or other third party, we would have no right to prevent them from using that technology or information to compete with us. If any of our trade secrets were to be disclosed to or independently developed by a competitor or other third party, our competitive position would be materially and adversely harmed.

Risks Related to Regulatory Approval and Other Legal Compliance Matters

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 of our product candidates. 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, our product candidates, and our ability to generate revenue will be materially impaired.

Any of our product candidates and the activities associated with their development and commercialization, including their design, testing, manufacture, safety, efficacy, recordkeeping, labeling, storage, approval, advertising, promotion, sale, and distribution, are subject to comprehensive regulation by the FDA 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-party CROs 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 biologic product candidate's safety, purity, and potency. Securing regulatory approval also requires the submission of information about the product manufacturing process to, and inspection of manufacturing facilities by, the relevant regulatory authority. Any of our product candidates 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 additional clinical trials are required, 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.

Further, changes in or the enactment of additional statutes, promulgation of regulations or issuance of guidance during preclinical or clinical development, or comparable changes in the regulatory review process 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 Food and Drug Omnibus Reform Act ("FDORA"), Congress required sponsors to develop and submit a diversity action plan ("DAP") for each Phase 3 clinical trial or any other "pivotal study" of a new drug or biological product. These plans were 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.

In February 2026, the Commissioner of FDA and the Director of Center for Biologics Evaluation and Research published an editorial in the New England Journal of Medicine in which they declared that, in most cases, the new default requirement for FDA approval of a new product will be one adequate and well-controlled pivotal clinical trial plus confirmatory evidence, rather than two pivotal clinical trials. In determining whether to rely on one trial, the FDA will focus on the single trial's quality, including magnitude of effect, appropriateness of control arms, endpoint selection, statistical power, blinding, handling of missing data, biological plausibility and alignment with intermediate biomarkers. The FDA has long had authority to approve new products on the basis of one trial plus confirmatory evidence and, in recent years, the agency has exercised that authority with respect to certain types of products. The FDA now takes the position that this will be the new official default standard for most product candidates. At this point, it is unclear how this new policy will be implemented by the FDA and how, if at all, it will affect our clinical development programs.

Further, on January 31, 2022, the new Clinical Trials Regulation (EU) No 536/2014 became applicable in the EU 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 ("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 EMA and available to clinical trial sponsors, competent authorities of the EU Member States and the public. We have not previously secured authorization to conduct clinical studies in the EU pursuant to this new regulation and, accordingly, there is a risk that we may be delayed in commencing any such studies.

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

We may seek certain designations for our product candidates, including Breakthrough Therapy, Fast Track and Priority Review designations in the U.S., and PRIME Designation in the EU, 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.

We may seek certain designations for one or more of our product candidates that could expedite review and approval by the FDA. A Breakthrough Therapy product is defined as a product that is intended, alone or in combination

with one or more other products, to treat a serious condition, and preliminary clinical evidence indicates that the product may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development. For products that have been designated as Breakthrough Therapies, interaction and communication between the FDA and the sponsor of the trial can help to identify the most efficient path for clinical development while minimizing the number of patients placed in ineffective control regimens.

The FDA may also designate a product for Fast Track review if it is intended, whether alone or in combination with one or more other products, for the treatment of a serious or life-threatening disease or condition, and it demonstrates the potential to address unmet medical needs for such a 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 application before the application is complete. This rolling review may be available if the FDA determines, after preliminary evaluation of clinical data submitted by the sponsor, that a Fast Track product may be effective.

We may also seek a priority review designation for one or more of our product candidates. If the FDA determines that a product candidate is intended to treat a serious disease or condition and if approved, would provide a significant improvement in the safety or effectiveness of the treatment, prevention or diagnosis of such disease or condition, the FDA may designate the product candidate for priority review. A priority review designation means that the goal for the FDA to review an application is six months, rather than the standard review period of ten months.

These designations are within the discretion of the FDA. Accordingly, even if we believe that one of our product candidates meets the criteria for these designations, the FDA may disagree and instead determine not to make such designation. Further, even if we receive a designation, the receipt of such designation for a product candidate may not result in a faster development or regulatory review or approval process compared to products considered for approval under conventional FDA procedures and does not assure ultimate approval by the FDA. In addition, even if one or more of our product candidates qualifies for these designations, the FDA may later decide that the product candidates no longer meet the conditions for qualification or decide that the time period for FDA review or approval will not be shortened.

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 the applicant 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 an applicant 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.

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 ("BPCIA") was enacted as part of the Patient Protection and Affordable Care Act ("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 regulatory 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. The law is complex and is still being interpreted and implemented by the FDA. As a result, its ultimate impact, implementation, and meaning are subject to uncertainty, and any new policies or processes adopted by the FDA could have a material adverse effect on the future commercial prospects for our biological 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 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 that 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 would have a material adverse impact on our business due to increased competition and pricing pressure.

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 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 products by the EMA in the EU. Generally, if a product candidate 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 the same product for the same 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.

In order for the FDA to grant orphan drug exclusivity to one of our products, the agency must find that the product is indicated for the treatment of a condition or disease with a patient population of fewer than 200,000 individuals annually 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, that exclusivity may not effectively protect the product from competition because different products can be approved for the same condition. In particular, the concept of what constitutes the "same drug" for purposes of orphan drug exclusivity remains in flux in the context of gene therapies.

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, even after an orphan drug is approved, the FDA can subsequently approve the same product for the same condition if the FDA concludes that the later product is clinically superior in that it is shown to be safer, more effective or makes a major contribution to patient care. 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.

On August 3, 2017, Congress passed the FDA Reauthorization Act of 2017 ("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 by President Trump on December 27, 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 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. The court in *Catalyst Pharms, Inc. v. Becerra* ("Catalyst") 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." Thus, the court concluded, orphan drug exclusivity applies to the entire designated disease or condition rather than the approved "indication or use." Although there have been legislative proposals to overrule this decision, they have not been enacted into law. 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.

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

In order to market and sell any of our product candidates in the EU and many other foreign jurisdictions, we or our collaborators must obtain separate marketing approvals 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 be approved for reimbursement before the product 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 UK as a result of the withdrawal of the UK from the EU, commonly referred to as Brexit. The UK 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 ("MHRA") is responsible for approving all medicinal products destined for the UK market (i.e., Great Britain and Northern Ireland). On April 28, 2025, the UK Parliament adopted amendments to improve and strengthen the UK's clinical trials regulatory regime; they will take effect on April 28, 2026. These changes were needed since the current UK 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 UK left the EU prior to the date on which the EU CTR took effect, the UK legal framework did not benefit from the same revisions as occurred at EU level.

Further, as of January 1, 2025, a new international recognition procedure ("IRP") will apply, which intends to facilitate approval of pharmaceutical products in the UK. 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 ("RRs"). The RRs notably include EMA and regulators in the EU/European Economic Area ("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, as a result of Brexit or otherwise, may force us or our collaborators to restrict or delay efforts to seek regulatory approval in the UK 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 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.

We expect that we will be subject to additional risks in commercializing any of our product candidates that receive marketing approval outside the United States, including tariffs, trade barriers and regulatory requirements; economic weakness, including inflation, or political instability in particular foreign economies and markets (such as the ongoing conflict between Ukraine and Russia); compliance with tax, employment, immigration and labor laws for employees living or traveling abroad; foreign currency fluctuations, which could result in increased operating expenses and reduced revenue, and other obligations incident to doing business in another country; and workforce uncertainty in countries where labor unrest is more common than in the United States.

Even if we, or any collaborators we may have, obtain marketing approvals for any of our product candidates, the terms of approvals and ongoing regulation of our products could require the substantial expenditure of resources and may limit how we, or they, manufacture and market our products, which could materially impair our ability to generate revenue.

Any product candidate for which we obtain marketing approval, along with the manufacturing processes, post-approval clinical data, labeling, advertising, and promotional activities for such medicine, will be subject to continual 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, cGMP requirements relating to quality control, quality assurance and corresponding maintenance of records and documents, and requirements regarding the distribution of samples to physicians and recordkeeping. Even if marketing approval of a product candidate is granted, the approval may be subject to limitations on the indicated uses for which the medicine may be marketed or to the conditions of approval, or contain requirements for costly post-marketing testing and surveillance to monitor the medicine.

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 ("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 the 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.

Accordingly, assuming we, or any collaborators we may have, receive marketing approval for one or more of our product candidates, we, and such collaborators, and our and their contract manufacturers will continue to expend time, money, and effort in all areas of regulatory compliance, including manufacturing, production, product surveillance, and quality control. If we and such collaborators are not able to comply with post-approval regulatory requirements, we and such collaborators could have the marketing approvals for our products withdrawn by regulatory authorities and our, or such collaborators', ability to market any future products could be limited, which could adversely affect our ability to achieve or sustain profitability. Further, the cost of compliance with post-approval regulations may have a negative effect on our business, operating results, financial condition, and prospects.

Disruptions at the FDA and other government agencies from funding cuts, personnel losses, regulatory reform, government shutdowns and other developments could hinder our ability to obtain guidance from the FDA regarding our clinical development programs and develop and secure approval of our product candidates in a timely manner, which would negatively impact our business.

The FDA and comparable regulatory agencies in foreign jurisdictions, such as the European Medicines Agency and Committee for Medicinal Products for Human Use, play an important role in the development of our product candidates by providing guidance on our clinical development programs and reviewing our regulatory submissions, including investigational new drug applications, requests for special designations and marketing applications. If these oversight and review activities are disrupted, then correspondingly our ability to develop and secure timely approval of our product candidates could be impacted in a negative manner.

For example, the recent loss and retirement of FDA leadership and personnel could lead to disruptions and delays in FDA guidance, or review and approval of our product candidates. Pursuant to President Trump's E.O. 14210, "Implementing the President's 'Department of Government Efficiency' Workforce Optimization Initiative," the Secretary of HHS announced on March 27, 2025, a reorganization and reduction in force across HHS of approximately 20,000 employees (82,000 to 62,000), with FDA's workforce of approximately 20,000 to decrease by 3,500 full-time employees. Subsequently, the FDA indicated that roughly a quarter of those employees who received reduction in force notices had been reinstated. On July 14, 2025, following litigation reaching the U.S. Supreme Court, the administration began to carry out these layoffs across HHS, including the FDA. In November 2025, a Congressional Continuing Resolution ended the government shutdown, providing full-year funding for the FDA for fiscal year 2026 through September 30, 2026 at approximately $7 billion with a slight increase in user fees for drug and device companies.

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 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. For example, while the FDA reduction in force did not reportedly specifically target FDA reviewers, many operations, administrative and policy staff that help support such reviews were affected and those losses could lead to delays in PDUFA reviews and related activities. There have been several reports in which the FDA failed to meet a PDUFA goal date for approval of an NDA or BLA due to heavy workload and limited resources. In addition, while currently unclear, there is a risk that the reduction in force and budget cutbacks could threaten the integrity of the PDUFA program itself. That is because, for the FDA to obligate user fees collected under PDUFA in the first place, a certain amount of non-user fee appropriations must be spent on the process for the review of applications plus certain other costs during the same fiscal year.

There is also substantial uncertainty as to how regulatory reform measures being implemented by the Trump Administration across the government will impact the FDA and other federal agencies with jurisdiction over our activities. For example, since taking office, President Trump has issued a number of executive orders that could have a significant impact on the manner in which the FDA conducts its operations and engages in regulatory and oversight activities. These executive orders include E.O. 14192, "Unleashing Prosperity Through Deregulation," January 31, 2025; E.O. 14212, "Establishing the President's Make America Healthy Again Commission," February 13, 2025; and E.O. 14219, "Ensuring

Lawful Governance and Implementing the President's 'Department of Government Efficiency' Deregulatory Initiative," February 21, 2025. If these or other orders or executive actions impose constraints on the FDA's ability to engage in oversight and implementation activities in the normal course, our business may be negatively impacted.

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.

During the last several years the U.S. government has shut down several times and certain regulatory agencies, such as the FDA and the SEC, have had to furlough critical employees and stop critical activities. If a prolonged government shutdown occurs, it could significantly impact the ability of the FDA to timely review and process our regulatory submissions and could impact our ability to access the public markets and obtain necessary capital to properly capitalize and continue our operations.

For example, the federal government shut down on October 1, 2025, and did not reopen for 43 days. With the shutdown, the FDA issued a public notice stating that agency operations would continue to the extent permitted by law, such as activities necessary to address imminent threats to the safety of human life and activities funded by carryover user fee funds. The FDA declared that, during the shutdown period, it did not have legal authority to accept user fees assessed for FY 2026 until an FY 2026 appropriation or Continuing Resolution for the FDA was enacted. As a result, the FDA was not able to accept any regulatory submissions for FY 2026 that required a fee payment and that was submitted during the lapse period. In addition, the FDA indicated that some of its regulatory science research, crucial for advancing product innovation, safety, and quality, would be curtailed during the lapse period.

At the same time, disruptions at the FDA and other government agencies may result from public health events similar to the COVID-19 pandemic. For example, during the 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.

Accordingly, if any of the foregoing developments and others impact the ability of the FDA to provide us guidance regarding our clinical development programs or delay the agency's review and processing of our regulatory submissions, including INDs, NDAs, or BLAs, our business would be negatively impacted. Further, any future government shutdown could impact our ability to access the public markets and obtain necessary capital to properly capitalize and continue our operations.

Even if our product candidates receive regulatory approval, they will be subject to significant post-marketing regulatory requirements and oversight.

Any regulatory approvals that we may receive for our product candidates will require the submission of reports to regulatory authorities and ongoing surveillance to monitor the safety and efficacy of the product candidate, may contain significant limitations related to use restrictions for specified age groups, warnings, precautions or contraindications, and may include burdensome post-approval study or risk management requirements and regulatory inspection. For example, the FDA may require a REMS in order to approve our product candidates, which could entail requirements for a medication guide, physician training and communication plans or additional elements to ensure safe use, such as restricted distribution methods, patient registries and other risk minimization tools. In addition, if the FDA, EMA or foreign regulatory authorities approve our product candidates, the manufacturing processes, labelling, packaging, distribution, adverse event reporting, storage, advertising, promotion, import, export and recordkeeping for our product candidates will be subject to extensive and ongoing regulatory requirements. These requirements include submissions of safety and other post-marketing information and reports, registration, as well as ongoing compliance with cGMPs and good clinical practices ("GCP") for any clinical trials that we conduct post-approval.

In addition, manufacturers of drug products and their facilities are subject to continual review and periodic, unannounced inspections by the FDA, EMA and other regulatory authorities for compliance with cGMP regulations and standards. 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 U.S. prior to being imported

or offered for import into the U.S. If we or a regulatory authority discover previously unknown problems with a product, such as adverse events of unanticipated severity or frequency, or problems with the facilities where the product is manufactured, a regulatory authority may impose restrictions on that product, the manufacturing facility or us, including requiring recall or withdrawal of the product from the market or suspension of manufacturing. In addition, failure to comply with FDA, EMA and other comparable foreign regulatory requirements may subject our company to administrative or judicially imposed sanctions, including:

- delays in or the rejection of product approvals;
- restrictions on our ability to conduct clinical trials, including full or partial clinical holds on ongoing or planned trials;
- restrictions on the products, manufacturers or manufacturing process;
- warning or untitled letters;
- civil and criminal penalties;
- injunctions;
- suspension or withdrawal of regulatory approvals;
- product seizures, detentions or import bans;
- voluntary or mandatory product recalls and publicity requirements;
- total or partial suspension of production;
- imposition of restrictions on operations, including costly new manufacturing requirements;
- revisions to the labelling, including limitation on approved uses or the addition of additional warnings, contraindications or other safety information, including boxed warnings;
- imposition of a REMS, which may include distribution or use restrictions; and
- requirements to conduct additional post-market clinical trials to assess the safety of the product.

The FDA strictly regulates marketing, labeling, advertising, and promotion of products that are placed on the U.S. market, and the relevant foreign regulatory agencies do the same in their respective jurisdictions. The FDA and other agencies actively enforce the laws and regulations prohibiting the promotion of off-label uses and a company that is found to have improperly promoted off-label uses may be subject to significant liability. 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.

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, January 2025, the FDA published final guidance outlining the agency's 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, 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 "HHS"), the FDA, the Federal Trade Commission ("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 Federal Food, Drug and Cosmetic Act, 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.

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 requirements 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.

The FDA, EMA and other regulatory authorities' policies may change and additional government regulations may be enacted that could prevent, limit or delay regulatory approval of our product candidates. We cannot predict the likelihood, nature or extent of government regulation that may asrise from future legislation or administrative action, either in the U.S. or abroad. If we are slow or unable to adapt to changes in existing requirements or the adoption of new requirements or policies, or if we are not able to maintain regulatory compliance, we may lose any marketing approval that we may have obtained and we may not achieve or sustain profitability.

Our relationships with healthcare providers, physicians, and third-party payors will be subject to applicable anti-kickback, fraud and abuse, 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 of our product candidates 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 include the following:

- the federal healthcare anti-kickback statute prohibits, among other things, persons from knowingly and willfully soliciting, offering, receiving, or providing remuneration, directly or indirectly, in cash or in kind, to induce or reward either the referral of an individual for, or the purchase, order, or recommendation of, any good or service, for which payment may be made under federal and state healthcare programs such as Medicare and Medicaid;

- the federal False Claims Act imposes criminal and civil penalties, including civil whistleblower or qui tam actions, against individuals or entities for knowingly presenting, or causing to be presented, to the federal government, claims for payment or approval from Medicare, Medicaid, or other government payors that are false or fraudulent or making a false statement to avoid, decrease, or conceal an obligation to pay money to

the federal government, with potential liability including mandatory treble damages and significant per-claim penalties;

- the federal Health Insurance Portability and Accountability Act of 1996, as further amended by the Health Information Technology for Economic and Clinical Health Act, which imposes certain requirements, including mandatory contractual terms, with respect to safeguarding the privacy, security, and transmission of individually identifiable health information without appropriate authorization by entities subject to the rule, such as health plans, health care clearinghouses, and health care providers;

- the federal false statements statute, which prohibits knowingly and willfully falsifying, concealing, or covering up a material fact or making any materially false statement in connection with the delivery of or payment for healthcare benefits, items, or services;

- the federal transparency requirements under the federal Physician Payment Sunshine Act, which requires manufacturers of drugs, devices, biologics, and medical supplies to report to the HHS information related to payments and other transfers of value to physicians, other healthcare providers and teaching hospitals, and ownership and investment interests held by physicians and their immediate family members and applicable group purchasing organizations; and

- analogous state laws and regulations, such as state anti-kickback and false claims laws, which may apply to sales or marketing arrangements and claims involving healthcare items or services reimbursed by non-governmental third-party payors, including private insurers, and certain state laws that require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government in addition to requiring drug manufacturers to report information related to payments to physicians and other health care providers or marketing expenditures.

Because of the breadth of these laws and the narrowness of the statutory exceptions and safe harbors available, it is possible that some of our business activities could be subject to challenge under one or more of such laws. 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 UK 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.

Efforts to ensure that our business arrangements with third parties will comply with applicable healthcare laws and regulations will involve substantial costs. It is possible that governmental authorities will conclude that our business practices may not comply with current or future statutes, regulations, or case law involving applicable fraud and abuse or other healthcare laws and regulations. If our operations are found to be in violation of any of these laws or any other governmental regulations that may apply to us, we may be subject to significant civil, criminal, and administrative penalties, damages, fines, exclusion from government funded healthcare programs, such as Medicare and Medicaid, and the curtailment or restructuring of our operations. If any of the physicians or other providers or entities with whom we expect to do business are found to be not in compliance with applicable laws, they may be subject to criminal, civil, or administrative sanctions, including exclusions from government funded healthcare programs. Liabilities they incur pursuant to these laws 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.

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 our product candidates, 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, President Obama signed into law the ACA. In addition, other legislative changes have been proposed and adopted since the ACA 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 the Coronavirus Aid, Relief, and Economic Security Act.

Under current legislation, the actual reductions in Medicare payments may vary up to 4%. The Consolidated Appropriations Act (the "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 ("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 Cuts for Jobs 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. In June 2021, the U.S. Supreme Court dismissed the most recent judicial challenge to the ACA brought by several states without specifically ruling on the constitutionality of the statute. Litigation and legislation over the ACA are likely to continue, with unpredictable and uncertain results.

The prices of prescription pharmaceuticals in the United States and foreign jurisdictions is 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 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, the HHS and the FDA published a final rule allowing states and other entities to develop a Section 804 Importation Program ("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 ("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 the HHS. Several states have passed laws allowing for the importation of drugs from Canada. On January 5, 2024, the FDA approved Florida's plan for Canadian drug importation. That state now has authority to import certain drugs 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 drug selected for importation, which must be approved by the FDA. The state will also need to relabel the drugs 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 agency 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 agency and ultimately shortening the review timeline.

Further, on November 20, 2020, the 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 final rule would also eliminate the current safe harbor for Medicare drug rebates and create new safe harbors for beneficiary point-of-sale discounts and pharmacy benefit manager service fees. It originally was set to go into effect on January 1, 2022, but has been delayed by Congress 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 2025); and replaces the Part D coverage gap discount program with a new discounting program (beginning in 2025). The IRA permits the Secretary of the HHS to implement many of these provisions through guidance, as opposed to regulation, for the initial years.

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. The 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 nine 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 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. Since the 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.

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 will become effective January 1, 2026. On January 17, 2025, the 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, the 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 the 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., Inc., 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 U.S. Constitution. Subsequently, other parties, including the U.S. Chamber of Commerce, or Chamber of Commerce, Bristol Myers Squibb Company, the PhRMA, Astellas Pharma US, Inc., Novo Nordisk Inc., Janssen Pharmaceuticals, Inc., Novartis Pharmaceutical Corporation, AstraZeneca L.P. and Boehringer Ingelheim Pharmaceuticals, Inc. 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. On April 15, 2025, President Trump issued an Executive Order which directs HHS to take steps to reduce the prices of pharmaceutical products. The new Order repeats many of the proposals advanced during the first Trump Administration, including directing the FDA to streamline and improve its existing drug importation program so as to make it easier for states to obtain approval without sacrificing the safety or quality of drug products. Other provisions of the Order relate to the 340B program. With respect to the IRA's Medicare drug pricing program, the Order, among other things, calls for alignment in "the treatment of small molecule prescription drugs with that of biological products, ending the distortion that undermines relative investment in small molecule prescription drugs, coupled with other reforms to prevent any increase in overall costs to Medicare and its beneficiaries."

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 directs the Secretary of HHS to communicate most-favored-nation ("MFN") price targets to pharmaceutical manufacturers to bring prices in line with comparably developed nations. The Executive Order further provides that if such actions do not lower the costs of pharmaceuticals, the Secretary of HHS would pursue other actions, including proposing a rulemaking that imposes MFN pricing in the United States. The implications of these actions remain unclear and are likely to result in litigation if the administration pursues an MFN regulatory pricing requirement.

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 health care 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 health care 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.

In addition, in some countries, including EU Member States, the pricing of prescription pharmaceuticals is subject to governmental control. In these countries, pricing negotiations with governmental authorities can take a significant amount of time after receipt of marketing approval for a product. In addition, there can be considerable pressure by governments and other stakeholders on prices and reimbursement levels, including as part of cost containment measures. 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 distribution, or arbitrage between low-priced and high-priced member states, can further reduce prices, and in certain instances render commercialization in certain markets infeasible or disadvantageous from a financial perspective. In some countries, we or our collaborators may be required to conduct a clinical trial or other studies that compare the cost-effectiveness of our product and/or our product candidates to other available products in order to obtain or maintain reimbursement or pricing approval. Publication of discounts by third party payors or government authorities may lead to further pressure on the prices or reimbursement levels. If reimbursement of our products is unavailable or limited in scope or amount, or if pricing is set at unsatisfactory levels, the commercial launch of our product and/or product candidates could be delayed, possibly for lengthy periods of time, we or our collaborators may not launch at all in a particular country, we may not be able to recoup our investment in one or more product candidates, and there could be a material adverse effect on our business.

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, principal investigators, consultants, and partners. 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 European Commission, 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 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 any international operations we may have in the future 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 ("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 the company 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.

Compliance with the FCPA 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.

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, contractual obligations and failure to comply with such requirements could subject us to significant fines and penalties, 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 and UK. 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, 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 the Health Insurance Portability and Accountability Act of 1996 ("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. These obligations may be applicable to some or all of our business activities now or in the future.

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 ("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, the 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 agency 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 US 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. In 2018, California passed into law the California Consumer Privacy Act (the "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 General Data Protection Regulation (the "GDPR"), 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 (the "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 – whose sole responsibility is to enforce the CPRA, 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. Other states will be considering similar laws in the future, 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. 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 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 European Commission to offer adequate data protection legislation, such as the U.S. There are ongoing concerns about the ability of companies to transfer personal data from the EU to other countries. Following the July 2020 Court of Justice of the European Union judgment invalidating the so-called EU-U.S. Privacy Shield, the European Commission adopted an adequacy decision for the EU-U.S. Data Privacy Framework in July 2023. This adequacy decision permits U.S. companies who self-certify under the EU-U.S. Data Privacy Framework to rely on it as a valid data transfer mechanism for data transfers from the European Union to the United States. However, some privacy advocacy groups have already suggested that they will be challenging the EU-U.S. Data Privacy Framework, and there is currently one pending litigation against the EU-U.S. Data Privacy Framework 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 EU-U.S. Data Privacy Framework, 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 European Commission 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 UK from the EU, the UK Data Protection Act 2018 applies to the processing of personal data that takes place in the UK and includes parallel obligations to those set forth by GDPR. In

relation to data transfers, both the UK and the EU have determined, through separate "adequacy" decisions, that data transfers between the two jurisdictions are in compliance with the UK Data Protection Act and the GDPR, respectively. The UK and the U.S. have also agreed to a U.S.-UK "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 UK to the U.S.

Following Brexit, there are open questions about how personal data will be protected in the UK and whether personal information can transfer from the EU to the UK. The European Commission decided in June 2021 that the level of data protection in the UK is "essentially adequate" for purposes of data transfer from the EU to the UK. On December 19, 2025, the European Commission renewed this decision until December 27, 2031. The UK and the U.S. have also agreed to a U.S.- UK "Data Bridge," which functions similarly to the EU-U.S. Data Privacy Framework and provides an additional legal mechanism for companies to transfer personal data from the UK to the U.S. Switzerland has also taken 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.-UK Data Bridge in relation to data transfers from Switzerland to the U.S.).

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.

Risks Related to Employee Matters, Managing Growth, Public Health and Information Technology

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

We are highly dependent on the principal members of our management and scientific teams. Each of these individuals is employed "at will," meaning we or the individual may terminate the employment relationship 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. 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 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. In addition, our ability to recruit and retain qualified personnel could be impacted by other factors, such as remote or hybrid working arrangements, which could impact employees' productivity and morale, as well as any failure to succeed in preclinical or clinical trials. The inability to recruit or the loss of the services of any executive, key employee, consultant or advisor may impede the progress of our research, development and commercialization objectives.

Security breaches and other disruptions to our information technology structure could compromise our information, disrupt our business and expose us to liability, which would cause our business and reputation to suffer.

In the ordinary course of our business, we collect, process and store sensitive data, including intellectual property, as well as our proprietary business information and that of our suppliers and business partners, employee data, and we may collect personally identifiable information of clinical trial participants in connection with clinical trials. We also rely to a large extent on information technology systems to operate our business, including our financial systems. We have outsourced elements of our confidential information processing and information technology structure, and as a result, we are managing independent vendor relationships with third parties who may or could have access to our confidential information. Similarly, our business partners and other third-party providers possess certain elements of our sensitive data. The secure maintenance of this information is important to our operations and business strategy. Despite our security measures, our information technology infrastructure (and those of our partners, vendors and third-party providers) may be vulnerable to attacks by hackers or breached due to employee error, malfeasance or other disruptions. We, our partners, vendors, and other third-party providers could be susceptible to third party attacks on our, and their, information security systems, which attacks are of ever-increasing levels of sophistication and are made by groups and individuals with a wide range of motives and expertise, including organized criminal groups, hacktivists, nation states and others. We have implemented a hybrid work model, which may place our information technology infrastructure and data at increased risk as employees may work from home utilizing network connections outside our premises. Additionally, sensitive data could be leaked, disclosed, or revealed as a result of or in connection with our employees', vendors' or partners' use of generative artificial intelligence ("AI") technologies or public AI platforms. We have invested in information technology security measures and the protection of confidential and sensitive information, but there can be no assurance that our efforts will prevent system failures, accidents or security breaches. While we believe we have not experienced any such material system failure, accident or security breach to date, any such event may substantially impair our ability to operate our business and would compromise our, and their, networks and the information stored could be accessed, publicly disclosed, lost, or stolen. In addition, if a ransomware attack or other cybersecurity incident occurs, either internally or at our vendors or third-party technology service providers, we could be prevented from accessing our data or systems, which may cause interruptions or delays in our business operations, cause us to incur remediation costs, subject us to demands to pay a ransom, or damage our reputation, regardless of whether we pay the ransom amount. Any such event, or other loss of information, could result in legal claims or proceedings, liability under laws that protect the privacy of personal information, disrupt our operations, and damage our reputation, any of which could adversely affect our business.

Although we have general liability and cybersecurity insurance coverage, our insurance may not cover all claims, continue to be available on reasonable terms or be sufficient in amount to compensate us fully for potential significant losses; additionally, the insurer may disclaim coverage as to any claim. The successful assertion of one or more large claims against us that exceed or are not covered by our insurance coverage or changes in our insurance policies, including premium increases or the imposition of large deductible or co-insurance requirements, could materially harm our business, financial condition, results of operations and prospects.

Risks Related to Our Common Stock

The market price of our common stock has been, and is likely to remain, volatile.

Our stock price has been, and is likely to remain, volatile. For example, since January 1, 2024, the trading price of our common stock on the Nasdaq Global Select Market has ranged from a low of $0.91 to a high of $11.58 through January 31, 2026. Some of the factors that may cause the market price of our common stock to fluctuate include:

- the success of existing or new competitive products or technologies;
- the timing and results of our preclinical studies for any product candidates that we develop;
- commencement or termination of collaborations for our product development and research programs;
- 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 genomic medicines, including those that involve gene editing;
- 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 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;
- 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;
- general economic, industry, and market conditions; and
- the other factors described in this "Risk Factors" section.

The stock market in general, and the market for pharmaceutical and biotechnology companies in particular, has experienced extreme price and volume fluctuations that have often been unrelated or disproportionate to changes in the operating performance of the companies whose stock is experiencing those price and volume fluctuations. 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 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 and trading volume could decline.

The trading market for our common stock depends, in part, on the research and reports that industry or financial analysts publish about us or our business. If one or more of the analysts covering our business downgrade their evaluations of our stock, the price of our stock could decline. If one or more of these analysts cease to cover our stock or fail to regularly publish reports on us, we could lose visibility in the market for our stock, which in turn could cause our stock price to decline.

Future sales and issuances of our common stock or rights to purchase common stock, including pursuant to our equity incentive plans and "at-the-market" offerings, could result in additional dilution of the percentage ownership of stockholders and could cause our stock price to fall.

We will need additional capital in the future to continue our planned operations. To the extent we raise additional capital by issuing equity securities, our stockholders may experience substantial dilution. We may sell common stock, convertible securities or other equity securities in one or more transactions at prices and in a manner we determine from time to time. If we sell common stock, convertible securities or other equity securities in more than one transaction, investors may be materially diluted by subsequent sales. These sales may also result in material dilution to our existing stockholders, and new investors could gain rights superior to our existing stockholders.

In addition, sales of a significant number of shares of our common stock in the public market could occur at any time. These sales, or the perception in the market that the holders of a large number of shares of common stock intend to sell shares, could reduce the market price of our common stock.

We have registered substantially all shares of common stock that we may issue under our equity compensation plans. These shares can be freely sold in the public market upon issuance and once vested, subject to volume limitations applicable to affiliates. If any of these additional shares are sold, or if it is perceived that they will be sold, in the public market, the market price of our common stock could decline.

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

As a public company we incur, and will 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 Global Select 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 increase our legal and financial compliance costs and make some activities more time-consuming and costly.

We have broad discretion in the use of our cash reserves and may not use them effectively, including that we may be exposed to liquidity issues and other systemic financial risks at the financial institutions holding our cash and cash equivalents.

Our management has broad discretion to use our cash reserves and could use our cash reserves in ways that do not improve our results of operations or enhance the value of our common stock. The failure by our management to apply these funds effectively could result in financial losses that could have a material adverse effect on our business, cause the price of our common stock to decline, and delay the development of our product candidates. Pending their use, we may invest our cash reserves in a manner that does not produce income or that loses value.

A portion of our cash may be held by financial institutions that may have been, or could in the future become, exposed to liquidity issues, bank failures or other systemic financial risks. Our uninsured cash deposits with such financial institutions may be at risk in the event they experience liquidity problems or other financial losses. We assess our banking relationships as we believe necessary or appropriate, but our business, our business partners, or industry as a whole may be adversely impacted in ways that we cannot predict at this time, including our ability to access cash in amounts adequate to finance or capitalize our current and/or projected business operations could be significantly impaired by factors that affect the financial institutions with which we have banking relationships, and in turn, us. These factors could include, among others, events such as liquidity constraints or failures, the ability to perform obligations under various types of financial, credit or liquidity agreements or arrangements (including cash management arrangements), disruptions or instability in the financial services industry or financial markets, or concerns or negative expectations about the prospects for companies in the financial services industry. In addition, our vendors, such as our CMOs, CROs or business partners, may be susceptible to the foregoing liquidity or other financial risks and factors, which could, in turn, have a material adverse effect on our current and/or projected business operations and results of operations and financial condition.

We do not expect to pay any dividends for the foreseeable future. Accordingly, stockholders must rely on capital appreciation, if any, for any return on their investments.

We have never declared or paid cash dividends on our common stock. We currently intend to retain all of our future earnings, if any, to finance the growth and development of our business. In addition, the terms of any future debt agreements may preclude us from paying dividends. As a result, capital appreciation, if any, of our common stock will be stockholders' sole source of gain for the foreseeable future.

Provisions in our restated certificate of incorporation and amended and restated bylaws or Delaware law might discourage, delay, or prevent a change in control of our company or changes in our management and, therefore, depress the trading price of our common stock.

Provisions in our restated certificate of incorporation and amended and restated bylaws or Delaware law may discourage, delay, or prevent a merger, acquisition, or other change in control that stockholders may consider favorable,

including transactions in which you might otherwise receive a premium for your shares of our common stock. These provisions may also prevent or frustrate attempts by our stockholders to replace or remove our management. These provisions include:

- limitations on the removal of directors;
- a classified board of directors so that not all members of our Board of Directors are elected at one time;
- advance notice requirements for stockholder proposals and nominations;
- the inability of stockholders to act by written consent or to call special meetings;
- the requirement that at least 75% of the votes cast by all our stockholders approve the amendment or repeal of certain provisions of our amended and restated bylaws or restated certificate of incorporation;
- the ability of our board of directors to make, alter, or repeal our amended and restated bylaws; and
- the ability of our board of directors to designate the terms of and issue new series of preferred stock without stockholder approval, which could be used to institute a rights plan, or a poison pill, that would work to dilute the stock ownership of a potential hostile acquirer, likely preventing acquisitions that have not been approved by our board of directors.

In addition, Section 203 of the General Corporation Law of the State of Delaware prohibits a publicly held Delaware corporation from engaging in a business combination with an interested stockholder, generally a person which together with its affiliates owns, or within the last three years has owned, 15% of our voting stock, for a period of three years after the date of the transaction in which the person became an interested stockholder, unless the business combination is approved in a prescribed manner.

The existence of the foregoing provisions could deter potential acquirers of our company, thereby reducing the likelihood that our stockholders could receive a premium for their shares of common stock in an acquisition.

Our restated certificate of incorporation designates the state courts in the State of Delaware or, if no state court located within the State of Delaware has jurisdiction, the federal court for the District of Delaware, as the sole and exclusive forum for certain types of actions and proceedings that may be initiated by our stockholders, which could discourage lawsuits against the company and our directors and officers.

Our restated certificate of incorporation provides that, unless our board of directors otherwise determines, the state courts in the State of Delaware or, if no state court located within the State of Delaware has jurisdiction, the federal court for the District of Delaware, will be the sole and exclusive forum for any derivative action or proceeding brought on our behalf, any action asserting a claim of breach of a fiduciary duty owed by any of our directors or officers to our company or our stockholders, any action asserting a claim against us or any of our directors or officers arising pursuant to any provision of the General Corporation Law of the State of Delaware or our restated certificate of incorporation or amended and restated bylaws, or any action asserting a claim against us or any of our directors or officers governed by the internal affairs doctrine. This exclusive forum provision may limit the ability of our stockholders to bring a claim in a judicial forum that such stockholders find favorable for disputes with us or our directors or officers, which may discourage such lawsuits against us and our directors and officers. This exclusive forum provision would not apply to suits brought to enforce a duty or liability created by the Exchange Act of 1934, which provides for exclusive jurisdiction of the federal courts. It could apply, however, to a suit that falls within one or more of the categories enumerated in the exclusive forum provision and asserts claims under the Securities Act of 1933, as amended (the "Securities Act"), inasmuch as Section 22 of the Securities Act creates concurrent jurisdiction for federal and state courts over all suits brought to enforce any duty or liability created by the Securities Act or the rules and regulations thereunder; provided, that with respect to claims under the Securities Act, our stockholders will not be deemed to have waived our compliance with the federal securities laws and the rules and regulations thereunder.

We are a "smaller reporting company" within the meaning of the Securities Exchange Act of 1934, and if we decide to take advantage of certain exemptions from various reporting requirements applicable to smaller reporting companies, our common stock could be less attractive to investors.

For so long as we qualify as a "smaller reporting company," we will have the option to take advantage of certain exemptions from various reporting and other requirements that are applicable to other public companies that are not "smaller reporting companies," including but not limited to reduced disclosure obligations regarding executive compensation in our periodic reports and proxy statements and later effective dates for compliance with certain new disclosure obligations. In addition, for as long as we are deemed neither a large accelerated filer nor an accelerated filer, we will continue to use the exemption from compliance with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act of 2002, as amended, or the Sarbanes-Oxley Act. We will remain a smaller reporting company if we have either (i) a public float of less than $250 million held by non-affiliates as of the last business day of the second quarter of our then-current fiscal year or (ii) annual revenues of less than $100 million during such recently completed fiscal year with less than $700 million in public float as of the last business day of the second quarter of such fiscal year.

In the event we are eligible to and do rely on the exemptions available to smaller reporting companies, we cannot predict if investors will find our common stock less attractive because we may or do rely on these exemptions. If some investors find our common stock less attractive as a result, there may be a less active trading market for our common stock and our stock price may be more volatile.

Item 1B. Unresolved Staff Comments

Not applicable.

Item 1C. Cybersecurity

Risk Management and Strategy

We have established certain processes for assessing, identifying and managing cybersecurity risks, which are built into our information technology functions and are designed to help protect our information, assets and operations from internal and external cyber threats.

Our cybersecurity risk management program is informed by recognized industry frameworks (e.g., NIST Cybersecurity Framework) which includes a risk assessment methodology designed to escalate cybersecurity risks to the appropriate channels within our organization to help identify material cybersecurity risks to our critical systems, information, products, services and our broader enterprise information technology ("IT") environment. The IT and legal departments help identify, assess and manage our cybersecurity threats and risks. The IT department, in coordination with the legal department, identifies and assesses risks from cybersecurity threats by monitoring and evaluating our threat environment and our risk profile using various methods, including evaluating threats reported to us, conducting audits, performing threat assessments, conducting vulnerability assessments and penetration tests.

Depending on the environment, we implement and maintain various technical, physical, and organizational measures, processes, standards and policies designed to manage and mitigate material risks from cybersecurity threats to our information systems and data, including an incident response plan that we periodically review and test to ensure it is well understood and the processes are being properly followed. The incident response plan includes procedures for responding to cybersecurity incidents and escalating cybersecurity incidents to cross-functional teams, management and our Board of Directors. Other controls include business continuity plans, encryption of data, network security controls, systems monitoring, mandatory annual employee training, and cybersecurity insurance. Our assessment and management of material risks from cybersecurity threats are integrated into our risk management protocols. Our cybersecurity risk management program shares common methodologies, reporting channels and governance processes that apply across the enterprise risk management program to other legal, compliance, strategic, operational and financial risk areas, including the involvement of cross-functional teams and, depending on the nature and severity of an incident, an escalation path to notify our executive and senior management teams and our Board of Directors. Material cybersecurity incidents are escalated to the Audit Committee and, as appropriate, the Board of Directors.

We use third-party service providers to assist us to identify, assess, and manage material risks from cybersecurity threats, including professional service firms, legal counsel, cybersecurity software providers and managed cybersecurity service providers. We use third-party service providers to perform a variety of functions throughout our business, such as application providers, hosting companies, contract research organizations, contract manufacturing organizations and supply chain resources. Depending on the nature of the services provided, the sensitivity of the information systems and data at issue, and the identity of the provider, our vendor management process may involve different levels of assessment which may include security questionnaires, review of System and Organization Controls reports, contractual requirements, and

periodic reassessments designed to help identify cybersecurity risks associated with a provider and impose contractual obligations related to cybersecurity on the provider.

We do not believe that there are currently any risks from known cybersecurity threats that have materially affected or are reasonably likely to materially affect us, including our business strategy, results of operations or financial condition. For additional information regarding risks we face, please refer to Part I, Item 1A "Risk Factors—Risks Related to Employee Matters, Managing Growth, Public Health and Information Technology—Security breaches and other disruptions to our information technology structure could compromise our information, disrupt our business and expose us to liability, which would cause our business and reputation to suffer" of this Annual Report on Form 10-K.

Governance

Our Board of Directors considers cybersecurity risk as part of its risk oversight function and has delegated to the Audit Committee of our Board oversight of cybersecurity and other information technology risks. The Audit Committee of our Board of Directors oversees our cybersecurity and data privacy risk management activities, and reports to the Board regarding such oversight as appropriate. The Audit Committee receives updates from management, including our Head of IT, regarding cybersecurity matters not less than twice per year, and is notified between such updates regarding any significant new cybersecurity threats or incidents.

Our Head of IT, who reports to our Chief Technical and Quality Officer and is a member of our senior leadership team, has primary responsibility for day-to-day management of our cybersecurity risk management program, including leading a dedicated team of IT professionals to monitor and assess cybersecurity risks, and is responsible for strategic leadership of our cybersecurity risk management program. The Head of IT role is currently held by an individual who has over 15 years of professional IT management experience in the life sciences industry. The Head of IT collaborates closely and regularly with an external consulting firm that provides a fractional Chief Information Security Officer, who has over 25 years of experience, including in senior information technology and cybersecurity management positions. Our Head of IT provides regular updates on our cybersecurity risk to our executive leadership team and other management responsible for IT and cybersecurity risk management.

Item 2. Properties.

We lease 59,783 square feet of office and laboratory space in Cambridge, Massachusetts under a lease that expires in October 2028. 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.

From time to time, we may become involved in litigation or other legal proceedings relating to claims arising from the ordinary course of business. There can be no assurance that any proceedings that result from these third-party actions will be resolved in our favor. In addition, if they are not resolved in our favor, there can be no assurance that the result will not have a material adverse effect on our business, financial condition, results of operations, or prospects. Certain of our intellectual property rights, including ones licensed to us under our licensing agreements, are subject to, and from time to time may be subject to, priority and validity disputes. For additional information regarding these matters, see Part I, Item 1A. "Risk Factors—Risks Related to Our Intellectual Property" of this Annual Report on Form 10-K. Regardless of outcome, litigation or other legal proceedings can have an adverse impact on us because of defense and settlement costs, diversion of management resources, and other factors.

Item 4. Mine Safety Disclosures.

Not applicable.

PART II

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

Market Information

Our common stock trades on the Nasdaq Global Select Market under the symbol "EDIT."

Holders

As of February 27, 2026, we had approximately 19 holders of record of our common stock. This number does not include beneficial owners whose shares were held in street name.

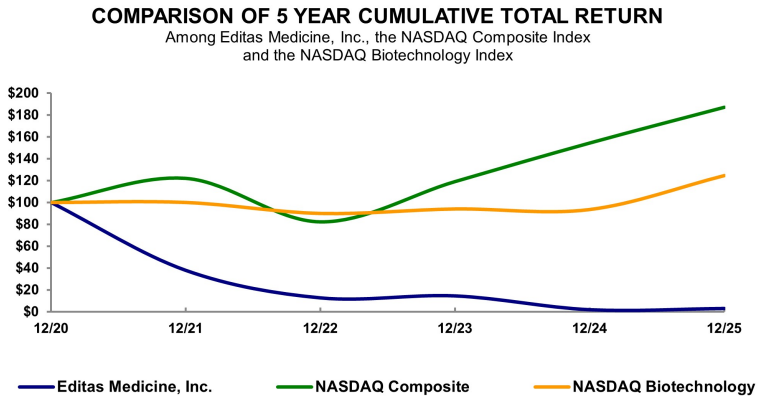
Dividend Policy

We have never declared or paid any cash dividends on our common stock. We currently intend to retain future earnings to fund the development and growth of our business. We do not expect to pay any cash dividends in the foreseeable future.

Performance Graph

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

The following graph compares the performance of our common stock to The Nasdaq Composite Index and to The Nasdaq Biotechnology Index from December 31, 2020 through December 31, 2025. The comparison assumes $100 was invested after the market closed on December 31, 2020 in our common stock and in each of the foregoing indices, and it assumes reinvestment of dividends, if any. The stock price performance included in this graph is not necessarily indicative of future stock price performance.



Recent Sales of Unregistered Securities

None.

Purchases of Equity Securities by the Issuer and Affiliated Purchasers

Neither we nor any affiliated purchaser or anyone acting on behalf of us or 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 together with our consolidated financial statements and related notes appearing elsewhere in this Annual Report on Form 10-K.

Some of the information contained in this discussion and analysis or set forth elsewhere in this Annual Report on Form 10-K contains forward-looking statements that involve substantial risks and uncertainties. The words "anticipate," "believe," "estimate," "expect," "intend," "may," "plan," "predict," "project," "would," and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. There are a number of important risks and uncertainties that could cause our actual results to differ materially from those indicated by forward-looking statements. We may not actually achieve the plans, intentions or expectations disclosed in our forward-looking statements, and you should not place undue reliance on our forward-looking statements. Actual results or events could differ materially from the plans, intentions, and expectations disclosed in the forward-looking statements we make. We have included important factors in the cautionary statements included in this Annual Report on Form 10-K, particularly in the section entitled "Risk Factors" in Part I, Item 1A that could cause actual results or events to differ materially from the forward-looking statements that we make. Our forward-looking statements do not reflect the potential impact of any future acquisitions, mergers, dispositions, joint ventures or investments that we may make.

You should read this Annual Report on Form 10-K and the documents that we have filed as exhibits to this Annual Report on Form 10-K completely and with the understanding that our actual future results may be materially different from what we expect. The forward-looking statements contained in this Annual Report on Form 10-K are made as of the date of this Annual Report on Form 10-K, and we do not assume any obligation to update any forward-looking statements, whether as a result of new information, future events or otherwise, except as required by applicable law.

Overview

We are a pioneering gene editing company dedicated to developing potentially transformative genomic medicines to treat a broad range of serious diseases. We have developed a proprietary gene editing platform based on CRISPR technology and we continue to expand its capabilities. Our product development strategy is to target diseases where gene editing can be used to enable or enhance therapeutic outcomes for patients, while maximizing probability of technical, regulatory and commercial success. We are focused on the development of *in vivo* gene editing medicines utilizing functional upregulation, which aims to increase the expression of a normal gene copy and its normal protein function to treat diseases caused by genetic mutations that eliminate or disrupt normal function. We believe the ability to provide *in vivo* gene editing, in which the medicine is injected or infused into the patient to edit the cells inside their body, and functionally upregulates normal gene expression and normal protein function in the target tissues holds the potential to significantly expand the addressable therapeutic possibilities of CRISPR-based gene editing. To that end, our preclinical efforts are also focused on the creation of a "plug 'n play" lipid nanoparticle ("LNP") platform to enable targeted delivery of *in vivo* gene editing medicines to multiple cells and tissues, including the liver, hematopoietic stem cells ("HSCs"), and other cells and tissues.

In September 2025, we announced the nomination of our lead *in vivo* development candidate, EDIT-401, an experimental, potential best-in-class, one-time therapy to significantly reduce LDL-cholesterol ("LDL-C") through upregulation of the LDL receptor ("LDLR"). EDIT-401 is designed to treat elevated levels of LDL-C, or hyperlipidemia, by directly editing the noncoding region of the LDLR gene to increase LDLR protein expression and reduce LDL-C levels. This targeted approach has demonstrated an approximately 90% mean reduction of LDL-C in non-human primates ("NHPs") in our preclinical studies with favorable tolerability data, and supports the potential of EDIT-401 to deliver

meaningful clinical outcomes for patients underserved by current lipid-lowering therapies. We are on track to submit an investigational new drug application ("IND") or foreign equivalent to conduct a clinical trial of EDIT-401 in patients with heterozygous familial hypercholesteremia by mid-2026 with the expectation of achieving early human proof-of-concept data for EDIT-401 by the end of 2026. We plan to complete enrolling the dose-finding portion of the first-in-human clinical trial with topline data results available in 2027. We expect to present additional preclinical data for EDIT-401 by mid-2026.

Our discovery and development efforts further include HSCs and other cells and tissues. Building on our experience in our clinical trials of renizgamglogene autogedtemcel ("reni-cel"), we have achieved *in vivo* preclinical proof-of-concept data of HSC editing in NHPs. In addition, we previously announced *in vivo* delivery to two additional cell types in humanized mice using our proprietary LNP targeting platform, demonstrating the "plug 'n play" potential of our proprietary extrahepatic LNP platform. We intend to continue optimizing candidates for our HSC program and exploring other cell types and tissues for development, but plan to focus our resources on the advancement of our lead EDIT-401 program to human proof-of-concept.

We are pursuing the right combination of gene editing and targeted delivery tools through internal development and the in-licensing of complementary technologies to build our preclinical pipeline and accelerate the achievement of our goal of delivering lifesaving medicines to patients with previously untreatable diseases. Through in-licensing of complementary technologies, we can expand our existing gene editing platform and further drive the development of our *in vivo* pipeline. This was demonstrated with our entry in 2024 into a collaboration and license agreement to access LNPs targeting the liver, including the LNP we are using in our EDIT-401 program. We also actively seek opportunities to out-license and partner our robust intellectual property portfolio to drive the development of CRISPR-based medicines in therapeutic areas outside of our core focus and to provide non-dilutive capital. For example, we are leveraging partnerships to progress engineered cell medicines to treat various cancers, including in our collaboration with Bristol Myers Squibb Company ("BMS") through its wholly owned subsidiary, Juno Therapeutics, Inc. ("Juno Therapeutics"). This collaboration, which leverages our Cas9 and AsCas12a platform technologies, seeks to advance alpha-beta T-cell experimental medicines for the treatment of solid tumors, liquid tumors, and autoimmune disease, and has resulted in 14 total programs to date, including BMS' CD19 HD Allo CAR T program for the treatment of autoimmune disease currently in Phase I clinical development.

In addition, in December 2023, we and Vertex Pharmaceuticals Incorporated ("Vertex") entered into a license agreement (the "Vertex License Agreement"), under which Vertex obtained a non-exclusive license for our Cas9 gene editing technology for *ex vivo* gene editing medicines targeting the BCL11A gene in the fields of SCD and TDT, including Vertex's CASGEVY™ (exagamglogene autotemcel). We received a $50.0 million upfront cash payment in the fourth quarter of 2023 and the 2024 annual license fee of $10.0 million in the first quarter of 2024. The Vertex License Agreement further provides for the payment by Vertex of a potential additional $50.0 million contingent upfront payment and further future fixed and sales-based annual license fees, ranging from $5.0 million to $40.0 million annually, inclusive of certain sales-based annual license fee increases, through 2034. We are required to pay The Broad Institute, Inc. ("Broad") and the President and Fellows of Harvard College ("Harvard") a mid-double-digit percentage of amounts payable to us from Vertex under the Vertex License Agreement as it relates to Cas9 technology licensed by us from Broad and Harvard. In October 2024, we entered into an agreement (the "DRI Agreement") with a wholly owned subsidiary of DRI Healthcare Trust ("DRI") providing for an upfront cash payment by DRI to us of $57.0 million. Under the DRI Agreement, DRI is purchasing up to 100% of certain future fixed and sales-based annual license fees that the Company is entitled to receive under the Vertex License Agreement, which fees range from $5.0 million to $40.0 million per year, including increases based on sales. In addition, DRI is purchasing a mid-double-digit percentage of a $50.0 million contingent upfront payment that the Company may receive under the Vertex License Agreement. All amounts above will be adjusted to exclude payments that the Company owes to Broad and Harvard. The Company has retained rights to certain portions of certain other sales-based annual license fees and the contingent upfront payment that may become due under the Vertex License Agreement, and the amounts that correspond to our licensor obligations.

Our operations to date have focused on organizing and staffing our company, business planning, raising capital, establishing our intellectual property portfolio, assembling our core capabilities in gene editing, seeking to identify potential product candidates, and undertaking preclinical studies and clinical trials. All of our ongoing research programs are still in the preclinical or research stage of development and the risk of failure of all of our research programs is high. We have not generated any revenue from product sales. We have primarily financed our operations through various equity financings, payments received under our research collaboration with BMS, our former strategic alliance with Allergan Pharmaceuticals International Limited (together with its affiliates, "Allergan"), which was terminated in August 2020, payments received under the DRI Agreement in connection with the Vertex License Agreement, and payments under the Vertex License Agreement.

We have incurred significant operating losses since inception. Our net losses were $160.1 million and $237.1 million for the years ended December 31, 2025 and 2024, respectively. As of December 31, 2025, we had an accumulated deficit of $1.6 billion. We expect to continue to incur significant expenses and operating losses for the foreseeable future. Our net losses may fluctuate significantly from quarter to quarter and from year to year. We anticipate that our expenses will increase as we continue to support preclinical studies and prepare for the clinical development of EDIT-401; commence and conduct clinical trials of EDIT-401; continue our current research programs and our preclinical development activities; seek to identify additional research programs and additional product candidates; initiate preclinical testing for other product candidates we identify and develop; maintain, expand, and protect our intellectual property portfolio, including reimbursing our licensors for such expenses related to the intellectual property that we in-license from such licensors; further develop our gene editing platform; and hire personnel. We do not expect to be profitable for the year ending December 31, 2026 or for the foreseeable future.

Financial Operations Overview

Revenue

To date, we have not generated any revenue from product sales and we do not expect to generate any revenue from product sales for the foreseeable future.

In connection with our collaboration with BMS, we have received an aggregate of $159.0 million in payments, which have primarily consisted of the initial upfront and amendment payments, development milestone payments, research funding support, and certain opt-in fees. We no longer receive research funding support. During the year ended December 31, 2025, we recognized $23.2 million of revenue related to our collaboration with BMS of which $9.7 million was previously deferred revenue. As of December 31, 2025, we had $40.5 million of deferred revenue related to BMS, all of which is classified as long-term deferred revenue on our consolidated balance sheet. Under this collaboration, we recognize revenue upon delivery of option packages to BMS or upon receipt of development milestone payments. We expect that our revenue will fluctuate from quarter-to-quarter and year-to-year as a result of the timing of when we deliver such option packages or receive such milestone payments.

Pursuant to the Vertex License Agreement, we received a $50.0 million upfront cash payment in the fourth quarter of 2023 upon execution of the agreement and the 2024 and 2025 annual license fees of $10.0 million in each of the first quarters of 2024 and 2025. The license agreement further provides for the payment by Vertex of a potential additional $50.0 million contingent upfront payment and further annual license fees, ranging from $5.0 million to $40.0 million annually, inclusive of certain sales-based annual license fee increases, through 2034. For the year ended, December 31, 2025, we recorded $10.0 million of revenue related to the annual license fee under the agreement.

For additional information about our revenue recognition policy related to the Vertex License Agreement and BMS collaboration, see Part II, Item 7 “Management’s Discussion and Analysis of Financial Condition and Results of Operations—Critical Accounting Policies and Estimates—Revenue Recognition” included in this Annual Report on Form 10-K.

For the foreseeable future we expect substantially all of our revenue will be generated from the Vertex License Agreement, our collaboration with BMS, and any other collaborations or license agreements we may enter into.

Operating Expenses

Research and Development Expenses

Research and development expenses consist primarily of costs incurred for our research, preclinical development, process and scale-up development, manufacture and clinical development of our product candidates, and the performance of development activities under our collaboration agreements. These costs are expensed as incurred and include:

- costs associated with our continued development of EDIT-401 as we progress EDIT-401 to IND and/or foreign equivalent submission and commence clinical trials;
- employee-related expenses including salaries, benefits, and stock-based compensation expense;

- costs associated with conducting our other preclinical, process and scale-up development, manufacturing, quality, clinical and regulatory activities, including fees paid to third-party professional consultants, service providers and suppliers;
- costs of purchasing lab supplies and non-capital equipment used in our preclinical activities and in manufacturing preclinical and clinical study materials;
- costs incurred for the research and development activities under our collaboration agreements;
- facility costs including rent, depreciation, and maintenance expenses; and
- fees for acquiring and maintaining licenses under our third-party licensing agreements, including any sublicensing or success payments made to our licensors.

At this time, we cannot reasonably estimate or know the nature, timing, and estimated costs of the efforts that will be necessary to complete the development of any product candidates we may identify and develop. This is due to the numerous risks and uncertainties associated with developing such product candidates, including the uncertainty of:

- successful completion of preclinical studies, IND-enabling studies and natural history studies;
- successful initiation of, enrollment in, and completion of, clinical trials;
- receipt of marketing approvals from applicable regulatory authorities;
- establishing commercial manufacturing capabilities or making arrangements with third-party manufacturers;
- obtaining and maintaining patent and trade secret protection and non-patent exclusivity;
- launching commercial sales of a product, if and when approved, whether alone or in collaboration with others;
- acceptance of a product, 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 following approval;
- enforcing and defending intellectual property and proprietary rights and claims; and
- achieving desirable medicinal properties for the intended indications.

A change in the outcome of any of these variables with respect to the development of any product candidates we develop would significantly change the costs, timing, and viability associated with the development of that product candidate.

Research and development activities are central to our business model. We expect research and development expenses to decrease in future periods compared to prior periods, due to the discontinuation of clinical development of our *ex vivo* reni-cel program that contributed significantly to expense in prior periods.

General and Administrative Expenses

General and administrative expenses consist primarily of salaries and other related costs, including stock-based compensation for personnel in executive, finance, investor relations, business development, legal, corporate affairs, information technology, facilities, and human resource functions. Other significant costs include corporate facility costs not otherwise included in research and development expenses, legal fees related to intellectual property and corporate matters, and fees for accounting and consulting services.

We anticipate that our general and administrative expenses that support continued research and development activities will decrease in the near future. We anticipate that expenses associated with operating as a public company, including costs for audit, legal, regulatory, and tax-related services, director and officer insurance premiums, and investor relation costs will remain flat in the near future. With respect to reimbursement of third-party intellectual property-related expenses specifically, given the ongoing nature of the opposition and interference proceedings involving the patents licensed to us under our license agreement with Broad and Harvard, we anticipate general and administrative expenses associated with reimbursement of third-party intellectual property-related expense will continue to fluctuate as the interference proceedings continue.

Restructuring and Impairment Charges

In December 2024, our board of directors approved the discontinuation of the clinical development of our *ex vivo* reni-cel program (the "Discontinuation"). As part of the Discontinuation, our Board of Directors approved a reduction in our employee workforce by approximately 180 positions, or by approximately 65% (the "Reduction"). Restructuring charges associated with the Discontinuation consist primarily of expenses in connection with the wind-down of various activities related to clinical development of reni-cel, including contract termination costs, impairment charges and non-cash charges, and expenses related to the Reduction, primarily consisting of severance payments and employee benefit costs. The actions associated with the Discontinuation and Reduction commenced in December 2024 and were substantially completed by December 31, 2025.

Other Income (Expense), Net

For the year ended December 31, 2025, other income (expense), net consisted primarily of interest income on cash and cash equivalents and marketable securities as well as interest expense accretion related to the liability for the sale of future revenues. For the year ended December 31, 2024, other income (expense), net was primarily attributable to interest income and accretion of discounts associated with marketable securities.

Critical Accounting Policies and Estimates

Our management's discussion and analysis of our financial condition and results of operations is based on our consolidated financial statements, which have been prepared in accordance with United States generally accepted accounting principles. The preparation of our consolidated financial statements requires us to make judgments and estimates that affect the reported amounts of assets, liabilities, revenues, and expenses, and the disclosure of contingent assets and liabilities in our consolidated financial statements. We base our estimates on historical experience, known trends and events, and various other factors that we believe to be reasonable under the circumstances. Actual results may differ from these estimates under different assumptions or conditions. On an ongoing basis, we evaluate our judgments and estimates in light of changes in circumstances, facts, and experience. The effects of material revisions in estimates, if any, will be reflected in the consolidated financial statements prospectively from the date of change in estimates.

While our significant accounting policies are described in more detail in the notes to our consolidated financial statements appearing elsewhere in this Annual Report on Form 10-K, we believe the following accounting policies used in the preparation of our consolidated financial statements require the most significant judgments and estimates.

Revenue Recognition

We recognize revenue in accordance with Financial Accounting Standards Board ("FASB") Accounting Standards Codification ("ASC"), Topic 606, *Revenue Recognition* ("ASC 606"). Accordingly, we recognize revenue following the five step model prescribed under Accounting Standards Updates No. 2014-09, *Revenue from Contracts with Customers*: (i) identify contract(s) with a 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 revenues when (or as) we satisfy the performance obligation. We only apply the five-step model to contracts when it is probable that we will collect the consideration we are entitled to in exchange for the goods or services we transfer to the customer. At contract inception, once the contract is determined to be within the scope of ASC 606, we assess the goods or services promised within each contract and determine those that are performance obligations, and whether each promised good or service is distinct. We then recognize as revenue the amount of the transaction price that is allocated to the respective performance obligation when (or as) the performance obligation is satisfied. As part of the accounting for these arrangements, we must develop assumptions that require judgment to determine the standalone selling price for each performance obligation identified in the contract and use judgment in the determination of the transaction price and the application of the

constraint. The determination of standalone selling price has not had a significant impact on the accounting for our revenue arrangements given the nature of the performance obligations. We have also not been required to apply significant judgment in determining the transaction price given the nature of the variable consideration and the application of the constraint.

Accrued Research and Development Expenses

As part of the process of preparing our financial statements, we are required to estimate our accrued expenses. 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 clinical research organizations, to investigative sites in connection with clinical trials, to sponsored research organizations, to service providers in connection with preclinical development activities and to service providers related to product manufacturing, development and distribution of clinical supplies.

We base our accrued expenses related to clinical trials on our estimates of the services performed and efforts expended pursuant to our contractual arrangements, including those with clinical research organizations. The financial terms of these agreements are sometimes 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 service providers will exceed the level of services performed and result in a prepayment of the clinical expense. Payments under some of these contracts depend on factors such as the successful enrollment of patients and the completion of clinical milestones. In accruing service fees, we estimate the time period over which services will be performed and the level of effort to be expended in each period. If the actual timing of the performance of services or the level of effort varies from our estimate, we adjust the accrual or prepaid accordingly.

Although we do not expect our estimates to be materially different from expenses actually incurred, if our estimates of the status and timing of services performed differs from the actual status and timing of services performed, we may report amounts that are too high or too low in any particular period. To date, there have been no material differences from our estimates to the amounts actually incurred.

Restructuring

We record liabilities for costs associated with restructuring activities in the period in which the liability is incurred. Typical costs associated with restructuring activities include employee termination benefits, contract termination costs and on-going contract costs for which there is no economic benefit. For costs associated with employee terminations in which the employee is subject to an existing benefit arrangement, the post-employment benefits are recognized when probable and estimable. Other employee termination costs are measured and recognized on the communication date, unless there is a required future service period, in which case, the expense is recognized over the service period. Contract termination costs are recognized upon termination of the contract and costs for on-going contracts for which there is no future benefit are recognized at fair value on the cease-use date.

We have made estimates and judgments regarding the amount and timing of our restructuring expense and liability, including current and future period termination benefits and other exit costs to be incurred when related actions take place. Restructuring charges are reflected in our consolidated statements of operations. Actual results may differ from these estimates.

Results of Operations

Comparison of Years Ended December 31, 2025 and 2024

The following table summarizes our results of operations for the years ended December 31, 2025 and 2024, together with the changes in those items in dollars (in thousands) and the respective percentages of change:

	Year Ended December 31,			
	2025	2024	Dollar Change	Percentage Change
Collaboration and other research and development revenues	$ 40,520	$ 32,314	$ 8,206	25 %
Operating expenses:				
Research and development	89,953	199,247	(109,294)	(55) %
General and administrative	49,903	71,987	(22,084)	(31) %
Restructuring and impairment charges	60,674	12,232	48,442	n/m
Total operating expenses	200,530	283,466	(82,936)	(29) %
Operating loss	(160,010)	(251,152)	91,142	(36) %
Other income (expense), net:				
Interest expense related to sale of future revenues	(6,171)	(2,190)	(3,981)	n/m
Interest income, net	8,310	16,252	(7,942)	(49) %
Other expense, net	(2,189)	(3)	(2,186)	n/m
Total other income (expense), net	(50)	14,059	(14,109)	n/m
Net loss	$ (160,060)	$ (237,093)	$ 77,033	32 %

For our results of operations, we have included the respective percentage of changes, unless greater than 100% or less than (100)%, in which case we have denoted such changes as not meaningful (n/m).

Collaboration and Other Research and Development Revenues

Collaboration and other research and development revenues increased by $8.2 million, to $40.5 million for the year ended December 31, 2025, from $32.3 million for the year ended December 31, 2024. The increase was attributable to recognition of the remaining deferred revenue upon the conclusion of a collaboration agreement with a strategic partner, as well as recognition of revenue related to a milestone achieved in 2025 under our collaboration with BMS.

Research and Development Expenses

Research and development expenses decreased by $109.2 million, to $90.0 million for the year ended December 31, 2025 from $199.2 million for the year ended December 31, 2024. 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) and the respective percentages of change:

	Year Ended December 31,			
	2025	2024	Dollar Change	Percentage Change
Employee related expenses	$ 30,183	$ 54,231	$ (24,048)	(44) %
External research and development expenses	27,282	78,453	(51,171)	(65) %
Facility expenses	15,300	26,430	(11,130)	(42) %
Stock-based compensation expenses	2,968	8,642	(5,674)	(66) %
Sublicense and license fees	7,440	18,953	(11,513)	(61) %
Other expenses	6,780	12,538	(5,758)	(46) %
Total research and development expenses	$ 89,953	$ 199,247	$ (109,294)	(55) %

The decrease in research and development expenses for the year ended December 31, 2025 compared to the year ended December 31, 2024 was primarily attributable to:

- approximately $51.2 million in decreased external research and development expenses primarily resulting from reduced clinical and manufacturing costs due to the Discontinuation, partially offset by costs attributable to *in vivo* research and discovery;
- approximately $24.0 million in decreased employee related expenses related to reduced headcount associated with the Reduction;
- approximately $11.5 million in decreased sublicense and license fees related to reduced licensing activity in 2025 compared to 2024;
- approximately $11.1 million in decreased facility expenses primarily due to the end of leases for manufacturing space due to the Discontinuation;
- approximately $5.8 million in decreased other expenses attributable to professional services to support our reni-cel program due to the Discontinuation; and
- approximately $5.7 million in decreased stock-based compensation expense primarily related to expense in connection with the achievement of certain performance-based vesting milestones for restricted stock units recognized in 2024 for which there was no equivalent expense in 2025, a reduction in the market price of our common stock year-over-year resulting in lower fair value, and a reduction in headcount associated with the Reduction.

General and Administrative Expenses

General and administrative expenses decreased by approximately $22.1 million, to $49.9 million for the year ended December 31, 2025 from $72.0 million for the year ended December 31, 2024. The following table summarizes our general and administrative expenses for the years ended December 31, 2025 and December 31, 2024, together with the changes in those items in dollars (in thousands) and the respective percentages of change:

	Year Ended December 31,			
	2025	2024	Dollar Change	Percentage Change
Employee related expenses	$ 12,211	$ 20,766	$ (8,555)	(41) %
Professional service expenses	8,303	14,278	(5,975)	(42) %
Intellectual property and patent related fees	16,839	14,016	2,823	20 %
Stock-based compensation expenses	7,032	12,775	(5,743)	(45) %
Facility and other expenses	5,518	10,152	(4,634)	(46) %
Total general and administrative expenses	$ 49,903	$ 71,987	$ (22,084)	(31) %

The decrease in general and administrative expenses for the year ended December 31, 2025 compared to the year ended December 31, 2024 was primarily attributable to:

- approximately $8.6 million in decreased employee related expenses related to reduced headcount associated with the Reduction;
- approximately $6.0 million in decreased professional services expenses primarily related to reduced licensing and strategic business activities in 2025 relative to 2024;
- approximately $5.7 million in decreased stock-based compensation expense primarily related to expense in connection with the achievement of certain performance-based vesting milestones for restricted stock units recognized in 2024 for which there was no equivalent expense in 2025, a reduction in the market price of our common stock year-over-year resulting in lower fair value, and a reduction in headcount associated with the Reduction; and

- approximately $4.6 million in decreased facility and other expenses primarily related to the end of a lease.

These decreases were partially offset by approximately $2.8 million in increased intellectual property and patent related fees for legal activity.

Restructuring Charges

Restructuring charges increased by approximately $48.4 million, to $60.7 million for the year ended December 31, 2025, from $12.2 million for the year ended December 31, 2024. The following table summarizes our restructuring charges for the years ended December 31, 2025 and December 31, 2024, together with the changes in those items in dollars (in thousands) and the respective percentages of change:

	Year Ended December 31,			
	2025	2024	Dollar Change	Percentage Change
Employee termination benefits	$ 3,723	$ 10,475	$ (6,752)	(64) %
Costs for ongoing contracts and terminated contracts	46,645	1,757	44,888	n/m
Acceleration of expense for change in useful life estimate and lease termination charges	6,548	—	6,548	100 %
Impairment charges	3,758	—	3,758	100 %
Total restructuring and impairment charges	$ 60,674	$ 12,232	$ 48,442	100 %

The increase in restructuring and impairment charges for the year ended December 31, 2025 compared to the year ended December 31, 2024 was primarily attributable to reni-cel related contract costs recognized at the contract cease-use-date in 2025, accelerated expense recognized due to changes in useful life estimates for leasehold improvements, software, and a right-of-use asset, and impairment charges related to the sale of certain assets, resulting from the actions associated with the Discontinuation and the Reduction. The increase was partially offset by decreased employee termination benefits due to the settlement of costs accrued as of December 31, 2024 during the year ended December 31, 2025. Refer to Note 17, Restructuring and Impairment Charges, for further information.

Other Income (Expense), Net

For the years ended December 31, 2025, and 2024, other income (expense), net was $0.1 million, which was primarily attributable to interest income and interest accretion related to the liability for the sale of future revenues. For the year ended December 31, 2024, other income (expense), net was $14.1 million, which was primarily attributable to interest income and accretion of discounts associated with marketable securities. The decrease is attributable to the interest accretion related to the liability for the sale of future revenues and reductions in investment income due to a decrease in our investments.

Liquidity and Capital Resources

Sources of Liquidity

As of December 31, 2025, we have raised an aggregate of $1.1 billion in net proceeds through the sale of shares of our common stock in public offerings and at-the-market offerings. We also have funded our business from our research collaboration with BMS, our former strategic alliance with Allergan, which was terminated in August 2020, payments received under the DRI Agreement in connection with the Vertex License Agreement, and payments under the Vertex License Agreement. As of December 31, 2025, we had cash, cash equivalents and marketable securities of $146.6 million.

In May 2021, we entered into a common stock sales agreement with TD Securities (USA) LLC (as successor to Cowen and Company, LLC) (“TD Cowen”) under which we from time to time can issue and sell shares of our common stock through TD Cowen in at-the-market offerings for aggregate gross sale proceeds of up to $300.0 million. We amended the common stock sales agreement with TD Cowen in February 2024 in connection with filing a new registration statement. In March 2025, we further amended our common stock sales agreement with TD Cowen in connection with amending our existing shelf registration statement following the loss of our status as a “well-known seasoned issuer” (as defined under Rule 450 of the Securities Act of 1933, as amended), reducing the amount of shares of common stock we

may issue and sell through TD Cowen to aggregate gross sale proceeds of up to $150.0 million (the "ATM Facility"). As of December 31, 2025, we have sold 14,327,365 shares of our common stock under the ATM Facility at a weighted average price of $3.07 per share for aggregate gross proceeds of $43.9 million and have $106.1 million of shares of our common stock remaining available for sale under the ATM Facility.

In addition to our existing cash and cash equivalents, we are eligible to earn milestone and other payments under our collaboration with BMS and our other collaboration and license agreements. Our ability to earn applicable milestone and other payments and the timing of earning these amounts are dependent upon the timing and outcome of development, regulatory and commercial activities and, as such, are uncertain at this time. As of December 31, 2025, our right to contingent payments under our collaboration with BMS, as well as the retained portions of the contingent upfront payment and other amounts under the Vertex License Agreement, are our only significant committed potential external source of funds.

Cash Flows

The following table provides information regarding our cash flows for the years ended December 31, 2025 and 2024, respectively (in thousands):

	Year Ended December 31,	
	2025	2024
Net cash (used in) provided by:		
Operating activities	$ (165,241)	$ (210,284)
Investing activities	138,668	162,146
Financing activities	40,470	56,027
Net increase (decrease) in cash, cash equivalents, and restricted cash	$ 13,897	$ 7,889

Net Cash Used in Operating Activities

The use of cash in all periods resulted primarily from our net losses adjusted for non-cash charges and changes in components of working capital.

Net cash used in operating activities was approximately $165.2 million for the year ended December 31, 2025, which primarily consisted of operating expenses related to our ongoing research and pre-clinical efforts, the wind-down of clinical and manufacturing activities related to our former reni-cel program and supporting business operations.

Net cash used in operating activities was approximately $210.3 million for the year ended December 31, 2024, which primarily consisted of operating expenses related to increasing our research efforts, the progression of clinical and manufacturing activities in support of our former reni-cel program and supporting business operations.

Net Cash Provided by Investing Activities

Net cash provided by investing activities was approximately $138.7 million for the year ended December 31, 2025, primarily related to maturities of marketable securities of $139.0 million and the proceeds from the sale of property and equipment of $0.3 million. This was offset by the purchases of property and equipment of $0.6 million.

Net cash provided by investing activities was approximately $162.1 million for the year ended December 31, 2024, primarily related to maturities of marketable securities of $257.2 million. This was offset by $86.2 million of purchases of marketable securities and purchases of property and equipment of $8.8 million.

Net Cash Provided by Financing Activities

Net cash provided by financing activities was approximately $40.5 million for the year ended December 31, 2025 primarily related to net proceeds from issuance of common stock from our at-the-market offering program of $42.8 million, proceeds received from issuance of common stock under our employee stock purchase plan of $0.4 million, and proceeds from the exercise of stock options of $0.1 million. This was offset by the repayment on the sale of future revenues of $2.9 million.

Net cash provided by financing activities was approximately $56.0 million for the year ended December 31, 2024, primarily related to net proceeds received from the sale of future revenue of $55.2 million, proceeds received from issuance of common stock under our employee stock purchase plan of $0.6 million and proceeds from the exercise of stock options of $0.2 million.

Funding Requirements

We expect expenses to decrease in future periods compared to prior periods, due to the Discontinuation. Our expenses for the foreseeable future will support preclinical studies and prepare for the clinical development of EDIT-401; commence clinical trials for EDIT-401; continue our current research programs and our preclinical development of product candidates from our current research programs; seek to identify additional product candidates and research programs; initiate preclinical testing and clinical trials for other product candidates we identify and develop; maintain, expand, and protect our intellectual property portfolio, including reimbursing our licensors for expenses related to the intellectual property that we in-license from such licensors; and incur costs associated with operating as a public company. In addition, if we obtain marketing approval for any product candidate that we identify and develop, we expect to incur significant commercialization expenses related to product sales, marketing, manufacturing, and distribution to the extent that such sales, marketing, and distribution are not the responsibility of a collaborator. We do not expect to generate significant recurring revenue unless and until we obtain regulatory approval for and commercialize a product candidate. Accordingly, we will need to obtain substantial additional funding in connection with our continuing operations. If we are unable to raise capital when needed or on attractive terms, we would be forced to delay, reduce, or eliminate our research and development programs or future commercialization efforts.

We expect that our existing cash and cash equivalents on December 31, 2025 will enable us to fund our operating expenses and capital expenditure requirements into the third quarter of 2027. Our forecast of the period of time through which our existing cash and cash equivalents will be adequate to support our operations is a forward-looking statement and involves significant risks and uncertainties. We have based this forecast on assumptions that may prove to be wrong, and actual results could vary materially from our expectations, which may adversely affect our capital resources and liquidity. We could utilize our available capital resources sooner than we currently expect. The amount and timing of future funding requirements, both near- and long-term, will depend on many factors, including, but not limited to:

- the costs of progressing the preclinical and clinical development of EDIT-401;
- the scope, progress, results, and costs of drug discovery, preclinical development, laboratory testing, and any clinical or natural history study trials for product candidates we 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 develop;
- the costs of establishing and maintaining a supply chain for the development and manufacture of our product candidates;
- the costs of future activities, including product sales, medical affairs, marketing, manufacturing, and distribution, for any product candidates for which we receive regulatory approval;
- the success of our collaboration with BMS, including whether BMS exercises any of its options to extend the research program term and/or to additional research programs under our collaboration;
- our ability to establish and maintain additional collaborations on favorable terms, if at all;
- the extent to which we acquire or in-license other medicines and technologies;
- the costs of reimbursing our licensors for the prosecution and maintenance of the patent rights in-licensed by us; and
- our ability to establish and maintain healthcare coverage and adequate reimbursement for any product candidates for which we receive regulatory approval.

Identifying potential product candidates and conducting preclinical studies and clinical trials is a time-consuming, expensive, and uncertain process that takes many 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 that are approved, we will require significant additional amounts in order to launch and commercialize our product candidates and may not achieve commercial success. Our commercial revenues, if any, will be derived from sales of genomic medicines that we do not expect to be commercially available for many years, if at all. 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.

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. To the extent that we raise additional capital through the sale of equity or convertible debt securities, our stockholders' ownership interests will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect the rights of our stockholders. Debt financing, if available, would result in increased fixed payment obligations and may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures, or declaring dividends.

If we raise 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 or 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.

Contractual Obligations

As of December 31, 2025, we had operating leases with future minimum lease payments for a total of $20.5 million, of which $7.4 million will be payable in 2026. These minimum lease payments exclude our share of the facility operating expenses, real-estate taxes and other costs that are reimbursable to the landlord under the leases.

In 2023, we entered into a license and service agreement pursuant to which we leased manufacturing space for our continued research and development activities. The lease commenced April 1, 2024. In September 2024, we modified the lease, and as a result of the modification the lease payments decreased and the notification period for the termination of the license and service agreement increased from 12 months' prior written notice to 18 months' prior written notice. In January 2025, we gave our termination notice on the license and service agreement, which resulted in the end of the term of the agreement being July 2026, and $8.9 million of remaining payments owed. In April 2025, we modified the lease to terminate on April 30, 2025 with a final fixed payment of $3.7 million.

In October 2024, we entered into the DRI Agreement under which we sold, transferred, assigned, and conveyed to DRI certain future license fees and other payments owed to us by Vertex under the Vertex License Agreement in exchange for an upfront cash payment by DRI to us of $57.0 million. Under the DRI Agreement, DRI is purchasing up to 100% of certain future fixed and sales-based annual license fees that the Company is entitled to receive under the Vertex License Agreement, which fees range from $5.0 million to $40.0 million per year including increases based on sales. In addition, DRI is purchasing a mid-double-digit percentage of the $50.0 million contingent upfront payment that the Company may receive under the Vertex License Agreement, in each case after subtracting amounts owing by us to our licensors, The Broad Institute, Inc. and the President and Fellows of Harvard College. The Company has retained rights to certain portions of certain other sales-based annual license fees and the contingent upfront payment that may become due under the Vertex License Agreement, and the amounts that correspond to our licensor obligations.

Our agreements with certain institutions to license intellectual property include potential milestone and success fees, sublicense fees, royalty fees, licensing maintenance fees, and reimbursement of patent maintenance costs that we may be required to pay. Our agreements to license intellectual property 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 milestones. These potential obligations are contingent upon future events and the timing and likelihood of such potential obligations are not known with certainty. For further information regarding these agreements, please see Part I, Item 1 "Business—Our Collaborations and Licensing Strategy" of this Annual Report on Form 10-K.

We also enter into contracts in the normal course of business with contract research organizations, contract manufacturing organizations 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 at any time upon prior notice.

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 and cash equivalents of $146.6 million, primarily held in money market mutual funds. 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, including cash equivalents, are in the form, or may be in the form of, money market funds or marketable securities and are or may be invested in U.S. Treasury and U.S. government agency obligations. Due to the short-term maturities and low risk profiles of our investments, an immediate 100 basis point change in interest rates would not have a material effect on the fair market value of our investments.

While we contract with certain vendors and institutions internationally, substantially all of our total liabilities as of December 31, 2025 were denominated in the United States dollar and we believe that we do not have any material exposure to foreign currency exchange rate risk.

Item 8. Financial Statement and Other Supplementary Information.

EDITAS MEDICINE, INC.

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Report of Independent Registered Public Accounting Firm

To the Stockholders and the Board of Directors of Editas Medicine, Inc.

Opinion on the Financial Statements

We have audited the accompanying consolidated balance sheets of Editas Medicine, Inc. (the Company) as of December 31, 2025 and 2024, the related consolidated statements of operations, comprehensive loss, stockholders' equity and cash flows for each of the two years in the period ended December 31, 2025, and the related notes (collectively referred to as the "consolidated financial statements"). In our opinion, the consolidated financial statements present fairly, in all material respects, the financial position of the Company at December 31, 2025 and 2024, and the results of its operations and its cash flows for each of the two years in the period ended December 31, 2025, in conformity with U.S. generally accepted accounting principles.

Basis for Opinion

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

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

Our audits included performing procedures to assess the risks of material misstatement of the 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 the critical audit matter does not alter in any way our opinion on the

consolidated financial statements, taken as a whole, and we are not, by communicating the critical audit matter below, providing a separate opinion on the critical audit matter or on the accounts or disclosures to which it relates.

Accrued Research and Development Expenses

Description of the Matter

As of December 31, 2025, the Company's accrued research and development expenses totaled $9.7 million. As discussed in Note 2 to the consolidated financial statements, the Company expenses research and development costs as incurred. To accrue these costs, the Company estimates the time period over which services will be performed and the level of effort to be expended in each period. If the actual timing of the performance of services or the level of effort varies from the Company's estimate, the accrued expense is adjusted accordingly.

Auditing the Company's accrued research and development expenses was challenging and required a higher extent of audit effort to obtain sufficient audit evidence due to the large volume of transactions and information obtained from multiple third-party vendors used by management in estimating the accrued expenses. Additionally, due to the timing and pattern of invoices received from third-party vendors, actual amounts incurred were not always known by the date the financial statements were issued.

How We Addressed the Matter in Our Audit

To test the accrued research and development expenses, our audit procedures included, among others, obtaining an understanding of management's process of estimating accrued research and development expenses and testing the accuracy and completeness of the underlying data used to support the amounts recorded. We evaluated management's estimates by inspecting contracts and related amendments with third-party vendors, reviewing cost information obtained from third-party vendors, and corroborating the progress of research and development projects with the Company's research and development personnel responsible for overseeing the projects. We also recalculated the accrual balance based on the evidence obtained and tested invoices from third-party vendors subsequent to year-end to evaluate the completeness and valuation of the accrual amount.

/s/ Ernst & Young LLP

We have served as the Company's auditor since 2015.

Boston, Massachusetts
March 9, 2026

Editas Medicine, Inc.
Consolidated Balance Sheets
(amounts in thousands, except share and per share data)

	December 31,	
	2025	**2024**
ASSETS		
Current assets:		
Cash and cash equivalents	$ 146,645	$ 131,541
Marketable securities	—	138,372
Accounts receivable	15,176	16,266
Prepaid expenses and other current assets	2,074	3,136
Total current assets	163,895	289,315
Property and equipment, net	3,542	14,497
Right-of-use assets	16,121	32,554
Restricted cash and other non-current assets	2,976	5,223
Total assets	$ 186,534	$ 341,589
LIABILITIES AND STOCKHOLDERS' EQUITY		
Current liabilities:		
Accounts payable	$ 2,605	$ 5,493
Accrued expenses	32,610	45,859
Liability for sale of future revenues, current	5,000	5,000
Deferred revenue, current	—	6,221
Operating lease liabilities, current	6,031	14,652
Total current liabilities	46,246	77,225
Operating lease liabilities, net of current portion	12,071	20,380
Liability for sale of future revenues, net of current portion	53,605	52,434
Deferred revenue, net of current portion	44,509	54,204
Other non-current liabilities	2,815	3,072
Total liabilities	159,246	207,315
Commitments and contingencies (Note 8)		
Stockholders' equity		
Preferred stock, $0.0001 par value per share: 5,000,000 shares authorized; no shares issued or outstanding	—	—
Common stock, $0.0001 par value per share: 390,000,000 and 195,000,000 shares authorized at December 31, 2025 and December 31, 2024, respectively; 97,866,996 and 82,734,696 shares issued and outstanding, at December 31, 2025 and December 31, 2024, respectively	10	8
Additional paid-in capital	1,655,781	1,602,441
Accumulated other comprehensive income	—	268
Accumulated deficit	(1,628,503)	(1,468,443)
Total stockholders' equity	27,288	134,274
Total liabilities and stockholders' equity	$ 186,534	$ 341,589

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

Editas Medicine, Inc.
Consolidated Statements of Operations
(amounts in thousands, except per share and share data)

	Year Ended December 31,	
	2025	2024
Collaboration and other research and development revenues	$ 40,520	$ 32,314
Operating expenses:		
Research and development	89,953	199,247
General and administrative	49,903	71,987
Restructuring and impairment charges	60,674	12,232
Total operating expenses	200,530	283,466
Operating loss	(160,010)	(251,152)
Other income (expense), net:		
Interest expense related to sale of future revenues	(6,171)	(2,190)
Interest income, net	8,310	16,252
Other expense, net	(2,189)	(3)
Total other income (expense), net	(50)	14,059
Net loss	$ (160,060)	$ (237,093)
Net loss per share, basic and diluted	$ (1.80)	$ (2.88)
Weighted-average common shares outstanding, basic and diluted	88,745,908	82,338,220

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

Editas Medicine, Inc.
Consolidated Statements of Comprehensive Loss
(amounts in thousands)

	Year Ended December 31,	
	2025	2024
Net loss	$ (160,060)	$ (237,093)
Other comprehensive income (loss):		
Unrealized gain (loss) on marketable debt securities	(268)	70
Comprehensive loss	$ (160,328)	$ (237,023)

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

Editas Medicine, Inc.
Consolidated Statements of Stockholders' Equity
(amounts in thousands except share data)

	Common Stock		Additional Paid-In Capital	Accumulated Deficit	Accumulated Other Comprehensive Income (Loss)	Total Stockholders' Equity
	Shares	Amount				
Balance at December 31, 2023	81,767,263	$ 8	$ 1,580,241	$ (1,231,350)	$ 198	$ 349,097
Exercise of stock options	23,809	—	193	—	—	193
Stock-based compensation expense	—	—	21,417	—	—	21,417
Vesting of restricted common stock awards	730,157	—	—	—	—	—
Issuance of common stock under employee stock purchase plan	213,467	—	590	—	—	590
Unrealized gain on marketable debt securities	—	—	—	—	70	70
Net loss	—	—	—	(237,093)	—	(237,093)
Balance at December 31, 2024	82,734,696	$ 8	$ 1,602,441	$ (1,468,443)	$ 268	$ 134,274
Issuance of common stock from at-the-market equity offering, net	14,327,365	2	42,846	—	—	42,848
Exercise of stock options	41,430	—	70	—	—	70
Stock-based compensation expense	—	—	10,000	—	—	10,000
Vesting of restricted common stock awards	476,731	—	—	—	—	—
Issuance of common stock under employee stock purchase plan	286,774	—	424	—	—	424
Unrealized loss on marketable debt securities	—	—	—	—	(268)	(268)
Net loss	—	—	—	(160,060)	—	(160,060)
Balance at December 31, 2025	97,866,996	$ 10	$ 1,655,781	$ (1,628,503)	$ —	$ 27,288

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

Editas Medicine, Inc.
Consolidated Statements of Cash Flows
(amounts in thousands)

	Year Ended December 31,	
	2025	2024
Cash flow from operating activities		
Net loss	$ (160,060)	$ (237,093)
Adjustments to reconcile net loss to net cash used in operating activities:		
Stock-based compensation expense	10,000	21,417
Depreciation	5,277	5,809
Loss on disposal of fixed assets	2,193	149
Net accretion of discounts on marketable securities	(902)	(5,791)
Interest related to sale of future revenues	6,171	2,190
Impairment of held for sale assets	3,758	—
Other non-cash items, net	(449)	(415)
Changes in operating assets and liabilities:		
Accounts receivable	1,090	(6,079)
Prepaid expenses and other current assets	1,062	4,396
Right-of-use assets	11,584	1,126
Other non-current assets	1,040	3,366
Accounts payable	(2,888)	(2,286)
Accrued expenses	(13,184)	11,623
Accrued interest on sale of future revenues	(2,128)	—
Deferred revenue	(15,916)	(8,463)
Operating lease liabilities	(12,081)	(1,504)
Other non-current liabilities	192	1,271
Net cash used in operating activities	(165,241)	(210,284)
Cash flow from investing activities		
Purchases of property and equipment	(607)	(8,826)
Proceeds from the sale of equipment	269	—
Purchases of marketable securities	—	(86,217)
Proceeds from maturities of marketable securities	139,006	257,189
Net cash provided by investing activities	138,668	162,146
Cash flow from financing activities		
Repayment on sale of future revenues	(2,872)	—
Proceeds from issuance of common stock from at-the-market equity offering, net	42,848	—
Proceeds from sale of future revenues, net	—	55,244
Proceeds from exercise of stock options	70	193
Issuance of common stock under benefit plans	424	590
Net cash provided by financing activities	40,470	56,027
Net increase (decrease) in cash, cash equivalents, and restricted cash	13,897	7,889
Cash, cash equivalents, and restricted cash, beginning of period	135,418	127,529
Cash, cash equivalents, and restricted cash, end of period	$ 149,315	$ 135,418
Cash and cash equivalents, end of period	146,645	131,541
Restricted cash[1]	2,670	3,877
Cash, cash equivalents, and restricted cash, end of period	$ 149,315	$ 135,418

[1]As of December 31, 2025 and December 31, 2024, restricted cash of $2,670 and $3,877, respectively, was included in Restricted cash and other non-current assets on the Consolidated Balance Sheets.

Supplemental disclosure of cash and non-cash activities:		
Fixed asset additions included in accounts payable and accrued expenses	$ —	$ 65
Cash paid in connection with operating lease liabilities	14,968	18,447
Remeasurement of operating lease liabilities and right-of-use assets due to lease modification	766	794
Commencement of right-of-use asset	—	7,844
Modification of right-of-use asset	—	2,683
Non-cash termination of right-of-use asset	4,849	—
Cash paid for interest	2,128	—
Issuance costs associated with the sale of future revenues included in accounts payable and accrued expenses	—	1,479

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

Editas Medicine, Inc.
Notes to Consolidated Financial Statements

1. Nature of Business

Editas Medicine, Inc. (the "Company") is a pioneering gene editing company dedicated to developing transformative genomic medicines to treat a broad range of serious diseases. The Company was incorporated in the state of Delaware in September 2013. Its principal offices are in Cambridge, Massachusetts.

Since its inception, the Company has devoted substantially all of its efforts to business planning, research and development, recruiting management and technical staff, and raising capital. The Company has primarily financed its operations through various equity financings, payments received under a research collaboration with the Bristol-Myers Squibb Company ("BMS"), through its wholly owned subsidiary Juno Therapeutics, Inc. ("Juno Therapeutics"), payments received under its former strategic alliance with Allergan Pharmaceuticals International Limited (together with its affiliates, "Allergan"), which was terminated in August 2020, payments received under a purchase and sale agreement with DRI Healthcare Trust ("DRI," and such agreement, the "DRI Agreement") and payments received under the Company's license agreement with Vertex Pharmaceuticals, Inc ("Vertex," and such agreement, the "Vertex License Agreement").

The Company is subject to risks common to companies in the biotechnology industry, including but not limited to, risks of failure of preclinical studies and clinical trials, the need to obtain marketing approval for any drug product candidate that it may identify and develop, the need to successfully commercialize and gain market acceptance of its product candidates, dependence on key personnel, protection of proprietary technology, compliance with government regulations, development by competitors of technological innovations and ability to transition from pilot-scale manufacturing to large-scale production of products.

Liquidity

As of December 31, 2025, the Company has raised an aggregate of $1.1 billion in net proceeds through the sale of shares of its common stock in public offerings and at-the-market offerings. The Company also has funded its business from payments received under the DRI Agreement, payments received under the Vertex License Agreement, its research collaboration with BMS through its wholly owned subsidiary Juno Therapeutics and its former strategic alliance with Allergan, which was terminated in August 2020. As of December 31, 2025, the Company had cash and cash equivalents of $146.6 million.

In May 2021, the Company entered into a common stock sales agreement with TD Securities (USA) LLC (as successor to Cowen and Company, LLC) ("TD Cowen"), under which the Company from time to time can issue and sell shares of the Company's common stock through TD Cowen in at-the-market offerings for aggregate gross sale proceeds of up to $300.0 million. The Company amended the common stock sales agreement with TD Cowen in February 2024 in connection with filing a new registration statement. In March 2025, the Company further amended its common stock sales agreement with TD Cowen in connection with amending its existing shelf registration statement following the loss of the Company's status as a "well-known seasoned issuer" (as defined under Rule 405 of the Securities Act of 1933, as amended), reducing the amount of shares of common stock the Company may issue and sell through TD Cowen to aggregate gross sale proceeds of up to $150.0 million (the "ATM Facility"). As of December 31, 2025, the Company had sold 14,327,365 shares of common stock under the ATM Facility for gross proceeds of $43.9 million and has $106.1 million of shares of common stock remaining available for issuance and sale under the ATM Facility.

The Company has incurred annual net operating losses in every year since its inception. As of March 9, 2026, the issuance date of the consolidated financial statements, the Company expects that its existing cash and cash equivalents will be sufficient to fund its operating expenses and capital expenditure requirements for at least the next twelve months. The Company has an accumulated deficit of $1.6 billion at December 31, 2025, and will require substantial additional capital to fund its operations. The Company has never generated any product revenue. There can be no assurance that the Company will be able to obtain additional debt or equity financings or generate product revenue or revenues from collaborative partners, on terms acceptable to the Company, on a timely basis or at all. The failure of the Company to obtain sufficient funds on acceptable terms when needed could have a material adverse effect on the Company's business, results of operations, and financial condition.

2. Summary of Significant Accounting Policies

Principles of Consolidation

The accompanying consolidated financial statements include the accounts of Editas Medicine, Inc. and its wholly owned subsidiaries, Editas Securities Corporation, which is a Delaware subsidiary created to buy, sell, and hold securities, and Editas Medicine, LLC, a Delaware limited liability company. All intercompany transactions and balances have been eliminated.

Basis of Presentation

The accompanying consolidated financial statements have been prepared in conformity with accounting principles generally accepted in the United States of America ("GAAP"). Any reference in these notes to applicable guidance is meant to refer to the authoritative United States generally accepted accounting principles as found in the Accounting Standards Codification ("ASC") and Accounting Standards Updates ("ASUs") of the FASB. For the year ended December 31, 2024, interest expense related to sale of future revenues has been reclassified to conform to the current year presentation in the consolidated statements of operations.

Use of Estimates

The preparation of consolidated financial statements in conformity with GAAP requires management to make estimates and assumptions that affect the amounts reported in the consolidated financial statements and accompanying notes. On an ongoing basis, the Company's management evaluates its estimates, which include, but are not limited to, estimates related to revenue recognition, stock-based compensation expense, the accrual for research and development expenses, the accrual for restructuring charges, effective interest rates, and deferred tax valuation allowances. The Company bases its estimates on historical experience and other market-specific or relevant assumptions that it believes to be reasonable under the circumstances. Actual results may differ from those estimates or assumptions.

Fair Value Measurements

ASC Topic 820, *Fair Value Measurement* ("ASC 820"), establishes a fair value hierarchy for instruments measured at fair value and requires valuation technique(s) used to measure fair value to maximize the use of observable inputs and minimize the use of unobservable inputs. Observable inputs are inputs that market participants would use in pricing the asset or liability based on market data obtained from sources independent of the Company. Unobservable inputs are inputs that reflect the Company's assumptions about the inputs that market participants would use in pricing the asset or liability, and are developed based on the best information available in the circumstances.

ASC 820 identifies fair value as the exchange price representing the amount that would be received to sell an asset or paid to transfer a liability in an orderly transaction between market participants on the measurement date. ASC 820 establishes a three-tier fair value hierarchy that distinguishes between the following:

- *Level 1* – Quoted market prices in active markets for identical assets or liabilities.
- *Level 2* – Observable inputs (other than Level 1 quoted prices) such as quoted prices in active markets for similar assets or liabilities, quoted prices in markets that are not active for identical or similar assets or liabilities, or other inputs that are observable or can be corroborated by observable market data.
- *Level 3* – Unobservable inputs that are supported by little or no market activity and that are significant to determining the fair value of the assets or liabilities, including pricing models, discounted cash flow methodologies and similar techniques.

To the extent that the valuation technique used to measure fair value utilizes inputs that are unobservable in the market, the determination of fair value requires more judgment. Accordingly, the degree of judgment exercised by the Company in determining fair value is greatest for instruments categorized in 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.

The carrying amounts reflected in the consolidated balance sheets for cash and cash equivalents, restricted cash, accounts receivable, prepaid expenses and other current assets, accounts payable, accrued expenses, and other current liabilities approximate their fair values, due to their short-term nature.

Cash, Cash Equivalents, and Restricted Cash

The Company considers all highly liquid investments purchased with original maturities of 90 days or less at acquisition to be cash equivalents. Cash and cash equivalents include cash held in banks and amounts held in money market funds.

As of December 31, 2025, the Company had restricted cash of $2.7 million held as collateral for the Company's office and lab facilities and credit card program. The restricted funds are maintained in a traditional bank account. All of the Company's restricted cash was included in restricted cash and other non-current assets on the consolidated balance sheets.

Marketable Securities

The Company classifies marketable securities with a remaining maturity when purchased of greater than three months and less than one year from the balance sheet date as current. Marketable securities are classified as long-term assets in the consolidated balance sheets if the contractual maturity exceeds one year and the Company does not intend to utilize the marketable securities to fund current operations. As of December 31, 2025, the Company did not hold marketable securities. For the year ended December 31, 2024, the Company's marketable securities consisted of investments in available-for-sale debt securities.

Available-for-sale debt securities are carried at fair value with the unrealized gains and losses included in accumulated other comprehensive income (loss) as a component of stockholders' equity until realized. Any premium or discount arising at purchase is amortized or accreted, respectively, to interest income, net over the life of the underlying security. Realized gains and losses are included in other income (expense).

The Company evaluates securities for impairment at the end of each reporting period. Impairment is evaluated considering numerous factors, and their relative significance varies depending on the situation. Factors considered include whether a decline in fair value below the amortized cost basis is due to credit-related factors or non-credit-related factors, the financial condition and near-term prospects of the issuer, and the Company's intent and ability to hold the investment to allow for an anticipated recovery in fair value. A credit-related impairment is recognized as an allowance on the balance sheet with a corresponding adjustment to income or loss. Any impairment that is not credit-related is recognized in other comprehensive income (loss), net of applicable taxes.

Accounts Receivable

The Company's receivables primarily relate to amounts reimbursed under its collaboration agreements. The Company believes that credit risk associated with its collaborations partners is not significant. To estimate the allowance for credit losses, the Company determines the allowance based on ongoing credit evaluation, historical experience, and the aging of such receivables, among other factors. To date, the Company has not had any write-offs and did not have an allowance for credit losses related to its receivables as of December 31, 2025 and 2024.

Concentrations of Credit Risk and Off-Balance Sheet Risk

The Company has no financial instruments with off-balance sheet risk such as foreign exchange contracts, option contracts, or other foreign hedging arrangements. Financial instruments that potentially subject the Company to a concentration of credit risk are cash, cash equivalents, marketable securities and receivables owed to the Company from collaboration partners. The Company's cash, cash equivalents, and marketable securities are held in accounts at a financial institution that may exceed federally insured limits.

Property and Equipment

Property and equipment consists of computers, laboratory equipment, furniture and office equipment, and leasehold improvements and is stated at cost, less accumulated depreciation. Maintenance and repairs that do not improve or extend the lives of the respective assets are expensed to operations as incurred, while costs of major additions and

betterments are capitalized. Depreciation is calculated over the estimated useful lives of the assets using the straight-line method. The Company capitalizes laboratory equipment used for research and development if it has alternative future use in research and development or otherwise.

Asset:	Estimated Useful life
Laboratory equipment	5 years
Computer equipment and software	3 years
Furniture and equipment	5 years
Leasehold improvements	Shorter of useful life or remaining lease term

Impairment of Long-Lived Assets

The Company evaluates long-lived assets for potential impairment when events or changes in circumstances indicate the carrying value of the assets may not be recoverable. Recoverability is measured by comparing the book values of the assets to the expected future net undiscounted cash flows that the assets are expected to generate. If such assets are considered to be impaired, the impairment to be recognized is measured as the amount by which the book values of the assets exceed their fair value. During the year ended December 31, 2025 the Company recognized a $3.8 million impairment charge. Refer to Note 17, Restructuring and Impairment Charges, for further information.

Revenue Recognition

The Company recognizes revenue in accordance with ASC Topic 606, *Revenue from Contracts with Customers* ("ASC 606"). The Company enters into collaboration agreements and certain other agreements that are within the scope of ASC 606, under which the Company licenses, may license or grants an option to license rights to certain of the Company's product candidates and performs research and development services in connection with such arrangements. The terms of these arrangements typically include payment of one or more of the following: non-refundable, up-front fees; reimbursement of research and development costs; development, clinical, regulatory and commercial sales milestone payments; and royalties on net sales of licensed products.

Under ASC 606, an entity recognizes revenue when its customer obtains control of promised goods or services, in an amount that reflects the consideration which the entity expects to receive in exchange for those goods or services. To determine the appropriate amount of revenue to be recognized for arrangements determined to be within the scope of ASC 606, the Company performs the following five steps: (i) identification of the promised goods or services in the contract; (ii) determination of whether the promised goods or services are performance obligations, including whether they are distinct in the context of the contract; (iii) measurement of the transaction price, including the constraint on variable consideration; (iv) allocation of the transaction price to the performance obligations; and (v) recognition of revenue when (or as) the Company satisfies each performance obligation. The Company only applies the five-step model to contracts when it is probable that the entity will collect the consideration it is entitled to in exchange for the goods or services it transfers to the customer.

The promised goods or services in the Company's arrangements typically consist of a license, or option to license, rights to the Company's intellectual property or research and development services. The Company provides options to additional items in such arrangements, which are accounted for as separate contracts when the customer elects to exercise such options, unless the option provides a material right to the customer. Performance obligations are promised goods or services in a contract to transfer a distinct good or service to the customer and are considered distinct when (i) the customer can benefit from the good or service on its own or together with other readily available resources and (ii) the promised good or service is separately identifiable from other promises in the contract. In assessing whether promised good or services are distinct, the Company considers factors such as the stage of development of the underlying intellectual property, the capabilities of the customer to develop the intellectual property on its own or whether the required expertise is readily available and whether the goods or services are integral or dependent to other goods or services in the contract.

The Company estimates the transaction price based on the amount expected to be received for transferring the promised goods or services in the contract. The consideration may include fixed consideration or variable consideration. At the inception of each arrangement that includes variable consideration, the Company evaluates the amount of potential payment and the likelihood that the payments will be received. The Company utilizes either the most likely amount method or expected value method to estimate the amount expected to be received based on which method best predicts the amount expected to be received. The amount of variable consideration that is included in the transaction price may be constrained

and is included in the transaction price only to the extent that it is probable that a significant reversal in the amount of the cumulative revenue recognized will not occur in a future period.

The Company's contracts often include development and regulatory milestone payments that are assessed under the most likely amount method and constrained if it is probable that a significant revenue reversal would occur. Milestone payments that are not within the Company's control or the licensee's control, such as regulatory approvals, are not considered probable of being achieved until those approvals are received. At the end of each reporting period, the Company re-evaluates the probability of achievement of such development and clinical milestones and any related constraint, and if necessary, adjusts its estimate of the overall transaction price. Any such adjustments are allocated to the respective performance obligations and the amounts allocated to satisfied performance obligations are recognized in the periods when adjustments are made.

For arrangements that include sales-based royalties, including milestone payments based on the level of sales, and 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 the Company's collaboration or strategic alliance arrangements.

The Company allocates the transaction price based on the estimated standalone selling price. The Company must develop assumptions that require judgment to determine the stand-alone selling price for each performance obligation identified in the contract. The Company utilizes key assumptions to determine the stand-alone selling price, which may include other comparable transactions, pricing considered in negotiating the transaction and the estimated costs. Variable consideration is allocated specifically to one or more performance obligations in a contract when the terms of the variable consideration relate to the satisfaction of the performance obligation and the resulting amounts allocated are consistent with the amounts the Company would expect to receive for the satisfaction of each performance obligation.

The consideration allocated to each performance obligation is recognized as revenue when control is transferred for the related goods or services. For performance obligations which consist of licenses and 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. The Company evaluates the measure of progress each reporting period and, if necessary, adjusts the measure of performance and related revenue recognition.

The Company receives payments from its customers based on billing schedules established in each contract. Up-front payments and fees are recorded as deferred revenue upon receipt or when due until the Company performs its obligations under these arrangements. Amounts are recorded as accounts receivable when the Company's right to consideration is unconditional.

Research and Development Expenses

Research and development expenses are charged to expense as incurred in performing research and development activities. The costs include employee-related expenses, including salaries, benefits, and stock-based compensation expense, costs of funding research performed by third parties that conduct research and development and preclinical and clinical activities on the Company's behalf, the cost of purchasing lab supplies and non-capital equipment used in preclinical and clinical activities and in manufacturing preclinical and clinical study materials, consultant fees, facility costs, including rent, depreciation, and maintenance expenses, and fees for acquiring and maintaining licenses under third party licensing agreements, which are typically expensed when incurred if the technology licensed has no alternate future uses, including any sublicensing or success payments made to the Company's licensors. In accruing service fees, the Company estimates the time period over which services will be performed and the level of effort to be expended in each period. If the actual timing of the performance of services or the level of effort varies from the Company's estimate, the accrual or prepaid is adjusted accordingly. The Company defers and capitalizes non-refundable advance payments made by the Company for research and development activities until the related goods are received or the related services are performed. In circumstances where amounts have been paid in excess of costs incurred, the Company records a prepaid expense.

Patent Costs

The Company expenses patent and patent application costs and related legal costs for the prosecution and maintenance of such patents and patent applications, including patents and patent applications the Company in-licenses, as incurred and classifies such costs as general and administrative expenses in the accompanying consolidated statements of operations.

Liability for Sale of Future Revenues

On October 3, 2024, the Company entered into the DRI Agreement under which it sold, transferred, assigned, and conveyed to DRI certain future license fees and other payments owed to the Company by Vertex under the terms of the Vertex License Agreement in exchange for an upfront cash payment by DRI to the Company of $57.0 million. Under the DRI Agreement, DRI is purchasing up to 100% of certain future fixed and sales-based annual license fees that the Company is entitled to receive under the Vertex License Agreement, which fees range from $5.0 million to $40.0 million per year, including increases based on sales. In addition, DRI is purchasing a mid-double-digit percentage of a $50.0 million contingent upfront payment that the Company may receive under the Vertex License Agreement. All amounts above will be adjusted to exclude payments that the Company owes to Broad and Harvard, as defined in Note 8, Commitments and Contingencies. The Company has retained rights to certain portions of certain sales-based annual license fees and the contingent upfront payment that may become due under the Vertex License Agreement, and the amounts that correspond to its licensor obligations.

In accordance with ASC Topic 470, *Borrower's Accounting for Debt Modification*, the Company has accounted for the transaction as debt. The gross proceeds of $57.0 million were recorded as a liability for the sale of future revenues, net of transaction costs of $1.8 million, which will be amortized over the estimated life of the arrangement using the effective interest method.

The Company estimates the effective interest rate used to record non-cash interest expense under the DRI Agreement based on the estimate of future revenue payments to be made to DRI. As of December 31, 2025, the estimated effective interest rate under the agreement was 7.7%. Over the life of the arrangement, the actual effective interest rate will be affected by the amount and timing of the payments made to DRI and changes in the Company's revenue forecasts. At each reporting date, the Company will reassess its estimate of total future payments to be made to DRI, and prospectively adjust the effective interest rate and amortization of the liability as necessary. Refer to Note 16, Debt, for further information.

Leases

The Company accounts for leases in accordance with ASC Topic 842, *Leases*. At the inception of an arrangement the Company determines whether the arrangement contains a lease. If a lease is identified in an arrangement, the Company recognizes a right-of-use asset and a lease liability on its balance sheet and determines whether the lease should be classified as a finance or operating lease. The Company does not recognize assets or liabilities for leases with lease terms of less than 12 months. Lease payments for short-term leases are recorded to operating expense on a straight-line basis over the lease term and variable lease payments are recorded in the period in which the obligation for those payments is incurred.

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

Finance and operating lease assets and liabilities are recognized at the lease commencement date based on the present value of the lease payments over the lease term using the discount rate implicit in the lease. If the rate is not readily determinable, the Company utilizes its incremental borrowing rate at the lease commencement date. Lease assets are further adjusted for prepaid or accrued lease payments. Operating lease payments are expensed using the straight-line method as an operating expense over the lease term. Finance lease assets are amortized to depreciation expense using the straight-line method over the shorter of the useful life of the related asset or the lease term. Finance lease payments are

bifurcated into (i) a portion that is recorded as imputed interest expense and (ii) a portion that reduces the finance liability associated with the lease.

The Company does not separate lease and non-lease components when determining which lease payments to include in the calculation of its lease assets and liabilities. Variable lease payments are expensed as incurred. If a lease includes an option to extend or terminate the lease, the Company reflects the option in the lease term if it is reasonably certain it will exercise the option.

Stock-based Compensation Expense

The Company accounts for all stock-based awards granted to employees and non-employees as stock-based compensation expense at fair value in accordance with ASC Topic 718, *Compensation—Stock Compensation* ("ASC 718"). The Company estimates the grant date fair value of restricted stock based on the market value of the Company's common stock on the date of the grant. The Company estimates the grant date fair value of each option award using the Black-Scholes option-pricing model.

The Black-Scholes option pricing model requires the input of certain assumptions, including (1) the expected stock price volatility, (2) the calculation of expected term of the award, (3) the risk-free interest rate, and (4) the expected dividend yield. The Company used its own historical volatility data for its estimates of expected volatility. The Company calculates historical volatility based on a period of time commensurate with the expected term. The Company uses the simplified method as prescribed by ASC 718 to calculate the expected term for options granted to employees as the Company is unable to rely on historical exercise data to provide a reasonable basis upon which to estimate the expected term. For options granted to non-employees, the Company utilizes the contractual term of the arrangement as the basis for the expected term. The Company determines the risk-free interest rate based on a treasury instrument whose term is consistent with the expected term of the stock options. The Company uses an assumed dividend yield of zero as the Company has never paid dividends and does not have current plans to pay any dividends on its common stock.

Service-Based Awards

For stock-based awards issued to employees, non-employee service providers, and members of the Company's board of directors (the "Board"), the Company recognizes the grant date fair value of the service-based options or restricted stock units ("RSUs") on a straight-line basis over the requisite service period, which is generally the vesting period of the respective award. If an employee or non-employee service requirement is concluded to be non-substantive, the stock-based compensation expense would be expensed immediately.

Performance-Based Awards

For performance-based awards, the Company recognizes the grant date fair value of the performance-based options or RSUs over the requisite service period using the accelerated attribution method to the extent achievement of the performance condition is probable. Certain awards are subject to both performance and continued service conditions.

The Company classifies stock-based compensation expense in its consolidated statements of operations in the same manner in which the award recipient's salary or service payments are classified. Forfeitures are recorded as they occur. If factors change or different assumptions are used, the Company's stock-based compensation expense could be materially different in the future.

Success Payments, Research Funding Payments and Notes Payables

Certain arrangements require the Company to make payments, if and when, the Company's market capitalization reaches specified thresholds for a specific period of time or upon a sale of the Company for consideration in excess of those thresholds or above a specific amount. The payments are accounted for under the provisions of ASC 718, whereby the Company recognizes the expense and liability when it becomes probable that the amounts will become due. The Company records this expense as a research and development expense in its consolidated statements of operations. The arrangements and payments are described more fully in Note 8, Commitments and Contingencies.

The payments are payable in either cash, common stock, or promissory notes payable, depending upon the licensor and the Company's election. If the Company elects to issue a promissory note relating to contractual obligations, the promissory note bears interest at 4.8% per annum. Outstanding principal and accrued interest on the promissory notes

are typically payable on the earlier of five months or a specified period of time following a Company sale or change of control event, subject to certain exceptions.

Restructuring

The Company records liabilities for costs associated with restructuring activities in the period in which the liability is incurred. Typical costs associated with restructuring activities include, employee termination benefits, contract termination costs and on-going contract costs for which there is no economic benefit. For costs associated with employee terminations in which the employee is subject to an existing benefit arrangement, the post-employment benefits are recognized when probable and estimable. Other employee termination costs are measured and recognized on the communication date, unless there is a required future service period, in which case, the expense is recognized over the service period. Contract termination costs are recognized upon termination of the contract and costs for on-going contracts for which there is no future benefit, are recognized at fair value on the cease-use date.

The Company has made estimates and judgments regarding the amount and timing of its restructuring expense and liability, including current and future period termination benefits and other exit costs to be incurred when related actions take place. Restructuring charges are reflected in the Company's consolidated statements of operations. Actual results may differ from these estimates. Refer to Note 17, Restructuring and Impairment Charges, for further information.

Income taxes

Income taxes are recorded in accordance with ASC Topic 740, *Income Taxes* (“ASC 740”), which provides for deferred taxes using an asset and liability approach. Under this method, deferred tax assets and liabilities are determined based on the difference between the financial reporting and the tax reporting basis of assets and liabilities and are measured using the enacted tax rates and laws that are expected to be in effect when the differences are expected to reverse. The Company provides a valuation allowance against net deferred tax assets unless, based upon the weight of available evidence, it is more likely than not that the deferred tax assets will be realized.

The Company accounts for uncertain tax positions in accordance with the provisions of ASC 740. When uncertain tax positions exist, the Company recognizes the tax benefit of tax positions to the extent that the benefit will more likely than not be realized. The determination as to whether the tax benefit will more likely than not be realized is based upon the technical merits of the tax position as well as consideration of the available facts and circumstances.

The Company assesses the impact of various tax reform proposals and modifications to existing tax treaties in all jurisdictions where they have operations to determine the potential effect on the Company’s business and any assumptions they have made about their future taxable income. The Company cannot predict whether any specific proposals will be enacted, the terms of any such proposals or what effect, if any, such proposals would have on the Company if they were to be enacted.

Comprehensive Loss

Comprehensive loss consists of net loss and changes in unrealized gains and losses on marketable securities.

Segment Information

Operating segments are defined as components of an enterprise about which separate discrete information is available for evaluation by the chief operating decision maker (the “CODM”), in deciding how to allocate resources and assess performance. The Company and the Company’s CODM, the Company’s Chief Executive Officer (the “CEO”), view the Company’s operations and manage the Company’s business as a single operating segment, which is the business of developing and commercializing gene editing technology.

Recently Adopted Accounting Pronouncements

In December 2023, the FASB issued ASU 2023-09 *Income Taxes (Topic 740): Improvements to Income Tax Disclosures*. This guidance is intended to enhance the transparency and decision-usefulness of income tax disclosures. The amendments in ASU 2023-09 require more detailed information in the income tax rate reconciliation and income taxes paid disaggregated by jurisdiction, among other enhancements. ASU 2023-09 is effective for fiscal years beginning after

December 15, 2024 on a prospective basis, with the option to apply the standard retrospectively. Early adoption was permitted.

ASU 2023-09 applies to disclosure requirements only. The Company retrospectively adopted the ASU as of December 31, 2025 and provided the required annual disclosures in its consolidated financial statements for the periods ending December 31, 2025 and December 31, 2024. Refer to Note 14, Income Taxes, for additional information on the Company's income taxes.

Recently Issued Accounting Pronouncements

In November 2024, the FASB issued ASU 2024-03 *Income Statement - Reporting Comprehensive Income - Expense Disaggregation Disclosures (Subtopic 220-40): Disaggregation of Income Statement Expenses*, which is intended to improve the disclosures about a public business entity's expenses by providing more detailed information about the types of expenses in commonly presented expense captions. The amendments in ASU 2024-03 are effective in the first annual reporting period beginning after December 15, 2026, and interim reporting periods within annual reporting periods beginning after December 15, 2027 as clarified in ASU 2025-01 *Income Statement - Reporting Comprehensive Income - Expense Disaggregation Disclosures (Subtopic 220-40): Clarifying the Effective Date*. Early adoption is permitted. The amendments can be applied either prospectively or retrospectively. The Company is currently evaluating the impact of this new standard in its consolidated financial statements and related disclosures.

In October 2023, the FASB issued ASU 2023-06 *Disclosure Improvements: Codification Amendments in Response to the SEC's Disclosure Update and Simplification Initiative,* which incorporates certain SEC disclosure requirements into the ASC. The amendments in the ASU are expected to clarify or improve disclosure and presentation requirements of a variety ASC topics, allow investors to more easily compare entities subject to the SEC's existing disclosures with those entities that were not previously subject to the requirements, and align the requirements in the ASC with the SEC's regulations. The effective date for each amendment will be the date on which the SEC's removal of that related disclosure from Regulation S-X or Regulation S-K becomes effective, with early adoption prohibited. The amendments in this ASU should be applied prospectively. The Company does not expect ASU 2023-06 will have a material impact to its consolidated financial statements.

3. Cash Equivalents and Marketable Securities

Cash equivalents consisted of the following at December 31, 2025 (in thousands):

December 31, 2025	Amortized Cost	Allowance for Credit Losses	Gross Unrealized Gains	Gross Unrealized Losses	Fair Value
Cash equivalents:					
Money market funds	$ 146,645	$ —	$ —	$ —	$ 146,645
Total	$ 146,645	$ —	$ —	$ —	$ 146,645

Cash equivalents and marketable securities consisted of the following at December 31, 2024 (in thousands):

December 31, 2024	Amortized Cost	Allowance for Credit Losses	Gross Unrealized Gains	Gross Unrealized Losses	Fair Value
Cash equivalents and marketable securities:					
Money market funds	$ 131,541	$ —	$ —	$ —	$ 131,541
U.S. Treasuries	131,582	—	289		131,871
Government agency securities	—	—	—	—	—
Corporate notes/bonds	6,522	—	—	(21)	6,501
Total	$ 269,645	$ —	$ 289	$ (21)	$ 269,913

As summarized in the tables immediately above, the Company's marketable securities had immaterial unrealized losses as of December 31, 2024. As of December 31, 2025, the Company did not hold available-for-securities.

4. Fair Value Measurements

Assets measured at fair value on a recurring basis as of December 31, 2025 were as follows (in thousands):

Financial Assets	December 31, 2025	Quoted Prices in Active Markets for Identical Assets (Level 1)	Significant Other Observable Inputs (Level 2)	Significant Unobservable Inputs (Level 3)
Cash equivalents:				
Money market funds	$ 146,645	$ 146,645	$ —	$ —
Restricted cash and other non-current assets:				
Money market funds	2,670	2,670	—	—
Total financial assets	$ 149,315	$ 149,315	$ —	$ —

Assets measured at fair value on a recurring basis as of December 31, 2024 were as follows (in thousands):

Financial Assets	December 31, 2024	Quoted Prices in Active Markets for Identical Assets (Level 1)	Significant Other Observable Inputs (Level 2)	Significant Unobservable Inputs (Level 3)
Cash equivalents:				
Money market funds	$ 131,541	$ 131,541	$ —	$ —
Marketable securities:				
Government agency securities	—	—	—	—
Corporate bonds	6,501	—	6,501	—
U.S. Treasuries	131,871	131,871	—	—
Restricted cash and other non-current assets:				
Money market funds	3,877	3,877	—	—
Total financial assets	$ 273,790	$ 267,289	$ 6,501	$ —

The fair value of the Company's liability for sale of future revenues approximates the amount recorded on the Company's balance sheet as of December 31, 2025 and 2024, which represents a level 3 fair value measurement.

5. Property and Equipment, Net

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

	As of December 31,	
	2025	2024
Laboratory equipment	$ 21,558	$ 29,128
Leasehold improvements	8,403	11,749
Construction-in-progress	—	827
Computer equipment	1,399	1,490
Furniture and office equipment	191	264
Software	344	2,687
Total property and equipment	31,895	46,145
Less: accumulated depreciation	(28,353)	(31,648)
Property and equipment, net	$ 3,542	$ 14,497

The Company recorded $5.3 million and $5.8 million in depreciation expense during the years ended December 31, 2025 and 2024, respectively.

6. Accrued Expenses

Accrued expenses consisted of the following (in thousands):

	As of December 31,	
	2025	2024
External research and development expenses	$ 9,672	$ 11,630
Employee related expenses	5,220	11,516
Sublicense and license fees	5,501	6,609
Intellectual property and patent related fees	2,105	1,471
Professional service expenses	232	1,593
Employee termination benefits	1,555	10,475
Restructuring contract costs	7,769	1,757
Other expenses	556	808
Total accrued expenses	$ 32,610	$ 45,859

7. Leases

The Company has multiple lease agreements for office, laboratory, and manufacturing space with varying contractual terms set to expire between 2026 and 2028. Typically, base rent payments commence at the beginning of each lease term and continue through the term of the respective lease. The Company's lease agreements have escalating rent clauses, which require higher rent payments in future years. The Company has one significant lease for office and laboratory space located in Cambridge, Massachusetts. The term of the lease began on October 1, 2016 and continues until October 2028. The Company does not have the option to extend the term of the lease at the termination date.

In 2023, the Company entered into a license and service agreement under which it leased manufacturing space for its continued research and development activities to support the Company's former reni-cel program. The lease commenced on April 1, 2024. In January 2025, the Company provided termination notice for the license and service agreement, which resulted in a scheduled lease termination date in July 2026 and $8.9 million of remaining payments owed. In April 2025, the Company entered into a modification to the lease to accelerate the lease termination date to April 30, 2025 with a final fixed payment of $3.7 million. As the lease modification resulted from the Discontinuation, as defined in Note 17, Restructuring and Impairment Charges, the Company recorded an expense for the final fixed payment in restructuring and impairment charges in the consolidated statement of operations during the year ended December 31, 2025.

The Company's operating leases included in its consolidated balance sheets as follows (in thousands):

	As of	
	December 31, 2025	December 31, 2024
Right-of-use assets	$ 16,121	$ 32,554
Operating lease liabilities, current	$ (6,031)	$ (14,652)
Operating lease liabilities, non-current	$ (12,071)	$ (20,380)

During the years ended December 31, 2025 and 2024, the Company recorded $14.8 million and $21.0 million, respectively, of expense related to operating lease costs and $2.6 million and $2.8 million, respectively, related to variable costs associated with the Company's operating leases.

Maturities of the Company's lease liabilities as of December 31, 2025 were as follows (in thousands):

Maturity of lease liabilities:	Year Ended December 31, 2025
2026	$ 7,389
2027	$ 7,076
2028	$ 6,000
Total minimum lease payments	$ 20,465
Less: imputed interest	$ (2,363)
Total operating lease liabilities at December 31, 2025	$ 18,102

The weighted-average remaining lease term is 2.8 years and the weighted-average discount rate is 9.2%.

8. Commitments and Contingencies

The Company is a party to a number of license agreements under which the Company licenses patents, patent applications, and other intellectual property from third parties. As such, the Company is obligated to pay licensors for various costs including upfront license fees, annual license fees, certain licensor expense reimbursements, success payments, research funding payments, and milestones triggerable upon certain development, regulatory, and commercial events as well as royalties on future product sales. These contracts are generally cancellable, with notice, at the Company's option and do not have significant cancellation penalties.

Broad Sponsored Research Agreement

In 2018, the Company entered into a sponsored research agreement, which was amended in January 2021 (as amended, the "Sponsored Research Agreement") with The Broad Institute, Inc. ("Broad"). The Sponsored Research Agreement provides for Broad to conduct research useful or relevant to gene editing in the field of genomic medicines for the prevention or treatment of human disease with funding from the Company. Under the Sponsored Research Agreement, Broad granted to the Company an exclusive right of first negotiation for licenses from Broad with respect to patentable inventions developed by Broad in the course of the sponsored research, subject to certain limitations and retained rights ("Sponsored Invention Licenses").

Under the Sponsored Research Agreement, the Company is obligated to make payments ("Market Cap Research Funding") in the event the Company's market capitalization reaches certain amounts for a specified period of time. Unless the Company has undergone a change in control, Market Cap Research Funding is payable by the Company in cash, in shares of common stock, or in the form of promissory notes, which may be settled in shares of common stock at the election of the Company. In aggregate, the Company has triggered $25.0 million in Market Cap Research Funding and has primarily settled these amounts through the issuance of shares of its common stock. The remaining $100.0 million in Market Cap Research Funding may be triggered when the Company's market capitalization reaches various low-ten to eleven figure dollar amounts or in the event of a Company sale. The Company is not required to make additional Market Cap Research Funding payments if the Company, whether directly or through its affiliates or sublicensees, is not researching, developing, or commercializing products based on or incorporating inventions exclusively licensed to the Company from Broad subject to certain exclusions.

The Sponsored Research Agreement is terminable by each party upon the occurrence of specified bankruptcy events of the other party and otherwise will continue in effect until the remaining Market Cap Research Funding payments are received by Broad and such time as the Company has no further rights of first negotiation for Sponsored Invention Licenses, unless otherwise mutually agreed between the parties.

Broad and Harvard License Agreements

The Company has entered into agreements with Broad and the President and Fellows of Harvard College ("Harvard") to license certain patent rights owned or co-owned by the institutions. The foundational patent rights that were in-licensed by the Company include Cas9-I (as amended and restated to date, the "Cas9-I License Agreement"), Cas12a (formerly known as Cpf1) (as amended, the "Cpf1 License Agreement"), and Cas9-II (as amended, the "Cas9-II License Agreement") (collectively referred to herein as the "License Agreements"). The Company received exclusive, worldwide,

royalty-bearing, sublicensable licenses to certain patent rights to develop and commercialize licensed product and a non-exclusive, worldwide, royalty-bearing sublicensable license under the same patent rights for all other purposes, subject to certain limitations and retained rights. The Company is obligated to use commercially reasonable efforts to research, develop, and commercialize licensed products. The Company is also required to achieve certain development milestones within specified time periods for products covered by the License Agreements, with Broad or Harvard, as applicable, having the right to terminate the License Agreements, on a license agreement-by-license agreement basis, if the Company fails to achieve these milestones within the required time periods. Broad or Harvard may grant licenses under specified circumstances to third parties that wish to develop and commercialize products that target a particular gene that otherwise would fall within the scope of the exclusive licenses granted to the Company, provided that the Company is not, directly or through any of its affiliates, sublicensees, or collaborators, researching, developing, or commercializing a product directed toward the same gene target, or can demonstrate to Broad's and/or Harvard's, as applicable, reasonable satisfaction that the Company is interested in researching, developing, and commercializing a product directed toward the same gene target, that the Company has a commercially reasonable research, development, and commercialization plan to do so, and the Company commences and continues reasonable commercial efforts under such plan. The Company has the right to terminate each of the License Agreements at will with four-months written notice to Broad. Unless terminated earlier, the term of each of the License Agreements will expire on a country-by-country basis, upon the expiration of the last to expire valid claim of the licensed patent rights in such country.

Milestones

In aggregate, the Company may pay up to $14.8 million, $20.0 million, and $3.7 million in clinical and regulatory milestones under the Cas9-I License Agreement, Cpf1 License Agreement, and Cas9-II License Agreement, respectively. In addition, the Company owes aggregate sales milestones totaling $54.0 million, $54.0 million, and $13.5 million under the Cas9-I License Agreement, Cpf1 License Agreement, and Cas9-II License Agreement, respectively. If the licensed product or service prevents or treats a human disease that afflicts fewer than a specified number of patients in the aggregate in the U.S. or a specified number of patients per year in the U.S., the clinical and regulatory milestones reduce to $4.1 million, $5.5 million, and $1.1 million under the Cas9-I License Agreement, Cpf1 License Agreement, and Cas9-II License Agreement, respectively. In addition, the aggregated sales milestones reduce to $36.0 million, $36.0 million, and $9.0 million under the Cas9-I License Agreement, Cpf1 License Agreement, and Cas9-II License Agreement, respectively. Certain clinical and regulatory milestones are subject to a multiplier payout equivalent to a double-digit percentage in the event of a change of control.

Royalties

The Company is required to pay on a product-by-product and country-by-country basis, a mid single-digit percentage royalty on net sales of licensed products made by the Company, its affiliates, or its sublicensees. The royalty percentage depends on the product and service, and whether such licensed product or licensed service is covered by a valid claim. If the Company is legally required to pay royalties to a third party on net sales of the Company's products because such third party holds patent rights that cover such licensed product, then the Company can credit up to a specified percentage of the amount paid to such third party against the royalties due to the institutions. Such credit may not exceed 50% of the applicable royalties paid by the Company to the applicable third party. The Company's obligation to pay royalties will expire on a product-by-product and country-by-country basis upon the later of the expiration of the last to expire valid claim of the patent rights that covers each licensed product or service in each country or the tenth anniversary of the date of the first commercial sale of the licensed product or licensed service.

Licensor Expense Reimbursements

The Company is obligated to reimburse Broad and Harvard for expenses incurred by each of them associated with the prosecution and maintenance of the patent rights that the Company licenses from them pursuant to the Cas9-I License Agreement, including the interference and opposition proceedings involving patents licensed to the Company under the license agreement, and other license agreements between the Company and Broad. As such, the Company anticipates that it has a substantial commitment in connection with these proceedings until such time as these proceedings have been resolved, but the amount of such commitment is not determinable. The Company incurred an aggregate of $11.3 million and $8.2 million in expense during the years ended December 31, 2025 and 2024, respectively, for such reimbursement.

Success Payments

Under the Cpf1 License Agreement and Cas9-II License Agreement, the Company is obligated to make payments ("Success Payments") in the event the Company's market capitalization reaches certain thresholds for a specified period of time, or in the event of a change in control of the Company, if the consideration is in excess of those thresholds. Unless the Company has undergone a change in control, Success Payments are payable by the Company in cash, shares of the Company's common stock, or in the form of promissory notes, which may be settled in shares of common stock at the election of the Company. In the event of a change in control of the Company, the Success Payments are required to be paid in cash. The Success Payments under the Cpf1 License Agreement are triggered when the Company's market capitalization reaches certain amounts ranging from $750.0 million to $10.0 billion for a specified period of time. The Success Payments under the Cas9-II License Agreement are triggered when the Company's market capitalization reaches certain amounts ranging from $1.0 billion to $9.0 billion for a specified period of time. In aggregate, the Company has triggered $25.0 million and $7.5 million of Success Payments under the Cpf1 License Agreement and Cas9-II License Agreement, respectively. The Company has primarily settled these amounts through the issuance of shares of its common stock.

The remaining $100.0 million and $22.5 million in Success Payments under the Cpf1 License Agreement and Cas9-II License Agreement, respectively, are only payable if the market capitalization threshold are met and the Company or any affiliate or sublicensee has at least one product candidate covered by a claim of a patent right licensed to the Company that is or was subject of a clinical trial.

Other Payments

The Company pays nominal annual license fees to the institutions. If the Company sublicenses any of the patent rights to a third party, the institutions have the right to receive sublicense income, which may be offset by the licensor expense reimbursement payments that the Company has made to the institution subject to certain limitations.

Litigation

The Company is not a party to any litigation and did not have contingency reserves established for any litigation liabilities as of December 31, 2025 or 2024.

9. Collaboration Agreements

The Company has entered into multiple collaborations, out-licenses, and strategic alliances with third parties that typically involve payments to or from the Company, including up-front payments, payments for research and development services, option payments, milestone payments, and royalty payments to or from the Company.

Collaboration Revenue

As of December 31, 2025, the Company's contract liabilities were primarily related to the Company's collaboration with BMS. The following table presents changes in the Company's accounts receivable and contract liabilities for the year ended December 31, 2025 (in thousands):

For the year ended December 31, 2025	Balance at December 31, 2024	Additions	Deductions	Balance at December 31, 2025
Accounts receivable	$ 16,266	$ 24,997	$ (26,087)	$ 15,176
Contract liabilities:				
Deferred revenue	$ 60,425	$ —	$ (15,916)	$ 44,509

During the years ended December 31, 2025 and 2024, the Company recognized the following collaboration revenue (in thousands):

Revenue recognized in the period from:	December 31, 2025	December 31, 2024
Amounts included in deferred revenue at the beginning of the period	$ 15,916	$ 8,463
Performance obligations satisfied in previous periods	$ —	$ —

Amendment to BMS Collaboration Agreement

In November 2019, the Company entered into the Second Amended and Restated Collaboration and License Agreement with BMS (the "2019 Collaboration Agreement") to focus on the research, development, and commercialization of autologous and allogenic alpha-beta T cell medicines for the treatment of all diseases, subject to certain exceptions. The Company may develop genome editing tools, specific to a gene target and enzyme combination (or a "Program") that, following the exercise of BMS' option and the Company's grant of a license, BMS may use in its development of gene edited alpha-beta T-cell therapies and certain other T-cell derived from pluripotent stem cells or any other precursor cell for the treatment of all diseases, subject to certain exceptions (the "BMS Field"). To assess the Programs prior to opt-in, the Company granted BMS a non-exclusive perpetual research license in the BMS Field. If BMS exercises their option to the Program, they receive an exclusive, worldwide, development and commercialization license in the BMS Field for a nominal option exercise fee. The BMS License Agreement provided that the Company would manufacture clinical grade materials through a Phase 1 clinical trial if requested by BMS at an incremental cost to be negotiated by the parties. However, BMS has sole responsibility, at its own cost, for the worldwide research, development, manufacturing, and commercialization of its products. They must use commercially reasonable efforts and meet certain regulatory and commercial diligence requirements.

On a product-by-product basis, the Company is eligible to receive up to $27.5 million in development milestones and $107.5 million in regulatory milestones. The Company is also eligible to receive up to an aggregate of $60.0 million for each of the first two licensed products to reach certain sales milestones. The Company is entitled to a high-single digit to low double-digit percentage of royalties on net sales of licensed products, subject to reductions in certain circumstances, through the later of the expiration of the patent(s) related to the licensed products or six years post-first commercial sale of such licensed products.

In March 2024, the Company entered into an amendment ("2024 Amendment") to the 2019 Collaboration Agreement to extend the collaboration until November 2026, with options to extend the collaboration for up to an additional two years, and provided BMS the ability to select up to three new gene targets for research. As of December 31, 2025, one extension option has expired, and BMS retains the right to extend the collaboration for one additional year.

Accounting Assessment

The Company evaluated the 2024 Amendment and concluded that the agreement qualifies as a contract with a customer under ASC 606. The contract modification was accounted for on a prospective basis as if it were a termination of the existing contract and the creation of a new contract since the promised goods and services were distinct from the goods and services that were transferred on or before the effective date of the amendment.

The Company has identified the following performance obligations under the 2024 Amendment: eighteen material rights for development and commercialization licenses for other gene editing tools specific to a gene target and enzyme combination (or a "Program"). The Company also evaluated the (i) the research license, (ii) contract term extensions, (iii) clinical supply arrangement, (iv) participation by employees on the oversight committee, alliance and technology transfer teams and (v) certain intellectual property rights and concluded that none of these met the definition of a performance obligation as a result of the promise being quantitively and qualitatively immaterial in the context of the arrangement or the promise did not convey a material right to BMS. The Company also concluded that there was not an implicit promise to perform research and development services.

At the inception of the 2024 Amendment in March 2024, the total transaction price of the 2024 Amendment was approximately $56.7 million, comprised of the remaining deferred revenue balance that was not recognized pursuant to the 2019 Amended Collaboration Agreement. The Company utilized the most likely amount method to estimate any development and regulatory milestone payments to be received, as well as extension term fees. There were no milestone or

extension term fees included in the initial transaction price. The Company considers the stage of development and the risks associated with the remaining development required to achieve the milestone, as well as whether the achievement of the milestone is outside the control of the Company and BMS. The outstanding milestone payments and extension term fees were fully constrained at the 2024 Amendment inception, as a result of the uncertainty of whether any of the milestones will be achieved or the term would be extended. The Company has determined that any commercial milestones and sales-based royalties will be recognized when the related sales occur. The Company reevaluates the transaction price at each reporting period and as uncertain events are resolved or other changes in circumstances occur.

The Company concluded that the rights and attributes of each of the development and commercialization licenses are identical for both the license granted at inception and the licenses that may be issued in the future upon exercise of the associated option. Each development and commercialization license is differentiated only by the Program to which it relates. The Company has considered the early stage of the science and the uncertainty of success and concluded that the probability of scientific success and opt-in is equal amongst all Programs. In addition, each Program is multi-functional, and a combination of Programs can be utilized in the development of a product candidate. As such, the Company concluded that the standalone selling price of each material right is the same. The Company will recognize the transaction price allocated to each material right when the material right is exercised, lapsed, or expired.

During the years ended December 31, 2025 and 2024, the Company recognized $23.2 million and $18.1 million of revenue, respectively, related to BMS. As of December 31, 2025, the Company recorded $40.5 million of deferred revenue, of which $40.5 million is classified as long-term in the Company's consolidated balance sheets.

Beam Therapeutics License Agreement

In 2018, the Company entered into a license agreement with Beam Therapeutics Inc. (“Beam,” and such agreement, the “Beam License Agreement”). Pursuant to the Beam License Agreement, the Company granted to Beam a worldwide, exclusive (subject to certain exceptions), sublicensable (subject to certain conditions), development and commercialization license under certain intellectual property controlled by the Company for the use of base editing therapies for the treatment of any field of human diseases and conditions, such to certain exceptions. Additionally, the Company granted Beam a non-exclusive research license. Lastly, the Company provided to Beam with an exclusive option to obtain three development and commercialization licenses to additional groups of intellectual property owned or controlled by the Company, on a group-by-group basis, during the specified option period, subject to certain exceptions.

The Company received preferred stock valued at $3.6 million and received a nominal upfront cash payment. The Company subsequently sold its equity investment in Beam following Beam’s initial public offering in 2023. The Company is also eligible to receive additional consideration if Beam exercises its option to obtain additional licenses for a fee ranging from a mid-teen million-dollar amount to a low to mid-eight-digit dollar amount per license, depending on the timing of the option exercise. To the extent that any products are commercialized, the Company would be entitled to receive tiered low single-digit royalty payments, plus any royalties that would be due from the Company to any applicable licensors related to the sale of such licensed products.

Unless earlier terminated by either party pursuant to the terms of the agreement, the Beam 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 expiration of the royalty term with respect to such licensed product in such country. Beam has the right, at its sole discretion, at any time to terminate the Beam License Agreement in its entirety or on a group-by-group of intellectual property basis, upon ninety days written notice to the Company. Upon termination, all rights and licenses granted by the Company will immediately terminate.

Accounting Assessment

The Company identified the following performance obligations (i) the research license and (ii) the initial development and commercialization license. In addition, the Company concluded that the three options for the additional development and commercialization licenses are not discounted and therefore they do not represent material rights.

The total transaction price at the inception of the arrangement was determined to be approximately $3.8 million, consisting of the upfront cash payment and the non-cash value of the preferred shares received by the Company. The consideration associated with the exercise of the option(s) will be accounted for if and when Beam elects to exercise their options. The other forms of consideration, including nominal cost reimbursement for past patent and license fees and sublicense income reimbursement are based on the most-likely amount and were excluded from the initial transaction price

as the most likely amount was estimated to be zero or the amount was otherwise fully constrained due to the significant uncertainties surrounding each payment. The commercial-based milestone reimbursement and the sales-based royalty payments will be recognized when the related sales occur as they were determined to relate predominantly to the licenses granted and therefore have also been excluded from the transaction price. Since both of the performance obligations were delivered at the inception of the arrangement and the licenses were made available for Beam's use and benefit, the Company recognized the total transaction price at the inception of the agreement.

During the years ended December 31, 2025, the Company recognized revenue under the Beam License Agreement of approximately $0.2 million. During the years ended December 31, 2024, the Company recognized revenue under the Beam License Agreement of approximately $0.8 million.

Vertex Pharmaceuticals License Agreement

On December 12, 2023, the Company and Vertex entered into the Vertex License Agreement. Under terms of the agreement, Vertex obtained a non-exclusive license for the Company's Cas9 gene editing technology for *ex vivo* gene editing medicines targeting the *BCL11A* gene in the fields of sickle cell disease and beta thalassemia, including exagamglogene autotemcel (exa-cel). In connection with the Vertex License Agreement, the Company received an upfront payment of $50.0 million. The Company is also eligible to receive annual license fees ranging from $5.0 million to $40.0 million annually through 2034, inclusive of certain fixed and sales-based annual license fee increases, and other contingent fees of $50.0 million.

Accounting Assessment

The Company assessed this arrangement in accordance with Topic 606 and concluded that the contract counterparty, Vertex, is a customer.

The Company identified a single performance obligation, which is the non-exclusive license to certain Cas9-I patents.

The total transaction price at the inception of the arrangement was determined to be $60.0 million, consisting of an upfront cash payment and the first annual license fee installment. The remaining annual license fees and contingent fees represent variable consideration that was evaluated under the most likely amount method, and excluded from the initial transaction price because the amounts were fully constrained. As part of its evaluation of the constraint, the Company considered numerous factors, including that receipt of the annual license fees and contingent fees are outside the control of the Company. They will be recognized when each payment is determined to be probable. The Company will re-evaluate the transaction price in each reporting period, as uncertain events are resolved, or as other changes in circumstances occur. Since the single performance obligation was delivered at the inception of the arrangement and the license was made available for Vertex's use and benefit, the Company recognized the total transaction price at the inception of the agreement.

During the years ended December 31, 2025 and 2024, the Company recognized revenue under the Vertex License Agreement of $10.0 million and $10.0 million, respectively.

10. Preferred Stock

The Company's amended and restated certificate of incorporation authorizes 5,000,000 shares of undesignated preferred stock that may be issued from time to time by the Board of Directors in one or more series. As of December 31, 2025, the Company had no shares of preferred stock issued or outstanding.

11. Common Stock

The voting, dividend, and liquidation rights of the holders of the common stock are subject to and qualified by the rights, powers, and preferences of holders of the preferred stock that may be issued from time to time. The common stock had the following characteristics as of December 31, 2025:

Voting

The holders of shares of common stock are entitled to one vote for each share of common stock held at any meeting of stockholders and at the time of any written action in lieu of a meeting.

Dividends

The holders of shares of common stock are entitled to receive dividends, if and when declared by the Board of Directors. No dividends have been declared or paid by the Company since its inception.

2015 Stock Incentive Plan

The Board of Directors approved, and in May 2025, the Company's stockholders approved the amendment and restatement of the Company's 2015 stock incentive plan (the "2015 Plan"). The 2015 Plan provides for the grant of incentive stock options, nonstatutory stock options, restricted stock awards, restricted stock units, stock appreciation rights, and other stock-based awards. The Company's employees, officers, directors, and consultants and advisors are eligible to receive awards under the 2015 Plan.

The amendment and restatement of the 2015 Plan extended the term of the 2015 Plan for an additional 10 years from the date on which it was approved by the stockholders, eliminated the "evergreen" provisions of the original plan and made certain other updates as further described in the Company's definitive proxy statement for the 2025 annual meeting of stockholders, as filed on April 15, 2025. As of December 31, 2025, there were 12,263,083 shares reserved but unissued under the 2015 Plan.

2015 Employee Stock Purchase Plan

The Company's Board of Directors adopted and the Company's stockholders approved the 2015 employee stock purchase plan (the "2015 ESPP"). The number of shares reserved for issuance under the 2015 ESPP is subject to annual increases, to be added as of the first day of each fiscal year, from January 1, 2017 until, and including, January 1, 2026, in an amount equal to the least of (a) 769,230 shares of common stock, (b) 1% of the total number of shares of common stock outstanding on the first day of the applicable year, and (c) an amount determined by the Board of Directors. The first offering under the 2015 ESPP opened on December 1, 2017. As of December 31, 2025, there were 2,685,252 shares reserved but unissued under the 2015 ESPP. In January 2026, the Board of Directors determined that there should be no increase in shares available under the 2015 ESPP.

Inducement Awards

From time to time the Board of Directors approves inducement awards to certain employees outside of the existing equity compensation plans in connection with such employees commencing employment with the Company. Inducement awards are typically a service-based option and a restricted stock unit and are subject to the Company's typical vesting terms and the employee's continued service relationship with the Company through the applicable vesting dates. As of December 31, 2025, the outstanding inducement awards related to a June 2022 and a July 2023 grant to the Company's Chief Executive Officer and Chief Scientific Officer, respectively, totaling 1,085,709 shares.

Shares Reserved for Future Issuance

	As of December 31,	
	2025	2024
Shares reserved for outstanding stock option awards under the 2013 Plan, as amended	—	114,108
Shares reserved for outstanding stock option awards and restricted stock units under the 2015 Plan	7,977,746	8,395,995
Shares reserved for outstanding inducement stock option award and restricted stock units	1,085,709	1,536,461
Remaining shares reserved, but unissued, for future awards under the 2015 Plan	12,263,083	9,673,426
Remaining shares reserved, but unissued, for future awards under the 2015 ESPP	2,685,252	2,972,026
	24,011,790	22,692,016

12. Stock-Based Compensation

Total compensation cost recognized for all stock-based compensation awards in the consolidated statements of operations was as follows (in thousands):

	Year Ended December 31,	
	2025	2024
Research and development	$ 2,968	$ 8,642
General and administrative	7,032	12,775
Total stock-based compensation expense	$ 10,000	$ 21,417

Restricted Stock Units

The following table summarizes restricted stock units activity for the instruments discussed above as of December 31, 2025 and 2024 is as follows:

	Shares	Weighted Average Grant Date Fair Value Per Share
Unvested restricted stock units as of December 31, 2024	2,326,523	$ 9.78
Issued	1,560	$ 1.26
Vested	(476,731)	$ 10.23
Forfeited	(1,255,622)	$ 9.97
Unvested restricted stock units as of December 31, 2025	595,730	$ 9.11

The expense related to restricted stock units granted for the years ended December 31, 2025 and 2024 was $1.8 million and $5.3 million, respectively.

During the year ended December 31, 2025, there were no restricted stock units granted that contain performance-based vesting provisions. There was no expense recognized related to performance-based vesting of restricted stock units for the year ended December 31, 2025. The expense related to the performance-based vesting of restricted stock units was $3.5 million for the year ended December 31, 2024.

As of December 31, 2025, total unrecognized compensation expense related to unvested restricted stock units was $2.6 million, which the Company expects to recognize over a remaining weighted-average period of 1.4 years.

Stock Options

The following is a summary of stock option activity for the year ended December 31, 2025:

	Shares	Weighted Average Exercise Price	Remaining Contractual Life (years)	Aggregate Intrinsic Value (in thousands)
Outstanding at December 31, 2024	7,656,010	$ 14.70	7.19	$ —
Granted	5,755,642	$ 1.74		
Exercised	(41,430)	$ 1.70		
Expired	(1,900,337)	$ 18.26		
Forfeited	(3,066,191)	$ 5.69		
Outstanding at December 31, 2025	8,403,694	$ 8.37	7.65	$ 1,395
Exercisable at December 31, 2025	3,814,200	$ 14.63	6.10	$ 216

The total intrinsic value of options exercised for the year ended December 31, 2025, was $0.1 million. The total intrinsic value of options exercised for the year ended December 31, 2024 was less than $0.1 million.

Using the Black-Scholes option pricing model, the weighted average fair value of options containing service-based vesting granted during the years ended December 31, 2025 and 2024 was $1.30 and $5.62, respectively. The expense related to options containing service-based vesting was $8.1 million and $12.2 million, for the years ended December 31, 2025 and 2024, respectively.

The fair value of each service-based vesting option issued was estimated at the date of grant using the Black-Scholes option pricing model with the following weighted-average assumptions:

	Year Ended December 31,	
	2025	2024
Expected volatility	84.4 %	75.6 %
Expected option term (in years)	6.23	6.20
Risk free interest rate	4.2 %	4.2 %
Expected dividend yield	—	—

As of December 31, 2025, total unrecognized compensation expense related to stock options was $9.3 million, which the Company expects to recognize over a remaining weighted-average period of 2.22 years.

13. 401(k) Savings Plan

The Company has a defined-contribution savings plan under Section 401(k) of the Internal Revenue Code of 1986, as amended (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 pretax basis. The Company provides a 200% match of employee contributions up to a limit on the Company’s contributions of the lesser of $7,000 or 3% of the employee’s salary. The Company made $1.2 million and $1.7 million, in contributions to the 401(k) Plan for the years ended December 31, 2025 and 2024, respectively.

14. Income Taxes

The Company had no income tax expense due to operating losses incurred for the years ended December 31, 2025 and 2024. During the years ended December 31, 2025 and 2024, the Company did not make any material payments of U.S federal, state, or local income taxes.

A reconciliation of the income tax expense computed using the federal statutory income tax rate to the Company's effective income tax rate is as follows:

	Year Ended December 31, 2025		Year Ended December 31, 2024	
	Amount	Percent	Amount	Percent
U.S. federal statutory income tax rate	$ (33,584)	21.0 %	$ (49,743)	21.0 %
State and local income taxes, net of federal income tax effect[1]	—	— %	—	— %
Effects of changes in tax laws or rates enacted in the current period	—	— %	—	— %
Tax Credits				
Research and development tax credits	(3,541)	2.2 %	(5,072)	2.1 %
Changes in valuation allowance	32,515	(20.3)%	53,592	(22.6)%
Nontaxable or nondeductible items				
Stock-based compensation	4,441	(2.8)%	568	(0.2)%
Other	178	(0.1)%	688	(0.3)%
Changes in unrecognized tax benefits	—	— %	—	— %
Other adjustments	(9)	— %	(33)	— %
Effective income tax rate	—	— %	—	— %

[1] State taxes in New Jersey made up the majority (greater than 50 percent) of the tax effect in this category

The components of the Company's deferred tax assets and liabilities consist of the following at December 31, 2025 and 2024 (in thousands):

	Year Ended December 31,	
	2025	2024
Deferred tax assets:		
Net operating loss carryforwards	$ 231,981	$ 170,770
Tax credit carryforwards	35,947	31,766
Accrued expenses	3,475	6,763
Capitalized patent costs	48,075	52,178
Capitalized research and development expenses, net Sec. 174	64,678	84,703
Capitalized research and development expenses, net Sec. 59(e)	11,841	—
Lease liabilities	4,763	9,134
Deferred revenue	11,712	15,755
Depreciation and amortization	1,169	978
Stock compensation	7,377	10,431
Other	15,421	14,976
Total deferred tax assets	436,439	397,454
Less valuation allowance	(432,196)	(388,967)
Net deferred tax assets	4,243	8,487
Deferred tax liabilities:	(4,243)	(8,487)
Right-of-use assets	(4,243)	(8,487)
Net deferred taxes	$ —	$ —

For taxable years beginning after December 31, 2021, the Tax Cuts and Jobs Act (the "Tax Act") eliminated the option to deduct research and development expenditures in the current year and required taxpayers to capitalize such expenses pursuant to the Internal Revenue Code of 1986, as amended (the "IRC"), Section 174. For taxable years beginning after December 31, 2024, the One, Big, Beautiful Bill Act (the "OBBA") eliminates the requirement to capitalize domestic research and development expenditures effected by the Tax Act, but the requirement to capitalize foreign research and development expenditures was not revised. As a result of these provisions, deferred tax assets related to capitalized research expenses pursuant to IRC Section 174 were $64.7 million for the year ended December 31, 2025. Under IRC Section 59(e) taxpayers may elect to capitalize domestic research and development expenditures and amortize these expenses over a ten-year period. As such, deferred tax assets related to capitalized research expenses pursuant to IRC Section 59(e) were $11.8 million for the year ended December 31, 2025.

The Company has incurred net operating losses ("NOLs") since inception. The Company did not have any international operations as of December 31, 2025. As such, net loss before taxes for the years ended December 31, 2025 and 2024 of $160.1 million and $237.1 million, respectively, is domestic. At December 31, 2025 and 2024, the Company had federal NOL carryforwards of $851.9 million and $612.8 million, respectively. Of the amount as of December 31, 2025, $777.1 million will carryforward indefinitely, while $74.8 million will begin expiring in 2035 and will continue to expire through 2037. As of December 31, 2025 and 2024, the Company also had state NOL carryforwards of approximately $899.5 million and $710.5 million, respectively, which may be available to offset future income tax liabilities and will begin expiring in 2035 and will continue to expire through 2045.

Under the provisions of the IRC, the NOL and research and development tax credit carryforwards are subject to review and possible adjustment by the Internal Revenue Service ("IRS") and state tax authorities. NOL and research and development tax credit carryforwards may become subject to an annual limitation in the event of certain cumulative changes in the ownership interest of significant shareholders over a three-year period in excess of 50%, as defined under Sections 382 and 383 of the IRC, respectively, as well as other similar state provisions. The Company conducted an analysis under Section 382 to determine if historical changes in ownership through June 30, 2024 would limit or otherwise restrict its ability to utilize its NOL and research and development tax credit carryforwards. As a result of this analysis, the Company does not believe there are any significant limitations on its ability to utilize these carryforwards. However, future changes in ownership occurring after June 30, 2024 could affect the limitation in future years, and any limitation may result in expiration of a portion of the NOL or research and development tax credit carryforwards before utilization.

The Company has evaluated the positive and negative evidence bearing upon the realizability of its deferred tax assets, which is principally comprised of NOL carryforwards, research and development tax credit carryforwards, and capitalized license and patent costs. The Company has determined that it is more likely than not that the Company will not recognize the benefits of its federal and state deferred tax assets, and as a result, a valuation allowance of $432.2 million and $389.0 million has been established at December 31, 2025 and 2024, respectively. The increase in the valuation allowance of $43.2 million for the year ended December 31, 2025 was primarily due to current period pre-tax losses incurred and research tax credits generated.

The Company applies ASC 740 related to accounting for uncertainty in income taxes. The Company's reserves related to income taxes are based on a determination of whether, and how much of, a tax benefit taken by the Company in its tax filings or positions is more likely than not to be realized following resolution of any potential contingencies present related to the tax benefit.

The following table summarizes the activity related to the Company's gross unrecognized tax benefits at the beginning and end of the years ended December 31, 2025 and 2024 (in thousands):

	Gross Unrecognized Tax Benefits
Balance as of December 31, 2023	**$ 13,659**
Gross increases for tax positions related to current year	2,020
Gross increases for tax positions related to prior year	—
Balance as of December 31, 2024	**$ 15,679**
Gross increases for tax positions related to current year	1,420
Gross increases for tax positions related to prior year	—
Balance as of December 31, 2025	$ 17,099

At December 31, 2025 and 2024, the Company had unrecognized tax benefits of $17.1 million and $15.7 million, respectively. The Company will recognize interest and penalties related to uncertain tax positions in income tax expense. As of December 31, 2025 and 2024, the Company had no accrued interest or penalties related to uncertain tax positions and no amounts have been recognized in the Company's statement of operations. The Company does not anticipate a material change to unrecognized tax benefits in the next twelve months.

The Company has not as of yet conducted a study of its research and development tax credit carryforwards. This study may result in an adjustment to the Company's research and development tax credit carryforwards; however, until a study is completed and any adjustment is known, no amounts are being presented as an uncertain tax position. A full valuation allowance has been provided against the Company's research and development tax credits, and if an adjustment is required, this adjustment would be offset by an adjustment to the valuation allowance. Thus, there would be no impact to the consolidated balance sheets or statements of operations if an adjustment were required.

The Company files income tax returns as prescribed by the tax laws of the jurisdictions in which it operates. In the normal course of business, the Company is subject to examination by federal and state jurisdictions, where applicable. Since the Company is in a loss carryforward position, the Company is generally subject to examination by the U.S. federal, state, and local income tax authorities for all tax years in which an NOL carryforward is available. There is a 2023 IRS audit in process.

15. Net Loss per Share

Basic net loss per common share is calculated by dividing the net loss attributable to common stockholders by the weighted average number of shares of common stock outstanding during the period, without consideration for potentially dilutive securities. Diluted net loss per share is computed by dividing the net loss attributable to common stockholders by the weighted average number of shares of common stock and potentially dilutive securities outstanding for the period determined using the treasury stock and if converted methods. Contingently issuable shares are included in the calculation of basic loss per share as of the beginning of the period in which all the necessary conditions have been satisfied. Contingently issuable shares are included in diluted loss per share based on the number of shares, if any, that would be issuable under the terms of the arrangement if the end of the reporting period was the end of the contingency period, if the results are dilutive.

For purposes of the diluted net loss per share calculation, unvested restricted stock units and outstanding stock options are considered to be common stock equivalents, but they were excluded from the Company's calculation of diluted net loss per share allocable to common stockholders because their inclusion would have been anti-dilutive. Therefore, basic and diluted net loss per share applicable to common stockholders were the same for all periods presented.

The following common stock equivalents were excluded from the calculation of diluted net loss per share allocable to common stockholders because their inclusion would have been anti-dilutive:

	As of December 31,	
	2025	2024
Unvested restricted stock units	595,730	2,326,523
Outstanding stock options	8,403,694	7,656,010
Total	8,999,424	9,982,533

16. Debt

Liability for the Sale of Future Revenues

The following table presents the changes in the liability related to the sale of future revenues under the DRI Agreement as of December 31, 2025 (in thousands):

	December 31, 2025
Liability for the sale of future revenues, net as of December 31, 2024	$ 57,434
Payments for sale of future revenues	(5,000)
Non-cash interest expense associated with sale of future revenues	5,781
Amortization of issuance costs	390
Liability for the sale of future revenues, net as of December 31, 2025	$ 58,605

17. Restructuring and Impairment Charges

On December 11, 2024, the Company's Board of Directors approved the discontinuation of the clinical development of the Company's renizgamglogene autogedtemcel ("reni-cel") program to treat sickle cell disease and transfusion-dependent beta thalassemia (the "Discontinuation"). As a result of the Discontinuation, the Company ceased activities towards the filing of a biologic license application and potential commercialization of reni-cel. In connection with the Discontinuation, the Company's Board of Directors also approved a reduction in the Company's employee workforce by approximately 180 positions, or approximately 65% (the "Reduction").

The Company incurred the following restructuring and impairment charges in connection with the Discontinuation and Reduction for the years ended December 31, 2025 and 2024, which are recorded in the consolidated statements of operations (in thousands):

	Year Ended December 31,	
	2025	2024
Employee termination benefits	$ 3,723	$ 10,475
Costs for ongoing contracts and terminated contracts	46,645	1,757
Acceleration of expense for change in useful life estimate and lease termination charges	6,548	—
Impairment charges	3,758	—
Total restructuring and impairment charges	$ 60,674	$ 12,232

The actions associated with the Discontinuation and Reduction commenced in December 2024 and were substantially completed by December 31, 2025.

Employee Termination Benefits

Employees affected by the Reduction received involuntary termination benefits pursuant to either a one-time benefit or arrangement or salary continuation for a set period of time in accordance with the Company's Amended and Restated Severance Benefit Plan (the "Benefit Plan"). For employees who were notified of their termination in December 2024 and had no requirements to provide future services or were subject to the Benefit Plan, the Company recognized the liability for the termination benefits in full at fair value in the fourth quarter of 2024. For employees who are required to render services beyond a minimum retention period to receive their one-time termination benefits or salary continuation, the Company recognized the termination benefits ratably over their future service periods. The service periods began in December 2024 and were completed in 2025.

The following table shows the liability related to employee termination benefits as of December 31, 2025 (in thousands):

	Employee Termination Benefits
Accrued employee termination benefits as of December 31, 2024	$ 10,475
Employee termination benefits charges incurred during period	3,723
Amounts paid or otherwise settled during the period	(12,643)
Accrued employee termination benefits as of December 31, 2025	$ 1,555

Costs for Ongoing Contracts and Terminated Contracts

The Discontinuation resulted in contract termination costs from vendor contracts before the end of their term, as well as costs that continue to be incurred under certain contracts with no future economic benefit to the Company. In accordance with ASC Topic 420, *Exit or Disposal Cost Obligations*, the Company recognized these unavoidable contract costs when incurred for terminated contracts or at the cease-use date as it relates to contract costs that continue to be incurred with no future economic benefit.

The following table shows the liability related to costs for ongoing contracts and contract termination costs as of December 31, 2025 (in thousands):

	Costs for Ongoing Contracts and Contract Termination Costs
Accrued contract costs as of December 31, 2024	$ 1,757
Contract costs incurred during the period	46,645
Amounts paid or otherwise settled during the period	(38,206)
Accrued contract costs as of December 31, 2025	$ 10,196

At December 31, 2025, $2.4 million of accrued ongoing contract costs were included in other non-current liabilities in the consolidated balance sheet.

These costs are subject to estimation based on the Company's expectation of the costs that will continue to be incurred on the contracts, as well as negotiation of contract changes and terminations with its vendors. Changes in this estimate will be made as the information becomes determinable and could be material.

Impairment and Accelerated Depreciation Charges

In conjunction with the Discontinuation, the Company committed to a plan to actively sell specific assets within its asset group, primarily certain of its laboratory and manufacturing equipment. The Company recorded a $3.8 million impairment charge during the year ended December 31, 2025 related to the sale of the specified assets. The sale was completed in April 2025.

Additionally, the Company abandoned certain other leasehold improvements, software, and right-of-use assets in the second quarter of 2025, and as a result, the Company accelerated depreciation and rent expense and recorded the following in the consolidated statements of operations (in thousands):

	Year Ended December 31,	
	2025	2024
Accelerated depreciation related to leasehold improvements and software	$ 1,790	$ —
Charges related to termination of lease	4,758	—
Total	$ 6,548	$ —

18. Segments

Operating segments are identified as components of an enterprise about which separate discrete financial information is available for evaluation by the CODM or decision-making group in making decisions on how to allocate resources and assess performance. The Company’s CODM is its CEO. The CEO views the Company’s operations and manages the Company’s business as one operating segment, which is the business of developing and commercializing gene editing technology.

The Company’s CEO manages and allocates resources to the operations of the Company on a total company basis by assessing the overall level of resources available and how to best deploy these resources across functions and research and development projects that are in line with our long-term company-wide strategic goals. In making these decisions, the

Company's CEO uses consolidated financial information for purposes of evaluating performance, forecasting future period financial results, allocating resources and setting incentive targets. The CODM performs this assessment based on the Company's consolidated net loss. Through this analysis, the CODM assesses performance by comparing actual consolidated net loss versus the budget, and then decides how to allocate resources to invest in the Company's research and development programs. The measure of segment assets is reported in the consolidated balance sheets as total assets.

The following table contains additional information on our consolidated revenue and net loss, including significant segment expenses (in thousands):

	Year Ended December 31,	
	2025	2024
Collaboration and other research and development revenues	$ 40,520	$ 32,314
Operating expenses:		
Research and development[1]		
Employee related expenses	33,076	61,136
External research and development expenses	71,676	79,900
Facility expenses	20,519	26,430
Stock-based compensation expenses	2,968	8,642
Sublicense and license fees	7,440	18,953
Other expenses[3]	12,106	12,768
General and administrative[2]		
Employee related expenses	13,041	24,335
Professional service expenses	8,673	14,358
Intellectual property and patent related fees	16,838	14,016
Stock-based compensation expenses	7,032	12,775
Facility and other expenses[4]	7,161	10,153
Interest expense related to sale of future revenues	(6,171)	(2,190)
Other expense, net	(2,189)	(3)
Interest income, net	8,310	16,252
Net loss	$ (160,060)	$ (237,093)

[1] The years ended December 31, 2025 and 2024 include $57,832 and $8,582 of restructuring charges, respectively.

[2] The years ended December 31, 2025 and 2024 include $2,842 and $3,650 of restructuring charges, respectively

[3] Other expenses primarily consists of consultant fees and office expenses

[4] Facility and other expenses primarily consists of rent expense, insurance premiums, software licenses and office expenses

For the year ended December 31, 2025, there were three customers for which revenues amount to 10% or more of the Company's consolidated collaboration and other research and development revenues, representing $23.2 million, $10.0 million, and $4.2 million of the Company's consolidated collaboration and other research and development revenues. For the year ended December 31, 2024, there were two customers for which revenues amounts to 10% or more of the Company's consolidated collaboration and other research and development revenues, representing $18.1 million and $10.0 million of the Company's consolidated collaboration and other research and development revenues. For the years ended December 31, 2025 and 2024, 100% and 100%, respectively, of the Company's consolidated collaboration and other research and development revenues was attributed to the U.S.

19. Subsequent Events

The Company evaluated subsequent events from the audited balance sheet date of December 31, 2025 through the issuance of the financial statements to provide additional evidence relative to certain estimates or to identify matters that require additional disclosure. The Company did not identify any subsequent events requiring further disclosure.

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 the effectiveness of our disclosure controls and procedures as of December 31, 2025. The term "disclosure controls and procedures," as defined in Rules 13a-15(e) and 15d-15(e) under the Securities Exchange Act of 1934 (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 Securities and Exchange Commission'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. Our management recognizes that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving their objectives and our management necessarily applies its judgment in evaluating the cost-benefit relationship of possible controls and procedures. Based on the evaluation of our disclosure controls and procedures as of December 31, 2025, our principal executive officer and principal financial officer concluded that, as of such date, our disclosure controls and procedures 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 as defined in Rules 13a-15(f) and 15d-15(f) under the Exchange Act. Our internal control over financial reporting is a process designed to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with general accepted accounting principles. Because of its inherent limitations, internal control over financial reporting may not prevent or detect misstatements. Projections of any evaluation of effectiveness to future periods are subject to the risk that controls may become inadequate because of changes in conditions, or that the degree of compliance with the policies or procedures may deteriorate.

Under the supervision and with the participation of our management, including our principal executive officer and principal financial officer, we conducted an evaluation of the effectiveness of our internal control over financial reporting based on the 2013 framework in Internal Control–Integrated Framework issued by the Committee of Sponsoring Organizations of the Treadway Commission. Based on our evaluation under that framework, our management concluded that our internal control over financial reporting was effective as of December 31, 2025.

As a "smaller reporting company," as defined by Rule 12b-2 under the Exchange Act, we are not required to provide an attestation report of our registered public accounting firm on the effectiveness of our internal control over financial reporting.

Changes in Internal Control over Financial Reporting

No change in our internal control over financial reporting (as defined in Rules 13a-15(f) and 15d-15(f) under the Exchange Act) occurred during our fiscal quarter ended December 31, 2025 that has materially affected, or is reasonably likely to materially affect, our internal control over financial reporting.

Item 9B. Other Information.

Director and Officer Trading Arrangements

A portion of the compensation of our directors and officers (as defined in Rule 16a-1(f) under the Securities Exchange Act of 1934 (the "Exchange Act")) is in the form of equity awards and, from time to time, directors and officers may engage in open-market transactions with respect to the securities acquired pursuant to such equity awards or other of our securities, including to satisfy tax withholding obligations when equity awards vest or are exercised, and for diversification or other personal reasons.

Transactions in our securities by directors and officers are required to be made in accordance with our insider trading policy, which requires that the transactions be in accordance with applicable U.S. federal securities laws that prohibit trading while in possession of material nonpublic information. Rule 10b5-1 under the Exchange Act provides an affirmative defense that enables directors and officers to prearrange transactions in our securities in a manner that avoids concerns about initiating transactions while in possession of material nonpublic information.

None of our directors or officers adopted or terminated a Rule 10b5-1 trading arrangement or a non-Rule 10b5-1 trading arrangement (as defined in Item 408(c) of Regulation S-K) during the fourth quarter of 2025.

Item 9C. Disclosure Regarding Foreign Jurisdictions that Prevent Inspections.

Not applicable.

PART III

Item 10. Directors, Executive Officers and Corporate Governance.

Except to the extent provided below, the information required by this Item 10 will be included in the section captioned "Corporate Governance" and the subsections thereof, "Nominees for Election as Class I Directors," "Directors Continuing in Office," "Executive Officers Who Are Not Directors," "Delinquent Section 16(a) Reports," if applicable, and "Insider Trading Policy," in our definitive proxy statement to be filed with the Securities and Exchange Commission ("SEC") with respect to our 2026 Annual Meeting of Stockholders, which information is incorporated herein by reference.

We have adopted a written code of business conduct and ethics that applies to our directors, officers, and employees, including our principal executive officer, principal financial officer, principal accounting officer or controller, or persons performing similar functions. A copy of the code is posted on the Corporate Governance section of our website, which is located at www.editasmedicine.com. If we make any substantive amendments to, or grant any waivers from, the code of business conduct and ethics for any officer or director, we will disclose the nature of such amendment or waiver on our website or in a current report on Form 8-K. We will provide any person, without charge, a copy of such Code of Business Conduct and Ethics upon written request, which may be mailed to 11 Hurley Street, Cambridge, MA 02141, Attn: Corporate Secretary.

We have adopted an Insider Trading Policy governing the purchase and sale and/or dispositions of our securities by our directors, officers, employees and other covered persons. We believe the Insider Trading Policy is reasonably designed to promote compliance with insider trading laws, rules and regulations, and Nasdaq listing standards. A copy of our Insider Trading Policy is filed as Exhibit 19.1 to this Annual Report on Form 10-K.

Item 11. Executive Compensation.

The information required by this Item 11 will be included in the section captioned "Executive Compensation" in our definitive proxy statement to be filed with the SEC with respect to our 2026 Annual Meeting of Stockholders, which information (other than the information required by Item 402(v) of Regulation S-K) 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 "Principal Stockholders" and "Securities Authorized for Issuance Under Our Equity Compensation Plans" in our definitive proxy statement to be filed with the SEC with respect to our 2026 Annual Meeting of Stockholders, which information 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 "Transactions with Related Persons" and "Director Independence" in our definitive proxy statement to be filed with the SEC with respect to our 2026 Annual Meeting of Stockholders, which information is incorporated herein by reference.

Item 14. Principal Accountant Fees and Services.

The information required by this Item 14 will be included in the sections captioned "Audit Fees" 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, which information is incorporated herein by reference.

PART IV

Item 15. Exhibits and Financial Statement Schedules.

(1) Financial Statements

Our consolidated financial statements are set forth in Part II, Item 8 “Financial Statements and Supplementary Data” of this Annual Report on Form 10-K and are incorporated herein by reference.

(2) Financial Statement Schedules

Schedules have been omitted since they are either not required or not applicable or the information is otherwise included herein.

(3) Exhibits

The exhibits filed as part of this Annual Report on Form 10-K are listed in the following Exhibit Index.

EXHIBIT INDEX

Exhibit Number	Description of Exhibit	Incorporated by Reference				Filed Herewith
		Form	File No.	Date of Filing	Exhibit Number	
3.1	Restated Certificate of Incorporation of the Registrant	8-K	001-37687	6/2/2025	3.1	
3.2	Amended and Restated By-laws of the Registrant	8-K	001-37687	12/10/2024	3.1	
4.1	Specimen Stock Certificate evidencing the shares of common stock	S-1	333-208856	1/4/2016	4.1	
4.2	Description of Registrant’s Securities Registered Pursuant to Section 12 of the Securities Exchange Act of 1934					X
10.1+	2013 Stock Incentive Plan, as amended	S-1	333-208856	1/4/2016	10.5	
10.2+	Form of Incentive Stock Option Agreement under 2013 Stock Incentive Plan, as amended	S-1	333-208856	1/4/2016	10.6	
10.3+	Form of Nonstatutory Stock Option Agreement under 2013 Stock Incentive Plan, as amended	S-1	333-208856	1/4/2016	10.7	
10.4+	Form of Early Exercise Nonstatutory Stock Option Agreement under 2013 Stock Incentive Plan, as amended	S-1	333-208856	1/4/2016	10.8	
10.5+	Form of Restricted Stock Agreement under 2013 Stock Incentive Plan, as amended	S-1	333-208856	1/4/2016	10.9	
10.6+	Amended and Restated 2015 Stock Incentive Plan	8-K	333-208856	6/2/2025	10.1	
10.7+	Form of Incentive Stock Option Agreement under 2015 Stock Incentive Plan	10-K	001-37687	2/28/2024	10.7	
10.8+	Form of Nonstatutory Stock Option Agreement under 2015 Stock Incentive Plan	10-K	001-37687	2/28/2024	10.8	
10.9+	Form of Restricted Stock Agreement under 2015 Stock Incentive Plan	10-K	001-37687	2/28/2024	10.9	
10.10+	Form of Restricted Stock Unit Award Agreement under the 2015 Stock Incentive Plan	10-K	001-37687	2/28/2024	10.10	

Exhibit Number	Description of Exhibit	Incorporated by Reference				Filed Herewith
		Form	File No.	Date of Filing	Exhibit Number	
10.11+	Employment Offer Letter, dated April 13, 2022, between the Registrant and Gilmore O'Neill	10-Q	001-37687	8/3/2022	10.1	
10.12+	Employment Offer Letter, dated July 7, 2022, between the Registrant and Amy Parison					X
10.13+	Promotion Letter, dated March 19, 2025, between the Registrant and Amy Parison					X
10.14+	Employment Offer Letter, dated July 3, 2023, between the Registrant and Linda C. Burkly	10-Q	001-37687	8/2/2023	10.3	
10.15+	Form of Inducement Stock Option Agreement for the Registrant's executive officers	10-K	001-37687	2/28/2024	10.19	
10.16+	Form of Inducement Restricted Stock Unit Award Agreement for the Registrant's executive officers	10-K	001-37687	2/28/2024	10.20	
10.17†	Amended and Restated Cas9-I License Agreement, dated December 16, 2016, among the Registrant, the President and Fellows of Harvard College ("Harvard"), and the Broad Institute, Inc. (the "Broad")	8-K	001-37687	1/23/2017	99.2	
10.18	Amendment No.1 to Amended and Restated Cas9-I License Agreement, by and among Editas Medicine, Inc., Harvard, and Broad, dated March 3, 2017	8-K	001-37687	3/7/2017	99.1	
10.19*	Second Amended and Restated License and Collaboration Agreement, dated November 11, 2019, between the Registrant and Juno Therapeutics, Inc. ("Juno")	10-K	001-37687	2/26/2020	10.20	
10.20*	First Amendment, dated March 21, 2024, to the Second Amended and Restated Collaboration and License Agreement, between the Registrant and Juno	10-Q	001-37687	5/8/2024	10.2	
10.21*	License and Agreement, dated November 11, 2019, between the Registrant and Juno	10-K	001-37687	2/26/2020	10.21	
10.22†	Sponsored Research Agreement, dated June 7, 2018, between the Registrant and Broad	10-Q/A	001-37687	10/23/2018	10.2	
10.23*	First Amendment to Sponsored Research Agreement, dated January 11, 2021, between the Registrant and Broad	10-K	001-37687	2/26/2021	10.24	
10.24*	Purchase and Sale Agreement, dated October 3, 2024, between the Registrant and DRI Healthcare Acquisitions LP	10.K	001-37687	3/5/2025	10.25	
10.25+	Summary of Director Compensation Program					X
10.26+	2015 Employee Stock Purchase Plan	S-1	333-208856	1/4/2016	10.25	
10.27+	Amended and Restated Severance Benefits Plan	8-K	001-37687	11/22/2023	10.1	
10.28	Form of Indemnification Agreement between the Registrant and each of its directors and executive officers	S-1	333-208856	1/4/2016	10.28	
10.29	Lease Agreement, dated February 12, 2016, between Registrant and ARE-MA Region No. 55 Exchange Holding LLC	8-K	001-37687	2/19/2016	99.1	

Exhibit Number	Description of Exhibit	Incorporated by Reference				Filed Herewith
		Form	File No.	Date of Filing	Exhibit Number	
10.30	First Amendment to Lease Agreement, dated November 15, 2022 between Registrant and ARE-MA Region No. 55, LLC	10-K	001-37687	2/22/2023	10.32	
10.32†	Cpf1 License Agreement, dated as of December 16, 2016, by and between the Registrant and Broad	8-K	001-37687	1/23/2017	99.1	
10.32†	Cas9-II License Agreement, dated as of December 16, 2016, by and between the Registrant and Broad	8-K	001-37687	1/23/2017	99.3	
10.33*	Omnibus Amendment, dated as of January 11, 2021, by and between the Registrant and Broad	10-K	001-37687	2/26/2021	10.32	
10.34	Omnibus Amendment, dated as of February 5, 2024, by and among the Registrant, Broad and Harvard	10-K	001-37687	2/28/2024	10.36	
10.35*	Letter Agreement, dated as of November 18, 2019, by and among, the Registrant, Broad and Harvard	10-K	001-37687	2/26/2020	10.30	
10.36*	Letter Agreement, dated as of December 16, 2019, by and among, the Registrant, Broad and Harvard	10-K	001-37687	2/26/2020	10.31	
10.37	Common Stock Sales Agreement, dated as of May 14, 2021, by and between the Company and TD Securities (USA) LLC, as amended on February 28, 2024 and March 5, 2025	S-3	333-277471	3/5/2025	1.2	
19.1	Insider Trading Policy	10-K	001-37687	3/5/2025	19.1	
21.1	Subsidiaries of the Registrant	10-K	001-37687	2/22/2023	21.1	
23.1	Consent of Ernst & Young					X
31.1	Rule 13a-14(a) Certification of Principal Executive Officer					X
31.2	Rule 13a-14(a) Certification of Principal Financial Officer					X
32.1	Certification of Principal Executive Officer and Principal Financial Officer pursuant to 18 U.S.C. §1350					X
97	Dodd-Frank Compensation Recovery Policy	10-K	001-37687	2/28/2024	97	
101	The following financial statements from the Company's Annual Report on Form 10-K for the year ended December 31, 2025, formatted in Inline XBRL (eXtensible Business Reporting Language): (i) Consolidated Balance Sheets, (ii) Consolidated Statements of Operations, (iii) Consolidated Statements of Comprehensive Loss, (iv) Consolidated Statement of Stockholders' Equity, (v) Consolidated Statements of Cash Flows and (vi) Notes to Consolidated Financial Statements, tagged as blocks of text and including detailed tags.					
104	The cover page from the Company's Annual Report on Form 10-K for the year ended December 31, 2025, formatted in Inline XBRL.					

† Confidential treatment has been granted as to certain portions, which portions have been omitted and filed separately with the Securities and Exchange Commission.

* Portions of this exhibit have been omitted pursuant to Item 601(b)(10)(iv) of Regulation S-K. Certain portions of this exhibit have been omitted because they are not material and are information of the type that the registrant customarily and actually treats as private or confidential.

+ Management contract or compensatory plan or arrangement.

Item 16. Form 10-K Summary.

None.

SIGNATURES

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

EDITAS MEDICINE, INC.

Dated: March 9, 2026

By: /s/ Gilmore O'Neill
Gilmore O'Neill
Principal Executive Officer

SIGNATURES

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

Signature	Title	Date
/s/ Gilmore O'Neill Gilmore O'Neill, M.B., M.M.Sc.	President and Chief Executive Officer, Director (principal executive officer)	March 9, 2026
/s/ Amy Parison Amy Parison	Chief Financial Officer (principal financial and accounting officer)	March 9, 2026
/s/ Jessica Hopfield Jessica Hopfield, Ph.D.	Chair of the Board	March 9, 2026
/s/ Bernadette Connaughton Bernadette Connaughton	Director	March 9, 2026
/s/ Andrew Hirsch Andrew Hirsch	Director	March 9, 2026
/s/ Elliott Levy Elliott Levy, M.D.	Director	March 9, 2026
/s/ David Scadden David Scadden, M.D.	Director	March 9, 2026

DESCRIPTION OF THE REGISTRANT'S SECURITIES REGISTERED PURSUANT TO SECTION 12 OF THE SECURITIES EXCHANGE ACT OF 1934

As of December 31, 2025, Editas Medicine, Inc. ("we" or "us") had one class of securities registered under Section 12 of the Securities Exchange Act of 1934, as amended: our common stock, par value $0.0001 per share.

Description of Capital Stock

The following description of our capital stock is intended as a summary only and therefore is not a complete description of our capital stock. This description is based upon, and is qualified by reference to, our restated certificate of incorporation, our amended and restated by-laws and applicable provisions of Delaware corporate law. You should read our restated certificate of incorporation and amended and restated by-laws, which are filed as exhibits to our most recent Annual Report on Form 10-K.

Our authorized capital stock consists of 390,000,000 shares of common stock, par value $0.0001 per share, and 5,000,000 shares of preferred stock, par value $0.0001 per share.

Common Stock

Annual Meeting. Annual meetings of our stockholders are held on the date designated in accordance with our amended and restated by-laws. Written notice must be mailed to each stockholder entitled to vote not less than ten nor more than 60 days before the date of the meeting. The presence in person or by proxy of the holders of record of a majority of our issued and outstanding shares entitled to vote at such meeting constitutes a quorum for the transaction of business at meetings of the stockholders. Special meetings of the stockholders may be called for any purpose, and may be called only by the board of directors, the chairman of the board, or the chief executive officer, and business to be transacted at any special meeting is limited to matters related to the purpose or purposes stated in the notice of the meeting. Except as may be otherwise provided by applicable law, our restated certificate of incorporation, or our amended and restated by-laws, when a quorum is present at any meeting, any matter other than the election of directors to be voted upon by the stockholders at such meeting shall be decided by the vote of the holders of shares of stock having a majority in voting power of the votes cast by the holders of all of the shares of stock present or represented at the meeting and voting affirmatively or negatively on such matter, and any election by stockholders of directors shall be determined by a plurality of the votes cast by the stockholders entitled to vote on the election.

Voting Rights. Each holder of common stock is entitled to one vote for each share held on all matters to be voted upon by stockholders.

Dividends. The holders of common stock, after any preferences of holders of any preferred stock, are entitled to proportionately receive dividends when and if declared by the board of directors out of legally available funds, subject to any preferential dividend or other rights of any series of preferred stock that we may designate and issue in the future.

Liquidation and Dissolution. If we are liquidated or dissolved, the holders of the common stock will be entitled to share in our assets available for distribution to stockholders in proportion

to the amount of common stock they own. The amount available for common stockholders is calculated after payment of all debts and other liabilities. Holders of any preferred stock will receive a preferential share of our assets before the holders of the common stock receive any assets.

Other Rights. Holders of the common stock have no right to:

- convert the stock into any other security;
- have the stock redeemed;
- purchase additional stock; or
- maintain their proportionate ownership interest.

The common stock does not have cumulative voting rights. Holders of shares of the common stock are not required to make additional capital contributions. The rights, preferences and privileges of holders of common stock are subject to and may be adversely affected by the rights of the holders of shares of any series of preferred stock that we may designate and issue in the future.

Provisions of Our Certificate of Incorporation and By-laws and Delaware Law That May Have Anti-Takeover Effects

Delaware law, our restated certificate of incorporation, and our amended and restated bylaws contain provisions that could have the effect of delaying, deferring or discouraging another party from acquiring control of us. These provisions, which are summarized below, are expected to discourage coercive takeover practices and inadequate takeover bids. These provisions are also designed to encourage persons seeking to acquire control of us to first negotiate with our board of directors.

Staggered Board; Removal of Directors. Our restated certificate of incorporation and amended and restated bylaws divide our board of directors into three classes with staggered three-year terms. In addition, a director may be removed only for cause and only by the affirmative vote of the holders of at least 75% of the votes that all of our stockholders would be entitled to cast in an annual election of directors. Any vacancy on our board of directors, including a vacancy resulting from an enlargement of our board of directors, may be filled only by vote of a majority of our directors then in office. The classification of our board of directors and the limitations on the removal of directors and filling of vacancies could make it more difficult for a third party to acquire, or discourage a third party from seeking to acquire, control of our company.

Stockholder Action by Written Consent; Special Meetings. Our restated certificate of incorporation provides that any action required or permitted to be taken by our stockholders must be effected at a duly called annual or special meeting of such holders and may not be effected by any consent in writing by such holders. Our restated certificate of incorporation and amended and restated bylaws also provide that, except as otherwise required by law, special meetings of our stockholders can only be called by the chairman of our board of directors, our Chief Executive Officer, or our board of directors.

Advance Notice Requirements for Stockholder Proposals. Our amended and restated bylaws establish an advance notice procedure for stockholder proposals to be brought before an annual meeting of stockholders, including proposed nominations of persons for election to our board of directors. Stockholders at an annual meeting may only consider proposals or nominations specified in the notice of meeting or brought before the meeting by or at the direction of our board of directors or by a stockholder of record on the record date for the meeting who is entitled to vote at the meeting and who has delivered timely written notice in proper form to our secretary of the stockholder's intention to bring such business before the meeting. These provisions could have the effect of delaying until the next stockholder meeting stockholder actions that are favored by the holders of a majority of our outstanding voting securities.

Delaware Business Combination Statute. We are subject to Section 203 of the DGCL ("Section 203"), which prohibits a Delaware corporation from engaging in business combinations with an interested stockholder. An interested stockholder is generally defined as an entity or person beneficially owning 15% or more of the outstanding voting stock of the corporation or any entity or person affiliated with or controlling or controlled by such entity or person ("interested stockholder"). Section 203 provides that an interested stockholder may not engage in business combinations with the corporation for a period of three years after the date that such stockholder became an interested stockholder, with the following exceptions:

- before such date, the board of directors of the corporation approved either the business combination or the transaction that resulted in the stockholder becoming an interested stockholder;
- upon completion of the transaction that resulted in the stockholder becoming an interested stockholder, the interested stockholder owned at least 85% of the voting stock of the corporation outstanding at the time the transaction began, excluding for purposes of determining the voting stock outstanding (but not the outstanding voting stock owned by the interested stockholder) those shares owned (i) by persons who are directors and also officers and (ii) employee stock plans in which employee participants do not have the right to determine confidentially whether shares held subject to the plan will be tendered in a tender or exchange offer; or
- on or after such date, the business combination is approved by the board of directors and authorized at an annual or special meeting of the stockholders, and not by written consent, by the affirmative vote of at least 66 2/3% of the outstanding voting stock that is not owned by the interested stockholder.

In general, Section 203 defines business combinations to include the following:

- any merger or consolidation involving the corporation and the interested stockholder;
- any sale, lease, transfer, pledge or other disposition of 10% or more of the assets of the corporation to or with the interested stockholder;
- subject to certain exceptions, any transaction that results in the issuance or transfer by the corporation of any stock of the corporation to the interested stockholder;

- any transaction involving the corporation that has the effect of increasing the proportionate share of the stock or any class or series of the corporation beneficially owned by the interested stockholder; or
- the receipt by the interested stockholder of the benefit of any loss, advances, guarantees, pledges or other financial benefits by or through the corporation.

Amendment of Certificate of Incorporation and Bylaws. The General Corporation Law of the State of Delaware provides generally that the affirmative vote of a majority of the shares entitled to vote on any matter is required to amend a corporation's certificate of incorporation or bylaws, unless a corporation's certificate of incorporation or bylaws, as the case may be, requires a greater percentage. Our amended and restated bylaws may be amended or repealed by a majority vote of our board of directors or by the affirmative vote of the holders of at least 75% of the votes that all of our stockholders would be entitled to cast in any annual election of directors. In addition, the affirmative vote of the holders of at least 75% of the votes that all of our stockholders would be entitled to cast in any annual election of directors is required to amend or repeal or to adopt any provisions inconsistent with any of the provisions of our restated certificate of incorporation described above under "—Staggered Board; Removal of Directors" and "—Stockholder Action by Written Consent; Special Meetings."

Exclusive Forum Selection. Our restated certificate of incorporation provides that the Court of Chancery of the State of Delaware shall be the sole and exclusive forum for (1) any derivative action or proceeding brought on behalf of our company, (2) any action asserting a claim of breach of a fiduciary duty owed by any of our directors, officers, or other employees to our company or our stockholders, (3) any action asserting a claim against our company arising pursuant to any provision of the General Corporation Law of the State of Delaware or our restated certificate of incorporation or amended and restated bylaws, or (4) any action asserting a claim against our company governed by the internal affairs doctrine. Although our restated certificate of incorporation contains the choice of forum provision described above, it is possible that a court could rule that such a provision is inapplicable for a particular claim or action or that such provision is unenforceable.

Blank Check Preferred Stock. Our restated certificate of incorporation provides for 5,000,000 authorized shares of preferred stock. The existence of authorized but unissued shares of preferred stock may enable our board of directors to render more difficult or to discourage an attempt to obtain control of our company by means of a merger, tender offer, proxy contest, or otherwise. For example, if in the due exercise of its fiduciary obligations, our board of directors were to determine that a takeover proposal is not in the best interests of our company, our board of directors could cause shares of preferred stock to be issued without stockholder approval in one or more private offerings or other transactions that might dilute the voting or other rights of the proposed acquiror or insurgent shareholder or shareholder group. In this regard, our restated certificate of incorporation grants our board of directors broad power to establish the rights and preferences of authorized and unissued shares of preferred stock. The issuance of shares of preferred stock could decrease the amount of earnings and assets available for distribution to holders of shares of common stock. The issuance may also adversely affect the rights and powers, including voting rights, of such holders and may have the effect of delaying, deterring,

or preventing a change in control of the company. Our board of directors currently does not intend to seek shareholder approval prior to any issuance of shares of preferred stock, unless otherwise required by law.

Authorized But Unissued Shares. Our authorized but unissued shares of common stock and preferred stock are available for future issuance without stockholder approval, subject to any limitations imposed by the listing standards of the Nasdaq Global Select Market. These additional shares may be used for a variety of corporate finance transactions, acquisitions and employee benefit plans. The existence of authorized but unissued and unreserved common stock and preferred stock could make more difficult or discourage an attempt to obtain control of us by means of a proxy contest, tender offer, merger, or otherwise.

11 Hurley Street
Cambridge, MA 02141
P 617-401-9000
F 617-494-0985

Thursday, Jul 07 2022

Amy Parison

Re: Offer of Employment

Dear Amy,

On behalf of Editas Medicine, Inc. (the "**Company**"), I am pleased to offer you employment with the Company. The purpose of this letter (the "**Offer Letter**") is to set forth the terms of your employment with the Company, should you accept our offer.

I am pleased to offer you the position of Vice President, Corporate Controller at the Company, reporting to the Chief Financial Officer. Your base salary will be at the rate of $10,769.23 per biweekly pay period (equivalent to an annualized base salary of $280,000), subject to tax and other withholdings as required by law. Such base salary may be adjusted from time to time in accordance with normal business practice and in the sole discretion of the Company. You will be employed on a full-time basis. Your effective date of hire as an employee (the "**Start Date**") will be Monday, Aug 01 2022. You shall work out of the Company's office at One Main Street, Cambridge, MA and shall travel as required by your job duties.

Following the end of each fiscal year and subject to the approval of the Company's Board of Directors (the "**Board**"), or a duly authorized committee thereof, you will be eligible for a retention and performance bonus, targeted at 30% of your annualized base salary, based on your and the Company's performance during the applicable fiscal year as determined by the Board (or such committee) and in accordance with certain corporate goals determined by the Board (or such committee), in each case, in its sole discretion. Such bonus shall be pro-rated for any partial year and shall not be payable if your Start Date is within the last quarter of the fiscal year. You must be an active employee of the Company on the date any bonus is distributed in order to be eligible for and to earn a bonus award, as it also serves as an incentive to remain employed by the Company, provided, that the Company will award and pay any bonus for the prior calendar year no later than March 15th of the next succeeding fiscal year.

You will receive a one-time sign on bonus of $30,000, less applicable taxes and withholdings (the "**Signing Bonus**"), which will be paid to you in the first regular payroll following your Start Date. Should, within one (1) year after your Start Date, you voluntarily terminate employment

with the Company for any reason or if the Company terminates your employment because it has determined in its sole discretion that you have (a) engaged in fraud, misappropriation, or embezzlement, (b) materially breached any Company policy or any agreement by and between you and the Company; (c) committed one or more acts constituting either a felony or any crime involving dishonesty or moral turpitude; or (d) failed to perform your duties and/or responsibilities to the Company's satisfaction, you agree to repay the Company within thirty (30) days of your separation from employment with the Company, the entire Signing Bonus paid by the Company. You further acknowledge and agree that the Company may deduct from any amounts due to you from the Company (including without limitation any salary, bonuses, severance or separation pay, and expense reimbursements) up to the full amount of the Signing Bonus owed to the Company, subject to applicable law. If such deduction does not fully satisfy the amount of reimbursement due, or if the Company elects not to take such deduction, you agree to repay the remaining unpaid balance to the Company within thirty (30) days of your separation from employment with the Company. By signing and returning this Offer Letter, you agree to repayment of the Signing Bonus as provided for in this paragraph, and you further agree to execute any documents that may be requested by the Company to memorialize any deductions that you have authorized herein.

Subject to approval of the Board, a duly authorized committee thereof or the Company's Chief Executive Officer, you may be granted (i) a stock option to purchase 26,850 shares of the Company's common stock (the "**Option**") at an exercise or purchase price equal to the fair market value of the Company's common stock on the date of grant and (ii) restricted stock units ("**RSU**," together with the Option, the "**Equity Awards**") in the amount of 5,967 units. The Option will vest over four (4) years at the rate of 25% on the first anniversary of the Start Date, and an additional 2.0833% of the original number of shares at the end of each successive month following the first anniversary of the Start Date until the fourth anniversary of such date. The RSU will vest over four (4) years at the rate of 25% of the original number of RSUs on the first anniversary of the Start Date, and an additional 25% of the original number of RSUs will vest at the end of each successive anniversary date of your Start Date until the fourth anniversary of such date. Notwithstanding the foregoing, the Equity Awards are subject to the standard terms and conditions of the Company's 2015 Stock Incentive Plan (as may be amended and/or restated from time to time). The Equity Awards may be brought to the Board of Directors, a duly authorized committee thereof or the Company's Chief Executive Officer for approval in the first full month following your Start Date.

You may participate in any benefit programs that the Company establishes and makes available to its employees from time to time, provided you are eligible under (and subject to all provisions of) the plan documents governing those programs. Additionally, you will be eligible for paid vacation and holidays in accordance with Company policy. Please see the enclosed "2022 Benefits Overview" for detailed information on our benefits and related policies, which currently include 13 paid holidays and a flexible time-off program. The benefit programs made available by the Company, and the rules, terms and conditions for participation in such benefit plans, may be changed by the Company at any time without advance notice.

You will be required to execute a Non-Solicitation, Non-Competition, Confidentiality and Assignment Agreement in the form attached hereto as Exhibit A (the "**Agreement**") and, prior to your Start Date, a Durable Automatic Sale Instruction Letter in the form attached hereto as Exhibit B. You acknowledge that your eligibility for the Signing Bonus and the Equity Awards referenced herein are contingent upon your agreement to the non-competition provisions set forth in the Agreement. You further acknowledge that such consideration was mutually agreed upon by you and the Company, is fair and reasonable, and is in exchange for your compliance with such non-competition obligations.

In making this offer, the Company understands, based on representations made by you, that you are not under any obligation to any former employer or any person or entity which would prevent, limit, or impair in any way your acceptance of this offer or employment or the performance by you of your duties as an employee of the Company. In accepting this offer you represent and warrant the foregoing to be true and correct (i) that in connection with providing services to the Company you will not use any confidential and/or proprietary information of any third party, including, without limitation, any former employer, or bring any biological or other materials to the Company and (ii) the Agreement was provided to you by the earlier of (A) the date we sent you this Offer Letter or (B) ten (10) business days before your Start Date.

You agree to provide to the Company, within three days of your hire date, documentation of your eligibility to work in the United States, as required by the Immigration Reform and Control Act of 1986. You may need to obtain a work visa in order to be eligible to work in the United States. If that is the case, your employment with the Company will be conditioned upon your obtaining a work visa in a timely manner as determined by the Company.

It is understood that you are an "at-will" employee. You are not being offered employment for a definite period of time or pursuant to an employment contract, and either you or the Company may terminate the employment relationship at any time and for any reason, with or without cause, or prior notice and without additional compensation to you.

This Offer Letter and the Agreement referenced above constitute the complete agreement between you and the Company, contain all of the terms of your employment with the Company and supersede any prior agreements, representations or understandings (formal or informal, whether written, oral or implied) between you and the Company. This Offer Letter may not be amended or modified except by an express written agreement signed by both you and a duly authorized officer of the Company, although your job duties, title, reporting relationship, compensation and benefits may change from time to time in the Company's sole discretion and provided that the "at-will" nature of your employment may only be changed by a written agreement signed by you and the Company's Chief Executive Officer, which expressly states the intention to modify the at-will nature of your employment. Nothing in this Offer Letter shall be construed as an agreement, either express or implied, to pay you any compensation or grant you any benefit beyond the end of your employment with the Company.

As an employee of the Company, you will be required to familiarize yourself and comply with all Company policies and procedures. Violations of the Company's policies may lead to immediate termination of your employment. Further, the Company's premises, including all workspaces, furniture, documents and other tangible materials, together with all information technology resources of the Company (including computers, portable devices, data and other electronic files (whether in hard copy or electronic form), and all internet and email communications) are subject to oversight and inspection by the Company at any time. Company employees shall have no expectation of privacy with regard to any Company premises, materials, resources or information.

The Company's offer of at-will employment is contingent upon your authorization and successful completion of background and reference checks as may be requested by the Company. If requested by the Company, you will be required to execute authorizations for the Company to obtain consumer reports and/or investigative consumer reports and use them in conducting background checks as a condition to your employment. The Company may obtain background reports both pre-employment and from time to time during your employment with the Company, as necessary.

Please indicate your acceptance of this offer by signing the enclosed copy of this Offer Letter and the Agreement via the electronic signature tool, no later than Thursday, Jul 14 2022.

Please know that we are truly excited at the prospect of you becoming part of the Editas team and at your leadership helping to build what we hope will be an exceptional organization, one that is both a scientific pioneer and that delivers transformative medicines to many patients. We believe that you will be a fundamental part of turning that aspiration into reality

Very truly yours,

Editas Medicine, Inc.

/s/ Sofia Morrow

Sofia Morrow

Vice President, Human Resources

The foregoing correctly sets forth the terms of my employment by the Company. I am not relying on any other representation, except as set forth in this Offer Letter.

/s/ Amy Parison

Amy Parison

11 Hurley Street
Cambridge, MA 02141
P 617-401-9000
F 617-494-0985

Exhibit A

Non-Solicitation, Non-Competition, Confidentiality and Assignment Agreement

Exhibit B

Durable Automatic Sale Instruction Letter

Amy Parison

Dear Amy,

Congratulations on your appointment to SVP, Chief Financial Officer. I am confident in this appointment and your ability to rise to the needs of the role. Your track record of financial decision-making, accounting acumen, and team leadership abilities make you the natural choice to succeed Erick Lucera. In addition, your intelligence and robust enterprise view position you for success. I look forward to seeing your continued growth and impact on Editas!

Below are details of your promotion and new 2025 compensation.

Base Salary Information	
Current Base Salary ($)	$340,000
Increase (%)	22.1%
Proposed New Salary ($)	$415,000
2025 Target Bonus (%)	40%
2025 Total Target cash ($)	$581,000
Salary Effective Date	March 28th, 2025
Equity Grant Information	
Annual Grant Stock Options*	34,672
Grant Date	April 8th, 2025

** The strike price will be equal to the closing price of the Company's common stock on the Nasdaq Global Select Market on the grant date.*

Your annual grant stock options will vest over 4 years at a rate of 2.0833% of the original number of shares per month, commencing one month after the Grant Date of April 8th, 2025 until fully vested on the fourth anniversary of the Grant Date.

Please indicate your acceptance of this offer by signing the enclosed copy of this Offer Letter and the Agreement via the electronic signature tool, no later than Monday, March 24, 2025.

Sincerely,

/s/ Gilmore ONeill_____

Gilmore O'Neill
President and Chief Executive Officer

The foregoing correctly sets forth the terms of my employment by the Company. I am not relying on any other representation, except as set forth in this Offer Letter.

/s/ Amy Parison_____ 3/29/2025
Signature Date

EDITAS MEDICINE

DIRECTOR COMPENSATION

Under this non-employee director compensation program, Editas Medicine (the "**Company**") pays its non-employee directors retainers in cash. Each non-employee director receives a cash retainer for service on the Board and for service on each committee of which the director is a member. The chairmen of the Board and of each committee receives higher retainers for such service. The amounts of the fees paid to each non-employee director for service on the board of directors and for service on each committee of the board of directors on which the director is a member are as follows:

	Member Annual Fee	Chairman Annual Fee
Board of Directors	$40,000	$ 75,000
Audit Committee	$8,750	$ 18,750
Organization, Leadership and Compensation Committee	$7,500	$ 15,000
Nominating and Corporate Governance Committee	$5,000	$ 10,000

Any non-employee director serving as the Board-appointed lead independent director also receives an annual fee of $25,000, in addition to any fees such director receives for his or her service on the Board or any committees thereof.

These fees are payable in arrears in four equal quarterly installments on the last day of each quarter, provided that the amount of such payment shall be prorated for any portion of such quarter during which the director was not serving. The Company also reimburses its non-employee directors for reasonable travel and other expenses incurred in connection with attending Board and committee meetings. Additionally, the Board may establish other committees from time to time that include fees for both members and chairpersons, as well as per meeting fees.

Under this non-employee director compensation program, each non-employee director shall be granted automatically and without the need for further Board action, under the Company's 2015 Stock Incentive Plan, on the date of his or her initial election to the Board, a stock option to purchase 103,400 shares of the Company's common stock (the "**Initial Option Grant**"). The Initial Option Grant shall vest as to one-third of the shares of the Company's common stock underlying such option on each anniversary of the grant date until the third anniversary of the grant date, subject to the non-employee director's continued service as a director through such vesting date. Further, on the date of the first Board meeting held after each annual meeting of stockholders, each non-employee director that has served on the Board for at least four months shall be granted automatically and without the need for further Board action, under the 2015 Stock Incentive Plan, a stock option to purchase 51,700 shares of the Company's common stock (the "**Annual Option Grant**). The Annual Option Grant shall vest in full on the one-year anniversary of the grant date, subject to the non-employee director's continued service as a director through such date. Each of the Initial Option Grant and the Annual Option Grant shall have an exercise price equal to the closing trading price of the Company's common stock on the

date of grant (or most recent preceding trading date if the grant date is not a trading day) and shall become exercisable in full upon a change in control of the Company.

Exhibit 23.1

Consent of Independent Registered Public Accounting Firm

We consent to the incorporation by reference in the following Registration Statements:

1. Registration Statements (Form S-3 No. 333-216528 and 333-277471) of Editas Medicine Inc.,
2. Registration Statement (Form S-8 No. 333-209351) pertaining to the Editas Medicine Inc. 2013 Stock Incentive Plan, 2015 Stock Incentive Plan and 2015 Employee Stock Purchase Plan of Editas Medicine Inc.,
3. Registration Statements (Form S-8 Nos. 333-216445, 333-223529, and 333-230266) pertaining to the 2015 Stock Incentive Plan and 2015 Employee Stock Purchase Plan of Editas Medicine, Inc.,
4. Registration Statement (Form S-8 No. 333-236662) pertaining to the Editas Medicine Inc. 2015 Stock Incentive Plan, 2015 Employee Stock Purchase Plan, Inducement Stock Option Awards (October 2019 – January 2020), and Inducement Restricted Stock Unit Awards (October 2019 – January 2020),
5. Registration Statement (Form S-8 No. 333-253716) pertaining to the Editas Medicine Inc. 2015 Stock Incentive Plan, 2015 Employee Stock Purchase Plan, Inducement Stock Option Award (November 2020), and Inducement Restricted Stock Unit Award (November 2020),
6. Registration Statement (Form S-8 No. 333-262977) pertaining to the Editas Medicine Inc. 2015 Stock Incentive Plan, 2015 Employee Stock Purchase Plan, Inducement Stock Option Awards (June 2021), and Inducement Restricted Stock Unit Awards (June 2021),
7. Registration Statement (Form S-8 No. 333-269917) pertaining to the Editas Medicine Inc. 2015 Stock Incentive Plan, and Inducement Stock Option Awards (June 2022 – July 2022),
8. Registration Statement (Form S-8 No. 333-277459) pertaining to the Editas Medicine Inc. 2015 Stock Incentive Plan, and Inducement Stock Option Awards (May 2023 – September 2023), and
9. Registration Statement (Form S-8 No. 333-285576) pertaining to the Editas Medicine Inc. 2015 Stock Incentive Plan;

of our report dated March 9, 2026, with respect to the consolidated financial statements of Editas Medicine, Inc. included in this Annual Report (Form 10-K) of Editas Medicine, Inc. for the year ended December 31, 2025.

/s/ Ernst & Young LLP
Boston, Massachusetts

March 9, 2026

Exhibit 31.1

CERTIFICATIONS

I, Gilmore O'Neill, certify that:

1. I have reviewed this Annual Report on Form 10-K of Editas Medicine, Inc.;

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:

    a. Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

    b. Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

    c. Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

    d. Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

    a. All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

    b. Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: March 9, 2026

By: /s/ Gilmore O'Neill

Gilmore O'Neill

Chief Executive Officer

Principal Executive Officer

Exhibit 31.2

CERTIFICATIONS

I, Amy Parison, certify that:

1. I have reviewed this Annual Report on Form 10-K of Editas Medicine, Inc.;

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:

    a. Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

    b. Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

    c. Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

    d. Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

    a. All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

    b. Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: March 9, 2026

By: /s/ Amy Parison

Amy Parison
Chief Financial Officer
(Principal Financial Officer)

Exhibit 32.1

CERTIFICATIONS OF CEO AND CFO 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 on Form 10-K of Editas Medicine, Inc. (the "Company") for the year ended December 31, 2025, as filed with the Securities and Exchange Commission on the date hereof (the "Report"), each of the undersigned officers of the Company hereby certifies, pursuant to 18 U.S.C. (section) 1350, as adopted pursuant to (section) 906 of the Sarbanes-Oxley Act of 2002, that to the best of her or his knowledge:

(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.

Dated: March 9, 2026

By: /s/ Gilmore O'Neill
Gilmore O'Neill
President and Chief Executive Officer

By: /s/ Amy Parison
Amy Parison
Chief Financial Officer