UNITED STATES
SECURITIES AND EXCHANGE COMMISSION

Washington, D.C. 20549

FORM 10-K

(Mark One)

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

For the fiscal year ended December 31, 2024

or

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

For the transition period from to

Commission file number: 001-37553

REGENXBIO Inc.

(Exact name of registrant as specified in its charter)

Delaware	**47-1851754**
(State or other jurisdiction of incorporation or organization)	(I.R.S. Employer Identification Number)

9804 Medical Center Drive
Rockville, MD 20850
(240) 552-8181

(Address of principal executive offices and Zip Code, and telephone number, including area code)

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

Title of each class	Trading symbol(s)	Name of each exchange on which registered
Common Stock, par value $0.0001 per share	RGNX	The Nasdaq Global Select Market

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

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

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

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

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

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

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

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

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

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

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

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

The aggregate market value of common stock held by non-affiliates of the registrant based on the closing price of the registrant's common stock as reported on The Nasdaq Global Select Market on June 30, 2024, the last business day of the registrant's most recently completed second quarter, was $536,344,977.

As of March 7, 2025, there were 50,086,138 shares of the registrant's common stock, par value $0.0001 per share, issued and outstanding.

DOCUMENTS INCORPORATED BY REFERENCE

Specified portions of the registrant's definitive proxy statement with respect to the registrant's 2025 Annual Meeting of Stockholders, which is to be filed pursuant to Regulation 14A within 120 days after the end of the registrant's fiscal year ended December 31, 2024, are incorporated by reference into Part III of this Annual Report on Form 10-K.

REGENXBIO INC.

Form 10-K

For the Year Ended December 31, 2024

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PART I

INFORMATION REGARDING FORWARD-LOOKING STATEMENTS

This Annual Report on Form 10-K contains "forward-looking statements" within the meaning of Section 27A of the Securities Act of 1933, as amended (the Securities Act), and Section 21E of the Securities Exchange Act of 1934, as amended (the Exchange Act). These statements express a belief, expectation or intention and are generally accompanied by words that convey projected future events or outcomes such as "anticipate," "assume," "believe," "continue," "could," "design," "estimate," "expect," "forecast," "goal," "intend," "may," "objective," "plan," "position," "potential," "predict," "project," "seek," "should," "will," "would" or variations of such words or by similar expressions. We have based these forward-looking statements on our current expectations, estimates and assumptions and analyses in light of our experience and our perception of historical trends, current conditions and expected future developments, as well as other factors we believe are appropriate under the circumstances. However, whether actual results and developments will conform with our expectations and predictions is subject to a number of risks, uncertainties, assumptions and other important factors, including, but not limited to:

- our ability to establish and maintain development partnerships, including our collaboration with AbbVie to develop and commercialize ABBV-RGX-314 and our collaboration with Nippon Shinyaku to develop and commercialize RGX-121 and RGX-111;
- our ability to obtain and maintain regulatory approval of our product candidates and the labeling for any approved products;
- the timing of enrollment, commencement, completion and the success of our AAVIATE®, AFFINITY BEYOND®, AFFINITY DUCHENNE®, ALTITUDE®, ASCENT™, ATMOSPHERE® and CAMPSIITE® clinical trials;
- the timing of commencement and completion and the success of preclinical studies conducted by us and our development partners;
- the timely development and launch of new products;
- the scope, progress, expansion and costs of developing and commercializing our product candidates;
- our ability to obtain, maintain and enforce intellectual property protection for our product candidates and technology, and defend against third-party intellectual property-related claims;
- our expectations regarding the development and commercialization of product candidates currently being developed by third parties that utilize our technology;
- our anticipated growth strategies;
- our expectations regarding competition;
- the anticipated trends and challenges in our business and the market in which we operate;
- our ability to attract or retain key personnel;
- the size and growth of the potential markets for our product candidates and the ability to serve those markets;
- the rate and degree of market acceptance of any of our products that are approved;
- our expectations regarding our expenses and revenue;
- our expectations regarding our need for additional financing and our ability to obtain additional financing;
- our expectations regarding the outcome of legal proceedings;
- our expectations regarding recent regulatory developments in the United States and foreign countries; and
- changes in the financial markets and banking system that may affect the availability and terms on which we may obtain financing and our ability to accurately predict how long our existing cash resources will be sufficient to fund our anticipated operating expenses.

You should carefully read the factors discussed in the sections titled "Risk Factors," "Management's Discussion and Analysis of Financial Condition and Results of Operations," and the factors discussed elsewhere in this Annual Report on Form 10-K and in our other filings with the U.S. Securities and Exchange Commission (the SEC) for additional discussion of the risks, uncertainties, assumptions and other important factors that could cause our actual results or developments to differ materially and adversely from

those projected in the forward-looking statements. The actual results or developments anticipated may not be realized or, even if substantially realized, they may not have the expected consequences to or effects on us or our businesses or operations. Such statements are not guarantees of future performance and actual results or developments may differ materially and adversely from those projected in the forward-looking statements. These forward-looking statements speak only as of the date of this Annual Report. Except as required by law, we disclaim any duty to update any forward-looking statements, whether as a result of new information, future events or otherwise.

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

AAVIATE, AFFINITY BEYOND, AFFINITY DUCHENNE, ALTITUDE, ATMOSPHERE, CAMPSIITE, NAV, NAVXpress, NAVXcell, REGENXBIO and the REGENXBIO logos are our registered trademarks. Any other trademarks appearing in this Annual Report on Form 10-K are the property of their respective holders.

INDUSTRY AND MARKET DATA

We obtained the industry, market and competitive position data used throughout this Annual Report on Form 10-K from our own internal estimates and research, as well as from industry and general publications, in addition to research, surveys and studies conducted by third parties. Internal estimates are derived from publicly-available information released by industry analysts and third-party sources, our internal research and our industry experience, and are based on assumptions made by us based on such data and our knowledge of our industry and market, which we believe to be reasonable. We have not independently verified industry, market and competitive position data from third-party sources, but we believe the sources of such information to be reliable. While we believe the industry, market and competitive position data included in this Annual Report on Form 10-K is reliable and is based on reasonable assumptions, such data involves risks and uncertainties and are subject to change based on various factors, including those discussed in "Risk Factors." These and other factors could cause results to differ materially from those expressed in the estimates made by the independent parties and by us.

ITEM 1. *BUSINESS*

Overview

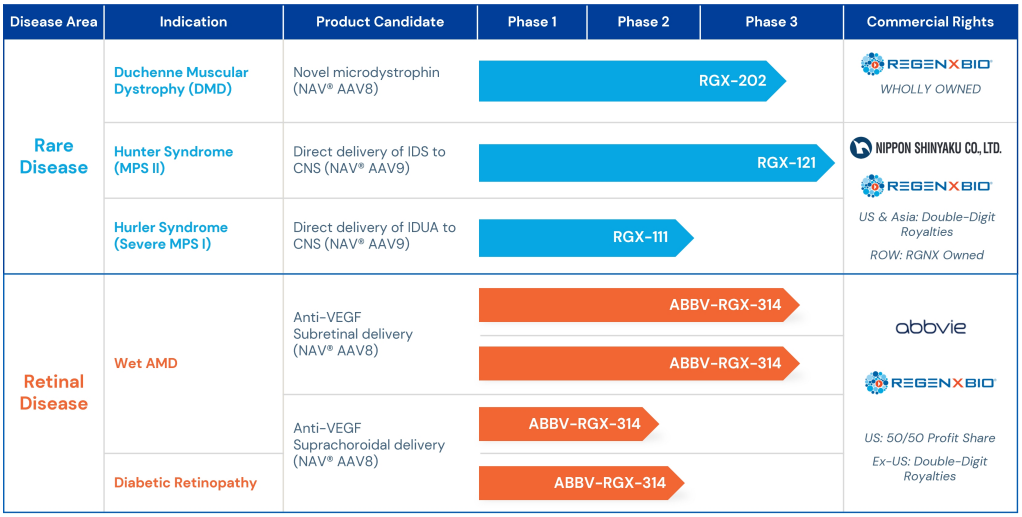
We are a leading clinical-stage biotechnology company seeking to improve lives through the curative potential of gene therapy. Our investigational gene therapies are designed to deliver functional genes to address genetic defects in cells, enabling the production of therapeutic proteins or antibodies that are intended to impact disease. Through a single administration, gene therapy could potentially alter the course of disease significantly and deliver improved patient outcomes with long-lasting effects.

Our investigational gene therapies use adeno-associated virus (AAV) vectors from our proprietary gene delivery platform, which we call our NAV® Technology Platform. AAV vectors are non-replicating viral delivery vehicles that are not known to cause disease. Our NAV Technology Platform consists of exclusive rights to a large portfolio of AAV vectors (NAV Vectors), including commonly used AAV8 and AAV9. We believe this platform forms a strong foundation for our current clinical-stage programs and, with our ongoing research and development, we expect to continue to expand our platform and pipeline of potential AAV vector-based gene therapies. We refer to commercial and investigational AAV vector-based gene therapies as AAV Therapeutics. Our NAV Technology Platform is the foundation for commercial and investigational AAV Therapeutics that have treated thousands of patients through our clinical pipeline and NAV licensees.

We have developed a broad pipeline of investigational AAV Therapeutics using our NAV Technology Platform as one-time treatments to address an array of diseases. We are currently focusing our internal development pipeline in three areas: retinal, neuromuscular and neurodegenerative diseases. We believe these product candidates are differentiated, can be expedited, and support meaningful near-term and long-term value generation. Our investigational AAV Therapeutics include:

- ABBV-RGX-314, which we are developing in collaboration with AbbVie to treat large patient populations impacted by wet age-related macular degeneration (wet AMD), diabetic retinopathy (DR) and other chronic retinal diseases characterized by loss of vision.
- RGX-202, which we are developing to treat Duchenne muscular dystrophy (Duchenne), one of the most common fatal genetic disorders affecting children.
- RGX-121 and RGX-111, which we are developing and plan to commercialize with Nippon Shinyaku to treat Mucopolysaccharidosis type II (MPS II) and Mucopolysaccharidosis type I (MPS I), both of which are progressive, neurodegenerative lysosomal storage disorders.

Our internal pipeline is shown below.



Since our founding, we have built a team of experts in research and development, scalable manufacturing, and preclinical and clinical development, enabling us to have integrated, end-to-end capabilities. We believe AAV Therapeutics represent a simplified and efficient potential new class of innovative medicines. Our experience and expertise distinguish us from other gene therapy companies and will help ensure value generation and our continued growth.

AAV Therapeutics

Historically, the primary challenge for gene therapy has been the safe and effective delivery of genes into cells. To address this challenge, scientists designed and developed a variety of gene vectors to facilitate gene delivery into cells.

Our gene therapies are designed to deliver working genes to cells using AAV vectors as the delivery "vehicles." We chose AAV vectors, modified viruses that cannot increase their numbers or reproduce themselves, based on several factors including their demonstrated efficiency in gene delivery to date and promising safety profiles for gene therapy.

Since our inception, we have built and advanced multiple promising AAV Therapeutics into late-stage clinical development. All of our AAV Therapeutics were built on our NAV Technology Platform. Together, with our manufacturing and end-to-end capabilities, we are excited about the prospect of bringing these assets to commercial stage and continuing to leverage our NAV Technology Platform to generate additional promising AAV Therapeutics.

Our NAV Technology Platform

In 2009, we acquired exclusive rights to our NAV Technology Platform. Our NAV Technology Platform includes over 100 NAV Vectors, as well as vectors that are at least 95% identical to any NAV Vector, that provide the foundation for the development of new AAV Therapeutics. We have observed that several of our NAV Vectors demonstrate preferential tropisms for a range of tissues, as well as efficient transgene delivery and expression that may produce a therapeutic effect. Our NAV Technology Platform has enabled the development of a number of AAV Therapeutics being investigated in clinical trials and one that is FDA-approved.

For many years, by sublicensing NAV Vectors from our NAV Technology Platform to other biopharmaceutical companies with disease-specific expertise, which we refer to as our NAV Technology Licensees, we received capital to advance our own research and capabilities. Our NAV Technology Platform is being applied to a number of programs over a broad range of therapeutic areas and disease indications by our NAV Technology Licensees. These partnered programs include Novartis' Zolgensma®, a gene therapy for the treatment of spinal muscular atrophy (SMA), which was approved by the U.S. Food and Drug Administration (FDA) in 2019, and has been used to treat over 4,500 patients suffering from SMA, a debilitating and potentially deadly disease. Our partnering strategy

provides us the flexibility to sublicense development of treatments designed to address significant unmet medical needs, while remaining focused on our own pipeline of AAV Therapeutics.

We believe we have extensive human safety experience to support the development of our investigational AAV Therapeutics based on data from thousands of patients dosed with AAV Therapeutics derived from our NAV Technology Platform in more than 15 different clinical-stage programs and with one FDA approved product. To date, we have observed that AAV Therapeutics derived from our NAV Technology Platform have been generally well tolerated.

Our AAV Therapeutic Platform

Discovery and Development of AAV Therapeutics

We have a team of scientists and engineers dedicated to expanding the understanding and applications of AAV vectors, applying the differentiated capabilities of the NAV Technology Platform and exploring the potential to generate new, innovative AAV Therapeutics. We endeavor to rapidly discover and develop a pipeline of investigational AAV Therapeutics with the potential, through a single administration, to alter the course of disease significantly and deliver improved patient outcomes with long-lasting effects. We believe that we have created a reproducible process and modular platform for the discovery and development of innovative AAV Therapeutics.

Our scientists are researching and evaluating NAV Vectors to identify and characterize new features that may be more clinically effective by improving tissue tropism and specificity, de-targeting the liver, increasing transduction efficiency and requiring a lower dose.

We are also engineering novel capsids by leveraging the natural diversity of our NAV Vectors and our detailed knowledge of AAV structure and function. We are designing new NAV Vectors with new features that may enhance tissue and cell type specificity, increase potency and potentially improve the safety profile of AAV Therapeutics. Through our internal efforts and collaborations, we are also designing novel vectors to which we add high affinity targeting domains with the goal of enabling them to deliver genes more precisely to specific tissues and cells.

With AAV Therapeutics, the transgene is eventually transported to the cell nucleus where it is transcribed into RNA. The production of RNA in the cell is controlled by transcriptional elements called enhancers and promoters that are linked to the gene. We have designed optimized enhancer and promoter combinations with the goal of enabling sustained gene expression in particular cell types and potentially increasing the durability of therapeutic effect.

We can design our AAV Therapeutics to deliver transgenes encoding a spectrum of therapeutic modalities. Our current pipeline of investigational AAV Therapeutics uses NAV Vectors to deliver transgenes encoding therapeutic antibodies, or able to compensate for a missing or non-functional gene.

We also conduct research studying the potential of NAV Vectors to deliver small RNAs, such as microRNA (miRNA) or antisense sequences, which could alter the structure or silence an RNA transcript. We have created a platform for designing efficient small RNA scaffolds to address targets of interest while avoiding off-target effects and cellular toxicity. In addition, NAV Vectors have been designed to enable *in vivo* gene editing, which involves the alteration of a gene via targeted insertion or deletion of DNA base pairs.

In addition to our research evaluating NAV Vectors, we also work on identifying potential indications for the development of new AAV Therapeutics, guided by our expertise and experience in bringing AAV Therapeutics to the clinical stage. Our early evaluation of targets includes scientific rationale and cross-functional analysis of technical feasibility. In our exploratory research, we work internally and through collaborations with external researchers to identify and optimize AAV Therapeutics based on AAV vector targeting, transgene optimization and evaluation of effective delivery devices. We then execute proof-of-concept research that informs the next steps in our pipeline strategy. While much of our research into potential AAV Therapeutics extends from our clinical expertise in eye diseases, AAV-mediated antibody delivery, neurodegenerative diseases and neuromuscular diseases, we are also able to research potential opportunities to advance AAV Therapeutics for new disease areas.

Our platform capabilities include a team of scientists that develop analytical assays and approaches to support our preclinical and clinical-stage pipeline. The ability to determine dose levels, biodistribution, and target engagement requires an understanding of complex variables that are related to properties of both the NAV Vector and the gene, and dependent on the delivery device. We believe that our analytical capabilities are at the forefront of AAV Therapeutic development.

AAV Therapeutic Manufacturing

Our research team works closely with our manufacturing team, allowing us to evaluate the manufacturability of AAV Therapeutics early in the discovery process. Through our ability to collaborate cross-functionally, we can move quickly from candidate selection to the manufacturing of clinical-grade material, which we believe allows us to accelerate the process of developing AAV Therapeutics.

We have invested in innovative manufacturing process development and analytical capabilities and use a suspension cell culture-based manufacturing process. We have deep in-house knowledge of biologics and gene therapy manufacturing, which has enabled us to scale manufacturing of our AAV Therapeutics while ensuring product quality for patients and improving cost-of-goods. We have developed systems which we believe will provide robust manufacturing and global supply of AAV Therapeutics to meet quality requirements and anticipated research, clinical and future commercial demand. Our Good Manufacturing Practices (cGMP) production facility, the REGENXBIO Manufacturing Innovation Center (RMIC), is located in our corporate headquarters in Rockville, Maryland. The RMIC has been designed to support clinical and future commercial production of AAV Therapeutics and has been in operation since mid-2022.

We have developed a proprietary, high-yielding manufacturing process platform for NAV vector production (NAVXpress®) that can be applied across multiple AAV Therapeutics. This manufacturing process platform approach improves development efficiency and shortens timelines by leveraging data across multiple programs. The suspension-based manufacturing platform has demonstrated robust scalability from bench-scale to 500 liter and 1,000-liter cGMP batches with consistent yield and product purity demonstrated via comparability studies. At the RMIC facility, we have demonstrated the ability to scale the manufacturing process to 2,000 liters. We believe this flexibility in manufacturing will support a wide range of potential commercial supply requirements for our AAV Therapeutics. The NAVXpress manufacturing process is fully characterized, and we have engaged in full-scale process validation to support a Biologics License Application (BLA) submission.

We have designed custom starting materials for use in the NAVXpress platform, including plasmids and cell lines, that increase the efficiency and productivity of NAV vectors. We have demonstrated that these cell lines, named NAVXcell®, enable high-yielding production processes while allowing for efficient purification.

We have developed product formulations specific to our different delivery devices and routes of administration. We aim to ensure that our formulations are designed and assessed to ensure product stability can be maintained for numerous years and that our AAV Therapeutics can be exposed to a variety of handling and delivery procedures.

We have endeavored to design our platform manufacturing process, formulations and devices to enable efficient transition from research to clinical trials to commercial readiness, while minimizing changes during product development. To support our platform, we have developed a comprehensive set of analytical methods to assess quality and characterize the product. We continue to expand and enhance internal analytical lab capabilities with the aim of improving quality and control and supporting accelerated development of AAV Therapeutics.

While we primarily rely upon internal manufacturing, we have agreements with biologics contract development and manufacturing organizations (CDMOs) for production of material under cGMP requirements to support our current and future clinical trials, as well as potential future commercialization of our investigational AAV Therapeutics. We select our CDMOs based on capability, capacity and expertise, and we believe partnering with multiple CDMOs provides us with flexibility and diversity in suppliers, as well as access to future capacity to accommodate clinical trials and commercialization.

AAV Therapeutic Delivery Devices

We believe that a critical component of AAV Therapeutic development is to deliver treatments safely, effectively and efficiently to the right part of the body. We leverage the differentiated characteristics of NAV Vectors to target different tissues and cells. To further enhance the profile of AAV Therapeutics, we have developed a platform of different devices to assist in the delivery of AAV Therapeutics using multiple routes of administration to tissues and cells.

We have developed significant expertise in designing delivery device systems for use with AAV Therapeutics and have also developed and in-licensed relevant intellectual property, including know-how, related to delivery devices. Our research and development activities have involved several delivery device advancements for AAV Therapeutics. We focus research on designing

features and implementing delivery device solutions that we believe have the potential to improve the effect, patient safety and caregiver usability of AAV Therapeutics.

We have advanced image-guided device delivery of AAV Therapeutics into the cerebrospinal fluid to target the brain and central nervous system for neurodegenerative diseases. We have also led the development of two different types of delivery devices of AAV Therapeutics into the eye for targeting the retina of patients. In 2020, in our clinical trial for the in-office treatment of wet AMD, an investigational AAV Therapeutic was delivered to a patient using a novel, suprachoroidal delivery device for the first time. In 2020, we initiated a pivotal phase program for ABBV-RGX-314 using a subretinal delivery device for the treatment of wet AMD. As part of our delivery device expertise, we have created teams of experts to support and train physicians to deliver AAV Therapeutics in operating room and physician office settings.

In recent years, a tremendous amount of progress has been made in the development of AAV Therapeutics, and we believe we are a leader in these advancements.

Our Investigational AAV Therapeutics

We are currently focusing our internal development pipeline in three areas: retinal diseases, neuromuscular diseases and neurodegenerative diseases.

ABBV-RGX-314 for the Treatment of Wet AMD and DR

We are developing ABBV-RGX-314 in collaboration with AbbVie as a potential one-time treatment for wet AMD and DR. These diseases are characterized by loss of vision due to excess fluid accumulation from new blood vessel formation and treated with anti-vascular endothelial growth factor (anti-VEGF) therapies.

Wet AMD is the leading cause of vision loss in people over 60, affecting more than 2 million patients in the United States, Europe and Japan. The risk for developing wet AMD increases with age and we anticipate that the incidence of new cases will continue to increase significantly with the growth of an aging population. In patients with wet AMD, fluid accumulation can result in physical changes in the structure of the retina and adverse changes in vision. As this process progresses, blindness can result from atrophy and scar formation.

DR is a complication of diabetes and is the leading cause of blindness in adults between 24 and 75 years of age worldwide. It is a progressive retinopathy, and the spectrum of DR severity ranges from non-proliferative diabetic retinopathy (NPDR) to proliferative diabetic retinopathy (PDR). As DR progresses, a large proportion of patients develop vision-threatening complications, including diabetic macular edema (DME) and neovascularization that can lead to blindness. An estimated 27 million patients are affected with DR across the US, Europe and Japan, and of those, there are more than 23 million DR patients without center-involved DME. DR is the leading cause of vision loss in working-age adults and the incidence is expected to continue to grow significantly with the prevalence of diabetes.

Frequent anti-VEGF injections in the eye have been shown to reduce the risk of blindness in randomized controlled clinical trials and are approved for the treatment of wet AMD and DR. The current standard-of-care anti-VEGF treatments require patients to receive injections in the eye every four to 16 weeks for the duration of the disease. Real world evidence shows that patients with wet AMD are severely undertreated, and DR patients with early non-proliferative disease are often not treated due to the unsustainable treatment burden of administering frequent injections required with currently approved anti-VEGF therapies. As a result, the majority of wet AMD patients experience significant vision loss over time and most patients with early non-proliferative DR progress to more severe forms of the proliferative disease, developing common vision-threatening complications such as center-involved DME and proliferative DR.

ABBV-RGX-314 is being developed as a novel, one-time treatment that includes the NAV AAV8 vector containing a gene for a monoclonal antibody fragment designed to inhibit VEGF activity, modifying the pathway for formation of new leaky blood vessels and retinal fluid accumulation. After delivery of ABBV-RGX-314, we believe retinal cells will continue to produce the anti-VEGF protein. Two separate routes of administration of ABBV-RGX-314 to the eye are being evaluated: a subretinal delivery procedure as well as a targeted, in-office administration to the suprachoroidal space. We have licensed certain exclusive rights to the SCS Microinjector® from Clearside Biomedical, Inc. (Clearside) to deliver gene therapy treatments to the suprachoroidal space of the eye.

Clinical Development of ABBV-RGX-314 Subretinal Delivery for the Treatment of Wet AMD

We have two ongoing pivotal trials, ATMOSPHERE® and ASCENT™, for the treatment of wet AMD using ABBV-RGX-314 (surabgene lomparvovec) delivered subretinally.

ATMOSPHERE and ASCENT are multi-center, randomized, active-controlled trials to evaluate the efficacy and safety of a single-administration of ABBV-RGX-314 versus standard of care in patients with wet AMD. Both trials are active and enrolling patients. To support future commercialization of ABBV-RGX-314, the cGMP material produced by our NAVXpress platform process has been incorporated in the ongoing pivotal trials, ATMOSPHERE and ASCENT, for the treatment of wet AMD using ABBV-RGX-314 delivered subretinally.

These trials are expected to support global regulatory submissions, including with the U.S. Food and Drug Administration (FDA) and the European Medicines Agency (EMA). Topline data from these trials are expected to be shared in 2026.

In October 2024, we reported positive data from a Phase II fellow eye sub-study at the American Academy of Ophthalmology (AAO) 2024 annual meeting. The sub-study evaluated subretinal delivery of ABBV-RGX-314 in patients who received ABBV-RGX-314 in the Phase I/IIa or bridging studies and elected to receive treatment in their second eye. As of September 11, 2024, ABBV-RGX-314 has been well tolerated in the treated second eye with no drug-related serious adverse events and no cases of intraocular inflammation observed. At nine months post-administration, patients saw a 97% reduction in anti-VEGF treatment burden and sustained vision and anatomy. We believe these data demonstrate the potential of ABBV-RGX-314 to preserve vision long-term for patients with wet AMD as a one-time treatment for both eyes. Bilateral disease impacts a significant number of patients with wet AMD.

In February 2024, we presented updated interim data at the Angiogenesis, Exudation and Degeneration annual meeting from the Phase II pharmacodynamic study designed to evaluate the same dose levels being used in the two pivotal trials. Data demonstrated that ABBV-RGX-314 manufactured using REGENXBIO's NAVXpress platform process was well tolerated at both dose levels. Through month 6, ABBV-RGX-314 in all cohorts exhibited similar and expected protein levels along with stable to improved best corrected visual acuity and central retinal thickness, as well as meaningful reductions in anti-VEGF burden, with the majority of subjects remaining injection-free.

In October 2022, we announced data from the Phase I/IIa long-term follow-up study of ABBV-RGX-314 for the treatment of wet AMD using subretinal delivery. As of August 29, 2022, ABBV-RGX-314 continued to be generally well-tolerated in the long-term follow-up study (n=37). Nine SAEs were reported in four patients, none of which were considered related to ABBV-RGX-314. Patients treated with ABBV-RGX-314 continued to demonstrate a long-term, durable treatment effect in Cohort 3 up to four years and in Cohort 4 up to three years. Stable to improved visual acuity was observed, with a mean BCVA of +12 letters from baseline at four years for Cohort 3 patients and -5 letters from baseline at three years for Cohort 4 patients following ABBV-RGX-314 administration.

Two-year findings from this study were published in *The Lancet* in March 2024, in a paper titled "Gene therapy for neovascular age-related macular degeneration by subretinal delivery of RGX-314: a phase 1/2a dose-escalation study."

Clinical Development of ABBV-RGX-314 Suprachoroidal Delivery for the Treatment of Wet AMD

We are also evaluating the efficacy, safety and tolerability of suprachoroidal delivery of ABBV-RGX-314 through AAVIATE®, a multi-center, open label, randomized, controlled, dose-escalation Phase II trial of ABBV-RGX-314 for the treatment of wet AMD.

In January 2024, we announced data from the AAVIATE trial demonstrating that patients treated with ABBV-RGX-314 continue to demonstrate stable BCVA and CRT at six months. In addition, a meaningful reduction in anti-VEGF treatment burden was observed following administration of ABBV-RGX-314. The highest reduction was seen in dose level 3, demonstrating an 80% reduction in annualized injection rate with 50% of patients remaining injection-free.

As of July 29, 2024, ABBV-RGX-314 at dose level 3 with short course prophylactic steroid eye drops continued to be well tolerated with no drug-related serious adverse events (SAEs) and no cases of intraocular inflammation, endophthalmitis, vasculitis, retinal artery occlusion, choroidal effusion, or hypotony. Mild episcleritis occurred in three patients, all resolved and completed treatment with topical steroids. There were no cases of elevated intraocular pressure. Based on this favorable safety profile, the Phase II AAVIATE trial is initiating enrollment in a new cohort to evaluate ABBV-RGX-314 at dose level 4 (1.5x10e12 GC/eye). Patients in this cohort will also receive short course prophylactic steroid eye drops.

Clinical Development of ABBV-RGX-314 Suprachoroidal Delivery for the Treatment of DR

We are evaluating the efficacy, safety and tolerability of suprachoroidal delivery of ABBV-RGX-314 for the treatment of DR in ALTITUDE®, a multi-center, open label, randomized, controlled, dose-escalation Phase II trial. We have enrolled 79 patients dosed in Cohorts 1-5 at three dose levels.

In November 2023, we presented data from the Phase II ALTITUDE trial. As of September 25, 2023, ABBV-RGX-314 was reported to be well tolerated at dose levels 1 and 2. Seven serious adverse events were reported, none of which were considered drug related. For patients in dose levels 1 and 2 (n=50), common ocular treatment-emergent adverse events in the study eye through one year included conjunctival hemorrhage and conjunctival hyperemia. Three patients had mild intraocular inflammation, which resolved on topical corticosteroids. Six patients had mild to moderate episcleritis and have resolved on topical corticosteroids. At one year, dose level 2 in NPDR patients prevented disease progression as measured by the Early Treatment Diabetic Retinopathy Study-Diabetic Retinopathy Severity Scale. Dose level 2 reduced the risk of developing vision-threatening events by 89% in these patients.

NPDR patients treated with ABBV-RGX-314 at dose levels 1 and 2 demonstrated clinically meaningful improvements in disease severity and reduction of vision-threatening events. In dose level 2, patients with baseline NPDR:

- 100% demonstrated stable to improved disease severity
    - 70.8% achieved ≥1 step improvement vs. 25.0% in control
    - 0% worsened ≥2 steps vs. 37.5% in control
- 4.2% developed vision-threatening events vs. 37.5% in control

In August 2024, we announced the ALTITUDE trial is enrolling a new cohort of patients with center-involved DME. DME is a vision-threatening complication of DR. Patients will receive a one-time, in-office injection of ABBV-RGX-314 at dose level 4 (1.5x10e12 GC/eye) with short course prophylactic steroid eye drops.

A successful end-of-Phase II meeting with the FDA occurred in the fourth quarter of 2024. In January 2025, AbbVie and REGENXBIO announced they will plan a Phase III program. The program is expected to support global regulatory submissions.

RGX-202 for the Treatment of Duchenne

We believe that RGX-202 has the potential to serve as a second-to-market, differentiated gene therapy treatment for Duchenne.

RGX-202 is our investigational AAV Therapeutic for the treatment of Duchenne, a rare disease caused by mutations in the gene responsible for making dystrophin, a protein of central importance for muscle cell structure and function. Without dystrophin, muscles throughout the body degenerate and become weak, eventually leading to loss of movement and independence, required support for breathing, cardiomyopathy and premature death. There is presently no cure for Duchenne, and there is a significant unmet need for disease modifying treatment options. Duchenne is one of the most common fatal genetic disorders affecting children, primarily boys. Duchenne is estimated to occur in approximately one in every 3,500-5,000 live male births and has an estimated prevalence of more than 30,000 cases in the U.S., Europe and Japan.

RGX-202 is designed to deliver a transgene for a novel microdystrophin that includes the functional elements of the C-Terminal (CT) domain found in naturally occurring dystrophin. Presence of the CT domain has been shown in preclinical studies to recruit several key proteins to the muscle cell membrane, leading to improved muscle resistance to contraction-induced muscle damage in dystrophic mice. Additional design features may potentially improve gene expression, increase translational efficiency and reduce immunogenicity. RGX-202 is designed to support the delivery and targeted expression of the transgene throughout skeletal and heart muscle using the NAV AAV8 vector, and a well-characterized muscle-specific promoter (Spc5-12).

We have received orphan drug product, Fast Track designation and rare pediatric disease designation from the FDA for RGX-202.

Clinical Development of RGX-202

AFFINITY DUCHENNE® is a multicenter, open-label Phase I/II/III trial of RGX-202 in ambulatory patients aged one and older. Patients receive a dose of $2x10^{14}$ GC/kg. The pivotal phase of the trial is expected to support a BLA submission using the accelerated approval pathway in 2026.

To support potential accelerated approval, the primary endpoint is the proportion of participants whose RGX-202 microdystrophin expression is ≥ 10% at Week 12. Secondary endpoints include change from baseline on timed function tests including time to stand, 10 meter walk/run and time to climb in participants ages 4 and older. Participants aged 1 to < 4 years will be evaluated using the Peabody Developmental Motor Scale-Third Edition and stride velocity 95th centile. Patients will be assessed on the North Star Ambulatory Assessment (NSAA) as an exploratory endpoint.

In March 2024 and May 2024, we reported interim safety and efficacy data from the Phase I/II portion of the trial, including positive RGX-202 microdystrophin expression results for the first two patients who received RGX-202 at dose level 2 (DL2), the pivotal dose.

In November 2024, we reported additional interim data from the Phase I/II portion of the AFFINITY DUCHENNE trial, including functional outcomes from the first five Phase I/II participants. Results include 12-month data from three dose level 1 patients aged 4-10 and nine-month data from two dose level 2 (pivotal dose) patients aged 8 and 12. In all five participants, across both dose levels, RGX-202 demonstrated evidence of positively impacting disease trajectory, with patients demonstrating stable or improved function on NSAA and timed function tests including time to stand, 10 meter walk/run and time to climb. Results were measured against external natural history controls matched for age and baseline function.

We also reported new biomarker and safety data in November 2024. The biomarker data continues to support consistent, high expression and transduction of RGX-202 microdystrophin. RGX-202 was appropriately localized to the sarcolemma, demonstrating the differentiated construct with the CT-Domain is appropriately targeting the muscle. In the 11 patients dosed as of November 1, 2024, RGX-202 was well tolerated with no SAEs or AESIs reported. Common drug-related AEs included nausea, vomiting and fatigue. All resolved and are typically anticipated with gene therapy administration.

Also in November 2024, we announced that a pivotal (Phase III) trial of AFFINITY DUCHENNE had been initiated and that the first patient had been dosed under the pivotal protocol. The trial will recruit approximately 30 ambulatory patients, aged 1 and above. In March 2025, we announced that the pivotal study was nearly 50% enrolled, and we expect to complete enrollment in the study in 2025, share top line data in the first half of 2026 and submit a BLA under the accelerated approval pathway in mid-2026.

We expect to share additional Phase I/II biomarker data at the 2025 Muscular Dystrophy Association (MDA) Clinical & Scientific Conference, including the first biomarker data from the cohort of patients aged 1-3. The company expects to share additional efficacy and safety data, including additional functional data, in the first half of 2025.

RGX-202 is manufactured using our proprietary, high-yielding NAVXpress platform process. This suspension-based manufacturing process has demonstrated scalability up to 2,000 liters with consistent yield and product purity. The RMIC has the capacity and yields to produce up to 2,500 doses of RGX-202 per year to support future commercialization.

Additionally, we are recruiting patients in the AFFINITY BEYOND® trial, an observational screening study. The primary objective is to evaluate the prevalence of AAV8 antibodies in patients with Duchenne up to 12 years of age. Information collected in this study may be used to identify potential participants for the AFFINITY DUCHENNE trial and potential future trials of RGX-202.

RGX-121 for the Treatment of MPS II

RGX-121 (clemidsogene lanparvovec) is our investigational AAV Therapeutic for the treatment of MPS II, which we are developing in collaboration with Nippon Shinyaku in the U.S. and certain countries in Asia. MPS II, also known as Hunter syndrome, is a rare disease caused by mutations in the gene responsible for making iduronate-2-sulfatase (IDS), which encodes the I2S enzyme. The I2S enzyme is responsible for the breakdown of the polysaccharides heparan sulfate (HS) and dermatan sulfate in lysosomes, structures that dispose of waste products inside cells. These polysaccharides, called glycosaminoglycans (GAGs), accumulate in tissues of MPS II patients, resulting in diverse clinical signs and symptoms. HS is a key biomarker of I2S enzyme activity and high amounts of HS accumulate in the central nervous system (CNS) of neuronopathic MPS II patients, which has been shown to correlate with neurocognitive manifestations of the disease. In severe forms of the disease, early developmental milestones may be met during the first year after birth, but developmental delay is readily apparent by 18 to 24 months. Developmental progression begins to plateau between three and five years of age, with regression reported to begin around six and a half years. By the time of death, most patients with CNS involvement are severely mentally handicapped and require constant care. MPS II is estimated to occur in approximately 1 in 100,000 to 1 in 170,000 births worldwide. Based on global population, this equates to approximately 500 to 1,000 MPS II patients born each year worldwide.

Enzyme replacement therapy (ERT), the current standard of care for patients with MPS II, does not treat CNS manifestations of the disease because the enzyme cannot cross the blood-brain barrier. We believe that specific treatment to address the neurological manifestations of MPS II and prevent or stabilize cognitive decline remains a significant unmet medical need.

RGX-121 is designed to use the NAV AAV9 vector to deliver the human *IDS* gene to cells in the CNS. Delivery of the gene therapy and expression of the enzyme that is deficient within cells in the CNS could provide a permanent source of secreted I2S enzyme on the CNS side of the blood-brain barrier, allowing for long-term cross-correction of cells throughout the CNS. We believe this strategy could provide rapid I2S enzyme delivery to the brain, potentially preventing the progression of cognitive deficits that otherwise occur in MPS II patients.

We have received orphan drug product designation, rare pediatric disease designation, regenerative medicine advanced therapy (RMAT) and Fast Track designation from the FDA, as well as orphan designation and advanced therapy medicinal products (ATMP) classification from the EMA for RGX-121.

Clinical Development of RGX-121 for the Treatment of MPS II

CAMPSIITE® is a Phase I/II/III multicenter, open-label trial enrolling boys with MPS II, aged 4 months up to 5 years of age.

In February 2024, we reported that the pivotal phase of the CAMPSIITE trial achieved its primary endpoint, Proportion of Patients with cerebrospinal fluid (CSF) D2S6 below maximum attenuated level at W16. Accurate and sensitive measurements of CSF GAGs, such as HS D2S6, have the potential to be considered a surrogate endpoint that is reasonably likely to predict clinical benefit in MPS II disease under the accelerated approval pathway, as buildup of GAGs in the CSF of MPS II patients correlates with clinical manifestations including neurodevelopmental deficits. MPS II patients treated with RGX-121 achieved decreased CSF levels of D2S6 below maximum attenuated disease levels at 16 weeks (p value of 0.00016). Patients receiving RGX-121 demonstrated an 86% median reduction in D2S6, approaching normal levels. Pivotal results were consistent with data from the dose-finding phase of CAMPSIITE, in which the majority of patients were shown to be exceeding expectations in neurodevelopmental function compared to natural history data up to four years.

The pivotal program used commercial-scale cGMP material from our proprietary, high-yielding NAVXpress process. In addition to measuring GAGs in the CSF, the trial will continue to collect neurodevelopmental data and caregiver-reported outcomes.

In June 2024, we announced the completion of a successful pre-BLA meeting for RGX-121, where we finalized details of our BLA with FDA. In that announcement, we shared that the FDA continues to be aligned with our plan to use CSF levels of HS D2S6 as a surrogate endpoint reasonably likely to predict clinical benefit to support accelerated approval of RGX-121. We expect an FDA inspection of our Manufacturing Innovation Center following the BLA submission for RGX-121.

In September 2024, we reported long term data showing that patients receiving RGX-121 at the pivotal dose level demonstrated an 85% median reduction of CSF levels of HS D2S6 approaching normal levels and sustained for up to two years. In the dose-finding part of the trial, investigators chose to discontinue standard-of-care intravenous enzyme replacement therapy (ERT) or to remain ERT-naïve for a majority of patients. At the pivotal dose level (dose level 3), 80% of patients were ERT-free at last time point, up to more than 18 months post-dosing. At dose level 2, 71% of patients were ERT-free at last time point, up to almost three years.

In January 2025, we announced a strategic partnership with Nippon Shinyaku to develop and commercialize RGX-121 in the United States and Asia. For more information, refer to "Nippon Shinyaku Partnership for MPS Diseases" below.

In March 2025, we submitted a BLA for RGX-121 seeking accelerated approval to the FDA, which we believe is likely to be eligible for priority review. We expect potential approval of RGX-121 in the second half of 2025.

Potential approval of the BLA for RGX-121 could result in receipt of a Rare Pediatric Disease Priority Review Voucher in 2025, assuming the statutory criteria are met. If approved, RGX-121 would be the first approved gene therapy and one-time treatment for MPS II.

RGX-111 for the Treatment of MPS I

RGX-111 is our investigational AAV Therapeutic for the treatment of MPS I, a rare disease caused by deficiency of IDUA, an enzyme required for the breakdown of polysaccharides in lysosomes. Similar to MPS II, many MPS I patients develop symptoms related to GAG accumulation in the CNS, which can include excessive accumulation of fluid in the brain, spinal cord compression and cognitive impairment. MPS I patients span a broad spectrum of disease severity and extent of CNS involvement. The severe form of MPS I is also referred to as Hurler syndrome. Hurler patients have two mutations in the IDUA gene, resulting in no active enzyme. These patients typically present with symptoms before two years of age and universally exhibit severe cognitive decline after an initial period of normal development. MPS I is estimated to occur in approximately 1 in 100,000 births worldwide. Based on global population, this equates to more than 1,000 MPS I patients born each year worldwide. The current standard of care for patients with an attenuated form of MPS I is a recombinant form of human IDUA, given as a weekly ERT infusion. This has demonstrated improvement in hepatosplenomegaly, growth, mobility and respiratory function. However, as the enzyme cannot cross the blood-brain barrier, ERT does not treat the CNS manifestations of MPS I. Patients are also often treated with hematopoietic stem cell transplantation (HSCT). Although this approach has demonstrated improvements in survival, growth, cardiac and respiratory function, mobility and intellect, it is also associated with clinically relevant morbidity and an estimated 10% to 20% mortality. Accordingly, the procedure is reserved for patients with severe disease before two years of age because the risk-benefit ratio is thought to be more favorable in younger patients who have not yet experienced advanced cognitive decline. Another critical limitation of HSCT is that cognitive decline continues for up to a year after transplant before stabilizing, leaving permanent cognitive deficits.

Overall, we believe the limitations of HSCT and ERT leave a significant unmet need for a method to safely achieve long-term IDUA reconstitution in the CNS for MPS I patients experiencing neurological complications. RGX-111 is designed to use the NAV AAV9 vector to deliver the human IDUA gene to the CNS. Delivery of the enzyme that is deficient within cells in the CNS could provide a permanent source of secreted IDUA beyond the blood-brain barrier, allowing for long-term cross-correction of cells throughout the CNS. We believe this strategy could also provide rapid IDUA delivery to the brain, potentially preventing the progression of cognitive deficits that otherwise occurs in MPS I patients. We have received orphan drug product designation, rare pediatric disease designation and fast track designation from the FDA, as well as orphan designation and ATMP classification from the EMA for RGX-111.

Clinical Development of RGX-111 for the Treatment of MPS I

A Phase I/II study evaluating RGX-111 initiated in 2017 and enrollment for that study completed in 2023. The multi-center, open-label, dose escalation trial evaluated the safety, tolerability and pharmacodynamics of RGX-111 delivered via single injection to the CNS to patients with MPS I. Interim results from the study, reported in February 2023, showed RGX-111 to be well tolerated across two dose levels in the eight patients enrolled in the Phase I/II trial as of January 17, 2023 and in the single-patient IND as of December 12, 2021, with no drug-related SAEs. Biomarker and neurodevelopmental assessments indicated an encouraging CNS profile in patients dosed with RGX-111.

In November 2023, future development of RGX-111 was largely halted as a result of a strategic pipeline prioritization and corporate restructuring. Efforts to continue development of RGX-111 are set to be reinitiated following our announcement in January 2025 of a strategic partnership with Nippon Shinyaku to develop and commercialize RGX-111 in the United States and Asia. For more information, refer to "Nippon Shinyaku Partnership for MPS Diseases" below.

Collaborations, Licensing and Company Formation

Collaborations, licensing and company formation are a key part of our commitment to enable the ongoing development of gene therapy treatments.

AbbVie Eye Care Collaboration

In September 2021, REGENXBIO and AbbVie announced a global strategic partnership to develop and commercialize ABBV-RGX-314, a potential one-time gene therapy for the treatment of wet AMD, DR and other chronic retinal diseases.

Under the terms of our Collaboration and License Agreement with AbbVie (the AbbVie Collaboration and License Agreement), we received an upfront payment of $370 million. Additionally, we are eligible to receive up to $1.38 billion in additional development, regulatory and commercial milestone payments.

In accordance with the AbbVie Collaboration and License Agreement, through December 31, 2022 we were responsible for development expenses related to certain ongoing clinical trials of ABBV-RGX-314 and the remaining ABBV-RGX-314 development expenses were shared with AbbVie. Beginning in 2023, AbbVie became responsible for the majority of the ABBV-RGX-314 development expenses.

In the United States, we will participate in commercialization of licensed products to the extent set forth in a commercialization plan to be determined in accordance with the AbbVie Collaboration and License Agreement, and the parties will equally share net profits and net losses associated with commercialization of the licensed products in the United States. Outside the United States, AbbVie will be responsible, at its sole cost, for the commercialization of licensed products. We will also be eligible to receive tiered royalties on net sales by AbbVie of licensed products outside the United States at percentages in the mid-teens to low twenties, subject to specified offsets and reductions.

We will lead the manufacturing of ABBV-RGX-314 for clinical development and U.S. commercial supply, and AbbVie will lead manufacturing of ABBV-RGX-314 for commercial supply outside the United States. Manufacturing expenses will be allocated between the parties in accordance with the terms of the AbbVie Collaboration and License Agreement and mutually agreed supply agreements.

Nippon Shinyaku Partnership for MPS Diseases

In January 2025, REGENXBIO and Nippon Shinyaku announced a strategic partnership to develop and commercialize RGX-121, a potential one-time gene therapy for the treatment of MPS II, and RGX-111, a potential one-time gene therapy for the treatment of MPS I, in the United States and certain countries in Asia. The Collaboration and License Agreement with Nippon Shinyaku (the Nippon Shinyaku Collaboration and License Agreement) became effective in March 2025.

Under the terms of the Nippon Shinyaku Collaboration and License Agreement, we will receive an upfront payment of $110 million. Additionally, we are eligible to receive up to $700 million in additional milestone payments, including $40 million in development and regulatory milestone payments and $660 million in sales milestones. We are eligible to receive meaningful double-digit royalties on net sales by Nippon Shinyaku of licensed products, subject to specified offsets and reductions. We retain all rights to, and one hundred percent of any potential proceeds related to the sale of, the Priority Review Voucher for RGX-121 received upon approval.

In accordance with the Nippon Shinyaku Collaboration and License Agreement, we will lead the development of RGX-121 and RGX-111 in the United States and Nippon Shinyaku will be responsible for development in licensed territories outside of the United States. We will lead the manufacturing of the licensed products for clinical development and commercial supply and reserve the right to develop and commercialize in countries outside of the licensed territories. Nippon Shinyaku will be responsible, at its sole cost, for the commercialization of licensed products. Manufacturing expenses will be allocated between the parties in accordance with the terms of the Nippon Shinyaku Collaboration and License Agreement and mutually agreed supply agreements.

NAV Technology Licensees

In addition to our internal product development efforts, we sublicense our NAV Vectors to other leading biotechnology and pharmaceutical companies. As of December 31, 2024, our NAV Technology Licensees are currently applying our NAV Technology Platform to a number of AAV Therapeutics over a broad range of therapeutic areas and disease indications. NAV Vectors have been extensively investigated in clinical trials registered in the National Institutes of Health (NIH) clinical trials database and are being used in one FDA-approved AAV Therapeutic in the United States (Novartis' Zolgensma). To date, thousands of patients have been treated by REGENXBIO and our NAV Technology Licensees using NAV Vectors across clinical trials, managed access and commercial settings.

Our NAV Technology Licensees are shown below.



We have also taken an active role in the formation of several of our NAV Technology Licensees, including being a founding shareholder in Dimension Therapeutics, Inc., Prevail Therapeutics Inc. and Corlieve Therapeutics SAS, all of which were acquired in strategic transactions since their formation. We entered into a license agreement with each of these NAV Technology Licensees upon their formation, for which we received equity in the NAV Technology Licensee in addition to other consideration.

NAV Technology licenses have been an important component of our strategy since REGENXBIO's formation, creating opportunity for the development of additional therapies for patients and potential for additional value generation from the platform. Equity ownership in certain NAV Technology Licensees has generated significant additional return for REGENXBIO shareholders, and we believe the acquisition of these NAV Technology Licensees in strategic transactions by biopharmaceutical companies is an important validation of the NAV Technology Platform.

Zolgensma License

In March 2014, we entered into an agreement with AveXis, Inc. (AveXis, now Novartis Gene Therapies) for an exclusive, worldwide commercial license, with rights to sublicense, to the NAV AAV9 vector for the treatment of SMA. In 2018, we amended the license to include additional intellectual property owned or in-licensed by us, including rights to the NAV Technology Platform beyond NAV AAV9, as well as additional AAV vectors we may discover or license for a certain period of time, for the treatment of SMA. Under the license agreement, as amended, we were entitled to receive over $270 million in fees, development and commercial milestones. In addition, we are entitled to receive mid-single to low double-digit royalties on net sales for Zolgensma or any product developed for the treatment of SMA using the NAV AAV9 vector. For any product developed for the treatment of SMA using a licensed vector other than NAV AAV9, we are entitled to receive a low double-digit royalty on net sales.

Novartis acquired AveXis for $8.7 billion in April 2018, and Zolgensma was subsequently approved by the FDA in May 2019. In December 2020, we sold a portion of our royalty rights from the net sales of Zolgensma to entities managed by Healthcare Royalty Management, LLC (HCR) for a gross purchase price of $200 million. As of December 31, 2024, Zolgensma is approved in 58 countries with more than 4,500 patients treated globally through clinical trials, early access programs and in the commercial setting. Novartis reported worldwide sales of Zolgensma of $1.21 billion in 2024.

Platform License Agreements and Other Licenses

Platform Licenses

We have exclusively licensed many of our rights in our NAV Technology Platform from the University of Pennsylvania (Penn) and GlaxoSmithKline LLC (GSK), which together we refer to as our Platform Licenses. We currently use our NAV Technology Platform to develop treatments in the areas of retinal, neuromuscular and neurodegenerative diseases. We also sublicense our NAV Technology Platform to third parties in order to develop and bring to market AAV Therapeutics for a range of severe diseases with significant unmet medical needs outside of our core disease indications and therapeutic areas.

The Trustees of the University of Pennsylvania. In February 2009, we entered into an exclusive, worldwide license agreement with Penn for patent and other intellectual property rights relating to a gene therapy technology platform based on AAVs discovered at Penn in the laboratory of James M. Wilson, M.D., Ph.D. This license was amended in September 2014, April 2016, April 2019, September 2020 and March 2022. In February 2009, we also entered into a sponsored research agreement with Penn (the 2009 SRA) under which we funded the nonclinical research of Dr. Wilson relating to AAV gene therapy and obtained an option to acquire an exclusive worldwide license in certain intellectual property created pursuant to such 2009 SRA. We entered into an additional sponsored research agreement (the 2013 SRA) with Penn in November 2013 which was funded entirely by our NAV Technology Licensee, Dimension Therapeutics, Inc. In December 2014, we entered into another SRA with Penn funding related nonclinical research of Dr. Wilson (the 2014 SRA).

Our license agreement with Penn, as amended, provides us with an exclusive, worldwide license under certain patents and patent applications in order to make, have made, use, import, offer for sale and sell products covered by the claims of the licensed patents and patent applications as well as all patentable inventions (to the extent they are or become available for license) that:

- were discovered by Dr. Wilson or other Penn researchers working under his direct supervision at Penn; and
- are related to the AAV technology platform discovered by Dr. Wilson at Penn prior to February 2009, pursuant to a sponsored research agreement or subsequent amendment to a sponsored research agreement; or
- are necessary or useful for the practice of Penn's patent rights in the treatment of CLN2 disease, a form of Batten disease, and conceived and reduced to practice since October 2015; and
- are owned and controlled by Penn.

Prior to entering into the license agreement with us, Penn had entered into two license agreements with third parties with respect to certain of the licensed patents and patent applications. Our license from Penn is subject to those preexisting license grants in addition to certain other retained fields. With respect to the first third-party license granted by Penn, our license is non-exclusive with respect to the patents and patent applications licensed to the third party for so long as that preexisting license grant remains in effect and will become exclusive upon the expiration or termination of that existing license agreement. The pre-existing licenses also include a license agreement Penn entered into with GSK in May 2002, granting a license to certain patents and patent applications, of which we subsequently sublicensed certain rights to from GSK in March 2009. For further information regarding our GSK sublicense, please see "Platform License Agreements and Other Licenses—Platform Licenses—GlaxoSmithKline LLC" located elsewhere in this Annual Report on Form 10-K. Our license agreement with Penn provides that should the rights Penn licensed to GSK ever revert to Penn, such rights shall automatically be included in our license agreement with Penn.

The Penn license agreement, as amended, also provides us with certain additional rights, including a non-exclusive, worldwide license to use (i) all data and information that was developed since October 2015 by Dr. Wilson, or other Penn researchers working under his direct supervision at Penn, that is related to Batten disease, owned by Penn, and necessary or useful for the practice of the licensed patent rights in the treatment of CLN2 disease; and (ii) all know-how that:

- was developed by Dr. Wilson, or other Penn researchers working under his direct supervision at Penn; and
- is related to the AAV technology platform discovered by Dr. Wilson prior to September 2014; or
- is related to the AAV technology platform discovered by Dr. Wilson at Penn after September 2014 during the performance of a research program we sponsored; and
- is owned by Penn; and
- is necessary or useful for the practice of the licensed patent rights.

Under the terms of the Penn license agreement, we issued equity to Penn and are also obligated to pay Penn:

- up to $20.5 million upon the achievement of various development and sales-based milestones, of which $0.5 million have been paid to date;
- low- to mid-single digit royalties on net sales of licensed pharmaceutical products sold by us or our affiliates;
- low-single digit to low-double digit royalty percentages of net sales on licensed products intended for research purposes only;
- low- to mid-double digit royalty percentage on royalties received from third parties on net sales of licensed pharmaceutical products by such third parties;
- certain sublicense fees, of which $6.0 million remain outstanding as of December 31, 2024; and
- reimbursements for ongoing patent prosecution and maintenance expenses.

Our Penn license agreement, as amended, will terminate with respect to licensed products in a field of use other than the treatment of familial hypercholesterolemia (FH) on a product-by-product and country-by-country basis on the date each particular licensed product ceases to be covered by at least one valid claim, issued or pending, under the licensed patent rights. We can terminate this license agreement by giving Penn prior written notice. Penn has the right to terminate:

- with notice if we are late in paying money due under the license agreement;
- with notice if we fail to achieve a diligence event on or before the applicable completion date or otherwise breach the license agreement;

•if we or our affiliates experience insolvency; or

•if we commence any action against Penn to declare or render any claim of the licensed patent rights invalid or unenforceable.

Under the 2014 SRA, as amended, we funded research at Penn, paid certain intellectual property legal and filing expenses and received the rights to certain research results. The Penn license agreement, as amended, and the 2014 SRA, as amended, provide that all patentable inventions conceived, created, or conceived and reduced to practice pursuant to the 2014 SRA, together with patent rights represented by or issuing from the U.S. patents and patent applications, including provisional patent applications, automatically become exclusively licensed to us and all research results become automatically licensed to us as know-how. Under the 2009 SRA, as amended, in consideration for our funding of research at Penn, we received an option to acquire a worldwide license on commercially reasonable terms to practice all patentable inventions conceived, created, or reduced to practice pursuant to the 2009 SRA, together with patent rights represented by or issuing from the U.S. patents and patent applications, including provisional patent applications.

GlaxoSmithKline LLC. In March 2009, we entered into a license agreement with GSK, which was amended in April 2009, in order to secure the exclusive rights to patents and patent applications covering NAV Technology that GSK had previously licensed from Penn (subject to certain rights retained by GSK and Penn). Under this GSK license agreement, we receive an exclusive, worldwide sublicense under the licensed patent rights to make, have made, use, import, sell and offer for sale products covered by the licensed patent rights anywhere in the world. Our rights under this GSK license agreement are subject to certain rights retained by GSK for the benefit of itself and other third parties, including rights relating to: domain antibodies; RNA interference and antisense drugs; internal research purposes and GSK's discovery research efforts with non-profit organizations and GSK collaborators; AAV8 for the treatment of hemophilia B; AAV9 for the treatment of Muscular Dystrophy, congestive heart failure suffered by Muscular Dystrophy patients and cardiovascular diseases by delivery of certain genes; and non-commercial research in the areas of Muscular Dystrophy, hemophilia B, congestive heart failure suffered by Muscular Dystrophy patients, and other cardiovascular disease. Under the terms of the license agreement, we issued equity to GSK and are obligated to pay GSK:

•up to $1.5 million in aggregate milestone payments, all of which have been paid;

•low- to mid-double digit percentages of any sublicense fees we receive from sublicensees for the licensed intellectual property rights; and

•reimbursements for certain patent prosecution and maintenance expenses.

In addition, under our GSK license agreement, we are obligated to pay low- to mid-single digit royalty percentages on net sales of licensed products. This payment has been assigned by GSK to Penn such that any royalties we are obligated to pay under the GSK license agreement will be paid to Penn rather than GSK. Under our GSK license agreement, we are required to use commercially reasonable efforts to develop and commercialize licensed products. Our GSK license agreement will terminate upon the expiration, lapse, abandonment or invalidation of the last licensed claim to expire, lapse, become abandoned or unenforceable in all the countries of the world where the licensed patent rights existed. The last to expire patent under the GSK license agreement, absent patent term extension, is January 2026. We may terminate this license agreement for any reason upon a specified number of days' written notice. GSK can terminate this license agreement if:

•we are late in paying GSK any money due under the agreement and do not pay in full within a specified number of days of GSK's written demand;

•we materially breach the agreement and fail to cure within a specified number of days; or

•we file for bankruptcy.

We have been notified of a potential dispute with GSK over the amount of sublicense fees paid by us to GSK under the GSK license agreement. We disagree with GSK's interpretation of the GSK license agreement. For additional information, please see Note 8, "Commitments and Contingencies" to the accompanying audited consolidated financial statements.

Other Licenses

Regents of the University of Minnesota. In November 2014, we entered into a license agreement with Regents of the University of Minnesota (Minnesota) for the exclusive rights to Minnesota's undivided interest in intellectual property jointly owned by Minnesota and us relating to the delivery of AAV vectors to the CNS. This license was amended in November 2016 and September 2021. Under this Minnesota license agreement, as amended, we receive an exclusive license under the licensed patent rights to make, have made, use, offer to sell or sell, offer to lease or lease, import or otherwise offer to dispose or dispose of products covered by the licensed patent rights in all fields of use in any country or territory in which a licensed patent has been issued and is unexpired or a licensed patent application is pending until November 2019, after which time the field of use would be limited to all fields of use using

our NAV Vectors in addition to certain additional indications and areas. Under the terms of the agreement, we are obligated to pay Minnesota upfront fees, annual maintenance fees, royalties on net sales, if any, sublicense fees and fees upon the achievement of various milestones.

Emory University. In August 2018, we entered into a license agreement with Emory University (Emory) for the exclusive rights to Emory's undivided interest in intellectual property jointly owned by Emory and us relating to the delivery of AAV vectors to the CNS. Under this Emory license agreement, we receive an exclusive license under the licensed patent rights to make, have made, use, import, offer to sell or sell licensed products in all fields of use in any country. Under the terms of the agreement, we are obligated to pay Emory an upfront fee, annual maintenance fees under certain circumstances, royalties on net sales, sublicense fees, and fees upon the achievement of various milestones for the first licensed product.

Clearside Biomedical, Inc. In August 2019, we entered into an option and license agreement with Clearside for the option to receive an exclusive, worldwide commercial license, with rights to sublicense, to Clearside's SCS Microinjector for the delivery of AAV gene therapies for the treatment of wet AMD, DR, and other conditions for which chronic anti-VEGF treatment is currently the standard of care. In October 2019, we exercised the option. This option and license agreement was amended in January and September 2023. Under the terms of the agreement, as amended, we are obligated to pay Clearside an upfront fee, royalties on net sales, and fees upon the achievement of various milestones. As between us and Clearside, we will be responsible for all development, regulatory and commercialization activities for our gene therapy product candidates. Clearside will be responsible for supplying the SCS Microinjector in support of our preclinical studies, clinical studies and commercial use.

Johns Hopkins University. In June 2022, we entered into a license agreement with Johns Hopkins University (JHU) for the exclusive rights to JHU's undivided interest in intellectual property jointly owned by JHU and us relating to suprachoroidal delivery of anti-VEGF vectors. Under this JHU license agreement, we receive an exclusive license under the licensed patent rights to make, have made, use, import, export, offer to sell and sell licensed products in all fields of use in any country. Under the terms of the agreement, we are obligated to pay JHU an upfront fee, royalties on net sales, minimum annual royalties, sublicense fees and fees upon the achievement of various milestones for the first two licensed products. Additionally, the Company is obligated to pay for certain costs incurred related to the maintenance of the licensed patents.

Intellectual Property

Our patent portfolio includes patents and patent applications that we own, co-own and license from third parties and covers all aspects of our NAV Technology Platform, clinical candidates and programs, formulations, devices, manufacturing and research programs. We believe this patent portfolio enables us to support our development of AAV Therapeutics to address significant unmet medical needs.

NAV Technology Platform

As of December 31, 2024, our patent portfolio included 25 issued U.S. patents and five European patents relating to the AAV7, AAV8, AAV9, AAVrh10 and AAVrh46 vectors and their uses. These patents have terms that will expire as late as 2027, not including patent term extensions.

Our Investigational AAV Therapeutics

As of December 31, 2024, in addition to the patents related to our NAV Technology Platform described above, our patent portfolio included a total of six issued U.S. patents, one issued European patent, two pending International Patent applications filed pursuant to the Patent Cooperation Treaty (PCTs) and 22 PCTs that have entered national stage relating to our product candidates, which are described below:

Retinal Diseases

In addition to our NAV Technology Platform patents covering the NAV AAV8 vector and manufacture of NAV AAV8 vectors used in our retinal disease programs, our patent portfolio includes more recent filings relating to our clinical candidate vectors, clinical protocols, routes of administration to the eye (subretinal and suprachoroidal), formulations and target diseases treated by our gene therapy vectors.

Our patent portfolio relating to ABBV-RGX-314 supports our clinical development and our collaboration with AbbVie for the clinical development of ABBV-RGX-314. Our patent portfolio covers the use of ABBV-RGX-314 for the treatment of wet AMD through subretinal or suprachoroidal administration and for the treatment of DR through suprachoroidal administration; it also covers formulations and devices used for suprachoroidal administration. Our patent portfolio relating to ABBV-RGX-314 includes one issued U.S. patent that will expire in 2037, one pending PCT and seven PCTs that have entered national stage for which any issued U.S. or European patent would expire in 2037, 2038, 2039, 2040 or 2043, in each case without taking into account any possible patent term adjustment or extension.

Neuromuscular Diseases

In addition to our NAV Technology Platform patents covering the NAV AAV8 vector and its manufacture, our patent portfolio includes more recent filings relating to RGX-202, the NAV AAV8 capsid carrying our microdystrophin construct used to treat Duchenne and the manufacture of RGX-202. Our patent portfolio also covers other AAV vectors carrying our microdystrophin transgene, as well as intravenous and other modes of administration, formulations and bioanalytical assays.

Our patent portfolio relating to RGX-202 includes five pending PCTs that have entered the national stage for which any issued U.S. or European patent would expire in 2040, 2042 or 2043 without taking into account any possible patent term adjustment or extension.

Neurodegenerative Diseases

In addition to our NAV Technology Platform patents covering the NAV AAV9 vector and the manufacture of NAV AAV9 vector used in our neurodegenerative disease program, our RGX-121 patent portfolio includes more recent filings that cover our clinical candidate vector, routes of administration used in our neurodegenerative disease clinical-stage program (intracisternal administration for intrathecal delivery, as well as lumbar puncture and intraventricular administration), formulations and clinical protocols.

Our patent portfolio relating to RGX-121 includes one issued U.S. patent that will expire in 2034, one issued U.S. patent that will expire in 2038, one issued U.S. patent that will expire in 2039, one issued U.S. patent that will expire in 2040, one issued U.S. patent that will expire in 2042, one issued European patent that will expire in 2036, one pending PCT application and ten PCTs that have entered national stage for which any issued U.S. or European patents would expire in 2034, 2036, 2037, 2038, 2039, 2041 or 2042, in each case without taking into account any possible patent term adjustment or extension.

Manufacturing

Our patent portfolio covers aspects of our manufacturing processes which support our ability to perform large scale manufacturing, increase yield and purity of AAV vector products and meet clinical supply requirements.

Our patent portfolio also includes protection for novel validation and potency assays that further support and streamline our manufacturing processes.

Customers

Our revenues for the years ended December 31, 2024, 2023 and 2022 consisted solely of license and royalty revenue. One customer (Novartis Gene Therapies) accounted for approximately 98%, 95% and 90% of our total revenues for the years ended December 31, 2024, 2023 and 2022, respectively. We expect future license and royalty revenue to continue to be derived from a limited number of licensees. Future license and royalty revenue is uncertain due to the contingent nature of our licenses granted to third parties and may fluctuate significantly from period to period.

Competition

We are aware of a number of companies focused on developing and commercializing gene therapies in various disease indications, including 4D Molecular Therapeutics, Inc., Adverum Biotechnologies, Inc., Amicus Therapeutics, Inc., Astellas Pharma, BioMarin Pharmaceutical, Inc., MeiraGTx Limited, Novartis AG, Passage Bio, Inc., PTC Therapeutics, Inc., Roche, Sanofi, Sarepta Therapeutics, Inc., Solid Biosciences, Inc., Taysha Gene Therapies, Inc., Tenaya Therapeutics, Inc., Ultragenyx Pharmaceutical Inc. and uniQure N.V., as well as a number of companies addressing other methods for modifying genes and regulating gene expression. Additionally, we have sublicensed our NAV Technology Platform for developing gene therapies in various disease indications to our NAV Technology Licensees. Not only must we compete with other companies that are focused on gene therapy products using earlier generation AAV technology and other gene therapy platforms, but any products that we may commercialize will have to compete with existing therapies and new therapies that may become available in the future.

There are other organizations working to improve existing therapies or to develop new therapies for our initially selected disease indications. Depending on how successful these efforts are, it is possible they may increase the barriers to adoption and success for our product candidates, if approved. These efforts include the following:

•**Wet AMD.** Marketed competition for wet AMD largely consists of anti-VEGF therapies developed by Roche/Genentech, Inc. (Lucentis, Susvimo, Vabysmo), Regeneron Pharmaceuticals, Inc. (Eylea, Eylea HD) and Novartis (Beovu). Lucentis biosimilars (Biogen - Byooviz, Coherus Biosciences - Cimerli) are also now marketed. The first Eylea biosimilar has also launched in the United States (Amgen - Pavblu). Companies with products in development for the treatment of wet AMD include, but may not be limited to, 4D Molecular Therapeutics, Adverum, Avirmax Bio, Exegenesis Bio, Eyepoint Pharmaceuticals, Kodiak Sciences, Inc., Ocular Therapeutix, Opthea, Outlook Therapeutics, Inc. and Skyline Therapeutics.

•**DR.** Currently marketed anti-VEGF competition for DR with DME include Novartis (Beovu), Roche/Genentech (Lucentis, Vabysmo), Regeneron (Eylea, Eylea HD) and Coherus Biosciences (Cimerli). Companies with products in development for the treatment of DR with DME include, but may not be limited to, 4D Molecular Therapeutics, Eyepoint, Kodiak Sciences, Oculis, and Roche. The principal marketed anti-VEGF competition for DR without DME is Roche/Genentech (Lucentis) and Regeneron (Eylea, Eylea HD). Companies with products in development for the treatment of DR without DME include, but may not be limited to, Kodiak Sciences, Novartis, Ocular Therapeutix and Roche.

•**DMD.** The principal marketed competition for the treatment of DMD is a gene therapy marketed by Sarepta/Roche (Elevidys). Currently marketed exon skipping competition for DMD includes Sarepta (Exondys, Vyondys, Amondys) and Nippon Shinyaku Co., Ltd. (Viltepso). There are two principal competitive gene therapy products in clinical development from Solid Biosciences (SGT-003) and Genethon (GNT0004). Other companies with gene therapies in early development for DMD include, but may not be limited to, Ultragenyx, Insmed, Huidagene, Novartis and Vertex.

•**MPS II.** The principal marketed competition for the treatment of MPS II is a systemic enzyme replacement therapy marketed by Takeda Pharmaceutical Company, Ltd. and Sanofi (Elaprase). Two additional products are marketed in select geographies in Asia by JCR Pharmaceuticals Co., Ltd. (Izcargo) and GC Pharma (Hunterase ICV). Companies with products in development for the treatment of MPS II include, but may not be limited to, Denali Therapeutics Inc., Esteve and Immusoft.

Many of our competitors, either alone or with their strategic partners, have substantially greater financial, technical and human resources than we do. Our competitors may be more successful than us in obtaining approval for treatments and achieving widespread market acceptance. Our competitors' treatments may be more effective, or more effectively marketed and sold, than any treatment we may commercialize and may render our treatments obsolete or non-competitive before we can recover the expenses of developing and commercializing any of our treatments.

Mergers and acquisitions in the biotechnology and pharmaceutical industries may result in even more resources being concentrated among a smaller number of our competitors. 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. Smaller or early-stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies.

We anticipate that we will face intense and increasing competition as new drugs and treatments enter the market and advanced technologies become available. We expect any treatments that we develop and commercialize to compete on the basis of, among other things, efficacy, safety, convenience of administration and delivery, price, level of biosimilar competition and availability of reimbursement from government and other third-party payors.

Government Regulation

In the United States, biological products, including gene therapy products, are subject to regulation under the Federal Food, Drug, and Cosmetic Act (FD&C Act), and the Public Health Service Act (PHS Act) and other federal, state, local and foreign statutes and regulations. Both the FD&C Act and the PHS Act and their corresponding regulations govern, among other things, the testing, manufacturing, safety, efficacy, labeling, packaging, storage, record keeping, distribution, reporting, advertising and other promotional practices involving biological products. Applications to the FDA are required before conducting clinical testing of biological products, and each clinical study protocol for a gene therapy product is reviewed by the FDA.

Within the FDA, the Center for Biologics Evaluation and Research (CBER) regulates gene therapy products as biological products. The FDA has published guidance documents related to, among other things, gene therapy products in general, their preclinical assessment, observing subjects involved in gene therapy studies for delayed adverse events, potency testing, and chemistry, manufacturing and control information in gene therapy INDs.

Ethical, scientific, social and legal concerns about gene therapy, genetic testing and genetic research could result in additional regulations restricting or prohibiting the processes we may use. Federal and state agencies, congressional committees and foreign governments have expressed interest in further regulating biotechnology. More restrictive regulations or claims that our products 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 our product candidates under development. In addition, while the FDA currently considers all gene therapy products to be biological products, this classification could come under scrutiny in the future, and it is possible that some gene therapies could be regulated as drug products (requiring a new drug application rather than a BLA for marketing).

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.

U.S. Biological Products Development Process

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

•completion of nonclinical laboratory tests, including evaluations of product chemistry, formulations, toxicity in animal studies in accordance with good laboratory practice (GLP) and applicable requirements for the humane use of laboratory animals or other applicable regulations;

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

•performance of adequate and well-controlled human clinical studies according to the FDA's requirements for good clinical practice (GCP) and additional requirements for the protection of human research subjects and their health information, to establish the safety and efficacy of the proposed biological product for its intended use;

•submission to the FDA of a BLA for marketing approval that includes substantive evidence of safety, purity, and potency from results of nonclinical testing and clinical studies, as well as information on the chemistry, manufacturing and controls to ensure product identity and quality, and proposed labeling;

•satisfactory completion of an FDA inspection of the manufacturing facility or facilities where the biological product is produced to assess compliance with cGMP, to assure that the facilities, methods and controls are adequate to preserve the biological 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;

•potential FDA inspection of the nonclinical and clinical study sites and the clinical study sponsor that generated the data in support of the BLA; and

•FDA review and approval, or licensure, of the BLA.

The clinical study sponsor must submit the results of the preclinical tests, together with manufacturing information, analytical data, any available clinical data or literature and a proposed clinical protocol, to the FDA as part of the IND. Some preclinical testing may continue even after the IND is submitted. The IND automatically becomes effective 30 days after receipt by the FDA, unless the FDA places the clinical study on a clinical hold within that 30-day time period. In such a case, the IND sponsor and the FDA must resolve any outstanding concerns before the clinical study can begin. The FDA may also impose clinical holds on a biological product candidate at any time before or during clinical studies due to safety concerns or non-compliance. If the FDA imposes a clinical hold, studies may not recommence without FDA authorization and then only under terms authorized by the FDA. Accordingly, we cannot be sure that submission of an IND will result in the FDA allowing clinical studies to begin, or that, once begun, issues will not arise that suspend or terminate such studies.

Clinical studies involve the administration of the biological product candidate to healthy volunteers or patients under the supervision of qualified investigators, generally physicians not employed by or under the study sponsor's control. Clinical studies are conducted under protocols detailing, among other things, the objectives of the clinical study, dosing procedures, subject selection and exclusion criteria, and the parameters to be used to monitor subject safety, including stopping rules that assure a clinical study will be stopped if certain adverse events should occur. Each protocol and any amendments to the protocol must be submitted to the FDA as

part of the IND. Clinical studies must be conducted and monitored in accordance with the FDA's regulations imposing the GCP requirements, including the requirement that all research subjects provide informed consent. Further, each clinical study must be reviewed and approved by an independent institutional review board (IRB) at or servicing each institution at which the clinical study will be conducted. An IRB is charged with protecting the welfare and rights of study participants and considers such items as whether the risks to individuals participating in the clinical studies are minimized and are reasonable in relation to anticipated benefits. The IRB also approves the form and content of the informed consent that must be signed by each clinical study subject or his or her legal representative and must monitor the clinical study until completed. Clinical studies generally also must be reviewed by an institutional biosafety committee (IBC), a local institutional committee that reviews and oversees basic and clinical research conducted at that institution. The IBC assesses the safety of the research and identifies any potential risk to public health or the environment. Some studies also employ a Data and Safety Monitoring Board (DSMB), which operates with independence from the study sponsor and has access to unblinded study data during the course of the study and may halt a study for ethical reasons such as undue safety risks.

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

•Phase I. The biological product is initially introduced into healthy human subjects and tested for safety. However, in the case of some products for rare, severe or life-threatening diseases, the initial human testing is often conducted in patients.

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

•Phase III. Clinical studies are undertaken to further evaluate dosage, clinical efficacy, potency, and safety in an expanded patient population at geographically dispersed clinical study sites. These clinical studies are intended to establish the overall risk/benefit ratio of the product and provide an adequate basis for product approval and labeling. Post-approval clinical studies, sometimes referred to as Phase IV clinical studies, may be conducted after initial marketing approval. These clinical studies are used to gain additional experience from the treatment of patients in the intended therapeutic indication, particularly for long-term safety follow-up. In some cases, Phase IV studies may be required by the FDA as a condition of approval. The FDA recommends that sponsors observe subjects for potential gene therapy-related delayed adverse events for as long as 15 years.

During all phases of clinical development, regulatory agencies require extensive monitoring and auditing of all clinical activities, clinical data, and clinical study investigators. Annual progress reports detailing the results of the clinical studies must be submitted to the FDA. Written IND safety reports must be promptly submitted to the FDA and the investigators for serious and unexpected adverse events, any findings from other studies, tests in laboratory animals or in vitro testing that suggest a significant risk for human subjects, or any clinically important increase in the rate of a serious suspected adverse reaction over that listed in the protocol or investigator brochure. The sponsor must submit an IND safety report within 15 calendar days after the sponsor determines that the information qualifies for expedited reporting. The sponsor also must notify the FDA of any unexpected fatal or life-threatening suspected adverse reaction within seven calendar days after the sponsor's initial receipt of the information. Phase I, Phase II and Phase III clinical studies may not be completed successfully within any specified period, if at all. The FDA or the sponsor or its DSMB may suspend a clinical study at any time on various grounds, including a finding that the research subjects or patients are being exposed to an unacceptable health risk. Similarly, an IRB can suspend or terminate approval of a clinical study at its institution if the clinical study is not being conducted in accordance with the IRB's requirements or if the biological product has been associated with unexpected serious harm to patients.

Human gene therapy products are a new category of therapeutics. Because this is a relatively new and expanding area of novel therapeutic interventions, there can be no assurance as to the length of the study period, the number of patients the FDA will require to be enrolled in the studies in order to establish the safety, efficacy, purity and potency of human gene therapy products, our ability to recruit sufficient numbers of study subjects for any trial, or that the data generated in these studies will be acceptable to the FDA to support marketing approval.

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

U.S. Review and Approval Processes

After the completion of clinical studies of a biological product, FDA approval of a BLA must be obtained before commercial marketing of the biological product. The BLA must include results of product development, laboratory and animal studies, human studies, information on the manufacture and composition of the product, proposed labeling and other relevant information. Under the Prescription Drug User Fee Act (PDUFA), the BLA must be accompanied by a substantial user fee payment unless an exception or waiver applies. In addition, under the Pediatric Research Equity Act (PREA), a BLA or supplement to a BLA must contain data to assess the safety and effectiveness of the biological product for the claimed indications in all relevant pediatric subpopulations and to support dosing and administration for each pediatric subpopulation for which the product is safe and effective. The FDA may grant deferrals for submission of pediatric data or full or partial waivers of pediatric requirements. Unless otherwise required by regulation, PREA does not apply to any biological product for an indication for which orphan designation has been granted. The testing and approval processes require substantial time and effort and there can be no assurance that the FDA will accept the BLA for filing and, even if filed, that any approval will be granted on a timely basis, if at all.

Within 60 days following submission of the application, the FDA reviews a BLA submitted to determine if it is substantially complete before the agency accepts it for filing. The FDA may refuse to file any BLA that it deems incomplete or not properly reviewable at the time of submission and may request additional information. In this event, the BLA must be resubmitted with the additional information. The resubmitted application also is subject to review before the FDA accepts it for filing. Once the submission is accepted for filing, the FDA begins an in-depth substantive review of the BLA. The FDA reviews the BLA to determine, among other things, whether the proposed product is safe and potent, including whether it is effective, for its intended use, and has an acceptable purity profile, and whether the product is being manufactured in accordance with cGMP to assure and preserve the product's identity, strength, quality, potency and purity as those factors relate to the safety or effectiveness of the product. The FDA may refer applications for novel biological products or biological products that present difficult questions of safety or efficacy to an advisory committee, typically a panel that includes clinicians and other experts, for review, evaluation and a recommendation as to whether the application should be approved and under what conditions. The FDA is not bound by the recommendations of an advisory committee, but it considers such recommendations carefully when making decisions. During the biological product approval process, the FDA also will determine whether a Risk Evaluation and Mitigation Strategy (REMS) is necessary to assure the safe use of the biological product upon marketing. If the FDA concludes a REMS is needed, the sponsor of the BLA must submit a proposed REMS; the FDA will not approve the BLA without a REMS, if required.

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

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

If a product receives regulatory approval, the approval may be significantly limited to specific diseases and dosages or the indications for use may otherwise be limited, which could restrict the commercial value of the product. Further, the FDA may require that certain contraindications, warnings or precautions be included in the product labeling. The FDA may impose restrictions and conditions on product distribution, prescribing, or dispensing in the form of a REMS, or otherwise limit the scope of any approval. In addition, the FDA may require post marketing clinical studies designed to further assess a biological product's safety and effectiveness, and testing and surveillance programs to monitor the safety of approved products that have been commercialized.

For new molecular entities, one of the performance goals agreed to by the FDA under PDUFA is to review 90% of standard BLAs within 10 months of the 60-day filing date and 90% of priority BLAs in six months of the 60-day filing date, whereupon a review decision is to be made. The FDA does not always meet its PDUFA goal dates for standard and priority BLAs and its review goals are subject to change from time to time. The review process and the PDUFA goal date may be extended by three months if the FDA requests or the BLA sponsor otherwise provides additional information, or clarification regarding information already provided in the submission, constituting a major amendment to the BLA.

Orphan Drug Designation

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

If a product that has orphan designation subsequently receives the first FDA approval for that product for the disease or condition for which it has such designation, the product is entitled to orphan product exclusivity, which means that the FDA may not approve any other applications to market the same drug or biological product for the same indication for seven years, except in limited circumstances, such as a showing of clinical superiority to the product with orphan exclusivity. Competitors, however, may receive approval of different products for the indication for which the orphan product has exclusivity or obtain approval for the same product but for a different indication for which the orphan product has exclusivity. Orphan product exclusivity also could block the approval of one of our products for seven years if a competitor obtains approval of the same biological product as defined by the FDA or if our product candidate is determined to be contained within the competitor's product for the same indication or disease. If a drug or biological product designated as an orphan product receives marketing approval for an indication broader than what is designated, it may not be entitled to orphan product exclusivity. Orphan drug status in the European Union (EU) has similar, but not identical, benefits.

Orphan drug products are also eligible for Rare Pediatric Disease Designation if greater than 50% of patients living with the disease are under age 18. A priority review voucher will be given to the sponsor of a product with a Rare Pediatric Disease Designation at the time of product approval that is transferable to another company.

Expedited Development and Review Programs

The FDA has a Fast Track program that is intended to expedite or facilitate the process for development and review of new drugs and biological products, including precision drugs or biological products, that meet certain criteria. Specifically, new drugs and biological 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 and the specific indication for which it is being studied. The sponsor of a new drug or biologic may request the FDA to designate the drug or biologic as a Fast Track product at any time during the clinical development of the product.

Any product submitted to the FDA for marketing, including under a Fast Track program, may be eligible for other types of FDA programs intended to expedite development and review, such as Breakthrough Therapy designation, priority review, and accelerated approval. Under the Breakthrough Therapy program, products intended to treat a serious or life-threatening disease or condition may be eligible for additional benefits when preliminary clinical evidence demonstrates that such product may have substantial improvement on one or more clinically significant endpoints over available therapies. Products with a Breakthrough Therapy designation are eligible for the benefits of Breakthrough Therapy, and the FDA will seek to ensure the sponsor of a breakthrough therapy product receives timely advice and interactive communications to help the sponsor design and conduct a development program as efficiently as possible. In addition, gene therapies, including genetically modified cells, that lead to a sustained effect on cells or tissues, may be eligible for regenerative medicine advanced therapy (RMAT) designation. Products with an RMAT designation are eligible for the benefits of Breakthrough Therapy in addition to allowing the sponsor the ability to participate in meetings with the FDA to discuss whether accelerated approval would be appropriate based on surrogate or intermediate endpoints reasonably likely to predict long-term clinical benefit.

A product with Fast Track, Breakthrough Therapy, or RMAT designation program may be eligible for "rolling review," which means the FDA may consider for review sections of the BLA on a rolling basis before the complete application is submitted, if the sponsor provides a schedule for the submission of the sections of the application, the FDA agrees to accept sections of the application and determines that the schedule is acceptable, and the sponsor pays any required user fees upon submission of the first section of the application.

A BLA may be eligible for priority review if the product has the potential to provide safe and effective therapy where no satisfactory alternative therapy exists or a significant improvement in the treatment, diagnosis or prevention of a serious or life-threatening disease or condition compared to marketed products. Specific priority review programs exist for material threat medical countermeasures, rare pediatric diseases and tropical diseases. The FDA will attempt to direct additional resources to the evaluation of an application for a new drug or biological product designated for priority review in an effort to facilitate the review, in accordance with FDA guidance.

Additionally, a product may be eligible for accelerated approval. Drug or biological products studied for their safety and effectiveness in treating serious or life-threatening illnesses may be eligible for accelerated approval, which means that they may be approved on the basis of 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 that can be measured earlier than irreversible morbidity or mortality or other clinical benefit, that is reasonably likely to predict an effect on irreversible morbidity or mortality or other clinical benefit, taking into account the severity, rarity or prevalence of the condition and the availability or lack of alternative treatments. Drugs granted accelerated approval generally provide a meaningful therapeutic advantage to patients over existing treatments. As a condition of approval, the FDA will often require that a sponsor of a drug or biological product receiving accelerated approval perform additional post-approval confirmatory trials to verify and describe the clinical benefit of the medicine. Any such confirmatory trial must be completed with due diligence and FDA may require that the trial be underway prior to approval. In addition, the FDA currently requires as a condition for accelerated approval pre-submission of promotional materials, which could adversely impact the timing of the commercial launch of the product.

The expedited programs, in general, do not change the standards for approval. Rather, these programs are intended to expedite the development and approval process, but they do not necessarily accomplish that intent.

Post-Approval Requirements

Maintaining substantial compliance with applicable federal, state, and local statutes and regulations requires the expenditure of substantial time and financial resources. Rigorous and extensive FDA regulation of biological products continues after approval, particularly with respect to cGMP. Accordingly, we and our CDMOs will be required to comply with applicable requirements in the cGMP regulations, including quality control and quality assurance and maintenance of records and documentation, with respect to the manufacturing and distribution of any of our product candidates that receive regulatory approval. Other post-approval requirements applicable to biological products, include reporting of cGMP deviations that may affect the identity, strength, quality, potency, or purity of a distributed product in a manner that may impact the safety or effectiveness of the product, record-keeping requirements, reporting of adverse effects, reporting updated safety and efficacy information, and complying with electronic record and signature requirements. After a BLA is approved, the product also may be subject to official lot release. As part of the manufacturing process, manufacturers are required to perform certain tests on each lot of the product before it is released for distribution. If the product is subject to official release by the FDA, the manufacturer submits samples of each lot of product to the FDA together with a release protocol showing a summary of the history of manufacture of the lot and the results of all of the manufacturer's tests performed on the lot. The FDA also may perform certain confirmatory tests on lots of some products, such as viral vaccines, before releasing the lots for distribution by the manufacturer. In addition, the FDA conducts laboratory research related to the regulatory standards on the safety, purity, potency, and effectiveness of biological products. We and our CDMOs will also be required to comply with all relevant FDA requirements and regulations and any applicable international agency regulatory requirements in our continued manufacturing and promotion of our approved products.

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

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

U.S. Patent Term Restoration and Marketing Exclusivity

Depending upon the timing, duration and specifics of the FDA approval of the use of our product candidates, some of our U.S. patents may be eligible for limited patent term extension under the Drug Price Competition and Patent Term Restoration Act of 1984, commonly referred to as the Hatch-Waxman Amendments. The Hatch-Waxman Amendments permit a patent restoration term of up to five years as compensation for patent term lost during product development and the FDA regulatory review process. However, patent term restoration cannot extend the remaining term of a patent beyond a total of 14 years from the product's approval date. The patent term restoration period is generally one-half the time between the effective date of an IND and the submission date of a BLA plus the time between the submission date of a BLA and the approval of that application. Only one patent applicable to an approved biological product is eligible for the extension and the application for the extension must be submitted prior to the expiration of the patent. The U.S. Patent and Trademark Office, in consultation with the FDA, reviews and approves the application for any patent term extension or restoration. In the future, we may apply for restoration of patent term for one of our currently owned or licensed patents to add patent life beyond its current expiration date, depending on the expected length of the clinical studies and other factors involved in the filing of the relevant BLA.

A biological product can obtain pediatric market exclusivity in the United States. Pediatric exclusivity, if granted in the case of a biologic approved under a BLA, adds six months to existing exclusivity periods. This six-month exclusivity, which runs from the end of other exclusivity protection, may be granted based on the voluntary completion of a pediatric study in accordance with an FDA-issued "Written Request" for such a study.

The Patient Protection and Affordable Care Act (PPACA) signed into law on March 23, 2010, includes a subtitle called the Biologics Price Competition and Innovation Act of 2009, which created an abbreviated approval pathway for biological products shown to be similar to, or interchangeable with, an FDA-licensed reference biological product. This amendment to the PHS Act attempts to minimize duplicative testing. Biosimilarity, which requires that there be no clinically meaningful differences between the biological product and the reference product in terms of safety, purity, and potency, can be shown through analytical studies, animal studies, and a clinical study or studies. Interchangeability requires that a product is biosimilar to the reference product and the product must demonstrate that it can be expected to produce the same clinical results as the reference product and, for products administered multiple times, 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. However, complexities associated with the larger, and often more complex, structure of biological products, as well as the process by which such products are manufactured, pose significant hurdles to interchangeability approval.

A reference biologic is granted 12 years of exclusivity from the time of first licensure of the reference product. The first biologic product submitted under the abbreviated approval pathway that is determined to be interchangeable with the reference product has exclusivity against other biologics submitting under the abbreviated approval pathway for the lesser of (i) one year after the first commercial marketing, (ii) 18 months after approval if there is no legal challenge, (iii) 18 months after the resolution in the applicant's favor of a lawsuit challenging the biologics' patents if an application has been submitted, or (iv) 42 months after the application has been approved if a lawsuit is ongoing within the 42-month period.

Additional Regulation

In addition to the foregoing, state and federal laws regarding environmental protection and hazardous substances, including the Occupational Safety and Health Act, the Resource Conservancy and Recovery Act and the Toxic Substances Control Act, affect our business. These and other laws govern our use, handling and disposal of various biological, chemical and radioactive substances used in, and wastes generated by, our 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. We believe that we are in material compliance with applicable environmental laws and that continued compliance therewith will not have a material adverse effect on our business. We cannot predict, however, how changes in these laws may affect our future operations. Equivalent laws have been adopted in other countries that impose similar obligations.

Other U.S. Healthcare Laws and Regulations

Healthcare providers, physicians and third-party payors play a primary role in the recommendation and use of pharmaceutical products that are granted marketing approval. Arrangements with third-party payors, existing or potential customers and referral sources are subject to broadly applicable fraud and abuse and other healthcare laws and regulations, and these laws and regulations may constrain the business or financial arrangements and relationships through which manufacturers market, sell and distribute the products for which they obtain marketing approval. Such restrictions under applicable federal and state healthcare laws and regulations include the following:

- the federal Anti-Kickback Statute, which prohibits, among other things, persons from knowingly and willfully soliciting, receiving, offering or paying remuneration, directly or indirectly, in cash or kind, in exchange for, or to induce, 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 healthcare programs such as the Medicare and Medicaid programs. This statute has been interpreted to apply to arrangements between pharmaceutical manufacturers, on the one hand, and prescribers, patients, purchasers and formulary managers on the other. PPACA amends the intent requirement of the federal Anti-Kickback Statute. A person or entity no longer needs to have actual knowledge of this statute or specific intent to violate it;

- the federal False Claims Act (FCA), which prohibits, among other things, individuals or entities from knowingly presenting, or causing to be presented, claims for payment from Medicare, Medicaid or other federal healthcare programs that are false or fraudulent. Federal Anti-Kickback Statute violations and certain marketing practices, including off-label promotion, also may implicate the FCA;

- federal criminal laws that prohibit executing a scheme to defraud any healthcare benefit program or making false statements relating to healthcare matters;

- the federal Physician Payment Sunshine Act, which requires certain manufacturers of drugs, devices, biologics and medical supplies to report annually to the Centers for Medicare & Medicaid Services (CMS) information related to payments and other transfers of value to physicians and teaching hospitals, and ownership and investment interests held by physicians and their immediate family members;

- the Health Insurance Portability and Accountability Act of 1996 (HIPAA) imposes criminal and civil liability for executing 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, which governs the conduct of certain electronic healthcare transactions and protects the security and privacy of protected health information; and

- state and foreign law equivalents of each of the above federal laws, such as anti-kickback and false claims laws which may apply to: items or services reimbursed by any third-party payor, including commercial insurers; 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 or otherwise restrict payments that may be made to healthcare providers and other potential referral sources; state laws that require drug manufacturers to report information related to payments and other transfers of value to physicians and other healthcare providers or marketing expenditures; and state laws governing the privacy and security of health information in certain circumstances. Many of these state and foreign laws differ from federal law and from each other in significant ways and may not have the same effect, thus complicating compliance efforts.

Violation of any of the laws described above or any other governmental laws and regulations may result in penalties, including civil and criminal penalties, damages, fines, the curtailment or restructuring of operations, the exclusion from participation in federal and state healthcare programs and imprisonment. Furthermore, efforts to ensure that business activities and business arrangements comply with applicable healthcare laws and regulations can be costly for manufacturers of branded prescription products.

Coverage and Reimbursement

Significant uncertainty exists as to the coverage and reimbursement status of any products for which we may obtain regulatory approval. In the United States and markets in other countries, sales of any product candidates for which regulatory approval for commercial sale is obtained will depend in part on the availability of coverage and adequate reimbursement from third-party payors. Third-party payors include government authorities, managed care providers, private health insurers and other organizations. The process for determining whether a payor will provide coverage for a drug product may be separate from the process for setting the reimbursement rate that the payor will pay for the drug product. Third-party payors may limit coverage to specific drug products on an approved list, or formulary, which might not include all FDA-approved drugs for a particular indication. Moreover, a payor's decision to provide coverage for a drug product does not imply that an adequate reimbursement rate will be approved.

Third-party payors are increasingly challenging the price and examining the medical necessity and cost-effectiveness of medical products and services, in addition to their safety and efficacy. New metrics frequently are used as the basis for reimbursement rates, such as average sales price, average manufacturer price and actual acquisition cost. In order to obtain coverage and reimbursement for any product that might be approved for sale, it may be necessary to conduct expensive pharmacoeconomic studies in order to demonstrate the medical necessity and cost-effectiveness of the products, in addition to the costs required to obtain regulatory approvals. If third-party payors do not consider a product to be cost-effective compared to other available therapies, they may not cover the product after approval as a benefit under their plans or, if they do, the level of payment may not be sufficient to allow a company to sell its products at a profit. Health Technology Assessment which is intended to take account of medical, social, economic and ethical issues when determining the suitability of a medicinal product for reimbursement has increasingly become an element of the pricing and reimbursement decisions of the competent authorities in EU Member States.

The U.S. government, state legislatures and foreign governments have shown significant interest in implementing cost containment programs to limit the growth of government-paid health care costs, including price controls, restrictions on reimbursement and requirements for substitution of generic products for branded prescription drugs. By way of example, the enactment of the Inflation Reduction Act (IRA) in August 2022 includes significant changes to potential Medicare drug product reimbursement through government negotiation of certain drug prices, as well as manufacturer discount and inflation rebate obligations. Adoption of government controls and measures, and tightening of restrictive policies in jurisdictions with existing controls and measures, could limit payments for pharmaceuticals.

U.S. Foreign Corrupt Practices Act

The U.S. Foreign Corrupt Practices Act (FCPA), to which we are subject, prohibits corporations and individuals from engaging in bribery and corruption when dealing with foreign government officials. It is illegal to pay, offer to pay, promise or authorize the payment of money or anything of value, directly or indirectly, to any foreign government official, political party or political candidate in an attempt to secure an improper advantage in order to obtain or retain business or to otherwise improperly influence a foreign official in his or her official capacity. Comparable laws have been adopted in other countries that impose similar obligations. We are also subject to the FCPA's accounting provisions, which require us to keep accurate books and records and to maintain a system of internal accounting controls sufficient to assure management's control, authority, and responsibility over our assets. The failure to comply with the FCPA and similar laws could result in civil or criminal sanctions or other adverse consequences.

Government Regulation Outside of the United States

In addition to regulations in the United States, we will be subject to a variety of regulations in other jurisdictions governing, among other things, clinical studies and any commercial sales and distribution of our products. Because biologically sourced raw materials are subject to unique contamination risks, their use may be restricted in some countries.

Whether or not we obtain FDA approval for a product, we must obtain the requisite approvals from regulatory authorities in foreign countries prior to the commencement of clinical studies or marketing of the product in those countries. Many countries outside of the United States have a similar process that requires the submission of a clinical study application much like the IND prior to the commencement of human clinical studies.

In the EU, for example, clinical trials are governed by the EU Regulation on Clinical Trials (Reg. EU No. 536/2014), or CTR, which became applicable in January 2022 and established a centralized process of obtaining competent authority approval for clinical trials in the EU. Under the CTR, trial sponsors submit their application for approval via an EU Portal. The approvals are still granted by the competent authorities of each EU Member State where the trial takes place; however, the procedure for approval is conducted in a coordinated manner among the concerned EU Member States as provided under the CTR. While the process for the application and granting of the approvals was streamlined, it remains a complex process that can significantly delay the start of a multinational clinical trial.

In the United Kingdom of Great Britain and Northern Ireland (UK), clinical trials are governed by the Medicines for Human Use (Clinical Trials) Regulations 2004 (UK CTRs). Under the UK CTRs, an approval is required from the Medicines and Healthcare products Regulatory Agency (MHRA) together with a positive ethics committee opinion. Clinical trials which take place in the UK and on NHS hospital sites, typically do so on the basis of standardized documentation which set out indemnification provisions. In the UK, proposals to replace the current UK CTRs with revised legislation (New UK CTRs) were laid before UK Parliament in January 2025 and propose changes with respect to transparency, approval pathways and regulatory requirements. The New UK CTRs will be debated in Parliament and, after a 12-month implementation period, are due to come into force in early 2026.

To obtain regulatory approval of a biological medicinal product in the EU, we must submit a marketing authorization application. 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 (EC) No 1394/2007 on advanced therapy medicinal products, read in combination with Directive 2001/83/EC on the Community code relating to medicinal products for human use and Regulation (EC) No 726/2004 laying

down Community procedures for the authorization and supervision of medicinal products for human and veterinary use and establishing the EMA. Regulation (EC) No 1394/2007 lays down specific rules concerning the authorization, supervision and pharmacovigilance of gene therapy medicinal products, somatic cell therapy medicinal products and tissue engineered products. The EMA's Committee for Advanced Therapies (CAT) is responsible for assessing the quality, safety and efficacy of ATMP. ATMP include gene therapy medicinal products, somatic cell therapy medicinal products and tissue engineered products. The role of the CAT is to prepare a draft opinion on an application for marketing authorization for an ATMP candidate that is submitted to the EMA. The EMA then provides a final opinion regarding the application for marketing authorization. The European Commission grants or refuses marketing authorization after the EMA has delivered its opinion.

Innovative medicinal products are authorized in the EU on the basis of a full marketing authorization application (as opposed to an application for marketing authorization that relies, in whole or in part, on data in the marketing authorization dossier for another, previously approved medicinal product). Applications for marketing authorization for innovative medicinal products must contain the results of pharmaceutical tests, preclinical tests and clinical trials conducted with the medicinal product for which marketing authorization is sought. Innovative medicinal products for which marketing authorization is granted are entitled to eight years of data exclusivity. During this period, applicants for approval of generics or biosimilars of these innovative products cannot rely on data contained in the marketing authorization dossier submitted for the innovative medicinal product to support their application. Innovative medicinal products for which marketing authorization is granted are also entitled to ten years of market exclusivity. During these ten years of market exclusivity, no generic or biosimilar medicinal product may be placed on the EU market even if a marketing authorization application for approval of a generic or biosimilar of the innovative product has been submitted to the EMA or to the competent regulatory authorities in the EU Member States and marketing authorization has been granted. The ten years of market exclusivity 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 their authorization, are held to bring a significant clinical benefit in comparison with existing therapies. However, there is no guarantee that a product will be considered by the EU's regulatory authorities to be an innovative medicinal product which is eligible for the relevant periods of data and market exclusivity.

Products authorized as "orphan medicinal products" in the EU are entitled to benefits additional to those granted in relation to innovative medicinal products. In accordance with Article 3 of Regulation (EC) No 141/2000 on orphan medicinal products, a medicinal product may be designated as an orphan medicinal product if (i) it is intended for the diagnosis, prevention or treatment of a life-threatening or chronically debilitating condition; (ii) either (a) such condition affects no more than five in 10,000 persons in the EU when the application is made, or (b) the product, without the incentives derived from orphan medicinal product status, would not generate sufficient return in the EU to justify investment; and (iii) there exists no satisfactory method of diagnosis, prevention or treatment of such condition authorized for marketing in the EU, or if such a method exists, the product will be of significant benefit to those affected by the condition. Further guidance on such criteria is provided in Regulation (EC) No 847/2000 laying down the provisions for implementation of the criteria for designation of a medicinal product as an orphan medicinal product and definitions of the concepts "similar medicinal product" and "clinical superiority". Orphan medicinal products are eligible for financial incentives such as reduction of fees or fee waivers and following grant of a marketing authorization, the EMA and the EU Member States' competent authorities are not permitted to accept another application for a marketing authorization, or grant a marketing authorization or accept an application to extend an existing marketing authorization, for the same therapeutic indication of a similar medicinal product for ten years following grant or authorization. The application for orphan drug designation must be submitted before the application for marketing authorization. The applicant may receive a fee reduction for the marketing authorization application if the orphan drug designation has been granted, but not if the designation is still pending at the time the marketing authorization is submitted. Orphan drug designation does not convey any advantage in, or shorten the duration of, the regulatory review and approval process.

The 10-year market exclusivity that an orphan drug enjoys may 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 designation, for example, if the product is sufficiently profitable not to justify maintenance of market exclusivity. Additionally, marketing authorization may be granted to a similar product during the 10-year period of market exclusivity for the same therapeutic indication at any time if:

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

Similar to obligations imposed in the United States, medicinal products authorized in the EU may be subject to post-authorization obligations, including the obligation to conduct Post Marketing Safety Studies (PASS) or Post Marketing Efficacy Studies (PAES).

In April 2024, the European Parliament adopted its position on the European Commission's proposal for a new Directive and a new Regulation, which would revise and replace the existing general pharmaceutical legislation. The proposed changes include a proposal to recast Directive 2001/83/EC, i.e. the Community code on medicinal products and the creation of a new Regulation laying down EU marketing authorization of medicinal products that will replace Regulation (EC) No 726/2004, Regulation (EC) No 141/2000 on orphan drugs and Regulation (EC) No 1901/2006 on pediatric medicines, and amend Regulation (EC) No 1394/2007 on ATMP and Regulation (EU) No 536/2014, i.e. the CTR. The proposals include significant changes to the EU pharmaceutical regulatory scheme, in particular with regard to the document protection and market exclusivity periods for medicinal products. Further changes may be expected while the legislative process continues.

Reimbursement for medicinal products is still an area that is not harmonized in the EU and is largely governed by EU Member States' laws. However, there are some EU level legal frameworks that must be taken into account, including Directive 89/105/EEC (the Price Transparency Directive). The aim of the Price Transparency Directive is to ensure that pricing and reimbursement mechanisms established in EU Member States are transparent and objective, do not hinder the free movement and trade of medicinal products in the EU and do not hinder, prevent or distort competition on the market. The Price Transparency Directive does not, however, provide any guidance concerning the specific criteria on the basis of which pricing and reimbursement decisions are to be made in individual EU Member States. Neither does it have any direct consequence for pricing or levels of reimbursement in individual EU Member States. The national authorities of the individual EU Member States are free to restrict the range of medicinal products for which their national health insurance systems provide reimbursement and to control the prices and/or reimbursement of medicinal products for human use. Individual EU Member States adopt policies according to which a specific price or level of reimbursement is approved for the medicinal product. Other EU Member States adopt a system of reference pricing, basing the price or reimbursement level in their territory either on the pricing and reimbursement levels in other countries, or on the pricing and reimbursement levels of medicinal products intended for the same therapeutic indication. Furthermore, some EU Member States impose direct or indirect controls on the profitability of the company placing the medicinal product on the market.

In 2011, Directive 2011/24/EU on the application of patients' rights in cross-border healthcare was adopted at the EU level. The Directive is intended to establish rules for facilitating access to safe and high-quality cross-border healthcare in the EU.

Health Technology Assessment (HTA) of medicinal products is becoming an increasingly common part of the pricing and reimbursement procedures in some EU Member States. HTA is the procedure according to which the assessment of the public health impact, therapeutic impact and the economic and societal impact of the use of a given medicinal product in the national healthcare systems of the individual country is conducted. HTA generally focuses on the clinical efficacy and effectiveness, safety, cost, and cost-effectiveness of individual medicinal products as well as their potential implications for the national healthcare system. Those elements of medicinal products are compared with other treatment options available on the market.

The outcome of HTA may influence the pricing and reimbursement status for specific medicinal products within individual EU member states. The extent to which pricing and reimbursement decisions are influenced by the HTA of a specific medicinal product vary between the EU Member States.

A new Regulation on HTA on EU level was adopted in December 2021: Regulation (EU) 2021/2282 on health technology assessment (the HTA Regulation) and applies from January 12, 2025. The HTA Regulation covers new medicines and certain new medical devices. Member states will be able to use common HTA tools, methodologies and procedures across the EU, working together in four main areas: (i) joint clinical assessments focusing on the most innovative health technologies with the most potential impact for patients; (ii) joint scientific consultations whereby developers can seek advice from HTA authorities; (iii) identification of emerging health technologies to identify promising technologies early; and (iv) continuing voluntary cooperation in other areas. Individual member states will continue to be responsible for assessing non-clinical (e.g., economic, social, ethical) aspects of health technology, and making decisions on pricing and reimbursement. For other countries outside of the EU, such as countries in Eastern Europe, Latin America or Asia, the requirements governing the conduct of clinical studies, product licensing, pricing and reimbursement vary from country to country. In all cases, again, the clinical studies are conducted in accordance with GCP and the applicable regulatory requirements and the ethical principles that have their origin in the Declaration of Helsinki.

The UK formally left the EU on January 31, 2020 and a transitional period applied until the UK's withdrawal from the EU became fully effective on December 31, 2020. As of January 1, 2021, the UK is a "third country" with respect to the EU (subject to the terms of the EU UK Trade Agreement), and EU law ceased to apply directly in the UK. However, the UK has retained the EU regulatory regime with certain modifications as standalone UK legislation, while the EU medicines laws remain applicable in Northern Ireland pursuant to the Northern Ireland Protocol. Therefore, the UK regulatory regime is currently substantially similar to EU regulations, but under the Medicines and Medical Devices Act 2021, the UK may adopt changed regulations for medicines, including their research, development and commercialization. Currently, new regulations with respect to clinical trials are due to come into force in 2026, as referred to above (the New UK CTRs).

In Great Britain, gene therapy medicinal products are classified as advanced therapy medicinal products. In order to place an advanced therapy medicinal product on the Great Britain market, a person must hold a marketing authorization for the medicinal product. There are various routes to applying for a marketing authorization in Great Britain. These include a national application for a

medicinal product, either on a 150-day assessment procedure or on a rolling review basis. From 1 January 2024, the UK has adopted an international recognition procedure which is a route open to applicants that have already received an authorization for the same product from one of the MHRA reference regulators. Reference regulators include the European Medicines Agency and the FDA. Under the international recognition procedure, there are two recognition routes (Route A and Route B). Advanced therapy medicinal products must follow Route B, which sets out a 110-day timetable, which runs from the date on which the submission has been validated by the MHRA. In order to make an application for a marketing authorization, the applicant must be established in the UK or the EU/EEA.

In the UK, following implementation of the Windsor Framework (an agreement which made changes to the Northern Ireland Protocol), from January 1, 2025, medicinal products may be designated as an orphan drug in the UK and will be valid UK-wide if the medicine meets criteria similar to those set out in European legislation. Unlike in the EU, there is no need to obtain orphan designation before the application for authorization is made, instead the criteria will be assessed with the application.

If we fail to comply with applicable foreign regulatory requirements, we may be subject to, among other things, fines, suspension or withdrawal of regulatory approvals, product recalls, seizure of products, operating restrictions and criminal prosecution.

Human Capital Resources

As of March 7, 2025, we employed 353 full-time employees, of which 285 were engaged in research and development activities, including preclinical, clinical and manufacturing related functions, and 68 were engaged in general administrative activities, including commercial, corporate development, finance, legal, human resources, information technology, facilities and other general and administrative functions. We have never had a work stoppage, and none of our employees are represented by a labor organization or under any collective bargaining arrangements. We consider our relationship with our employees to be good.

Talent, Growth and Retention

We appreciate the importance of retention, growth and development of our employees. We seek and value employees who have substantial experience in the discovery, development, manufacture and commercialization of innovative therapies in a complex regulatory environment. For certain key functions, especially in research and development and manufacturing activities, we require specialized scientific and gene therapy expertise. To attract and retain the talent we require, we believe we offer competitive compensation, including salary, cash incentive awards 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. All full-time employees are eligible to participate in the same health and welfare and retirement savings plans. Additionally, we provide professional development programs and on-demand learning opportunities to cultivate talent at all levels throughout our company.

Equal Opportunity

We believe that a diverse, equitable and inclusive culture fosters innovation, which is integral to our mission of improving lives through the curative potential of gene therapy. We have worked to ensure equal opportunity in all aspects of employment and appropriate merit-based representation of gender, race and ethnicity throughout our company. We emphasize an inclusive environment and equitable treatment as an important part of our company culture.

Available Information

Our principal offices are located at 9804 Medical Center Drive, Rockville, MD 20850, and our telephone number is (240) 552-8181. Our website address is www.regenxbio.com. The information contained in, or that can be accessed through, our website is not a part of, or incorporated by reference in, this Annual Report on Form 10-K. We file annual, quarterly, and current reports, proxy statements, and other documents with the SEC under the Exchange Act. You may obtain any reports, proxy and information statements, and other information that we file electronically with the SEC at www.sec.gov.

You also may view and download copies of our SEC filings free of charge at our website as soon as reasonably practicable after we electronically file such material with, or furnish it to, the SEC. The information contained on, or that can be accessed through, our website will not be deemed to be incorporated by reference in, and is not considered part of, this Annual Report on Form 10-K. Investors should also note that we use our website, as well as SEC filings, press releases, public conference calls and webcasts, to announce financial information and other material developments regarding our business. We use these channels, as well as any social media channels listed on our website, to communicate with investors and members of the public about our business. It is possible that the information that we post on our social media channels could be deemed material information. Therefore, we encourage investors, the media and others interested in our company to review the information that we post on our social media channels.

ITEM 1A. *RISK FACTORS*

You should carefully consider the risk factors set forth below as well as the other information contained in this Annual Report on Form 10-K and in our other public filings in evaluating our business. Any of the following risks could materially and adversely affect our business, financial condition or results of operations. In addition, these risks could cause actual results and developments to differ materially and adversely from those projected in the forward-looking statements contained in this Annual Report on Form 10-K (please read the Information Regarding Forward-Looking Statements appearing at the beginning of this Form 10-K). Additional risks and uncertainties not currently known to us or that we currently view to be immaterial may also have a material adverse effect on our business, financial condition or results of operations. In these circumstances, the market price of our common stock would likely decline and you could lose all or part of your investment.

Risk Factor Summary

Risks Related to Our NAV Technology Platform and the Development of Our Product Candidates

- It is difficult to predict the time and cost of development and of obtaining regulatory approval for our product candidates.
- Our business depends substantially on the success of our lead product candidates.
- We have limited clinical results for some of our product candidates.
- Regulatory authorities may not consider the endpoints of our clinical trials to provide clinically meaningful results.
- The results from our preclinical studies or clinical trials for our product candidates may not support as broad a marketing approval as we seek, and we may be required to conduct additional clinical trials or evaluate subjects for a follow-up period.
- We may encounter substantial delays in our planned clinical trials, or we may fail to demonstrate safety and efficacy to the satisfaction of applicable regulatory authorities.
- We may be negatively impacted if the results of our planned clinical trials are inconclusive or if there are safety concerns or serious adverse events associated with our product candidates.
- Undesirable side effects may delay or prevent our product candidates and those of our licensees or collaborators from obtaining regulatory approval, limit their commercial potential or result in significant negative consequences following approval.
- We cannot predict when, or if, we will obtain regulatory approval to commercialize a product candidate.

Risks Related to Our Financial Position

- We face significant competition and there is a possibility that our competitors may achieve regulatory approval before us or develop products that are safer, less expensive or more convenient or effective than ours.
- We expect to regularly incur losses for the foreseeable future and may never again achieve or maintain profitability.
- Failure to obtain additional funding when needed may force us to delay, limit or terminate certain of our licensing activities, product development efforts or other operations.
- We have never generated revenue from sales of our product candidates and may never do so in the future.

Risks Related to Third Parties

- If third parties do not meet our deadlines, our preclinical and clinical development programs could be delayed or unsuccessful.
- If our licensing arrangements or collaborations are not successful, our business could be harmed.

Risks Related to Manufacturing

- Products intended for use in gene therapies are novel, complex and difficult to manufacture.
- Delays in obtaining regulatory approval of our manufacturing process or disruptions in our manufacturing process may delay or disrupt our commercialization efforts.
- Third parties we rely upon to conduct our product manufacturing may not perform satisfactorily.

•We are required to comply with ongoing manufacturing regulatory requirements.

Risks Related to the Commercialization of Our Product Candidates

•If we are unable to establish sales and marketing capabilities or enter into agreements with third parties to market and sell our product candidates, if approved, we may be unable to generate any product revenue.

•We may not achieve our projected timelines that we announced.

•Even if we receive regulatory approval, we still may not be able to successfully commercialize our product candidates.

•Failure to obtain or maintain adequate insurance coverage and reimbursement for our products, if approved, or the imposition of price controls or other forms of pricing regulation could limit our ability to market those products and decrease our ability to generate product revenue.

Risks Related to Our Business Operations

•We may not be successful in our efforts to identify or discover additional product candidates.

•Our future success depends on our ability to retain key employees, consultants and advisors and to attract qualified personnel.

•We may face liability for our conduct and that of our employees, principal investigators, consultants or commercial partners.

•We may face product liability lawsuits.

•We could become subject to fines or penalties related to the failure to comply with environmental, health and safety laws.

•We and our collaborators or other contractors or consultants may suffer cybersecurity breaches.

•Our customers are concentrated and therefore the loss of a significant customer may harm our business.

Risks Related to Our Intellectual Property

•Our intellectual property rights may be limited by the terms and conditions of licenses granted to us by others.

•We must obtain and maintain patent protection for our products and technology to protect our intellectual property rights.

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

•Our intellectual property licenses with third parties may be subject to disagreements.

•We are required to comply with the agreements under which we license intellectual property rights from third parties.

•We may not be successful in obtaining necessary rights to our product candidates through acquisitions and in-licenses.

•We may not be able to protect our intellectual property rights in the United States and throughout the world.

•Issued patents covering our NAV Technology Platform or our product candidates could be found invalid or unenforceable.

•Third parties may initiate legal proceedings alleging that we are infringing their intellectual property rights.

•We may be subject to intellectual property claims.

•Changes in U.S. patent law could diminish the value of patents in general, thereby impairing our ability to protect our products.

•We may be unable to obtain patent term extension and data exclusivity for our product candidates.

Risks Related to Ownership of Our Common Stock

•Our operating results are difficult to predict and could cause the price of our common stock to fluctuate substantially.

•Raising additional capital may cause dilution to our existing stockholders, restrict our operations or require us to relinquish proprietary rights.

•Strategic partnerships and any other arrangements or acquisitions that we effect in the future may increase our capital requirements, dilute our stockholders, cause us to incur debt or assume contingent liabilities and subject us to other risks.

•Provisions in our certificate of incorporation and bylaws might discourage, delay or prevent a change in control.

•Our certificate of incorporation includes exclusive forum clauses for certain litigation.

•Our business could be negatively affected as a result of the actions of activist stockholders.

Risks Related to our NAV Technology Platform and the Development of Our Product Candidates

Our gene therapy product candidates are based on a novel technology that makes it difficult to predict the time and cost of development and of subsequently obtaining regulatory approval. Only a few gene therapy products have been approved in the United States, the European Union or elsewhere.

We have concentrated our research and development efforts on our proprietary AAV gene delivery platform (our NAV Technology Platform), and we have granted licenses to certain intellectual property related to our NAV Technology Platform to our NAV Technology Licensees. Our future success depends on our and our NAV Technology Licensees' successful development and commercialization of viable gene therapy product candidates. There can be no assurance that we or our NAV Technology Licensees will not experience problems or delays in developing current or future product candidates or that such problems or delays will not cause unanticipated costs, or that any such development problems can be solved. We also may experience unanticipated problems or delays in expanding our manufacturing capacity, and this may prevent us from completing our clinical trials, meeting the obligations of our collaborations or commercializing our products on a timely or profitable basis, if at all. For example, we, a partner or another group may uncover one or more previously unknown risks associated with AAV or our NAV Technology Platform, and this may prolong the period of observation required for obtaining regulatory approval, necessitate additional clinical testing or invalidate our NAV Technology.

In addition, the clinical trial requirements of the FDA, the EMA and other regulatory authorities and the criteria these regulators use to determine the quality, safety and efficacy of a product candidate vary substantially according to the type, complexity, novelty and intended use and market of such product candidates. The regulatory approval process for novel product candidates such as ours can be significantly more expensive and take longer than for other, better known or more extensively studied product candidates. Only a few gene therapy products have been approved in the United States, the European Union or elsewhere. It is difficult to determine how long it will take or how much it will cost to obtain regulatory approvals for our product candidates in the United States, the European Union or elsewhere, or how long it will take to commercialize our product candidates, if approved. Furthermore, approvals by one regulatory authority may not be indicative of what other regulatory authorities may require for approval, and approvals of *ex vivo* gene therapy products may not be indicative of what may be required for approval of *in vivo* gene therapy products.

Regulatory requirements governing gene and cell therapy products have changed frequently and may continue to change in the future. Additionally, we may seek regulatory approval in territories outside the United States and the European Union, which may have their own regulatory authorities along with frequently changing requirements or guidelines. The regulatory review committees and advisory groups in the United States, the European Union and elsewhere, 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 our product candidates or lead to significant post-approval limitations or restrictions. As we advance our product candidates, 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 certain of our product candidates. These additional processes may result in a review and approval process that is longer than we otherwise would have expected. 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 product revenue, and our business, financial condition, results of operations and prospects would be materially harmed.

Our business depends substantially on the success of our lead product candidates. If we are unable to obtain regulatory approval for, or successfully commercialize, our lead product candidates, our business will be materially harmed.

Our product candidates will require substantial clinical development and testing, manufacturing bridging studies and process validation and regulatory approval prior to commercialization. Successful continued development and ultimate regulatory approval of our lead product candidates is critical for our future business success and our ability to generate product revenue. We have invested, and will continue to invest, a significant portion of our financial resources in the development of our lead product candidates. We will need to raise sufficient funds for, and successfully complete, our clinical trials of our lead product candidates in appropriate subjects. The future regulatory and commercial success of these product candidates is subject to a number of risks, including the following:

•we may not have sufficient financial and other resources or patient availability to complete the necessary clinical trials for our lead product candidates;

•we may not be able to provide evidence of quality, efficacy and safety for our lead product candidates;

•we do not know the degree to which our lead product candidates will be accepted by patients, the medical community and third-party payors as a therapy for the respective diseases to which they relate, even if approved;

•the results of our clinical trials may not meet the level of statistical or clinical significance required by the FDA, EMA or comparable foreign regulatory bodies for marketing approval, and modifications to the design of our clinical trials could delay their enrollment, commencement or completion;

•subjects in our clinical trials may die or suffer other adverse effects for reasons that may or may not be related to our lead product candidates;

•subjects in clinical trials undertaken by our licensees or collaborators, or undertaken by others using AAV, may die or suffer other adverse effects for reasons that may or may not be related to our NAV Technology Platform or AAV;

•certain patients' immune systems might prohibit the successful delivery of certain gene therapy products to the target tissue, thereby limiting the treatment outcomes;

•we may not successfully establish commercial manufacturing capabilities;

•if approved for treatment of the expected conditions, our lead product candidates will likely compete with other treatments then available, including the off-label use of products already approved for marketing and other therapies currently available or which may be developed;

•our products and products developed by our licensees and collaborators may not maintain a continued acceptable safety profile following regulatory approval;

•we may not maintain compliance with post-approval regulation and other requirements; and

•we may not be able to obtain, maintain or enforce our rights under our licensed patents and other intellectual property rights.

Of the large number of biologics and drugs in development in the biopharmaceutical industry, only a small percentage result in the submission of a BLA to the FDA or marketing authorization application (MAA) to the EMA and even fewer are approved for commercialization. Furthermore, even if we do receive regulatory approval to market our lead product candidates, any such approval may be subject to limitations on the indicated uses for which we may market the product. Accordingly, even if we are able to obtain the requisite financing to continue to fund our development programs, we cannot assure you that our lead product candidates will be successfully developed or commercialized. If we or any of our future development partners are unable to develop, obtain regulatory approval for, or, if approved, successfully commercialize, our lead product candidates, we may not be able to generate sufficient revenue to continue our business.

We have limited clinical results for some of our product candidates and success in preclinical studies or early clinical trials may not be indicative of results obtained in later trials.

Gene therapy development has inherent risks. Some of our lead product candidates have limited clinical and preclinical results and we may experience unexpected results in the future. We or any of our future development partners will be required to demonstrate through adequate and well-controlled clinical trials that our product candidates containing our proprietary vectors are safe and effective, with a favorable benefit-risk profile, for use in their target indications before we can seek regulatory approvals for their commercial sale. Drug development is a long, expensive and uncertain process, and delay or failure can occur at any stage of development, including after commencement of any of our clinical trials.

The results of preclinical studies and early clinical trials are not always predictive of future results. Any product candidate we or any of our future development partners advance into clinical trials, including our lead product candidates, may not have favorable results in later clinical trials, if any, or receive regulatory approval. There is a high failure rate for drugs and biologic products proceeding through clinical trials. Data obtained from preclinical and clinical activities are subject to varying interpretations that may delay, limit or prevent regulatory approval. In addition, we may experience regulatory delays or rejections as a result of many factors, including due to changes in regulatory policy during the period of our product candidate development. Any such delays could materially harm our business, financial condition, results of operations and prospects.

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

During the FDA review process, we will need to identify success criteria and endpoints such that the FDA will be able to subsequently evaluate the clinical efficacy and safety profile of our product candidates. As we are developing novel treatments for diseases in which there is little clinical experience with new endpoints and methodologies, there is heightened risk that the FDA, the

EMA or other regulatory bodies may not consider the clinical trial endpoints that we select to provide clinically meaningful results (reflecting a tangible benefit to patients). In addition, the resulting clinical data and results may be difficult to analyze. Even if the FDA 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. Further, even if we do achieve the pre-specified criteria, we may produce results that are unpredictable or inconsistent with the results of the non-primary endpoints or other relevant data. The FDA also weighs the benefits of a product against its risks, and the FDA may view the efficacy results in the context of safety as not being supportive of regulatory approval. The EMA and other regulatory authorities in the European Union and other countries may make similar comments with respect to these endpoints and data, which may jeopardize or preclude our ability to obtain regulatory approvals in the European Union and other jurisdictions.

The results from our preclinical studies or clinical trials for our product candidates may not support as broad a marketing approval as we seek, and the FDA, the EMA or other regulatory authorities may require us to conduct additional clinical trials or evaluate subjects for an additional follow-up period.

While we believe our product candidates should be applicable for the treatment of patients with certain conditions, the results from our preclinical and planned clinical trials may not support as broad of a marketing approval as we seek. Even if we obtain regulatory approval for our product candidates, we may be required by the FDA, the EMA or other regulatory bodies to conduct additional clinical trials to support approval of our product candidates for patients diagnosed with different mutations of the respective diseases to which our product candidates relate. This could result in significant cost increases and substantial delays in obtaining, or never obtaining, marketing approval for our product candidates to treat patients. The inability to market our product candidates to treat patients for the intended indications would materially harm our business, financial condition, results of operations and prospects.

We may encounter substantial delays in our planned clinical trials, or we may fail to demonstrate safety and efficacy to the satisfaction of applicable regulatory authorities.

Before obtaining marketing approval from regulatory authorities for the sale of our product candidates, we must conduct extensive clinical trials to demonstrate their safety and efficacy. Clinical testing is expensive, time-consuming and uncertain as to outcome. A failure of one or more clinical trials can occur at any stage of testing. Events that may prevent successful or timely commencement and completion of preclinical and clinical development include:

- delays in reaching a consensus with regulatory authorities on trial design;
- delays in reaching agreement on acceptable terms with prospective CROs and clinical trial sites;
- delays in opening clinical trial sites or obtaining required institutional review board or independent Ethics Committee approval at each clinical trial site;
- delays in recruiting and enrolling suitable subjects to participate in our clinical trials, due to factors such as the size of the trial or subject population, process for identifying subjects, design or expansion of protocols, eligibility and exclusive criteria, perceived risks and benefits of the relevant product candidate or gene therapy generally, availability of competing therapies and trials, severity of the disease under investigation, need and length of time required to discontinue other potential therapies, availability of genetic testing, availability and proximity of trial sites for prospective subjects, ability to obtain subject consent and referral practices of physicians;
- imposition of a clinical hold by regulatory authorities;
- failure by us, any CROs we engage or any other third parties to adhere to clinical trial requirements;
- failure to perform in accordance with GCP, or applicable regulatory guidelines in the European Union and other countries;
- delays in the testing, validation, manufacturing and delivery of our product candidates to the clinical sites, including delays by third parties with whom we have contracted to perform;
- delays in having subjects complete participation in a trial or return for post-treatment follow-up;
- clinical trial sites or subjects dropping out of a trial;
- selection of clinical endpoints that require prolonged periods of clinical observation or analysis of the resulting data;
- occurrence of serious adverse events associated with the product candidate that are viewed to outweigh its potential benefits;

•occurrence of serious adverse events in trials of the same class of agents conducted by other sponsors; or

•changes in regulatory requirements and guidance that require amending or submitting new clinical protocols.

Any inability to successfully complete research studies and preclinical and clinical development could result in additional costs to us or impair our ability to generate revenues from product sales, regulatory and commercialization milestones and royalties. In addition, if we make manufacturing or formulation changes to our product candidates, we may need to conduct additional studies to bridge our modified product candidates to earlier versions. Clinical trial delays also could shorten any periods during which we may have the exclusive right to commercialize our product candidates, if approved, or allow our competitors to bring products to market before we do, which could impair our ability to successfully commercialize our product candidates, if approved, and may harm our business, financial condition, results of operations and prospects.

We may be negatively impacted if the results of our planned clinical trials are inconclusive or if there are safety concerns or serious adverse events associated with our product candidates.

If the results of our planned clinical trials are inconclusive or if there are safety concerns or serious adverse events associated with our product candidates, we may:

•receive a clinical hold for a particular product candidate, which, if not lifted, could require that we discontinue development of a product candidate;

•be required to conduct additional studies or clinical trials with respect to a product candidate or for a potential indication, which may result in additional significant expense and delays in seeking regulatory approval;

•be delayed in obtaining regulatory approval for our product candidates, if 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;

•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 or other requirements;

•have regulatory authorities withdraw, vary or suspend their approval of the product or impose restrictions on its distribution in the form of a modified risk evaluation and mitigation;

•be subject to the addition of labeling statements, such as warnings or contraindications;

•be sued; or

•experience damage to our reputation.

Our NAV Technology Platform, our product candidates or our licensees' or collaborators' product candidates, and the process for administering such product candidates, may cause undesirable side effects or have other properties that could delay or prevent regulatory approval of product candidates, limit the commercial potential or result in significant negative consequences following any potential marketing approval.

There have been several significant adverse side effects in gene therapy treatments in the past, including reported cases of leukemia in trials using lentivirus vectors and death seen in trials sponsored by other companies using adenovirus vectors and AAV vectors, including NAV vectors. Gene therapy is still a relatively new approach to disease treatment and additional adverse side effects could be identified. There also is the potential risk of delayed adverse events following exposure to gene therapy products due to persistent biologic activity of the genetic material or other components of products used to carry the genetic material. Possible adverse side effects that could occur with treatment with gene therapy products include an immunologic reaction early after administration which could substantially limit the effectiveness of the treatment. In previous clinical trials involving AAV vectors for gene therapy, some subjects experienced the development of a T-cell response, whereby after the vector is within the target cell, the cellular immune response system triggers the removal of transduced cells by activated T-cells. Furthermore, in clinical trials sponsored by other companies involving AAV vectors administered intravitreally for the treatment of retinal conditions, serious adverse reactions, such as panuveitis and loss of vision, have occurred. In addition to side effects caused by product candidates, the administration process or related procedures also can cause adverse side effects. If any such adverse events occur in our or third-party trials, our clinical trials could be suspended or terminated.

As a result of these concerns, we may decide, or the FDA, the European Commission, the EMA or other regulatory authorities could order us, to halt, delay or amend preclinical development or clinical development of our product candidates or we may be unable to receive regulatory approval of our product candidates 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 of our product candidates, the commercial prospects of such product candidates may be harmed and our ability to generate product revenues from any of these product candidates may be delayed or eliminated. Any of these occurrences may harm our ability to develop other product candidates and may harm our business, financial condition and prospects significantly.

Additionally, if any of our product candidates receives marketing approval, the FDA could require us to adopt a Risk Evaluation and Mitigation Strategy (REMS) and other regulatory authorities could impose other specific obligations as a condition of approval to ensure that the benefits of our product candidates outweigh their risks, which could delay approval of our product candidates. A REMS 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 or patients; and elements to assure safe use, which can severely restrict the distribution of a product by, for example, requiring that health care providers receive particular training and obtain special certification prior to prescribing and dispensing the product, limiting the healthcare settings in which the product may be dispensed and subjecting patients to monitoring and enrollment in a registry. If the FDA requires us to adopt a REMS for our products and we are unable to comply with its requirements, the FDA may deem our products to be misbranded and we may be subject to civil money penalties. The European Commission, the EMA and other regulatory authorities may, following grant of marketing authorization in their territory, impose similar obligations.

Any of these events could prevent us from achieving or maintaining market acceptance of our NAV Technology Platform and our product candidates and could materially harm our business, prospects, financial condition and results of operations.

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

We cannot commercialize a product candidate until the appropriate regulatory authorities have reviewed and approved the product candidate. Even if our product candidates 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. The ability of the FDA to review and approve new products can be affected by a variety of factors, including government budget and funding levels, ability to hire and retain key personnel and accept the payment of user fees, and statutory, regulatory and policy changes. Average review times at the FDA have fluctuated in recent years as a result. Disruptions at the FDA and other agencies may also slow the time necessary for new product candidates to be reviewed and approved by necessary government agencies, which could adversely affect our business. In addition, government funding of 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. 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 on additional government regulation from future legislation or administrative action or based on 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 our product candidates. Any of the foregoing scenarios could materially harm the commercial prospects for our product candidates and materially harm our business, financial condition, results of operations and prospects.

Further, the regulatory authorities may require concurrent approval or the CE mark (a mandatory conformity assessment marking for certain products sold within the European Economic Area (the EEA)) of a companion diagnostic device, since it may be necessary to use FDA-cleared or FDA-approved, or CE-marked, diagnostic tests or diagnostic tests approved by other comparable foreign regulatory authorities to diagnose patients or to assure the safe and effective use of our product candidates in trial subjects. FDA refers to such tests as *in vitro* companion diagnostic devices. The FDA has articulated a policy position that, when safe and effective use of a therapeutic product depends on a diagnostic device, the FDA generally will require approval or clearance of the companion diagnostic device at the same time that the FDA approves the therapeutic product. The FDA's guidance allows for two exceptions to the general rule of concurrent drug/device approval, namely, when the therapeutic product is intended to treat serious and life-threatening conditions for which no alternative exists, and when a serious safety issue arises for an approved therapeutic

agent, and no FDA-cleared or FDA-approved companion diagnostic test is yet available. It is unclear how the FDA will apply this policy to our current or future gene therapy product candidates. Should the FDA deem genetic tests used for diagnosing patients for our therapies to be *in vitro* companion diagnostics requiring FDA clearance or approval, we may face significant delays or obstacles in obtaining approval of a BLA for our product candidates.

In the European Union, companion diagnostics are subject to the European Union Directive on *in vitro* diagnostic medical devices and its implementation in the European Union Member States. Recently revised European Union laws on *in vitro* diagnostics applied beginning in 2022, which provide for stricter requirements for *in vitro* diagnostic medical devices and impose additional obligations on manufacturers of *in vitro* diagnostic medical devices that may impact the development and authorization of our product candidates in the European Union. For example, the new regulation extends the requirement for performance assessment procedures and requires greater involvement of notified bodies in the development of *in vitro* diagnostic medical devices. This may result in additional regulatory and premarket requirements to market new *in vitro* diagnostic medical devices. Companies producing *in vitro* diagnostic medical devices will be required to have a responsible person to oversee regulatory compliance. In addition, the new regulation introduces risk classification of *in vitro* diagnostic medical devices and significantly increases the number of products that will be subject to stricter regulation. It also introduces the requirement to involve a notified body in the conformity assessment procedure.

Approval of a product candidate in the United States by the FDA does not ensure approval of such product candidate by regulatory authorities in other countries or jurisdictions, and approval by one foreign regulatory authority does not ensure approval by regulatory authorities in other foreign countries or by the FDA. Sales of our product candidates outside of the United States will be subject to foreign regulatory requirements governing clinical trials and marketing approval. Even if the FDA grants marketing approval for a product candidate, comparable regulatory authorities of foreign countries also must approve the manufacturing and marketing of the product candidates in those countries. Approval procedures vary among jurisdictions and can involve requirements and administrative review periods different from, and more onerous than, those in the United States, including additional preclinical studies or clinical trials. In many countries outside the United States, a product candidate must be approved for reimbursement before it can be approved for sale in that country. In some cases, the price that we intend to charge for our products, if approved, is also subject to approval. We intend to submit a marketing authorization application to EMA for approval of our product candidates by the European Commission in the European Union. However, obtaining such approval from the European Commission following the opinion of EMA is a lengthy and expensive process. Additionally, the UK has its own separate approval procedures for our product candidates following the UK's exit from the European Union.

Even if a product candidate is approved, the FDA or the European Commission, as the case may be, may limit the indications for which the product may be marketed, require extensive warnings on the product labeling or require expensive and time-consuming additional clinical trials or reporting as conditions of approval. Regulatory authorities in countries outside of the United States and the European Union also have requirements for approval of product candidates with which we must comply prior to marketing in those countries. Obtaining foreign regulatory approvals and compliance with foreign regulatory requirements could result in significant delays, difficulties and costs for us and could delay or prevent the introduction of our product candidates in certain countries.

Further, clinical trials conducted in one country may not be accepted by regulatory authorities in other countries. Also, regulatory approval for any of our product candidates may be withdrawn. If we fail to comply with the regulatory requirements, our target market will be reduced and our ability to realize the full market potential of our product candidates will be harmed and our business, financial condition, results of operations and prospects will be harmed.

Risks Related to Our Financial Position

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

The biotechnology and pharmaceutical industries, including the gene therapy field, are characterized by rapidly changing technologies, significant competition and a strong emphasis on intellectual property. We face substantial competition from many different sources, including large and specialty pharmaceutical and biotechnology companies, academic research institutions, government agencies and public and private research institutions.

We are aware of a number of companies focused on developing gene therapies in various indications, as well as a number of companies addressing other methods for modifying genes and regulating gene expression. Any advances in gene therapy technology made by a competitor may be used to develop therapies that could compete against any of our product candidates.

Many of our potential competitors, alone or with their strategic partners, have substantially greater financial, technical and other resources, such as larger research and development, clinical, marketing and manufacturing organizations. Mergers and acquisitions in the biotechnology and pharmaceutical industries may result in even more resources being concentrated among a smaller number of competitors. Our commercial opportunity could be reduced or eliminated if 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. Competitors also may obtain FDA or other regulatory approval for their products more rapidly or earlier than we may obtain approval for ours, or have already obtained, 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 our product candidates against those of competitors. 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.

Even though we have obtained orphan drug exclusivity for certain product candidates, that exclusivity may not effectively protect the product candidate from competition because the FDA may subsequently approve another drug for the same condition if the FDA concludes that the latter drug is not the same drug or is clinically superior in that it is shown to be safer, more effective or makes a major contribution to patient care. In the European Union, marketing authorization may be granted to a similar medicinal product for the same orphan indication if:

- the second applicant can establish in its application that its medicinal product, although similar to the orphan medicinal product already authorized, is safer, more effective or otherwise clinically superior;
- the holder of the marketing authorization for the original orphan medicinal product consents to a second orphan medicinal product application; or
- the holder of the marketing authorization for the original orphan medicinal product cannot supply sufficient quantities of orphan medicinal product.

We have incurred cumulative net losses and have had few profitable quarters since inception. We expect to regularly incur losses until we have successfully commercialized one or more product candidates and may never achieve or maintain profitability in the future.

Since inception, we have incurred cumulative net losses. We have historically financed our operations primarily through private and public offerings of our equity securities, collaborations and licensing rights to our NAV Technology Platform, including milestone payments and royalties from our NAV Technology Licensees. We have devoted substantially all of our efforts to research and development, including preclinical and clinical development of our product candidates, and licensing our NAV Technology Platform, as well as to building out our team. We expect that it could be years before we commercialize most of our product candidates, and we can provide no assurance that we will ever be able to do so. We license certain intellectual property related to our NAV Technology Platform to our NAV Technology Licensees and collaborators. Our NAV Technology Licensees and collaborators have multiple preclinical studies and clinical trials in progress. However, only one gene therapy product based on our licensing program, Novartis AG's Zolgensma, has been approved or commercialized. Other than revenue in connection with sales of Zolgensma, we may generate only limited recurring revenue in the near term from our current NAV Technology Licensees and collaborators. We expect to continue to incur significant expenses and regularly incur 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 our research studies and preclinical and clinical development of our product candidates, including our lead product candidates;
- initiate additional preclinical studies and clinical trials for our lead product candidates and future product candidates, if any;
- initiate additional activities relating to manufacturing, including building out additional laboratory and manufacturing capacity;
- seek to identify additional product candidates;
- prepare our BLA and MAA for our lead product candidates and seek marketing approvals for any of our other product candidates that successfully complete clinical trials, if any;
- further develop our NAV Technology Platform;
- establish a sales, marketing and distribution infrastructure to commercialize any product candidates for which we may obtain marketing approval, if any;

•maintain, expand and protect our intellectual property portfolio and enforce our intellectual property rights; and

•acquire or in-license other product candidates and technologies.

For us to become consistently profitable, we and our licensees and collaborators must develop and commercialize product candidates with significant market potential. This will require us and our licensees and collaborators to be successful in a range of business challenges, including expansion of the licensing of our NAV Technology Platform, completing preclinical studies of product candidates, commencing and completing clinical trials of product candidates, obtaining marketing approval for these product candidates, manufacturing, marketing and selling those products for which we may obtain marketing approval and satisfying any post-marketing requirements. We may never succeed in any or all of these activities and, even if we do, we may never consistently generate revenues that are sufficient to achieve profitability, and we may not be able to sustain or increase profitability on a quarterly or annual basis. Our failure to become consistently profitable 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 also could cause you to lose all or part of your investment.

We will need to raise additional funding, which may not be available on acceptable terms, or at all. Failure to obtain this necessary capital when needed may force us to delay, limit or terminate certain of our licensing activities, product development efforts or other operations.

We expect to require substantial future capital in order to complete research studies, preclinical and clinical development for our current product candidates and any future product candidates, and potentially commercialize these product candidates, if approved. We expect our spending levels to increase in connection with our preclinical and clinical trials of our product candidates. In addition, if we obtain marketing approval for any of our product candidates, we expect to incur significant expenses related to product sales, medical affairs, marketing, manufacturing and distribution. 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 favorable terms, we could be forced to delay, reduce or eliminate certain of our licensing activities, our research and development programs or other operations.

Our operations have consumed significant amounts of cash since inception. Our future capital requirements will depend on many factors, including:

•the timing of enrollment, commencement and completion of our clinical trials;

•the results of our clinical trials;

•the results of our preclinical studies for our product candidates and any subsequent clinical trials;

•the scope, progress, results and costs of drug discovery, laboratory testing, preclinical development and clinical trials for our product candidates;

•the costs associated with building out additional laboratory and manufacturing capacity;

•the costs, timing and outcome of regulatory review of our product candidates;

•the impact of any government-imposed tariffs on cost of goods and services, particularly related to partnered product candidates;

•the costs of future product sales, medical affairs, marketing, manufacturing and distribution activities for any of our product candidates for which we receive marketing approval;

•revenue, if any, received from commercial sales of our products, should any of our product candidates receive marketing approval;

•revenue received from commercial sales of Zolgensma and the timing and amount of Zolgensma royalties paid to HCR under our royalty purchase agreement;

•revenue received from other commercial sales of our licensees' and collaborators' products, should any of their product candidates receive marketing approval, and other revenue received under our licensing agreements and collaborations;

•the costs of preparing, filing and prosecuting patent applications, maintaining and enforcing our intellectual property rights and defending any intellectual property-related claims;

•our current licensing agreements or collaborations remaining in effect, including the AbbVie Collaboration and License Agreement relating to ABBV-RGX-314 and the Nippon Shinyaku Collaboration and License Agreement relating to RGX-121 and RGX-111, and our ability to timely achieve any milestones set forth in such agreements or collaborations;

•our ability to establish and maintain additional licensing agreements or collaborations on favorable terms, if at all; and

•the extent to which we acquire or in-license other product candidates and technologies.

The issuance of additional securities, whether equity or debt, by us, including through our at-the-market program, or the possibility of such issuance, may cause the market price of our common stock to decline. Adequate additional financing may not be available to us on acceptable terms, or at all. We also could be required to seek funds through arrangements with partners or otherwise that may require us to relinquish rights to our intellectual property, our product candidates or otherwise agree to terms unfavorable to us.

Although we have generated significant revenues from licensing our NAV Technology Platform and our other intellectual property, such as our licensing pursuant to the AbbVie Collaboration and License Agreement and may do so under the Nippon Shinyaku Collaboration and License Agreement, we have never generated revenue from sales of our product candidates and may never do so in the future.

We have generated significant revenues from licensing our NAV Technology Platform, including sublicense fees, milestone payments and royalties on net sales of a licensed product, Zolgensma, and licensing our intellectual property to AbbVie pursuant to the AbbVie Collaboration and License Agreement and may do so in the future pursuant to the Nippon Shinyaku Collaboration and License Agreement. However, our ability to generate revenue from sales of our internal product candidates will depend on our ability, alone or with partners, to successfully complete the development of, and obtain the regulatory approvals necessary to commercialize, our product candidates.

Our ability to generate future revenues from sales of our product candidates and in connection with sales of our licensees' and collaborators' products depends heavily on our, and our licensees' and collaborators', success in:

•completing research studies and preclinical and clinical development of product candidates and identifying new gene therapy product candidates;

•obtaining regulatory and marketing approvals for product candidates for which clinical trials are completed;

•commercializing product candidates for which regulatory and marketing approval is obtained by establishing a sales force, marketing and distribution infrastructure or, alternatively, collaborating with a commercialization partner;

•negotiating favorable terms in any collaboration, licensing or other arrangements into which we or our licensees and collaborators may enter and performing our obligations in such collaborations;

•qualifying for adequate coverage and reimbursement by government and third-party payors for product candidates;

•maintaining and enhancing a sustainable, scalable, reproducible and transferable manufacturing process for our vectors and product candidates;

•establishing and maintaining supply and manufacturing relationships with third parties that can provide adequate, in both amount and quality, products and services to support clinical development and the market demand for product candidates, if approved;

•obtaining market acceptance of product candidates as a viable treatment option;

•competing effectively when other companies may develop products that are priced lower, reimbursed more favorably by government or other third-party payors, safer, more effective or more convenient to use than our products, if any, or our licensees' and collaborators' products;

•implementing additional internal systems and infrastructure, as needed;

•negotiating favorable terms in any collaboration, licensing or other arrangements into which we may enter and performing our obligations in such collaborations;

•maintaining, protecting and expanding our portfolio of intellectual property rights, including patents, trade secrets and know-how;

•avoiding and defending against third-party interference, infringement and other intellectual property related claims; and

•attracting, hiring and retaining qualified personnel.

Many of these factors as they relate to our licensees' and collaborators' products, including Zolgensma, will be outside our control, and future revenues in connection with sales of such products may be precluded or limited by any of these factors.

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

Risks Related to Third Parties

We rely on third parties to conduct aspects of our clinical trials and certain preclinical research development activities. If these third parties do not meet our deadlines or otherwise conduct the preclinical research and development activities and trials as required, our clinical and preclinical development programs could be delayed or unsuccessful.

We do not have the ability to conduct all aspects of our preclinical research and development activities or clinical trials ourselves. We are dependent on third parties to conduct certain aspects of our clinical trials and, therefore, the timing of the initiation and completion of these trials may be controlled by such third parties and may occur on substantially different timing from our estimates. We also rely on third parties to conduct a portion of our preclinical research and development activities and we may also rely on CROs, medical institutions, clinical investigators, consultants or other third parties to conduct our clinical trials in accordance with our clinical protocols and regulatory requirements. A loss or deterioration of our relationships with such third parties or the principal investigators for our preclinical and clinical programs could materially harm our business.

There is no guarantee that any third party on which we rely for our preclinical research and development activities and the administration and conduct of our clinical trials will devote adequate time and resources to such activities or trials or perform as contractually required. If any such third party fails to meet expected deadlines, fails to adhere to our preclinical or clinical protocols or otherwise performs in a substandard manner, our preclinical programs and clinical trials may be extended, delayed or terminated, which could materially harm our business. Additionally, if any of our clinical trial sites terminates for any reason, we may experience the loss of follow-up information on subjects enrolled in our ongoing clinical trials unless we are able to transfer those subjects to another qualified clinical trial site. Furthermore, principal investigators for our clinical trials may serve as scientific advisors or consultants to us from time to time and may receive cash or equity compensation in connection with such services. If these relationships and any related compensation result in perceived or actual conflicts of interest, the integrity of the data generated at the applicable clinical trial site may be jeopardized, which could result in substantial delays in our clinical trials and materially harm our business.

We have in the past, and in the future may, enter into licensing agreements or collaborations with third parties licensing parts of our NAV Technology Platform for the development of product candidates. If these licensing arrangements or collaborations are not successful, our business could be harmed.

We have entered into agreements involving the licensing of parts of our NAV Technology Platform and relating to the development and commercialization of certain product candidates and plan to enter into additional licensing agreements or collaborations in the future. We have limited control over the amount and timing of resources that our current and future licensees and collaborators dedicate to the development or commercialization of product candidates or of products utilizing licensed components of our NAV Technology Platform. Our ability to generate revenues from these arrangements will depend on our and our licensees' and collaborators' abilities to successfully perform the functions assigned to each of us in these arrangements. In addition, our licensees and collaborators have the ability to abandon research or development projects and terminate applicable agreements. Moreover, an unsuccessful outcome in any clinical trial for which our licensee or collaborator is responsible could be harmful to the public perception and prospects of our NAV Technology Platform or product candidates.

Any current or future licensing agreements or future collaborations we enter into may pose additional risks, including the following:

•subjects in clinical trials undertaken by our licensees and collaborators may suffer adverse effects, including death;

•our licensees and collaborators may not pursue development and commercialization of any product candidates that achieve regulatory approval or may elect not to continue or renew development or commercialization programs based on clinical trial results, changes in the licensees' or collaborators' strategic focus or available funding or external factors, such as an acquisition, that divert resources or create competing priorities;

•we may not have access to, or may be restricted from disclosing, certain information regarding product candidates being developed or commercialized under a collaboration and, consequently, may have limited ability to inform our stockholders about the status of such product candidates;

•our licensees or collaborators could independently develop, or develop with third parties, products that compete directly or indirectly with our product candidates if the licensees or 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;

•product candidates developed in collaboration with us may be viewed by our licensees or collaborators as competitive with their own product candidates or products, which may cause licensees or collaborators to cease to devote resources to the commercialization of our product candidates;

•a licensee or collaborator with marketing and distribution rights to one or more of our product candidates that achieve regulatory approval may not commit sufficient resources to the marketing and distribution of any such product candidate;

•our licensees or collaborators may breach their reporting, payment, intellectual property or other obligations to us, which could prevent us from complying with our contractual obligations to our upstream licensors;

•disagreements with licensees or collaborators, including disagreements over intellectual property and other proprietary rights, payment obligations, contract interpretation or the preferred course of development of any product candidates, may cause delays or termination of the research, development or commercialization of such product candidates, may lead to additional responsibilities for us with respect to such product candidates or may result in litigation or arbitration, any of which would be time-consuming and expensive and could potentially lessen the value of such agreements and collaborations;

•our licensees or collaborators may not properly maintain or defend our intellectual property rights or may use our proprietary information in such a way as to invite litigation that could jeopardize or invalidate our intellectual property or proprietary information or expose us to potential litigation;

•disputes may arise with respect to the ownership of our other rights to intellectual property developed pursuant to our licensing agreements or collaborations;

•depending on the terms of the licensing agreement, the licensee or collaborator may have sole discretion regarding material aspects of the development, marketing or sale of a product candidate;

•our licensees or collaborators may infringe or otherwise violate the intellectual property rights of third parties, which may expose us to litigation and potential liability; and

•licensing agreements or collaborations may be terminated for the convenience of the licensee or collaborator and, if terminated, we could be required to raise additional capital to pursue further development or commercialization of the applicable product candidates.

If our licensing agreements or collaborations do not result in the successful development and commercialization of products, or if one of our licensees or collaborators terminates its agreement with us, we may not receive any future milestone or royalty payments. If we do not receive the payments we expect under these agreements, our development of product candidates could be delayed and we may need additional resources to develop our product candidates. In addition, if one of our licensees or collaborators terminates its agreement with us, we may find it more difficult to attract new licensees or collaborators and the perception of us in the business and financial communities could be harmed. Each of our licensees and collaborators is subject to similar risks with respect to product development, regulatory approval and commercialization, and any such risk could result in its business being harmed, which could adversely affect our collaboration.

We may in the future decide to partner or collaborate with pharmaceutical and biotechnology companies for the development and potential commercialization of our product candidates. These relationships, or those like them, may require us to incur non-recurring and other charges, increase our near- and long-term expenditures, issue securities that dilute our existing stockholders or disrupt our management and business. In addition, we could face significant competition in seeking appropriate collaborators and the negotiation process is time-consuming and complex. Our ability to reach a definitive licensing agreement or collaboration agreement will depend, among other things, upon our assessment of the collaborator's resources and expertise, the terms and conditions of the proposed collaboration and the proposed collaborator's evaluation of a variety of factors.

We may not be successful in our efforts to establish such a strategic partnership or other alternative arrangements for our product candidates because our research and development pipeline may be insufficient, our product candidates may be deemed to be at too early of a stage of development for collaborative effort or third parties may not view our product candidates as having the requisite potential to demonstrate safety and efficacy or market opportunity. In addition, we may be restricted under existing collaboration agreements from entering into future agreements with potential collaborators. If we license rights to product candidates, we may not be able to realize the benefit of such transactions if we are unable to successfully integrate the licensed product candidates with our existing operations.

If we are unable to reach agreements with suitable licensees or collaborators on a timely basis, on acceptable terms or at all, we may have to curtail the development of a product candidate, reduce or delay its development program, delay its potential commercialization, 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 fund development or commercialization activities on our own, we may need to obtain additional expertise and additional capital, which may not be available to us on acceptable terms or at all.

Risks Related to Manufacturing

Products intended for use in gene therapies are novel, 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 currently have development, manufacturing and testing agreements with third parties to manufacture supplies of our product candidates, in addition to our internal manufacturing laboratory. Several factors could cause production interruptions, including equipment malfunctions, facility contamination, raw material shortages or contamination, a decline in stability of a product that reduces its shelf life, natural disasters, public health crises, disruption in utility services, human error or disruptions in the operations of suppliers.

Our product candidates require processing steps that are more complex than those required for most chemical pharmaceuticals. Moreover, unlike chemical pharmaceuticals, the physical and chemical properties of biologics such as ours 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. Accordingly, we employ multiple steps to control our manufacturing process to assure that the process works and the product candidate is made strictly and consistently in compliance with the process. Problems with the manufacturing process, even minor deviations from the normal process, could result in product defects or manufacturing failures that may not be detected in standard release testing, which could result in lot failures, product recalls, declines in stability, product liability claims or insufficient inventory. We may encounter problems achieving adequate quantities and quality of clinical-grade materials that meet FDA, EMA or other applicable foreign standards or specifications with consistent and acceptable production yields and costs.

In addition, the FDA, the EMA and other foreign 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 foreign regulatory authorities may require that we not distribute a lot or batch until the competent authority 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/batch failures or product recalls. Lot/batch failures, which we have experienced in the past, 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 operate our manufacturing process which could result in delays in our production or difficulties in maintaining compliance with applicable regulatory requirements.

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 processes or facilities also could restrict our ability to meet market demand for our products. Additionally, should our manufacturing agreements with third parties be terminated for any reason, there may be a limited number of manufacturers who would be suitable replacements and it could take a significant amount of time to transition the manufacturing to a replacement.

Delays in obtaining regulatory approval of our manufacturing process or disruptions in our manufacturing process, including the development of our cGMP production facility, may delay or disrupt our commercialization efforts.

Before we can begin to commercially manufacture our product candidates in third-party or our own facilities, we must obtain regulatory approval from the FDA, which includes a review of the manufacturing process and facility. A manufacturing authorization must also be obtained from the appropriate European Union Member State regulatory authorities and may be required by other foreign regulatory authorities. The timeframe required to obtain such approval or authorization is uncertain. In order to obtain approval, we will need to ensure that all of our processes, methods and equipment are compliant with cGMP, and perform extensive audits of vendors, contract laboratories and suppliers. If we or any of our vendors, contract laboratories or suppliers are found to be out of compliance with cGMP, we may experience delays or disruptions in manufacturing while we work to remedy the violation or while we work to identify suitable replacement vendors, contract laboratories or suppliers. The cGMP requirements govern quality control

of the manufacturing process and documentation policies and procedures. In complying with cGMP, we will be obligated to expend time, money and effort in production, record keeping and quality control to assure that the product meets applicable specifications and other requirements. If we fail to comply with these requirements, we would be subject to possible regulatory action, which could result in fines or reputational harm, and we may not be permitted to sell any products that we may develop.

We currently rely and expect to continue to rely on third parties to conduct parts of our product manufacturing, and these third parties may not perform satisfactorily.

We currently plan to have some of the material manufactured for our planned preclinical and clinical programs by third parties. We currently rely, and expect to continue to rely, on third parties for the production of a portion of our preclinical study and planned clinical trial materials and, therefore, we can control only certain aspects of their activities.

We rely on additional third parties to manufacture ingredients of our product candidates and to perform quality testing, and reliance on these third parties entails risks to which we would not be subject if we manufactured the product candidates ourselves, including:

- reduced control for certain aspects of manufacturing activities;
- termination or nonrenewal of manufacturing and service agreements with third parties in a manner or at a time that is costly or damaging to us;
- disruptions to the operations of our third-party manufacturers and service providers caused by conditions unrelated to our business or operations, including the bankruptcy of, or legal or regulatory actions against, the manufacturer or service provider;
- reduced capacity of our third-party manufacturers and service providers caused by increased demand by their other customers;
- discovery of data integrity issues with our third-party manufacturers and service providers which directly or indirectly impact our ability to use our product candidates; and
- legal or regulatory actions against our third-party manufacturers and service providers which adversely affect our ability to use our product candidates.

FDA, EMA or other regulatory authority action could include injunction, recall, seizure or total or partial suspension of product manufacture or manufacturing authorization. Any of these events could lead to clinical trial delays or failure to obtain regulatory approval, or impact our ability to successfully commercialize future product candidates, and therefore may cause our business, financial condition, results of operations and prospects to be materially harmed.

Failure to comply with ongoing manufacturing regulatory requirements could cause us to suspend production or put in place costly or time-consuming remedial measures, and shortages of resources or raw materials could result in delays in our research studies, preclinical and clinical development or marketing schedules.

Regulatory authorities may, at any time following approval of a product for sale, audit the manufacturing facilities for such product. If any such inspection or audit identifies a failure to comply with applicable regulations, or if a violation of product specifications or applicable regulations occurs independent of such an inspection or audit, the relevant regulatory authority may require remedial measures that may be costly or time-consuming to implement and that may include the temporary or permanent suspension of a clinical trial or commercial sales or the temporary or permanent closure of a manufacturing facility. Any such remedial measures imposed upon us or any of our third-party manufacturers could materially harm our business, financial condition, results of operations and prospects.

If we or any of our third-party manufacturers fail to comply with applicable cGMP regulations, regulatory authorities can impose sanctions including, among other things, refusal to approve a pending application for a new product candidate or suspension or revocation of a pre-existing approval. Such an occurrence may cause our business, financial condition, results of operations and prospects to be materially harmed.

Additionally, if supply from a manufacturing facility is interrupted, there could be a significant disruption in commercial supply of our products. An alternative manufacturer would need to be qualified, through a supplement to its regulatory filing, which could result in further delay. Regulatory authorities also may require additional trials if a new manufacturer is relied upon for commercial production. Switching manufacturers may involve substantial costs and could result in a delay in our desired clinical and commercial timelines.

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 resources, raw materials and components required in our manufacturing or research and development processes are derived from biologic sources, and we normally rely on suppliers to provide such resources, raw materials and components. These may be difficult to procure and subject to contamination or recall. Certain resources, raw materials and components, especially those that are specifically catered to the gene therapy industry, may become unavailable to us in sufficient quantities from time to time due to increased demand.

A material shortage, contamination, recall or restriction on the use of biologically derived substances in the manufacture of our product candidates may be beyond our control and 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.

Risks Related to the Commercialization of Our Product Candidates

If we are unable to adequately establish sales and marketing capabilities or enter into agreements with third parties to market and sell our product candidates, if approved, we may be unable to generate any product revenue.

We currently have no products to sell. We have a limited product sales and marketing organization. To successfully commercialize any products that may result from our development programs, we will need to further develop these capabilities, either on our own or with others. The expansion of our own commercial team or the establishment of a contract sales force to market any products we may develop will be expensive and time-consuming and could delay any product launch. Moreover, we cannot be certain that we will be able to successfully develop this capability. We may enter into additional collaboration arrangements regarding one or more of our product candidates with other entities to utilize their marketing and distribution capabilities, such as our collaboration with AbbVie and Nippon Shinyaku, but we may be unable to enter into such agreements on favorable terms, if at all. If any current licensees or collaborators, or future licensees or collaborators, do not commit sufficient resources to commercialize our products, if approved, or we are unable to develop the necessary capabilities on our own, we will be unable to generate sufficient product revenue to sustain our business. We compete with many companies that currently have extensive, experienced and well-funded marketing and sales operations to recruit, hire, train and retain marketing and sales personnel. We also face competition in our search for third parties to assist us with the sales and marketing efforts of our product candidates. Without an adequate internal team or the support of a third party to perform marketing and sales functions, we may be unable to compete successfully against these more established companies.

Our efforts to educate the medical community and third-party payors on the benefits of our product candidates may require significant resources and may never be successful. Such efforts may require more resources than are typically required due to the complexity and uniqueness of our potential products. If any of our product candidates is approved but fails to achieve market acceptance among physicians, patients or third-party payors, we will not be able to generate significant revenues from such product, which could materially harm our business, financial condition, results of operations and prospects.

If we do not achieve our projected development goals in the timeframes we announce and expect, the commercialization of our products may be delayed and, as a result, our stock price may decline.

From time to time, we estimate the timing of the accomplishment of various scientific, clinical, regulatory and other product development goals, which we sometimes refer to as milestones. These milestones may include, but are not limited to, the commencement or completion of scientific studies and clinical trials, the submission of regulatory filings, the announcement of results from scientific studies or clinical trials and the announcement of additional product candidates. From time to time, we may publicly announce the expected timing of some of these milestones. All of these milestones are and will be based on numerous assumptions. The actual timing of these milestones can vary dramatically compared to our estimates, in some cases for reasons beyond our control. If we do not meet these milestones as publicly announced, or at all, the commercialization of our products may be delayed or never achieved and, as a result, our stock price may decline.

Even if we receive regulatory approval, we still may not be able to successfully commercialize our lead product candidates or any future product candidate, and the revenue that we generate from any approved product's sales, if any, could be limited.

From time to time, public sentiment may be more adverse to commercialization of gene therapy as a therapeutic technique. Even with the requisite approvals from the FDA, the EMA and other regulatory authorities, the commercial success of our product candidates will depend, in part, on the acceptance of physicians, patients and health care payors of gene therapy products in general, and our product candidates in particular, as medically necessary, cost-effective and safe. Any product that we commercialize may not gain acceptance by physicians, patients, health care payors and others in the medical community. If these products do not achieve an adequate level of acceptance, we may not generate significant product revenue and may not become profitable. The degree of market acceptance of our product candidates will depend on a number of factors, including:

- demonstration of clinical efficacy and safety compared to other more-established products;
- limitation of our targeted patient population and other limitations or warnings contained in any FDA or European Commission labeling, or other comparable foreign regulatory authority-approved labeling;
- acceptance of a new formulation by health care providers and their patients;
- the prevalence and severity of any adverse effects;
- new procedures or methods of treatment that may be more effective in treating or may reduce the conditions which our products are intended to treat;
- pricing and cost-effectiveness;
- the effectiveness of our or any future collaborators' sales and marketing strategies;
- our ability to obtain and maintain sufficient third-party coverage and reimbursement from government health care programs, including Medicare and Medicaid, private health insurers and other third-party payors;
- unfavorable publicity and negative public opinion relating to product candidates or gene therapy generally, including due to serious adverse events in gene therapy trials; and
- the willingness of patients to pay out-of-pocket in the absence of third-party coverage or reimbursement.

If any product candidate is approved but does not achieve an adequate level of acceptance by physicians, hospitals, healthcare payors or patients, we may not generate sufficient revenue from that product candidate and may not become or remain profitable. Our efforts to educate the medical community and third-party payors on the benefits of our lead product candidates or any future product candidates may require significant resources and may never be successful. In addition, our ability to successfully commercialize our product candidates will depend on our ability to differentiate our products from competing products and defend and enforce our intellectual property rights relating to our products. Additionally, if the market opportunities for our lead product candidates or any future product candidates are smaller than we believe they are, our product revenues may be harmed and our business may suffer.

We focus our research and product development on treatments for severe genetic and orphan diseases. Our understanding of both the number of people who have these diseases, as well as the subset of people with these diseases who have the potential to benefit from treatment with our product candidates, are based on estimates. These estimates may prove to be incorrect and new studies may reduce the estimated incidence or prevalence of these diseases. The number of patients in the United States, the European Union and elsewhere may turn out to be lower than expected, may not be otherwise amenable to treatment with our products or patients may become increasingly difficult to identify and access, all of which would harm our business, financial condition, results of operations and prospects.

Further, there are several factors that could contribute to making the actual number of patients who receive any products we develop less than the potentially addressable market. These include the lack of widespread availability of, and limited reimbursement for, new therapies in many underdeveloped markets. Further, the severity of the progression of a disease up to the time of treatment, especially in certain degenerative conditions such as the conditions our lead product candidates are intended to treat, will likely diminish the therapeutic benefit conferred by a gene therapy due to irreversible cell death. Lastly, certain patients' immune systems might prohibit the successful delivery of certain gene therapy products to the target tissue, thereby limiting the treatment outcomes.

The insurance coverage and reimbursement status of newly approved products is uncertain. Failure to obtain or maintain adequate coverage and reimbursement for our products, if approved, or the imposition of price controls or other forms of pricing regulation could limit our ability to market those products and decrease our ability to generate product revenue.

We expect the cost of a single administration of gene therapy products, such as those we are developing, to be substantial, when and if they achieve regulatory approval. We expect that coverage and reimbursement by government and private payors will be essential for most patients to be able to afford these treatments. Accordingly, sales of our product candidates will depend substantially, both domestically and abroad, on the extent to which the prices of our product candidates 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. We may not be able to provide data sufficient to gain 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 our product candidates. Even if coverage is provided, the approved reimbursement amount may not be adequate to realize a sufficient return on our investment.

There is significant uncertainty related to third-party coverage and reimbursement of newly approved products, including potential one-time gene therapies. In the United States, third-party payors, including government payors such as the Medicare and Medicaid programs, play an important role in determining the extent to which new drugs and biologics will be covered and reimbursed. The Medicare and Medicaid programs increasingly are used as models for how private payors and other government payors develop their coverage and reimbursement policies. It is difficult to predict what the CMS, the agency responsible for administering the Medicare program, will decide with respect to coverage and reimbursement for fundamentally novel products such as ours, as there is little body of established practices and precedents for these types of products. We cannot be assured that Medicare or Medicaid will cover any of our products, if approved, or provide reimbursement at adequate levels to realize a sufficient return on our investment. In addition, government regulators and legislative bodies in the United States have enacted laws and are considering numerous proposals that may result in limitations on the prices at which we could charge customers for our products if we have products that are approved for sale. For example, the IRA enacted in 2022, permits HHS to engage in price-capped negotiation to set the price of certain drugs and biologics reimbursed under Medicare Part B and Part D. The IRA contains statutory exclusions to the negotiation program, including for certain orphan designated drugs for which the only approved indication is for the orphan disease or condition. Should our product candidates be approved and covered by Medicare Part B or Part D, and fail to fall within a statutory exclusion, such as that for an orphan drug, those products could, after a period of time (e.g., 11 years after FDA approval of biologics, including gene therapies), be selected for negotiation and become subject to prices representing a significant discount from average prices to wholesalers and direct purchasers. The IRA also establishes a rebate obligation for drug manufacturers that increase prices of Medicare Part B and Part D covered drugs at a rate greater than the rate of inflation. The inflation rebates may require us to pay rebates if we were to increase the cost of a covered Medicare Part B or Part D covered product faster than the rate of inflation. At this time, we are unable to predict how these recent legislative changes or any future legislation might affect our business. Moreover, reimbursement agencies in the European Union may be more conservative than CMS. It is difficult to predict what third-party payors will decide with respect to the coverage and reimbursement for our product candidates.

Outside the United States, international operations generally are subject to extensive government price controls and other market regulations, and increasing emphasis on cost-containment initiatives in the European Union and other countries may put pricing pressure on us. In many countries, the prices of medical products are subject to varying price control mechanisms as part of national health systems. It also can take a significant amount of time after approval of a product to secure pricing and reimbursement for such product in many countries outside the United States. In general, the prices of medicines under such systems are substantially lower than in the United States. Other countries allow companies to fix their own prices for medical products, but monitor and control company profits. Additional foreign price controls or other changes in pricing regulation could restrict the amount that we are able to charge for our product candidates. Accordingly, in markets outside the United States, the reimbursement for our products may be reduced compared with the reimbursement in the United States and may be insufficient to generate commercially reasonable product revenues.

Moreover, increasing efforts by government and third-party payors in the United States and abroad to cap or reduce healthcare costs has led to increased pressure on the healthcare industry to reduce costs and may, in the future, cause such organizations to limit both coverage and the level of reimbursement for new products approved and, as a result, they may not cover or provide adequate payment for our product candidates. Payors increasingly are considering new metrics as the basis for reimbursement rates, and the existing data for reimbursement based on some of these metrics is limited. Therefore, it may be difficult to project the impact of these evolving reimbursement metrics on the willingness of payors to cover candidate products that we or our partners are able to commercialize. We expect to experience pricing pressures in connection with the sale of any of our product candidates due to the trend toward managed healthcare, the increasing influence of health maintenance organizations and additional legislative changes.

Additionally, our lead product candidates are designed to provide therapeutic benefit after a single administration and, therefore, the pricing and reimbursement of a single administration of our lead product candidates, if approved, must be adequate to support our commercial infrastructure. The downward pressure on healthcare costs in general, particularly prescription drugs and surgical procedures and other treatments, has become intense. As a result, increasingly high barriers are being erected to the entry of new products. If we are unable to obtain adequate levels of reimbursement, our ability to successfully market and sell our product candidates will be harmed. The manner and level at which reimbursement is provided for services related to 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 limit our ability to market or sell our products.

We cannot predict the extent to which our business may be affected by these or other potential future legislative or regulatory developments. However, future price controls or other changes in pricing regulation or negative publicity related to the pricing of drugs and biologics generally could restrict the amount that we are able to charge for our future products, if any, which could adversely affect our revenue and results of operations.

Risks Related to Our Business Operations

We may not be successful in our efforts to identify or discover additional product candidates and may fail to capitalize on programs or product candidates that may be a greater commercial opportunity or for which there is a greater likelihood of success.

Our main goals continue to include the ability to identify, develop and commercialize product candidates based on our NAV Technology Platform. Research programs to identify new product candidates require substantial technical, financial and human resources. Although certain of our product candidates are currently in research studies or preclinical development, we may fail to identify potential product candidates for clinical development for several reasons. For example, our research may be unsuccessful in identifying potential product candidates or our potential product candidates may be shown to have harmful side effects, may be commercially impracticable to manufacture or may have other characteristics that may make the products unmarketable or unlikely to receive marketing approval.

Our spending on current and future research and development programs may not yield any commercially viable products. If we do not accurately evaluate the commercial potential for a particular product candidate, we may relinquish valuable rights to that product candidate through strategic collaboration, licensing or other arrangements in cases in which it would have been more advantageous for us to retain sole development and commercialization rights to such product candidate. Alternatively, we may allocate internal resources to a product candidate in a therapeutic area in which it would have been more advantageous to enter into a partnering arrangement.

If any of these events occur, we may be forced to abandon our development efforts with respect to a particular product candidate or fail to develop a potentially successful product candidate, which could materially harm our business, financial condition, results of operations and prospects.

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

Our executive team underwent significant transition in 2024, including the appointment of a new Chief Executive Officer and Chief Financial Officer, as well as the appointment of our former Chief Executive Officer as Chairman of the Board of Directors. If we are unable to successfully transition to our new leadership team, or if we were to lose the services of any of the members of our executive team, we may be unable to achieve our strategic priorities. In addition, our success depends in large part on the performance of our team. If we were to lose the services of a significant number of employees, consultants or advisors, or those who sit in key positions, including scientific and technical roles, such loss could impede our ability to achieve our research, development, licensing and commercialization objectives. We currently do not have “key person” insurance on any of our employees.

Recruiting and retaining other qualified employees, consultants and advisors for our business, including scientific and technical personnel is, and will continue to be, critical to our success. There currently is a shortage of skilled individuals with substantial gene therapy experience. As a result, competition for skilled personnel, including in gene therapy research and vector manufacturing, is intense and the turnover rate can be high. We may not be able to attract and retain personnel on acceptable terms given the competition among numerous pharmaceutical and biotechnology companies and academic institutions for individuals with similar skill sets. In addition, failure to succeed in preclinical studies or clinical trials or applications for marketing approval may make it more challenging to recruit and retain qualified personnel. The inability to recruit, or loss of services of any of our key executives, employees, consultants or advisors may impede the progress of our research, development, licensing and commercialization objectives and materially harm our business, financial condition, results of operations and prospects.

If we are successful in executing our business strategy, we will need to expand our managerial, operational, financial, compliance and other systems and resources to manage our operations, continue our research and development and licensing activities, and, in the longer term, build a sales and marketing infrastructure to support commercialization of any of our product candidates that are approved for sale. Future growth would impose significant added responsibilities on members of management. It is likely that our management, finance, development personnel, systems and facilities currently in place may not be adequate to support this future growth. Our need to effectively manage our operations, growth and product candidates requires that we continue to develop more robust business processes and improve our systems and procedures in each of these areas and to attract and retain sufficient numbers of talented employees. We may be unable to successfully implement these tasks on a larger scale and, accordingly, may not achieve our research, development and growth goals.

If our employees, principal investigators, consultants or commercial partners engage in misconduct, or if we are unable to comply with federal and state healthcare fraud and abuse laws, false claims laws, health information privacy and security laws or other applicable laws or regulations, then we could face substantial penalties.

We are exposed to the risk of fraud or other misconduct by our employees, principal investigators, consultants and commercial partners. Misconduct by these parties could include intentional failures to: comply with FDA regulations or the regulations applicable in the European Union 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 business 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 adverse impact on our business, financial condition, results of operations and prospects, including the imposition of significant fines or other sanctions.

If we obtain the approval of the FDA, the European Commission or other regulatory authorities for any of our product candidates and begin commercializing those products in the United States or outside the United States, our operations will be directly, or indirectly through our prescribers, customers and purchasers, subject to various federal, state and foreign fraud and abuse laws and regulations, including, without limitation, the federal Health Care Program Anti-Kickback Statute, the federal civil and criminal False Claims Act and Physician Payments Sunshine Act and regulations, and similar laws in foreign jurisdictions. These laws will impact, among other things, our proposed sales, marketing and educational programs. In addition, we may be subject to patient privacy laws by both the federal government and the states in which we conduct our business. The laws that will affect our operations include, but are not limited to:

- the federal Health Care Program Anti-Kickback Statute, which prohibits, among other things, persons or entities from knowingly and willfully soliciting, receiving, offering or paying any remuneration (including any kickback, bribe or rebate), directly or indirectly, overtly or covertly, in cash or in kind, in return for the purchase, recommendation, leasing or furnishing of an item or service reimbursable under a federal healthcare program, such as the Medicare and Medicaid programs. This statute has been interpreted to apply to arrangements between pharmaceutical manufacturers on the one hand, and prescribers, purchasers and formulary managers on the other. Liability may be established under the federal Anti-Kickback Statute without proving actual knowledge of the statute or specific intent to violate it;

•federal civil and criminal false claims laws and civil monetary penalty laws which prohibit, among other things, individuals or entities from knowingly presenting, or causing to be presented, claims for payment or approval from Medicare, Medicaid or other government payors that are false or fraudulent. PPACA provides and recent government cases against pharmaceutical and medical device manufacturers support the view that federal Anti-Kickback Statute violations and certain marketing practices, including off-label promotion, may implicate the False Claims Act;

•HIPAA, which created new federal criminal statutes that prohibit a person from knowingly and willfully executing a scheme or from making false or fraudulent statements to defraud any healthcare benefit program, regardless of the payor (e.g., public or private);

•HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act (HITECH), and its implementing regulations, and as amended again by the final HIPAA omnibus rule, Modifications to the HIPAA Privacy, Security, Enforcement, and Breach Notification Rules Under HITECH and the Genetic Information Nondiscrimination Act;

•Other Modifications to HIPAA, which imposes certain requirements relating to 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;

•federal transparency laws, including the federal Physician Payment Sunshine Act, that require disclosure of payments and other transfers of value provided to physicians and teaching hospitals, and ownership and investment interests held by physicians and other healthcare providers and their immediate family members and applicable group purchasing organizations;

•national laws, industry codes and professional codes of conduct applicable to certain European Union Member States which require payments made to physicians to be publicly disclosed and agreements with physicians to often be the subject of prior notification and approval by the physicians' employer, his or her competent professional organization and/or the regulatory authorities of the individual Member States;

•federal, state and foreign laws relating to the processing, storage and transfer of personal data, including, but not limited to, the California Consumer Privacy Act and the European Union's General Data Protection Regulation, which may require us to incur substantial costs or change our business practices with respect to the treatment of personal data; and

•state and foreign law equivalents of each of the above federal laws, state laws that require drug manufacturers to report information related to payments and other transfers of value to physicians and other healthcare providers or marketing expenditures and state laws governing the privacy and security of health information in certain circumstances, many of which differ from each other in significant ways and may not have the same effect, thus complicating compliance efforts in certain circumstances, such as specific disease states.

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, reputational harm, public reprimands, third-party actions, such as cease and desist letters or injunctions, and the curtailment or restructuring of our operations, any of which could harm our ability to operate our business and our results of operations.

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 European Union. The provision of benefits or advantages to physicians is also governed by the national anti-bribery laws of European Union Member States and other jurisdictions in which we may operate. Infringement of these laws could result in substantial fines and imprisonment.

Product liability lawsuits against us could cause us to incur substantial liabilities and could limit licensing of our NAV Technology Platform or commercialization of any product candidates that we may develop.

We face an inherent risk of product liability exposure related to our licensed NAV Technology Platform and the testing of our product candidates in clinical trials and may face an even greater risk if products utilizing our NAV Technology Platform are commercialized. If we cannot successfully defend ourselves against claims that our technology or product candidates caused injuries, we could incur substantial liabilities. Regardless of merit or eventual outcome, liability claims may result in:

•decreased demand for our technology, including any product candidates that we may develop;

•loss of revenue;

•substantial monetary awards to trial participants or patients;

•significant time and costs to defend the related litigation;

•withdrawal of clinical trial participants;

•the inability to license our NAV Technology Platform or commercialize any product candidates that we may develop; and

•injury to our reputation and significant negative media attention.

Although we maintain product liability insurance coverage, this insurance may not be adequate to cover all liabilities that we may incur. We anticipate that we will evaluate the need to increase our insurance coverage each time we commence a clinical trial and may from time to time purchase additional coverage for clinical trials. We may need to increase our product liability insurance coverage if we successfully commercialize any product candidates. Insurance coverage is increasingly expensive and 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, our development partners, including our licensees and collaborators, or our third-party manufacturers or suppliers fail to comply with environmental, health and safety laws and regulations, we could become subject to fines or penalties or incur costs that could materially harm the success of our business.

We, our development partners, including our licensees and collaborators, and our third-party manufacturers and suppliers are subject to numerous environmental, health and safety laws and regulations, including those governing laboratory procedures and the generation, handling, use, storage, treatment, manufacture, transportation and disposal of, and exposure to, hazardous materials and wastes, as well as laws and regulations relating to occupational health and safety. Our operations involve the use of hazardous and flammable materials, including chemicals and biologic and radioactive materials. Our operations and the operations of our development partners and third-party manufacturers and suppliers also produce hazardous waste products. We cannot eliminate the risk of contamination or injury from these materials. In the event of contamination or injury resulting from the use of hazardous materials by us, our development partners or our third-party manufacturers or suppliers, we could be held liable for any resulting damages, and any liability could exceed our resources. We also could incur significant costs associated with civil or criminal fines and penalties.

Although we maintain workers' compensation insurance for certain costs and expenses we may incur due to work-related injuries to our employees, this insurance may not provide adequate coverage against potential liabilities. Although we maintain insurance for claims that may be asserted against us in connection with our storage or disposal of biologic, hazardous or radioactive materials, this insurance may not be adequate to cover all liabilities that we may incur in connection with such claims.

In addition, we may incur substantial costs in order to comply with current or future environmental, health and safety laws and regulations, which have tended to become more stringent over time. These current or future laws and regulations may impair us or our development partners', including our licensees' and collaborators', research, development or production efforts. Failure to comply with these laws and regulations also may result in substantial fines, penalties or other sanctions or liabilities, which could materially harm our business, financial condition, results of operations and prospects.

Our information technology systems, or those of our vendors, collaborators or other contractors or consultants, may fail or suffer security breaches, which could result in a material disruption of our business or financial operations, including our licensing and product development programs.

Our information technology systems and those of our current and any future vendors, collaborators and other contractors or consultants are vulnerable to damage from computer viruses, unauthorized access, natural disasters, terrorism, war and telecommunication and electrical failures. Although we have experienced cybersecurity incidents from time to time in the past, we believe we have not experienced any incident that has had a material effect on our business. If such an incident were to occur in the future and cause a material interruption in our operations, it could result in a material disruption of our business or financial operations, including our licensing and development programs, and potentially subject us to liability. Unauthorized disclosure of sensitive or confidential patient or employee data, including personally identifiable information, whether through breach of information technology systems, systems failure, employee negligence, fraud or misappropriation, or otherwise, or unauthorized access to or through our information systems and networks, whether by our employees or third parties, could result in negative publicity, legal liability and damage to our reputation. Unauthorized disclosure of personally identifiable information could also expose us to sanctions for violations of data privacy laws and regulations around the world, especially since the regulatory environment surrounding data privacy laws are increasingly demanding, with frequent imposition of new and changing requirements. To the extent that any disruption or security breach results in a loss of, or damage to, our trade secrets, data or applications, or inappropriate disclosure of confidential or proprietary information, we could incur liability, our competitive position could be harmed

and the further licensing of our NAV Technology Platform and development and commercialization of our product candidates could be delayed. For example, the loss of, or damage to, clinical trial data for any of our product candidates could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data.

Security breaches pose a risk that sensitive data, including intellectual property, trade secrets or personal information, may be exposed to unauthorized persons or to the public. Cyber-attacks are increasing in their frequency, sophistication and intensity, and have become increasingly difficult to detect. Cybersecurity attacks include, but are not limited to, malicious software (malware, ransomware and viruses), phishing and social engineering, attempts to gain unauthorized access to networks, computer systems and data, malicious or negligent actions of employees (including misuse of information they are entitled to access), cyber extortion, electronic or wire fraud, and other forms of electronic security breaches. These incidents may be caused by failures during routine operations, such as system upgrades, or by user errors, as well as network or hardware failures, malicious or disruptive software, unintentional or malicious actions of employees or contractors, cyberattacks by hackers, criminal groups or nation-state organizations (which may include social engineering, business email compromise, cyber extortion, denial of service, or attempts to exploit vulnerabilities), geopolitical events, natural disasters, failures or impairments of telecommunications networks, or other catastrophic events. Our business partners face similar risks, and a security breach of their systems could adversely affect our security posture. While we have procedures in place for selecting and managing our relationships with third-party service providers and other business partners, we do not have control over their business operations or governance and compliance systems, practices and procedures, and our management of multiple third-party service providers and business partners increases our operational complexity. If we fail to adequately monitor our third-party service providers' and business partners' performance, including for compliance with our agreements and regulatory and legal requirements, we may have to incur additional costs to correct errors, our reputation could be harmed or we could be subject to litigation, claims, legal or regulatory proceedings, inquiries or investigations. Third-party service providers and business partners may experience cybersecurity incidents that may involve data we share with them or rely on them to provide to us, and the need to coordinate with such third-parties and business partners, including with respect to timely notification and access to personnel and information concerning an incident, may complicate our efforts to resolve any issues that arise. As a result, we are subject to the risk that the activities associated with our third-party service providers and business partners will adversely affect our business, even if the cyber incident does not directly impact our systems or information. While we continue to invest in data protection and information technology, including providing an information security training and compliance program to our employees, there can be no assurance that our efforts will prevent service interruptions or identify breaches in our systems that could adversely affect our business and operations and/or result in the loss of critical or sensitive information, which could result in financial, legal, business or reputational harm.

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 cover one or more large claims; 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.

Our customers are concentrated and therefore the loss of a significant customer may harm our business.

Our current revenues are derived from a concentrated customer base. Our revenues for the years ended December 31, 2024 and 2023 consisted solely of license and royalty revenue. One customer accounted for approximately 98% of our total revenues for the year ended December 31, 2024. One customer accounted for approximately 95% of our total revenues for the year ended December 31, 2023. We expect future license and royalty revenue to be derived from a limited number of licensees and collaborators. Future license and royalty revenue is uncertain due to the contingent nature of our licenses granted to third parties.

Risks Related to Our Intellectual Property

Our rights to license our NAV Technology Platform and to develop and commercialize our 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 technology and products, including technology related to our manufacturing process and our gene therapy 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 license our platform or 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 not included in all of our licenses. For example, under our license agreement with GSK, GSK retained certain exclusive and non-exclusive rights under the patent rights that it licensed from Penn.

Licenses to additional third-party technology that may be required for our licensing or development programs may not be available in the future or may not be available on commercially reasonable terms, or at all, which could materially harm our business and financial condition.

In some circumstances, we may not have the right to control the preparation, filing and prosecution of patent applications, or to maintain or enforce the patents, covering technology that we license from third parties. For example, under our license agreement with Penn, Penn is entitled to control the preparation, prosecution and maintenance of the patent rights licensed to us. However, if we determine that we desire a greater degree of control over such patent rights, the Penn license agreement provides that Penn will work in good faith with us to enter into an arrangement for such additional control with reimbursement by us of certain expenses. If our licensors fail to maintain 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 impacted. In addition to the foregoing, the risks associated with patent rights that we license from third parties will also apply to patent rights we may own in the future.

Furthermore, the research resulting in certain of our licensed patent rights and technology was funded by the U.S. government. As a result, the government may have certain rights, or march-in rights, to such patent rights and technology. When new technologies are developed with government funding, the government generally obtains certain rights in any resulting patents, including a non-exclusive license authorizing the government to use the invention for non-commercial purposes. These rights may permit the 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. The government can exercise its march-in rights if it determines that action is necessary because we fail to achieve practical application of the government-funded technology, because action is necessary to alleviate health or safety needs, to meet requirements of federal regulations or to give preference to U.S. industry. In addition, our rights in such inventions may be subject to certain requirements to manufacture products embodying such inventions in the United States. Any exercise by the government of such rights could harm our competitive position, business, financial condition, results of operations and prospects.

If we are unable to obtain and maintain patent protection for our products and 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 license our NAV Technology Platform and commercialize our products and technology may be harmed.

Our success depends, in part, on our ability to obtain and maintain patent protection in the United States and other countries with respect to our proprietary NAV Technology Platform, our product candidates and our manufacturing technology. Our licensors have sought and we intend to seek to protect our proprietary position by filing patent applications in the United States and abroad related to many of our novel technologies and product candidates that are important to our business.

The patent prosecution process is expensive, time-consuming and complex, and we may not be able to file, prosecute, maintain, enforce or license all necessary or desirable patent applications at a reasonable cost or in a timely manner. In addition, certain patents in the field of gene therapy that may have otherwise potentially provided patent protection for certain of our product candidates have expired or will soon expire. In some cases, the work of certain academic researchers in the gene therapy field has entered the public domain, which we believe precludes our ability to obtain patent protection for certain inventions relating to such work. It is also possible that we will fail to identify patentable aspects of our research and development output before it is too late to obtain patent protection.

We are a party to intellectual property license agreements with GSK and Penn, each of which is important to our business, and other entities and we expect to enter into additional license agreements in the future. Our existing license agreements impose, and we expect that future license agreements will impose, various diligence, development and commercialization timelines, milestone payments, royalties and other obligations on us. If we or our licensees or collaborators fail to comply with our obligations under these agreements, or we are subject to a bankruptcy, the licensor may have the right to terminate the license, in which event we would not be able to market products covered by the license.

The patent position of biotechnology and pharmaceutical companies generally is highly uncertain, involves complex legal and factual questions and has, in recent years, been the subject of much litigation. As a result, the issuance, scope, validity, enforceability and commercial value of our patent rights are highly uncertain.

Our pending and future patent applications may not result in patents being issued which protect our technology or product candidates or which effectively prevent others from commercializing competitive technologies and product candidates. Changes in either the patent laws or interpretation of the patent laws in the United States and other countries may diminish the value of our patents or narrow the scope of our patent protection.

We may not be aware of all third-party intellectual property rights potentially relating to our technology and product candidates. Publications of discoveries in the scientific literature often lag the actual discoveries, and patent applications in the United States and other jurisdictions are typically not published until 18 months after filing or, in some cases, not at all. Therefore, we cannot be certain that we were the first to make the inventions claimed in any owned or any licensed patents or pending patent applications, or that we were the first to file for patent protection of such inventions.

Even if the patent applications we license or may own in the future do 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. Our competitors or other third parties may avail themselves of safe harbor under the Drug Price Competition and Patent Term Restoration Act of 1984 (the Hatch-Waxman Amendments) to conduct research and clinical trials and may be able to circumvent our patents by developing similar or alternative technologies or products in a non-infringing manner.

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. Such challenges may result in 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. 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.

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

Because we rely on third parties, including contractors, to research, develop and manufacture our product candidates, we must, at times, share trade secrets with them. We seek to protect our proprietary technology in part by entering into confidentiality agreements and, if applicable, material transfer agreements, consulting agreements or other similar agreements with our advisors, employees, third-party contractors and consultants prior to beginning research or disclosing proprietary information. These agreements typically limit the rights of the third parties to use or disclose our confidential information, including our trade secrets. Despite the contractual provisions employed when working with third parties, these provisions may be breached, and the need to share trade secrets and other confidential information increases the risk that such trade secrets become known by our competitors, are inadvertently incorporated into the technology of others, or are disclosed or used in violation of these agreements. Any disclosure, either intentional or unintentional, by our employees, the employees of third parties with whom we share our facilities or third-party consultants and vendors that we engage to perform research, clinical trials or manufacturing activities, or misappropriation by third parties (such as through a cybersecurity breach) of our trade secrets or proprietary information could enable competitors to duplicate or surpass our technological achievements, thus eroding our competitive position in our market. Further, adequate remedies may not exist in the event of unauthorized use or disclosure. Given that our proprietary position is based, in part, on our know-how and trade secrets, a competitor's independent discovery of our trade secrets or other unauthorized use or disclosure would impair our competitive position and may materially harm our business.

In addition, these agreements typically restrict the ability of our advisors, employees, third-party contractors and consultants to publish data potentially relating to our trade secrets, although our agreements may contain certain limited publication rights. For example, any academic institution that we collaborate with, or may collaborate with in the future, will sometimes be granted rights to publish data arising out of such collaboration, provided that we are notified in advance and given the opportunity to delay publication for a limited time period in order for us to secure patent protection of intellectual property rights arising from the collaboration, in addition to the opportunity to remove confidential or trade secret information from any such publication. We may also conduct joint research and development programs that may require us to share trade secrets under the terms of our research and development or similar agreements. Despite our efforts to protect our trade secrets, our competitors may discover our trade secrets, either through breach of our agreements with third parties, independent development or publication of information by any of our third-party collaborators. A competitor's discovery of our trade secrets would impair our competitive position and harm our business.

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

The agreements under which we currently license intellectual property or technology from or to third parties, including the AbbVie Collaboration and License Agreement, the Nippon Shinyaku Collaboration and License Agreement and our license agreements with GSK and Penn, 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, increase what we believe to be our financial or other obligations under the relevant agreement, or decrease what we believe to be the financial or other obligations of our licensee under the relevant agreement, any of which could materially harm 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 or obtain additional licensing arrangements on commercially acceptable terms, we may be unable to successfully develop and commercialize the affected products or product candidates, which could have a material adverse effect on our business, financial conditions, 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 others to advance our research, to expand our licensing program or to allow commercialization of our product candidates. It is possible that we may be unable to obtain 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 or 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 redesign our platform technology or to develop or commercialize the affected product candidates, which could materially harm our business. We cannot provide any assurances that third-party patents do not exist or will not be issued, which might be enforced against our current platform technology, manufacturing methods, product candidates or future methods or products, resulting in either an injunction prohibiting our licensing, 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.

In many of our existing license agreements, patent prosecution of our licensed technology is controlled primarily by the licensor, and we are required to reimburse the licensor for certain costs of patent prosecution and maintenance. If our licensors fail to obtain and maintain patent or other protection for the proprietary intellectual property we license from them, we could lose our rights to the intellectual property or our exclusivity with respect to those rights, and our competitors could market competing products using the intellectual property. Further, in our license agreements, we could be responsible for bringing actions against any third party for infringing on the patents we have licensed. Certain of our license agreements in which we are the licensee also require us to meet development milestones to maintain the license, including establishing a set timeline for developing and commercializing products and minimum diligence obligations in developing and commercializing the product. 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 or otherwise violate intellectual property of the licensor that is not subject to the licensing agreement;
- the sublicensing and corresponding payment obligations of patent and other intellectual property rights under our collaborative development relationships;
- our diligence obligations under the license agreement and what activities satisfy those diligence obligations;
- the inventorship or ownership of inventions and know-how resulting from the joint creation or use of intellectual property by our licensors and us and our partners; and
- the priority of invention of patented technology.

If disputes over intellectual property that we have licensed prevent or impair our ability to maintain our current licensing arrangements on acceptable terms, we may be unable to successfully develop and commercialize the affected product candidates.

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

We currently have rights to intellectual property, through licenses from third parties, to develop our product candidates. Because our programs may require the use of intellectual property or other proprietary rights held by third parties, the growth of our business may depend, in part, on our ability to acquire, in-license or use such intellectual property and proprietary rights. We may be unable to acquire or in-license any compositions, methods of use, processes (and patents for such technology) or other intellectual property rights from third parties that we identify as necessary for our technology platform and product candidates. The licensing or acquisition of third-party intellectual property rights is a competitive area, and several more established companies may pursue strategies to license or acquire third-party intellectual property rights that we may consider attractive. These established companies may have a competitive advantage over us due to their size, capital resources and greater clinical development and commercialization capabilities. In addition, companies that perceive us to be a competitor may be unwilling to license rights to us. We also may be unable to license or acquire third party intellectual property rights on terms that would allow us to make an appropriate return on our investment.

We sometimes collaborate with non-profit and academic institutions to accelerate our preclinical research or development under written agreements with these institutions. Some of these institutions may provide us with an option to negotiate a license to any of the institution's rights in technology resulting from the collaboration. Regardless of such option, we may be unable to negotiate a license within the specified timeframe or under terms that are acceptable to us. If we are unable to do so, the institution may offer the intellectual property rights to other parties, potentially blocking our ability to pursue our program.

If we are unable to successfully obtain rights to required third-party intellectual property rights or maintain the existing intellectual property rights we have, we may have to abandon development of the relevant program or product candidate and our business, financial condition, results of operations and prospects could suffer.

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

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

Many companies have encountered significant problems in protecting and defending intellectual property rights in foreign jurisdictions. The legal systems of certain countries, particularly certain developing countries, do not favor the enforcement of patents, 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 or marketing of competing products in violation of our proprietary rights generally. Proceedings to enforce our patent rights in foreign jurisdictions could result in substantial costs and divert our efforts and attention from other aspects of our business, could put our patents at risk of being invalidated or interpreted narrowly and our patent applications at risk of not issuing and could provoke third parties to assert claims against us. We may not prevail in any lawsuits that we initiate and the damages or other remedies awarded, if any, may not be commercially meaningful. Accordingly, our efforts to enforce our intellectual property rights around the world may be inadequate to obtain a significant commercial advantage from the intellectual property that we develop or license. In this regard, we are engaged in patent litigation with Sarepta Therapeutics, Inc. (Sarepta) arising from its use of cultured host cell technology, which we believe is claimed in a patent we licensed from Penn, to make gene therapy products to treat Duchenne muscular dystrophy and Limb-girdle muscular dystrophy, among other products. In January 2024, the U.S. District Court for the District of Delaware granted Sarepta's motion for summary judgment dismissing the case. Although we have appealed this decision our litigation against Sarepta will have an uncertain outcome and may not result in the patent enforcement we desire.

Competitors may infringe our patents or the patents of our licensing partners, or we may be required to defend against claims of infringement or that our intellectual property is invalid or unenforceable. To counter infringement or unauthorized use claims or to defend against claims of infringement or other intellectual property related claims can be expensive and time consuming. Even if resolved in our favor, litigation or other legal proceedings relating to intellectual property claims may cause us to incur significant expenses, and could distract our technical and management personnel from their normal responsibilities. In addition, there could be

public announcements of the results of hearings, motions or other interim proceedings or developments and if securities analysts or investors perceive these results to be negative, it could materially harm the price of our common stock. Such litigation or proceedings could substantially increase our operating losses and reduce the resources available for development activities or any future sales, marketing or distribution activities. We may not have sufficient financial or other resources to adequately conduct such litigation or proceedings. Some of our competitors may be able to sustain the costs of such litigation or proceedings more effectively than we can because of their greater financial resources and more mature and developed intellectual property portfolios. An adverse result in any litigation proceeding could put one or more of our patents or licensed patents at risk of being invalidated, held unenforceable or interpreted narrowly, and could put any of our patent applications at risk of not yielding an issued patent. Furthermore, because of the substantial amount of discovery required in connection with intellectual property litigation, there is a risk that some of our confidential information or trade secrets could be compromised by disclosure during this type of litigation. Uncertainties resulting from the initiation and continuation of patent and other intellectual property litigation or proceedings could materially harm our ability to compete in the marketplace.

Periodic maintenance fees, renewal fees, annuity fees and various other government fees on patents and/or applications will be due to be paid to the U.S. Patent and Trademark Office (the USPTO) and various patent agencies outside of the United States over the lifetime of our licensed patents and/or applications and any patent rights we may own or license in the future. We may rely on our licensing partners to pay these fees due to non-U.S. patent agencies with respect to our licensed patent rights. The USPTO and various non-U.S. patent agencies require compliance with several procedural, documentary, fee payment and other similar provisions during the patent application process. We employ reputable law firms and other professionals to help us comply and 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 many 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 partial or complete loss of patent rights in the relevant jurisdiction. In such an event, potential competitors might be able to enter the market and this circumstance could materially harm our business. If we or our licensors fail to maintain the patents and patent applications covering our product candidates, it could have a material adverse effect on our business.

We have registered trademarks with the USPTO, including for the marks "AAVIATE," "AFFINITY," "AFFINITY BEYOND," "AFFINITY DUCHENNE," "ALTITUDE," "ATMOSPHERE," "CAMPSIITE," "NAV," "NAVXcell", "NAVXpress" and "REGENXBIO," as well as for the REGENXBIO logos. Our trademarks or trade names may be challenged, infringed, circumvented or declared generic or determined to be infringing on other marks. We may not be able to protect our rights to these trademarks and trade names, which we need to build name recognition among potential partners or customers in our markets of interest. At times, competitors may adopt trade names or trademarks similar to ours, thereby impeding our ability to build brand identity and possibly leading to market confusion. In addition, there could be potential trade name or trademark infringement claims brought by owners of other registered trademarks or trademarks that incorporate variations of our registered or unregistered trademarks or trade names. Over the long-term, if we are unable to establish name recognition based on our trademarks and trade names, then we may not be able to compete effectively and our business may be harmed. Our efforts to enforce or protect our proprietary rights related to trademarks, trade secrets, domain names, copyrights or other intellectual property may be ineffective and could result in substantial costs and diversion of resources and could harm our financial condition or results of operations.

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.

Issued patents covering our NAV Technology Platform or our product candidates could be found invalid or unenforceable if challenged in court. We may not be able to protect our trade secrets in court.

If one of our licensing partners or we initiate legal proceedings against a third party to enforce a patent covering our NAV Technology Platform or one of our product candidates, the defendant could counterclaim that the patent covering our product candidate is invalid or unenforceable. In patent litigation in the United States, defendant counterclaims alleging invalidity or unenforceability are commonplace. Grounds for a validity challenge could be an alleged failure to meet any of several statutory requirements, including subject-matter eligibility, novelty, non-obviousness, written description or enablement. Grounds for an unenforceability assertion could be an allegation that someone connected with prosecution of the patent withheld information material to patentability from the USPTO, or made a misleading statement, during prosecution. Third parties also may raise similar claims before administrative bodies in the United States or abroad, even outside the context of litigation. Such mechanisms include re-examination, post grant review, *inter partes* review and equivalent proceedings in foreign jurisdictions. Such proceedings could result in the revocation or cancellation of or amendment to our patents in such a way that they no longer cover our NAV Technology

Platform or our product candidates. The outcome following legal assertions of invalidity and unenforceability is unpredictable. With respect to the validity question, for example, we cannot be certain that there is no invalidating prior art, of which the patent examiner and we or our licensing partners were unaware during prosecution. If a defendant were to prevail on a legal assertion of invalidity or unenforceability, we could lose at least part, and perhaps all, of the patent protection on one or more of our product candidates. Such a loss of patent protection could materially harm our business.

In addition to the protection afforded by patents, we rely on trade secret protection and confidentiality agreements to protect proprietary know-how that is not patentable or that we elect not to patent, processes for which patents are difficult to enforce and any other elements of our technology, product candidate discovery and development processes that involve proprietary know-how, information or technology that is not covered by patents. However, trade secrets can be difficult to protect and some courts inside and outside the United States are less willing or unwilling to protect trade secrets. We seek to protect our proprietary technology and processes, in part, by entering into confidentiality agreements with our employees, consultants, scientific advisors and contractors. 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. We also seek to preserve the integrity and confidentiality of our data and trade secrets by maintaining physical security of our premises and physical and electronic security of our information technology systems. While we have confidence in these individuals, organizations and systems, agreements or security measures may be breached, and we may not have adequate remedies for any breach. In addition, our trade secrets may otherwise become known or be independently discovered by competitors.

Third parties may initiate legal proceedings alleging that we are infringing their intellectual property rights, the outcome of which would be uncertain and could materially harm our business.

Our commercial success depends, in part, upon our ability to license our NAV Technology Platform, and upon our ability and our licensees' and collaborators' ability to develop, manufacture, market and sell products and use our proprietary technologies without infringing or otherwise violating the proprietary rights and intellectual property of third parties. The biotechnology and pharmaceutical industries are characterized by extensive and complex litigation regarding patents and other intellectual property rights. We or our collaborators may in the future become party to, or be threatened with, adversarial proceedings or litigation regarding intellectual property rights with respect to our product candidates and technology, including interference proceedings, post grant review and *inter partes* review before the USPTO. Third parties may assert infringement claims against us or our collaborators based on existing patents or patents that may be granted in the future, regardless of their merit. There is a risk that third parties may choose to engage in litigation with us or our collaborators to enforce or to otherwise assert their patent rights against us. Even if we believe such claims are without merit, a court of competent jurisdiction could hold that these third-party patents are valid, enforceable and infringed, which could materially harm our ability to license our technology platform or commercialize our lead product candidates or any future product candidates or technologies covered by the asserted third-party patents. In order to successfully challenge the validity of any such U.S. patent in federal court, we would need to overcome a presumption of validity. As this burden is a high one requiring us to present clear and convincing evidence as to the invalidity of any such U.S. patent claim, there is no assurance that a court of competent jurisdiction would invalidate the claims of any such U.S. patent. If we are found to infringe a third party's valid and enforceable intellectual property rights, we could be required to obtain a license from such third party to continue licensing, developing, manufacturing and marketing our product candidates and 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 or our collaborators could be forced, including by court order, to cease licensing, developing, manufacturing and commercializing the infringing technology or product candidates. In addition, we could be found liable for monetary damages, including treble damages and attorneys' fees, if we are found to have willfully infringed a patent or other intellectual property right or if our collaborators are found to have willfully infringed a patent or other intellectual property right, for which we may be required to indemnify our collaborators. A finding of infringement could prevent us from licensing our technology platform or manufacturing and commercializing our product candidates or force us to cease some of our business operations, which could materially harm our business. Claims that we have misappropriated the confidential information or trade secrets of third parties could similarly harm our business, financial condition, results of operations and prospects.

We may be subject to claims asserting 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 or 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 these individuals or we 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 of intellectual property rights may not be self-executing or the assignment agreements may be breached, and we may be forced to bring claims against third parties, or defend claims that they may bring against us, to determine the ownership of what we regard as our intellectual property.

Changes in U.S. patent law could diminish the value of patents in general, thereby impairing our ability to protect our products.

The patent positions of companies engaged in the development and commercialization of biologics and pharmaceuticals are particularly uncertain. On March 20, 2012, the Supreme Court issued a decision in *Mayo Collaborative Services v. Prometheus Laboratories, Inc. (Prometheus)*, a case involving patent claims directed to a process of measuring a metabolic product in a patient to optimize a drug dosage for the patient. According to the Supreme Court, the addition of well-understood, routine or conventional activity such as "administering" or "determining" steps was not enough to transform an otherwise patent-ineligible natural phenomenon into patent-eligible subject matter. On June 13, 2013, the Supreme Court issued its decision in *Association for Molecular Pathology v. Myriad Genetics, Inc. (Myriad)*, a case involving patent claims held by *Myriad* relating to the breast cancer susceptibility genes BRCA1 and BRCA2. *Myriad* held that an isolated segment of naturally occurring DNA, such as the DNA constituting the BRCA1 and BRCA2 genes, is not patent eligible subject matter, but that complementary DNA, which is an artificial construct that may be created from RNA transcripts of genes, may be patent eligible.

The USPTO has issued a number of guidance memoranda and updates to instruct USPTO examiners on the ramifications of the *Prometheus*, *Myriad* and other court rulings and the application of the rulings to natural products and principles including all naturally occurring nucleic acids. USPTO guidance may be further updated in view of developments in the case law and in response to public feedback. Patents for certain of our product candidates contain claims related to specific DNA sequences that are naturally occurring and, therefore, could be the subject of future challenges made by third parties. In addition, USPTO guidance or changes in guidance or procedures issued by the USPTO could make it impossible for us to pursue similar patent claims in patent applications we may prosecute in the future.

We cannot assure you that our efforts to seek patent protection for our technology and products will not be negatively impacted by the decisions described above, rulings in other cases or changes in guidance or procedures issued by the USPTO. We cannot fully predict what ongoing impact the Supreme Court's decisions in *Prometheus* and *Myriad* may have on the ability of life science companies to obtain or enforce patents relating to their products and technologies in the future. These decisions, the guidance issued by the USPTO and rulings in other cases or changes in USPTO guidance or procedures could materially harm our existing patent portfolio and our ability to protect and enforce our intellectual property in the future.

Moreover, although the Supreme Court has held in *Myriad* that isolated segments of naturally occurring DNA are not patent-eligible subject matter, certain third parties could allege that activities that we may undertake infringe other gene-related patent claims, and we may deem it necessary to defend ourselves against these claims by asserting non-infringement and/or invalidity positions, or paying to obtain a license to these claims. In any of the foregoing or in other situations involving third-party intellectual property rights, if we are unsuccessful in defending against claims of patent infringement, we could be forced to pay damages or be subjected to an injunction that would prevent us from utilizing the patented subject matter. Such outcomes could harm our business, financial condition, results of operations or prospects.

If we do not obtain patent term extension and data exclusivity for our product candidates, our business may be materially harmed.

Depending upon the timing, duration and specifics of any FDA marketing approval of our product candidates, one or more of our U.S. patents may be eligible for limited patent term extension under 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, we may not be granted an extension because of, for example, failing to exercise due diligence during the testing phase or regulatory review process, failing to apply within applicable deadlines, failing to apply prior to expiration of relevant patents or otherwise failing to satisfy applicable requirements. Moreover, the applicable time period or the scope of patent protection afforded could be less than we request. If we are unable to obtain patent term extension or the term of any such extension is less than we request, our competitors may obtain approval of competing products following our patent expiration, and our revenue could be reduced, possibly materially. Further, for our licensed patents, we may not have the right to control prosecution, including filing with the U.S. Patent and Trademark Office, a petition for patent term extension under the Hatch-Waxman Act. Thus, if one of our licensed patents is eligible for patent term extension under the Hatch-Waxman Act, we may not be able to control whether a petition to obtain a patent term extension is filed, or obtained, from the U.S. Patent and Trademark Office.

Risks Related to Ownership of Our Common Stock

Our operating results may fluctuate substantially, which makes our future operating results difficult to predict and could cause the price of our common stock to fluctuate substantially.

We expect our operating results to be subject to fluctuations. Our net income or loss and other operating results may be affected by numerous factors, including:

- any variations in the level of expenses related to our NAV Technology Platform, lead product candidates or future product candidates and technologies;
- the addition or termination of any clinical trials and the timing and outcomes of clinical trials;
- any regulatory or clinical developments affecting our lead product candidates, any future product candidates or our licensees' product candidates;
- our execution of any collaborative, licensing or similar arrangements and the timing of any payments we may make or receive under these arrangements;
- changes in the competitive landscape of our industry, including consolidation among our competitors or partners;
- the nature and terms of any stock-based compensation grants;
- any intellectual property infringement lawsuits in which we may become involved;
- our ability to adequately support future growth;
- potential unforeseen business disruptions that increase our costs or expenses;
- future accounting pronouncements or changes in our accounting policies; and
- the changing and volatile global economic environment.

The cumulative effect of these factors could result in large fluctuations and unpredictability in our quarterly and annual operating results. As a result, we believe that comparing our operating results on a period-to-period basis is not necessarily meaningful. Investors should not rely on our past results as an indication of our future performance. This variability and unpredictability could also result in our failure to meet the expectations of securities or industry analysts or investors for any period. If our operating results fall below the expectations of investors or analysts, the price of our common stock could decline substantially. Furthermore, any quarterly or annual fluctuations in our operating results may, in turn, cause the price of our stock to fluctuate substantially. Such a stock price decline could occur even when we have met any previously publicly stated revenue or earnings guidance we have provided.

Raising additional capital may cause dilution to our existing stockholders, restrict our operations or require us to relinquish proprietary rights.

We have raised significant capital through public offerings of our common stock in order to fund our operations, which has caused dilution to our stockholders. We may seek to raise additional capital through public or private equity offerings, debt financings, strategic partnerships, licensing arrangements or other means. We have an effective shelf registration statement on file with the SEC, which allows us to access capital in a timely manner. To the extent that we raise additional capital by issuing equity securities, including through our at-the-market program, the share ownership of existing stockholders will be diluted. Any future debt financing may involve covenants that restrict our operations, including limitations on our ability to incur liens or additional debt, pay dividends, redeem our stock, make certain investments or engage in certain merger, consolidation, or asset sale transactions. In addition, if we seek funds through arrangements with collaborative partners, these arrangements may require us to relinquish rights to some of our technologies or products or otherwise agree to terms unfavorable to us.

If we engage in future acquisitions or strategic partnerships, this may increase our capital requirements, dilute our stockholders, cause us to incur debt or assume contingent liabilities and subject us to other risks.

We may evaluate various acquisitions and strategic partnerships, including licensing or acquiring complementary products, intellectual property rights, technologies, or businesses. Any potential acquisition or strategic partnership may entail numerous risks, including:

- increased operating expenses and cash requirements;

•the assumption of additional indebtedness or contingent liabilities;

•the issuance of our equity securities;

•assimilation of operations, intellectual property and products of an acquired company, including difficulties associated with integrating new personnel;

•the diversion of our management's attention from our existing product programs and initiatives in pursuing such a strategic merger or acquisition;

•retention of key employees, the loss of key personnel, and uncertainties in our ability to maintain key business relationships;

•risks and uncertainties associated with the other party to such a transaction, including the prospects of that party and their existing products or product candidates and regulatory approvals; and

•our inability to generate revenue from acquired technology and/or products sufficient to meet our objectives in undertaking the acquisition or even to offset the associated acquisition and maintenance costs.

In addition, if we undertake acquisitions, we may issue dilutive securities, assume or incur debt obligations, incur large one-time expenses and acquire intangible assets that could result in significant future amortization expense. Moreover, we may not be able to locate suitable acquisition opportunities and this inability could impair our ability to grow or obtain access to technology or products that may be important to the development of our business.

Provisions in our certificate of incorporation and bylaws and under Delaware law might discourage, delay or prevent a change in control of our company or changes in our board of directors and, therefore, depress our stock price.

Our restated certificate of incorporation and amended and restated bylaws contain provisions that could depress the market price of our common stock by acting to discourage, delay or prevent a change in control of our company or changes in our board of directors that the stockholders of our company may deem advantageous. Among other things, these provisions:

•establish a classified board of directors so that not all members of our board are elected at one time;

•permit the board of directors to establish the number of directors;

•provide that directors may only be removed "for cause" and only upon the vote of the holders of at least two-thirds of our outstanding shares;

•require super-majority voting to amend some provisions in our restated certificate of incorporation and amended and restated bylaws;

•authorize the issuance of "blank check" preferred stock that our board of directors could use to implement a stockholder rights plan;

•eliminate the ability of our stockholders to call special meetings of stockholders;

•prohibit stockholder action by written consent, which requires all stockholder actions to be taken at a meeting of our stockholders;

•provide that the board of directors is expressly authorized to adopt, amend or repeal our bylaws; and

•establish advance notice requirements for nominations for election to our board of directors or for proposing matters that can be acted upon by stockholders at annual stockholder meetings.

In addition, Section 203 of the Delaware General Corporation Law may discourage, delay or prevent a change in control of our company. Section 203 imposes certain restrictions on merger, business combinations and other transactions between us and holders of 15% or more of our common stock.

Our restated certificate of incorporation includes exclusive forum clauses for certain litigation that may be initiated by our stockholders, which could limit our stockholders' ability to obtain a favorable judicial forum for disputes with us or our directors, officers or employees.

Pursuant to our restated certificate of incorporation, unless we consent in writing to the selection of an alternative forum, the Court of Chancery of the State of Delaware (or, if the Court of Chancery does not have jurisdiction, the federal district court for the District of Delaware), will be the sole and exclusive forum for (i) any derivative action or proceeding brought on our behalf, (ii) any action asserting a claim of breach of a fiduciary duty owed by any of our directors, officers or other employees to us or our stockholders, (iii) any action asserting a claim arising pursuant to any provision of the Delaware General Corporation Law, our restated certificate of incorporation or our amended and restated bylaws or (iv) any action asserting a claim governed by the internal affairs doctrine. Additionally, if the subject matter of any action within the scope of the preceding sentence is filed in a court other than a court located with the State of Delaware (a Foreign Action) in the name of any stockholder, such stockholder shall be deemed to have consented to (a) the personal jurisdiction of the state and federal courts located within the State of Delaware in connection with any action brought in any such court to enforce the preceding sentence and (b) having service of process made upon such stockholder in any such action by service upon such stockholder's counsel in the Foreign Action as agent for such stockholder.

Additionally, our restated certificate of incorporation provides that, unless we consent in writing to the selection of an alternative forum, the federal district courts of the United States of America shall, to the fullest extent permitted by law, be the exclusive forum for the resolution of any complaint asserting a cause of action arising under the Securities Act. Any person or entity purchasing or otherwise acquiring any interest in any of our securities shall be deemed to have notice of and to have consented to this provision.

The forum selection clauses in our restated certificate of incorporation may limit our stockholders' ability to obtain a favorable judicial forum for disputes with us, our directors, officers or other employees. Alternatively, if a court were to find the choice of forum provisions contained in our restated certificate of incorporation to be inapplicable or unenforceable in an action, we may incur additional costs associated with resolving such action in other jurisdictions, which could adversely affect our business and financial condition.

Our business could be negatively affected as a result of the actions of activist stockholders.

Proxy contests have been waged against many companies in the biopharmaceutical industry over the last several years, and proxy advisory firms or investors may recommend changes to our business operations, provisions in our restated certificate of incorporation or amended and restated bylaws, or the composition of our board of directors or its committees. If faced with a proxy contest or other type of stockholder activism, or a proxy advisory firm recommendation that is adverse to a management proposal, we may not be able to respond successfully to the contest or dispute, which could be disruptive to our business. Even if we are successful, our business could be adversely affected by such a contest or dispute involving us or our partners because:

- responding to proxy contests or other actions by activist stockholders, or adverse proxy advisory firm recommendations, can be costly and time-consuming, disrupting operations and diverting the attention of management and employees;
- perceived uncertainties as to future direction may result in the loss of potential acquisitions, collaborations or licensing opportunities, and may make it more difficult to attract and retain qualified personnel and business partners; and
- if individuals are elected to our board of directors with a specific agenda, it may adversely affect our ability to effectively and timely implement our strategic plan and create additional value for our stockholders.

These actions could cause our stock price to decrease and experience periods of increased volatility.

ITEM 1B. ***UNRESOLVED STAFF COMMENTS***

None.

ITEM 1C. ***CYBERSECURITY***

We regularly assess risks from cybersecurity threats; monitor our information systems for potential vulnerabilities; and test those systems pursuant to our cybersecurity policies, processes and practices. To protect our information systems from cybersecurity threats, we use various security tools that are designed to help identify, escalate, investigate, resolve and recover from security incidents in a timely manner. We require annual information security training to be completed by our employees, and we maintain a limited cybersecurity liability insurance policy.

Our Senior Vice President of Information Technology (SVP, IT) is responsible for the establishment and maintenance of our cybersecurity program, as well as the assessment and management of cybersecurity risks. Our current SVP, IT has over 25 years of experience in information technology and possesses the requisite education, skills and experience expected of an individual assigned to these duties. We also engage third-party consultants and auditors to assess the effectiveness of our cybersecurity prevention and response systems and processes. Where applicable, third-party service providers are contractually obligated to notify us of material incidents arising from cybersecurity events within their purview.

We identify, assess and manage material risks from cybersecurity threats by following written policies and procedures, which are in compliance with the International Organization for Standardization (ISO) 27001 Information Security Management System. The output of this process is then integrated with our enterprise risk management (ERM) program. The ERM program is managed cross-functionally, with input from various senior management representatives across our business operations, and is used to assess risks to our business based on their potential likelihood and magnitude of impact. Our information technology organization provides the inputs to our ERM process related to material cybersecurity risks and mitigation plans. The information technology team is responsible for the prevention, detection, mitigation and remediation of cybersecurity incidents. Cybersecurity incidents are documented and triaged in accordance with a defined process. Incidents deemed to be significant are escalated to the Audit Committee of our Board of Directors after appropriate assessment by the information technology organization and other internal stakeholders. In the event an incident highlights an emerging or previously unidentified cybersecurity risk, such risk is then synthesized into the ERM process.

The Audit Committee of our Board of Directors oversees our ERM program regarding material risks arising from cybersecurity threats impacting our business. Management provides quarterly reporting on our material enterprise risks to the Audit Committee. In addition to material risks identified through the ERM process, our information technology management provides periodic reporting, at least semi-annually, on our cybersecurity risk profile and risk mitigation strategies to the Audit Committee. This reporting is also made available to the full Board of Directors.

In the last three years, we have not identified any cybersecurity incidents which have materially affected, or are reasonably likely to materially affect, our business. For further information regarding cybersecurity risks, please refer to "Risk Factors – Risks Related to Our Business Operations" and other risks described in the "Risk Factors" section of this Annual Report on Form 10-K.

ITEM 2. ***PROPERTIES***

Our corporate headquarters are located in Rockville, Maryland. We occupy approximately 186,000 square feet of office, laboratory and manufacturing space at this location, including a fully operational cGMP manufacturing facility, under a lease that expires in September 2036, subject to certain extension and termination options that we hold under the lease agreement.

We also occupy approximately 78,000 square feet of office, laboratory and warehousing space at other locations in Rockville, Maryland and Washington, D.C., under leases that expire at various dates through 2029, some of which are renewable for additional years.

We believe that our facilities are adequate to meet our operating needs for the foreseeable future.

ITEM 3. ***LEGAL PROCEEDINGS***

From time to time, we are party to various lawsuits, claims or other legal proceedings that arise in the normal course of our business. We do not believe that we are currently party to any pending legal actions that could reasonably be expected to have a material adverse effect on our business, financial condition, results of operations or cash flows.

ITEM 4. ***MINE SAFETY DISCLOSURES***

Not applicable.

PART II

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

Market Information

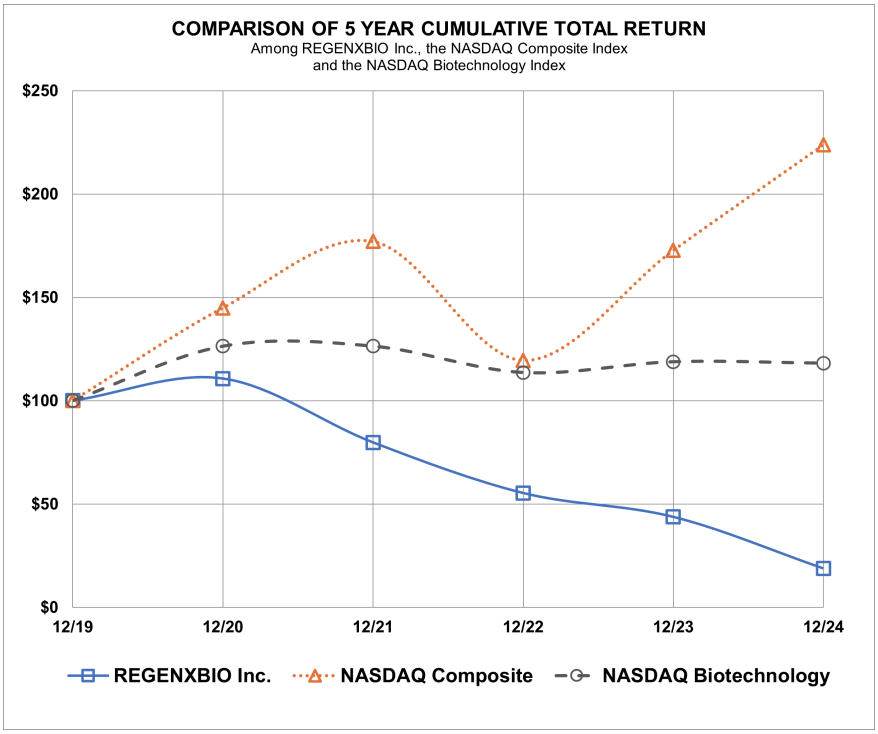
Our common stock is traded on The Nasdaq Global Select Market under the symbol "RGNX."

Stock Performance Graph

The graph set forth below compares the cumulative total stockholder return on our common stock between December 31, 2019 and December 31, 2024, with the cumulative total return of (a) the Nasdaq Composite Index and (b) the Nasdaq Biotechnology Index, over the same period. The figures below assume an investment of $100 in our common stock, the Nasdaq Composite Index and the Nasdaq Biotechnology Index at the closing price on December 31, 2019 and assumes the reinvestment of dividends, if any.

The comparisons shown in the graph below are based upon historical data. We caution that the stock price performance shown in the graph below is not necessarily indicative of, nor is it intended to forecast, the potential future performance of our common stock. Information used in the graph was obtained from the Nasdaq Stock Market LLC, a financial data provider and a source believed to be reliable. The Nasdaq Stock Market LLC is not responsible for any errors or omissions in such information.

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



$100 investment in stock or index	December 31, 2019	December 31, 2020	December 31, 2021	December 31, 2022	December 31, 2023	December 31, 2024
REGENXBIO Inc.	$ 100	$ 111	$ 80	$ 55	$ 44	$ 19
Nasdaq Composite	$ 100	$ 145	$ 177	$ 119	$ 173	$ 224
Nasdaq Biotechnology	$ 100	$ 126	$ 126	$ 114	$ 119	$ 118

Holders

As of March 7, 2025, there were five holders of record of our common stock. The actual number of stockholders is greater than this number of record holders, as stockholders who are beneficial owners of our common stock hold such shares in street name through brokers and other nominees that are record holders of our common stock.

Dividends

We have not declared or paid any cash dividends on our common stock since our inception. We do not plan to pay dividends in the foreseeable future.

ITEM 6. [RESERVED]

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

You should read the following discussion and analysis of our financial condition and results of operations in conjunction with the audited financial statements and the notes thereto included elsewhere in this Annual Report on Form 10-K. In addition, you should read the "Risk Factors" and "Information Regarding Forward-Looking Statements" sections of this Annual Report on Form 10-K for a discussion of important factors that could cause actual results to differ materially from the results described in or implied by the forward-looking statements contained in the following discussion and analysis.

For a full discussion and analysis of financial condition and results of operations for the year ended December 31, 2023, including a year-over-year comparison to the year ended December 31, 2022, please read the "Management's Discussion and Analysis of Financial Condition and Results of Operations" section of our Annual Report on Form 10-K for the year ended December 31, 2023, which we filed with the SEC on February 27, 2024.

Overview

We are a leading clinical-stage biotechnology company seeking to improve lives through the curative potential of gene therapy. Our investigational gene therapies are designed to deliver functional genes to address genetic defects in cells, enabling the production of therapeutic proteins or antibodies that are intended to impact disease. Through a single administration, gene therapy could potentially alter the course of disease significantly and deliver improved patient outcomes with long-lasting effects.

Overview of Product Candidates

We have developed a broad pipeline of gene therapy programs using our proprietary adeno-associated virus (AAV) gene therapy delivery platform (NAV Technology Platform) as a one-time treatment to address an array of diseases. Our lead programs and product candidates are described below:

•**ABBV-RGX-314:** We are developing ABBV-RGX-314 (surabgene lomparvovec) in collaboration with AbbVie as a potential one-time treatment for chronic retinal conditions that cause total or partial vision loss, including wet age-related macular degeneration (wet AMD) and diabetic retinopathy (DR). ABBV-RGX-314 is currently being evaluated in multiple clinical trials, including two pivotal trials (ATMOSPHERE and ASCENT), one Phase II bridging study, one long-term follow-up study and a fellow eye sub-study in patients with wet AMD, all utilizing subretinal delivery. Additionally, two Phase II clinical trials in patients with wet AMD (AAVIATE) and DR (ALTITUDE) are ongoing along with two corresponding long-term follow-up studies, all utilizing in-office suprachoroidal delivery. Within the Phase II study in DR, we are also evaluating ABBV-RGX-314 in diabetic macular edema (DME). ABBV-RGX-314 uses the NAV® AAV8 vector to deliver a gene encoding a therapeutic antibody fragment to inhibit vascular endothelial growth factor (VEGF). We have licensed certain exclusive rights to the SCS Microinjector® from Clearside Biomedical, Inc. (Clearside) to deliver gene therapy treatments to the suprachoroidal space of the eye.

Wet AMD

Subretinal Delivery

Enrollment continues to be on track in the ATMOSPHERE® and ASCENT™ pivotal trials for the treatment of patients with wet AMD using subretinal delivery. These trials are expected to support global regulatory submissions with the U.S. Food and Drug Administration (FDA) and the European Medicines Agency (EMA). Topline data from these trials are expected to be shared in 2026.

Suprachoroidal Delivery

The AAVIATE® trial is a multi-center, open label, randomized, controlled, dose-escalation Phase II trial to evaluate the efficacy, safety and tolerability of suprachoroidal delivery of ABBV-RGX-314 for the treatment of wet AMD. As of July 29, 2024, ABBV-RGX-314 at dose level 3 with short course prophylactic steroid eye drops continues to be well tolerated with no drug-related serious adverse events (SAEs) and no cases of intraocular inflammation, endophthalmitis, vasculitis, retinal artery occlusion, choroidal effusion or hypotony. Mild episcleritis occurred in three patients, all resolved and completed treatment with topical steroids. There were no cases of elevated intraocular pressure. Based on this favorable safety profile, the Phase II AAVIATE trial is initiating enrollment in a new cohort to evaluate ABBV-RGX-314 at dose level 4 (1.5x10e12 GC/eye). Patients in this cohort will also receive short course prophylactic steroid eye drops.

DR and DME

The ALTITUDE® trial is a multi-center, open label, randomized, controlled, dose-escalation Phase II trial to evaluate the efficacy, safety and tolerability of ABBV-RGX-314 using suprachoroidal delivery for the treatment of DR. Based on positive interim results from this trial, we, with partner AbbVie, conducted an End-of-Phase II meeting with the FDA in the fourth quarter of 2024. In January 2025, we and AbbVie announced we will plan a Phase III program for ABBV-RGX-314 in DR. The program is expected to support global regulatory submissions.

The ALTITUDE trial is now enrolling a new cohort of patients with center-involved DME. DME is a vision-threatening complication of DR; an estimated 34 million people globally have DME. Patients will receive a one-time, in-office injection of ABBV-RGX-314 at dose level 4 (1.5x10e12 GC/eye) with short course prophylactic steroid eye drops.

•**RGX-202:** We are developing RGX-202 as an investigational AAV therapeutic for the treatment of Duchenne muscular dystrophy (Duchenne), using the NAV AAV8 vector to deliver a transgene for a novel microdystrophin that includes the functional elements of the C-Terminal domain as well as a muscle-specific promoter to support a targeted therapy for improved resistance to muscle damage associated with Duchenne.

AFFINITY DUCHENNE® is a multicenter, open-label Phase I/II/III trial to evaluate the safety, tolerability and clinical efficacy of a one-time intravenous dose of RGX-202 in patients with Duchenne aged one and older. The initiation of the pivotal study as well as positive safety and efficacy data from the Phase I/II portion of the study, including the first functional data, were announced in November 2024. In March 2025, we announced that the pivotal study was nearly 50% enrolled, and we expect to complete enrollment in the study in 2025, share top line data in the first half of 2026 and submit a Biologics License Application (BLA) under the accelerated approval pathway in mid-2026.

We expect to share additional Phase I/II biomarker data at the 2025 Muscular Dystrophy Association (MDA) Clinical & Scientific Conference, including the first biomarker data from the cohort of patients aged 1-3. The company expects to share additional efficacy and safety data, including additional functional data, in the first half of 2025.

We are also recruiting patients in the AFFINITY BEYOND® trial, an observational screening study. The primary objective is to evaluate the prevalence of AAV8 antibodies in patients with Duchenne up to 12 years of age. Information collected in this study may be used to identify potential participants for the AFFINITY DUCHENNE trial and potential future trials of RGX-202.

•**RGX-121:** We are developing RGX-121 (clemidsogene lanparvovec) in collaboration with Nippon Shinyaku in the United States and certain countries in Asia as an investigational one-time AAV therapeutic for the treatment of Mucopolysaccharidosis Type II (MPS II), also known as Hunter syndrome, using the NAV AAV9 vector to deliver the gene that encodes the iduronate-2-sulfatase enzyme.

In the pivotal phase of the Phase I/II/III CAMPSIITE® trial, RGX-121 achieved its primary endpoint, a reduction in cerebrospinal fluid Heparan sulfate levels of D2S6, a biomarker indicative of brain disease activity, with statistical significance. In September 2024, we announced positive data from the pivotal dose level of RGX-121 demonstrating long-term systemic effect. We plan to use levels of cerebrospinal fluid Heparan sulfate D2S6 as a surrogate endpoint reasonably likely to predict clinical benefit for accelerated approval. A BLA for RGX-121 seeking accelerated approval was submitted to the FDA in March 2025, which we believe is likely to be eligible for priority review. We expect potential approval of RGX-121 in the second half of 2025.

Potential approval of the BLA for RGX-121 could result in receipt of a Rare Pediatric Disease Priority Review Voucher in 2025, assuming the statutory criteria are met. If approved, RGX-121 would be the first approved gene therapy and one-time treatment for MPS II.

•**RGX-111:** We are developing RGX-111 in collaboration with Nippon Shinyaku in the United States and certain countries in Asia as an investigational one-time AAV therapeutic for the treatment of Mucopolysaccharidosis Type I (MPS I), also known as Hurler syndrome, using the NAV AAV9 vector to deliver the IDUA gene.

In November 2023, future development of RGX-111 was halted as a result of a strategic pipeline prioritization and corporate restructuring. Prior to that announcement, RGX-111 was demonstrated to be well tolerated and indicated encouraging biomarker and neurodevelopmental results in a Phase I/II study. Efforts to continue development of RGX-111 are set to be reinitiated following our announcement in January 2025 of a strategic partnership with Nippon Shinyaku.

Strategic Pipeline Prioritization and Restructuring

In November 2023, we implemented a strategic pipeline prioritization and corporate restructuring designed to prioritize the development of ABBV-RGX-314, RGX-202 and RGX-121, and to seek strategic alternatives for our other clinical stage product candidates: (i) RGX-111 for the treatment of MPS I, (ii) RGX-181 for the treatment of late-infantile neuronal ceroid lipofuscinosis type 2 (CLN2) disease, and (iii) RGX-381 for the treatment of the ocular manifestations of CLN2 disease. The restructuring plan included a reduction in workforce and other planned operating expenses, primarily in rare neurodegenerative disease development, early research and other general and administrative areas. We implemented a reduction in workforce of approximately 15%, which was substantially completed in the fourth quarter of 2023. For additional information regarding the corporate restructuring, please refer to Note 14, "Restructuring" to the accompanying audited consolidated financial statements.

Collaboration and License Agreement with AbbVie

In September 2021, we entered into a collaboration and license agreement with AbbVie Global Enterprises Ltd. (AbbVie), a subsidiary of AbbVie Inc., to jointly develop and commercialize ABBV-RGX-314 (the AbbVie Collaboration Agreement). Pursuant to the AbbVie Collaboration Agreement, both we and AbbVie are active participants in the development of ABBV-RGX-314 and development expenses are shared between the parties in accordance with the agreement. The Company will lead the manufacturing of ABBV-RGX-314 for clinical development and U.S. commercial supply, and AbbVie will lead the global commercialization of ABBV-RGX-314. We received an up-front fee of $370.0 million from AbbVie upon the effective date of the AbbVie Collaboration Agreement in November 2021, and we are eligible to receive up to $1.38 billion from AbbVie upon the achievement of specified development and sales-based milestones. Additionally, the parties will share equally in the net profits and net losses associated with the commercialization of ABBV-RGX-314 in the United States, and we are eligible to receive tiered royalties on net sales by AbbVie of ABBV-RGX-314 outside the United States. For additional information regarding the AbbVie Collaboration Agreement, please refer to Note 10, "License and Collaboration Agreements—AbbVie Collaboration and License Agreement" to the accompanying audited consolidated financial statements.

Collaboration and License Agreement with Nippon Shinyaku

In January 2025, we entered into a collaboration and license agreement with Nippon Shinyaku Co., Ltd. (Nippon Shinyaku) for the development and commercialization of RGX-121 and RGX-111 (the Nippon Shinyaku Collaboration Agreement). Pursuant to the Nippon Shinyaku Collaboration Agreement, we are responsible for the development of RGX-121 and RGX-111 in the United States, and Nippon Shinyaku is responsible for development in licensed territories outside the United States. We are responsible for the manufacturing of RGX-121 and RGX-111 for clinical development and commercial supply, and manufacturing expenses will be allocated between the parties in accordance with the terms of the Nippon Shinyaku Collaboration Agreement. Nippon Shinyaku will be responsible, at its sole cost, for the commercialization of RGX-121 and RGX-111 in the licensed territories. Under the terms of the Nippon Shinyaku Collaboration Agreement, we will receive an up-front payment of $110.0 million from Nippon Shinyaku following the effective date of the agreement in March 2025 and we are eligible to receive up to $700.0 million from Nippon Shinyaku upon the achievement of specified development and sales-based milestones. We are also eligible to receive double-digit royalties on net sales of RGX-121 and RGX-111 by Nippon Shinyaku, subject to specified offsets and reductions. We also retain all rights to, and any proceeds related to the sale of, any priority review vouchers that may be issued upon the potential approvals of RGX-121 and RGX-111. For additional information regarding the Nippon Shinyaku Collaboration Agreement, please refer to Note 10, "License and Collaboration Agreements—Nippon Shinyaku Collaboration and License Agreement" to the accompanying audited consolidated financial statements.

Overview of Our NAV Technology Platform

In addition to our internal product development efforts, we also selectively license the NAV Technology Platform and other intellectual property rights to other leading biotechnology and pharmaceutical companies, which we refer to as NAV Technology Licensees. As of December 31, 2024, our NAV Technology Platform was being applied in one commercial product, Zolgensma®, and the preclinical and clinical development of a number of other licensed products. Licensing the NAV Technology Platform allows us to maintain our internal product development focus on our core disease indications and therapeutic areas while still expanding the NAV gene therapy pipeline, developing a greater breadth of treatments for patients, providing additional technological and potential clinical proof-of-concept for our NAV Technology Platform and creating potential additional revenue opportunities.

Financial Overview

Revenues

Our revenues to date consist primarily of license and royalty revenue resulting from the licensing of our NAV Technology Platform and other intellectual property rights. We have not generated any revenues from commercial sales of our own products. If we fail to complete the development of our product candidates in a timely manner or obtain regulatory approval and adequate labeling, our ability to generate future revenues will be materially compromised.

We license our NAV Technology Platform and other intellectual property rights to other biotechnology and pharmaceutical companies, including collaborators for the joint development and commercialization of our product candidates. The terms of the licenses vary, and licenses may be exclusive or non-exclusive and may be sublicensable by the licensee. Licenses may grant intellectual property rights for purposes of internal and preclinical research and development only, or may include the rights, or options to obtain future rights, to commercialize drug therapies for specific diseases using the NAV Technology Platform and other licensed rights. License agreements generally have a term at least equal to the life of the underlying patents, but are terminable at the option of the licensee. Consideration from licensees under our license agreements may include: (i) up-front and annual fees, (ii) milestone payments based on the achievement of certain development and sales-based milestones, (iii) sublicense fees, (iv) royalties on sales of licensed products, (v) fees for services related to the development of licensed products and (vi) other consideration payable upon optional goods and services purchased by licensees.

Future license and royalty revenues are dependent on the successful development and commercialization of licensed products, which is uncertain, and revenues may fluctuate significantly from period to period. Additionally, we may never receive consideration in our license agreements that is contemplated on option fees, development and sales-based milestone payments, royalties on sales of licensed products or sublicense fees, given the contingent nature of these payments. Our revenues are concentrated among a low number of licensees and licenses are terminable at the option of the licensee. The termination of our licenses by licensees may materially impact the amount of revenue we recognize in future periods. Please refer to Note 17, "Segment and Geographical Information" to the accompanying audited consolidated financial statements for a description of segment and geographical information regarding our revenues.

Zolgensma Royalties

Royalty revenue to date consists primarily of royalties on net sales of Zolgensma, which is marketed by Novartis Gene Therapies, Inc. (formerly AveXis, Inc.) (Novartis Gene Therapies), a wholly owned subsidiary of Novartis AG (Novartis), for the treatment of spinal muscular atrophy (SMA). Zolgensma is a licensed product under our license agreement with Novartis Gene Therapies for the development and commercialization of treatments for SMA using the NAV Technology Platform.

Operating Expenses

Our operating expenses consist primarily of cost of revenues, research and development expenses and general and administrative expenses. Personnel costs including salaries, wages, benefits, bonuses and stock-based compensation expense, comprise a significant component of research and development and general and administrative expenses. We allocate indirect expenses associated with our facilities, information technology costs, depreciation and other overhead costs between research and development and general and administrative categories based on employee headcount and the nature of work performed by each employee or using other reasonable allocation methodologies.

Cost of Revenues

Our cost of revenues consists primarily of upstream fees due to our licensors as a result of revenue generated from the licensing of our NAV Technology Platform and other intellectual property rights, including sublicense fees and royalties on net sales of licensed products. Sublicense fees are based on a percentage of license fees received by us from licensees and are recognized in the period that the underlying license revenue is recognized. Royalties are based on a percentage of net sales of licensed products by licensees and are recognized in the period that the underlying sales occur. Future costs of revenues are uncertain due to the nature of our license agreements and significant fluctuations in cost of revenues may occur from period to period.

Research and Development Expense

Our research and development expenses consist primarily of:

•salaries, wages and personnel-related costs, including benefits, travel and stock-based compensation, for our scientific personnel and others performing research and development activities;

•costs related to executing preclinical studies and clinical trials;

•costs related to acquiring, developing and manufacturing materials for preclinical studies and clinical trials;

•fees paid to consultants and other third parties who support our product candidate development;

•other costs in seeking regulatory approval of our product candidates; and

•direct costs and allocated costs related to laboratories and facilities, depreciation expense, information technology and other overhead.

Up-front fees incurred in obtaining technology licenses for research and development activities, as well as associated milestone payments, are charged to research and development expense as incurred if the technology licensed has no alternative future use.

We expect to continue to incur significant research and development expenses for the foreseeable future as we continue the development of our product candidates and engage in early research and development for prospective product candidates and new technologies. The following table summarizes our research and development expenses incurred during the years ended December 31, 2024, 2023 and 2022 (in thousands):

	Years Ended December 31,		
	2024	2023*	2022*
Direct Expenses			
ABBV-RGX-314	$ 36,401	$ 24,453	$ 50,246
RGX-202	14,419	10,803	17,102
RGX-121	12,143	19,128	8,436
Other product candidates	7,103	9,908	6,663
Total direct expenses	70,066	64,292	82,447
Unallocated Expenses			
Platform and early research	27,885	46,045	48,950
Personnel	65,950	73,356	67,466
Facilities	11,410	11,653	10,284
Stock-based compensation	17,985	20,568	21,368
Depreciation and amortization	15,226	16,352	11,938
Total unallocated expenses	138,456	167,974	160,006
Total research and development	$ 208,522	$ 232,266	$ 242,453

* Certain amounts reported in prior years have been reclassified to conform to the current year's presentation.

Direct expenses related to the development of ABBV-RGX-314 for the years ended December 31, 2024, 2023 and 2022 include net cost reimbursement from AbbVie under our eye care collaboration of $78.3 million, $74.2 million and $19.3 million, respectively, which were recorded as a reduction of research and development expenses. Net cost reimbursement from AbbVie includes reimbursement of personnel and overhead costs attributable to the development of ABBV-RGX-314, the underlying costs of which are reported as unallocated expenses in the table above. We typically utilize our employee and infrastructure resources across our development programs. As a result, we generally do not allocate personnel and other internal costs, such as facilities and other overhead costs, to specific product candidates or development programs.

Platform and early research reported in the table above includes direct costs not identifiable with a specific lead product candidate, including costs associated with our research and development platform used across programs, process and analytical development, early research and development for prospective product candidates and new technologies, and other costs in support of research and development activities.

Direct expenses related to the development of product candidates for which we have discontinued internal development are included in other product candidates in the table above. We expect to continue to incur minor development expenses associated with long-term follow up studies for certain discontinued product candidates.

General and Administrative Expense

Our general and administrative expenses consist primarily of salaries, wages and personnel-related costs, including benefits, travel and stock-based compensation, for employees performing functions other than research and development. This includes certain personnel in executive, commercial, corporate development, finance, legal, human resources, information technology, facilities and administrative support functions. Additionally, general and administrative expenses include costs associated with accounting, legal, commercial and other corporate advisory services, obtaining and maintaining patents, insurance, information systems and other general corporate activities, as well as facility-related costs and other corporate overhead costs not otherwise allocated to research and development expense. We expect that our general and administrative expenses will increase as we continue to develop, and potentially commercialize, our product candidates.

Other Income (Expense)

Interest Income from Licensing

In accordance with our revenue recognition policy, interest income from licensing consists of imputed interest recognized from significant financing components identified in our license agreements with NAV Technology Licensees.

Investment Income

Investment income consists of interest income earned and gains and losses realized from our cash and cash equivalents, marketable securities and non-marketable equity securities. Cash equivalents are comprised of money market mutual funds and highly liquid debt securities with original maturities of 90 days or less at acquisition. Marketable securities are comprised of available-for-sale debt securities.

Interest Expense

Interest expense consists primarily of interest imputed on the liability related to the sale of future Zolgensma royalties to entities managed by Healthcare Royalty Management, LLC (collectively, HCR). Interest expense is recognized using the effective interest method, based on our estimate of total royalty payments expected to be received by HCR under the royalty purchase agreement. For further information regarding the royalty purchase agreement with HCR, please refer to Note 7, "Liability Related to Sale of Future Royalties" to the accompanying audited consolidated financial statements.

Critical Accounting Policies and Estimates

This Management's Discussion and Analysis of Financial Condition and Results of Operations is based on our consolidated financial statements, which we have prepared in accordance with accounting principles generally accepted in the United States of America (GAAP). The preparation of financial statements in conformity with GAAP requires us to make estimates and assumptions that affect the reported amounts of assets, liabilities, revenues and expenses and related disclosure of contingent assets and liabilities for the periods presented. We base our estimates on historical experience and on various other factors that we believe are reasonable under the circumstances, the results of which form the basis for making judgments about the carrying value of assets and liabilities, and other reported amounts, that are not readily apparent from other sources. Actual results may differ materially from these estimates under different assumptions or conditions.

Our significant accounting policies are fully described in Note 2, "Summary of Significant Accounting Policies" to our audited consolidated financial statements appearing elsewhere in this Annual Report on Form 10-K. We believe the following accounting policies are critical to the process of making significant judgments and estimates in the preparation of our financial statements and understanding and evaluating our reported financial results.

Revenue Recognition

We recognize revenue in accordance with Accounting Standards Codification (ASC) 606, *Revenue from Contracts with Customers* (ASC 606). ASC 606 requires entities to recognize revenue when control of the promised goods or services is transferred to customers at an amount that reflects the consideration to which the entity expects to be entitled to in exchange for those goods or services. The following five steps are performed to determine the appropriate revenue recognition for arrangements within the scope

of ASC 606: (i) identify the 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 revenue when (or as) the entity satisfies the performance obligations.

We apply the five-step model to contracts that are within the scope of ASC 606 only 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, for contracts 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 respective performance obligations when (or as) the respective performance obligations are satisfied.

We evaluate our contracts with customers for the presence of significant financing components. If a significant financing component is identified in a contract and provides a financing benefit to the customer, the transaction price for the contract is adjusted to account for the financing portion of the arrangement, which is recognized as interest income over the financing term using the effective interest method. In determining the appropriate interest rates for significant financing components, we evaluate the credit profile of the customer and prevailing market interest rates and select an interest rate in which we believe would be charged to the customer in a separate financing arrangement over a similar financing term.

License and Royalty Revenue

We license our NAV Technology Platform and other intellectual property rights to other biotechnology and pharmaceutical companies. The terms of the licenses vary, and licenses may be exclusive or non-exclusive and may be sublicensable by the licensee. Licenses may grant intellectual property rights for purposes of internal and preclinical research and development only, or may include the rights, or options to obtain future rights, to commercialize drug therapies for specific diseases using our NAV Technology Platform and other licensed rights. License agreements generally have a term at least equal to the life of the underlying patents, but are terminable at the option of the licensee. Consideration payable to us under our license agreements may include: (i) up-front and annual fees, (ii) milestone payments based on the achievement of certain development and sales-based milestones, (iii) sublicense fees, (iv) royalties on sales of licensed products, (v) fees for services related to the development of licensed products and (vi) other consideration payable upon optional goods and services purchased by licensees.

Our license agreements are accounted for as contracts with customers within the scope of ASC 606, with the exception of transactions for which the counterparty is determined not to be a customer. At the inception of each license agreement, we determine the contract term for purposes of applying the requirements of ASC 606. Licenses are generally terminable at the option of the licensee with advance notice to us. For each license granted, including licenses granted upon the exercise of license options, we evaluate these termination rights to determine whether a substantive termination penalty would be incurred by the licensee upon termination. If the licensee incurs a substantive termination penalty upon termination, the contract term for revenue recognition purposes is generally equal to the stated term of the license, which is the life of the underlying licensed patents. Alternatively, if the licensee does not incur a substantive termination penalty upon termination, the contract term for revenue recognition purposes may be shorter than the stated term of the license, in which case the termination rights may be accounted for as contract renewal options. The determination of whether a substantive termination penalty is associated with the termination rights requires significant judgment. In making this determination, we consider, among other things, the nature of the intellectual property rights that would be returned to us upon termination, including the exclusivity of the licensed rights and the stage of development of the licensed products, the payment terms, including the amount and timing of non-refundable or guaranteed payments, and the business purpose of the termination rights granted to the licensee. Generally, the most significant judgment in determining whether a substantive termination penalty exists relates to the amount of any up-front or guaranteed non-refundable payments relative to the amount of annual payments that may be avoided by the licensee upon termination of the license. We consider all of the facts and circumstances relevant to each license when making this determination.

Performance obligations under our license agreements may include (i) the delivery of intellectual property licenses, (ii) options granted to licensees to acquire additional licenses, to the extent the options represent material rights to the licensee, and (iii) research and development services to be performed by us related to licensed products. License agreements may provide licensees with contract renewal options or options to acquire additional licenses, goods or other services. Options are evaluated at the inception of the license agreement to determine whether they provide material rights to the licensee. In making this determination, we consider whether the options are priced at an incremental discount to the standalone selling price for the underlying licenses, goods or services, in which case the option is considered to be a material right to the licensee and is accounted for as a separate performance obligation under the current license agreement. At the inception of each license agreement which contains performance obligations for research and development services, we evaluate whether the license is distinct from the research and development services, which requires judgment. In making this determination, we consider, among other things, the stage of development of the licensed products and whether the research and development services will significantly impact further development of the licensed products. If it is

determined that the license is not distinct from the research and development services, the license is combined with the research and development services into a single performance obligation.

We evaluate the transaction price of our license agreements at the inception of each agreement and at each reporting date. The transaction price includes the fixed consideration payable to us during the contract term, as well as any variable consideration to the extent that it is probable that a significant reversal of revenue will not occur in the future. Fixed consideration under the license agreements may include up-front and annual fees payable during the contract term and fees for development services related to licensed products. Variable consideration under the license agreements may include development and sales-based milestone payments, sublicense fees and royalties on sales of licensed products. Consideration contingent upon the exercise of options by a licensee is excluded from the transaction price and not accounted for as part of the license agreement until the option is exercised.

The transaction price for each license agreement is allocated to the underlying performance obligations based on their relative standalone selling prices and recognized as revenue when (or as) the performance obligations are satisfied. Consideration allocated to performance obligations for the delivery of an intellectual property license is recognized as revenue in full upon the delivery of the license to the licensee. Consideration allocated to performance obligations for license options is recognized as revenue in full upon the earlier of the option exercise or expiration. The exercise of a license option by a licensee is accounted for as a new license for revenue recognition purposes. Consideration allocated to performance obligations for research and development services is recognized as revenue as the services are performed by us.

Up-front and annual licenses fees payable to us over the contract term of each license are included in the transaction price, and the portion of this consideration allocated to the performance obligation for the delivery of the intellectual property license is recognized as revenue in full upon the delivery of the license to the licensee. If annual license fees are payable to us in periods beyond 12 months from the delivery of the license, a significant financing component is deemed to exist which provides a financing benefit to the licensee. If a significant financing component is identified, we adjust the transaction price for the license to include only the present value of the annual license fees payable to us over the contract term. The discounted portion of the license fees is recognized as interest income from licensing over the financing period of the license.

Development milestone payments are payable to us upon the achievement of specified development milestones. At the inception of each license agreement that contains development milestone payments, we evaluate whether the milestones are considered probable of achievement and estimate the amount to be included in the transaction price using the most likely amount method. If it is probable that a significant revenue reversal will not occur in the future, milestone payments are included in the transaction price and recognized as revenue upon the delivery of the license. Milestone payments contingent on the achievement of development milestones that are not within our control or the control of the licensee, such as regulatory approvals, are not considered probable of being achieved and are excluded from the transaction price until the milestone is achieved. At each reporting date, we re-evaluate the probability of achievement of each outstanding development milestone and, if necessary, adjust the transaction price for any milestones for which the probability of achievement has changed due to current facts and circumstances. Any such adjustments are recorded on a cumulative catch-up basis and recognized as revenue in the period of the adjustment.

Royalties on sales of licensed products, sales-based milestone payments, including milestones payable upon first commercial sales of licensed products, and sublicense fees based on the receipt of certain fees by licensees from any sublicensees are excluded from the transaction price of each license and recognized as revenue in the period that the related sales or sublicenses occur, provided that the associated license has been delivered to the licensee.

Royalty revenue to date consists primarily of royalties on net sales of Zolgensma, which is a licensed product under our license agreement with Novartis Gene Therapies for the development and commercialization of treatments for SMA. We recognize royalty revenue from net sales of Zolgensma in the period in which the underlying products are sold by Novartis Gene Therapies, which in certain cases may require us to estimate royalty revenue for periods of net sales which have not yet been reported to us. Estimated royalties are reconciled to actual amounts reported in subsequent periods, and any differences are recognized as an adjustment to royalty revenue in the period the royalties are reported.

We receive payments from licensees based on the billing schedules established in each license agreement. Amounts recognized as revenue which have not yet been received from licensees, including unbilled royalties, are recorded as accounts receivable when our rights to the consideration are conditional solely upon the passage of time. Amounts recognized as revenue which have not yet been received from licensees are recorded as contract assets when our rights to the consideration are not unconditional. Contract assets are recorded as other current assets on the consolidated balance sheets. If a licensee elects to terminate a license prior to the end of the license term, the licensed intellectual property is returned to us and any consideration recorded as accounts receivable or contract assets which is not contractually payable by the licensee is charged off as a reduction of license revenue in the period of the termination. Amounts received by us prior to the delivery of underlying performance obligations are deferred and recognized as

revenue upon the satisfaction of the performance obligations. Deferred revenue which is not expected to be recognized within 12 months from the reporting date is recorded as non-current on the consolidated balance sheets.

Collaborative Arrangements

We evaluate our agreements with collaboration partners to determine whether they are within the scope of ASC 808, *Collaborative Arrangements* (ASC 808). Such arrangements are within the scope of ASC 808 if they involve joint operating activities performed by parties that are both active participants in the activities and exposed to significant risks and rewards dependent on the commercial success of such activities. This evaluation is performed throughout the life of the arrangement based on any changes in the roles and responsibilities of the parties under the arrangement. For collaboration arrangements within the scope of ASC 808 that contain multiple elements, we identify the various transactions with the counterparty and determine if any unit of account is more reflective of a transaction with a customer and therefore should be accounted for within the scope of ASC 606. For transactions that are accounted for pursuant to ASC 808, an appropriate method of recognition and presentation is determined and consistently applied. For transactions that are accounted for pursuant to ASC 606, we apply the five-step model as described in our revenue recognition policies.

For additional information regarding our collaborative arrangements, including our collaborations with AbbVie and Nippon Shinyaku, refer to Note 10, "License and Collaboration Agreements" to the accompanying audited consolidated financial statements.

Accrued Research and Development Expenses

We estimate our accrued research and development expenses as of each balance sheet date. This process involves reviewing contracts and purchase orders with service providers, identifying services that have been performed on our behalf and estimating the level of service performed, the expected remaining period of performance and the associated expenses incurred for the service when we have not yet been invoiced or otherwise notified of actual cost. Depending on the timing of payments to the service providers and the estimated expenses incurred, we may record net prepaid or accrued research and development expenses relating to these costs.

Examples of estimated accrued research and development expenses include fees paid to:

•Contract research organizations (CROs) and other vendors in connection with preclinical development and clinical studies;

•Contract manufacturing organizations (CMOs) and other vendors related to process development and manufacturing of materials for use in preclinical development and clinical studies; and

•service providers for professional service fees such as consulting and other research and development related services.

Our understanding of the status and timing of services performed relative to the actual status and timing may vary and may result in us reporting changes in estimates in any particular period. To date, there have been no material differences from our estimates to the amount actually incurred.

Stock-based Compensation

We account for our stock-based compensation awards in accordance with ASC 718, *Compensation—Stock Compensation* (ASC 718). ASC 718 requires all stock-based awards to employees and nonemployees to be recognized as expense based on the grant date fair value of the awards. Our stock-based awards include stock options and restricted stock units granted to employees and nonemployees and shares issued to employees under our employee stock purchase plan.

Our stock-based awards may be subject to either service or performance-based vesting conditions. Compensation expense related to awards with service-based vesting conditions is recognized on a straight-line basis based on the estimated grant date fair value over the requisite service period of the award, which is generally the vesting term. Compensation expense related to awards with performance-based vesting conditions is recognized based on the estimated grant date fair value over the requisite service period using the accelerated attribution method to the extent achievement of the performance condition is probable.

We have elected to not estimate forfeitures of stock-based awards and account for forfeitures as they occur.

We estimate the fair value of our stock option awards using the Black-Scholes option-pricing model, which requires the input of subjective assumptions, including (i) the fair value of the underlying common stock, (ii) the expected stock price volatility, (iii) the expected term of the award, (iv) the risk-free interest rate and (v) expected dividends. The fair value of our common stock, as used as an input to determine the fair value of our stock option awards, is based on the closing price of our common stock on the date of the

grant. We estimate expected stock price volatility based on the historical volatility of our common stock over a period of time commensurate with the expected term of our stock option awards. Due to the lack of sufficient historical data, we estimate the expected term of our employee stock options using the "simplified" method, whereby the expected term equals the arithmetic average of the vesting term and the original contractual term of the option. For stock options granted to nonemployees, we use the contractual term of the award rather than expected term to estimate the fair value of the award. We estimate the risk-free interest rates for periods within the expected term of its options based on the rates of U.S. Treasury securities with maturity dates commensurate with the expected term of the associated awards. We assume a dividend yield of zero for our common stock as we have never paid dividends and do not expect to pay dividends for the foreseeable future.

We estimate the fair value of our restricted stock units based on the closing price of our common stock on the date of the grant.

Interest Expense on Liability Related to Sale of Future Royalties

We recorded a liability for the net proceeds received from the sale of our Zolgensma royalty payments to HCR. The liability is amortized over the estimated life of the arrangement using the effective interest method. The total amount of royalty payments received by HCR under the agreement, less the net proceeds we received from the sale, is recorded as interest expense over the life of the arrangement. We estimate the effective interest rate based on our estimate of total future royalty payments to be received by HCR under the agreement. We reassess these estimates at each reporting date and adjust the effective interest rate and amortization of the liability on a prospective basis as necessary.

Income Taxes

We account for income taxes in accordance with ASC 740, *Income Taxes,* which provides for deferred taxes using an asset and liability approach. We recognize deferred tax assets and liabilities for the expected future tax consequences of events that have been included in the consolidated financial statements or tax returns. Deferred tax assets and liabilities are determined based on the difference between the financial statement and tax bases of assets and liabilities using enacted tax rates in effect for the year in which the differences are expected to reverse. Valuation allowances are provided if based upon the weight of available evidence, it is more likely than not that some or all of the deferred tax assets will not be realized.

As of December 31, 2024, we had federal net operating loss (NOL) carryforwards of $373.6 million, U.S. state NOL carryforwards of $408.9 million and federal and state research and development tax credit carryforwards of $87.2 million (net of unrecognized tax benefits of $0.1 million) which may be available to offset future income tax liabilities. Our federal NOL carryforwards and a portion of our state NOL carryforwards as of December 31, 2024 may be carried forward indefinitely. The remaining portion of our state NOL carryforwards and our federal and state credit carryforwards as of December 31, 2024 expire at various dates between 2029 and 2044.

We have evaluated the positive and negative evidence bearing upon the realizability of our deferred tax assets, including our NOL and credit carryforwards. Based on our history of operating losses, and other relevant facts and circumstances, we concluded that it is more likely than not that the benefit of our deferred tax assets will not be realized. Accordingly, we provided a full valuation allowance for our net deferred tax assets as of December 31, 2024 and 2023.

Results of Operations

Our consolidated results of operations were as follows (in thousands):

	Years Ended December 31,			Change	
	2024	2023	2022	2024 vs. 2023	2023 vs. 2022
Revenues					
License and royalty revenue	$ 83,328	$ 90,242	$ 112,724	$ (6,914)	$ (22,482)
Total revenues	83,328	90,242	112,724	(6,914)	(22,482)
Operating Expenses					
Cost of revenues	33,567	37,213	54,545	(3,646)	(17,332)
Research and development	208,522	232,266	242,453	(23,744)	(10,187)
General and administrative	76,619	88,494	85,281	(11,875)	3,213
Credit losses (recoveries)	(5,000)	—	—	(5,000)	—
Impairment of long-lived assets	2,101	—	—	2,101	—
Other operating expenses (income)	865	397	(6,679)	468	7,076
Total operating expenses	316,674	358,370	375,600	(41,696)	(17,230)
Loss from operations	(233,346)	(268,128)	(262,876)	34,782	(5,252)
Other Income (Expense)					
Interest income from licensing	174	25	342	149	(317)
Investment income	18,729	11,319	5,383	7,410	5,936
Interest expense	(12,659)	(6,862)	(23,254)	(5,797)	16,392
Total other income (expense)	6,244	4,482	(17,529)	1,762	22,011
Loss before income taxes	(227,102)	(263,646)	(280,405)	36,544	16,759
Income Tax Benefit	—	152	84	(152)	68
Net loss	$ (227,102)	$ (263,494)	$ (280,321)	$ 36,392	$ 16,827

Comparison of the Years Ended December 31, 2024 and 2023

License and Royalty Revenue. License and royalty revenue decreased by $6.9 million, from $90.2 million for the year ended December 31, 2023 to $83.3 million for the year ended December 31, 2024. The decrease was primarily attributable to non-recurring development milestone revenue recognized in 2023, and Zolgensma royalty revenues, which decreased from $85.3 million in 2023 to $81.5 million in 2024. As reported by Novartis, sales of Zolgensma in 2024 were $1.21 billion, consistent with 2023, and the product continues to treat mainly incident patients in established markets. The decrease in Zolgensma royalties was primarily attributable to fluctuations in the effective royalty rate under the license agreement with Novartis.

Research and Development Expense. Research and development expenses decreased by $23.7 million, from $232.3 million for the year ended December 31, 2023 to $208.5 million for the year ended December 31, 2024. The decrease was primarily attributable to the following:

- a decrease of $10.5 million in personnel-related costs for research and development personnel, including a $2.6 million decrease in stock-based compensation expense, primarily driven by the reduction in workforce associated with our corporate restructuring in the fourth quarter of 2023;
- a decrease of $10.3 million in manufacturing expenses and other costs of clinical supply for our lead product candidates, largely driven by ABBV-RGX-314 and RGX-121 clinical supply costs;
- a decrease of $6.2 million in preclinical activities and other early-stage research and development; and
- a decrease of $6.1 million in costs for laboratories and facilities used by research and development personnel, including a $1.1 million decrease in depreciation expense allocated to research and development functions, primarily driven by a decrease in laboratory supplies and consumables.

The decrease in research and development expenses was partially offset by an increase of $10.5 million in costs associated with clinical trial activities, largely driven by clinical trial expenses for ABBV-RGX-314 and RGX-202.

General and Administrative Expense. General and administrative expenses decreased by $11.9 million, from $88.5 million for the year ended December 31, 2023 to $76.6 million for the year ended December 31, 2024. The decrease was primarily attributable to professional services and consulting fees, including legal and other corporate advisory services, and other corporate overhead expenses.

Credit Losses (Recoveries). We recognized credit recoveries of $5.0 million during the year ended December 31, 2024 related to the full collection of amounts due under our settlement agreement with Abeona Therapeutics Inc. (Abeona), for which we had previously recorded an allowance for credit losses. For further information regarding the settlement agreement with Abeona and the allowance for credit losses, please refer to Note 10, "License and Collaboration Agreements – Settlement Agreement with Abeona Therapeutics" to the accompanying audited consolidated financial statements. We did not record any credit losses or recoveries during the year ended December 31, 2023.

Investment Income. Investment income increased by $7.4 million, from $11.3 million for the year ended December 31, 2023 to $18.7 million for the year ended December 31, 2024. The increase was largely attributable to the achievement of milestones associated with the acquisition of our non-marketable equity securities of Corlieve Therapeutics SAS (Corlieve) by uniQure N.V. (uniQure) in July 2021. The Company recognized realized gains of $6.6 million and $2.2 million upon the achievement of such milestones during 2024 and 2023, respectively. The remaining increase was primarily attributable to higher yields on investments in cash equivalents and marketable debt securities.

Liquidity and Capital Resources

Sources of Liquidity

As of December 31, 2024, we had cash, cash equivalents and marketable securities of $244.9 million, which were primarily derived from the sale of our common stock and pre-funded warrants and license fees received under the AbbVie Collaboration Agreement, as described below. We expect that our cash, cash equivalents and marketable securities as of December 31, 2024, along with the up-front payment of $110.0 million expected to be received under the Nippon Shinyaku Collaboration Agreement, will enable us to fund our operating expenses and capital expenditure requirements, and are sufficient to meet our financial commitments and obligations, for at least the next 12 months from the date of this report, based on our current business plan. Our recent sources of liquidity include the following events and transactions:

- In March 2024, we completed a public offering of 4,565,260 shares of our common stock at a price of $23.00 per share and 1,521,740 pre-funded warrants to purchase shares of our common stock at a price of $22.9999 per pre-funded warrant, which equaled the public offering price per share of the common stock less the $0.0001 exercise price of each pre-funded warrant. The aggregate net proceeds received from the offering were $131.1 million, net of underwriting discounts and commissions and offering expenses.
- In September 2021, we entered into the AbbVie Collaboration Agreement for the development and commercialization of ABBV-RGX-314. Pursuant to the AbbVie Collaboration Agreement, we received an up-front fee of $370.0 million from AbbVie upon the effective date of the agreement in November 2021, and we are eligible to receive up to $1.38 billion from AbbVie upon the achievement of specified development and sales-based milestones. Additionally, the parties will share equally in the net profits and net losses associated with the commercialization of ABBV-RGX-314 in the United States, and we are eligible to receive tiered royalties on net sales by AbbVie of ABBV-RGX-314 outside the United States.
- In January 2025, we entered into the Nippon Shinyaku Collaboration Agreement for the development and commercialization of RGX-121 and RGX-111 in the United States and certain countries in Asia. Pursuant the Nippon Shinyaku Collaboration Agreement, we will receive an up-front payment of $110.0 million following the effective date of the agreement in March 2025 and are eligible to receive up to $700.0 million upon the achievement of specified development and sales-based milestones. We are also eligible to receive double-digit royalties on net sales of RGX-121 and RGX-111 by Nippon Shinyaku, subject to specified offsets and reductions.

We intend to devote the majority of our current capital to preclinical research, clinical development, seeking regulatory approval of our product candidates and, if approved, commercialization of our product candidates, as well as additional capital expenditures needed to support these activities. Because of the numerous risks and uncertainties associated with the development and commercialization of gene therapy product candidates, we are unable to estimate the total amount of operating expenditures and capital outlays necessary to complete the development of our product candidates. Additionally, our estimates are based on assumptions that may prove to be wrong, and we may use our available capital resources sooner than we currently expect which could accelerate our liquidity needs.

At-the-Market Offering Programs

In September 2023, we entered into an ATM Equity Offering℠ Sales Agreement with BofA Securities, Inc. (BofA) pursuant to which we may offer and sell shares of our common stock having an aggregate offering price of up to $150.0 million from time to time through BofA, acting as our sales agent (the BofA ATM Program). We terminated the BofA ATM Program effective in November 2024. No shares of common stock were sold under the BofA ATM Program prior to its termination.

In December 2024, we entered into a Sales Agreement with Leerink Partners LLC (Leerink) pursuant to which we may offer and sell shares of our common stock having an aggregate offering price of up to $150.0 million from time to time through Leerink, acting as our sales agent (the Leerink ATM Program). As of December 31, 2024, no shares of common stock had been sold under the Leerink ATM Program. We intend to use proceeds obtained from the sale of shares under the Leerink ATM Program, if any, for general corporate purposes.

Private Placement

In July 2023, we sold 257,466 shares of our common stock in a private placement transaction for which we received aggregate net proceeds of $4.9 million, net of offering expenses.

Cash Flows

Our consolidated cash flows were as follows (in thousands):

	Years Ended December 31,		
	2024	2023	2022
Net cash used in operating activities	$ (173,125)	$ (218,407)	$ (207,488)
Net cash provided by (used in) investing activities	103,446	190,943	(11,929)
Net cash provided by (used in) financing activities	92,683	(34,966)	(28,840)
Net increase (decrease) in cash and cash equivalents and restricted cash	$ 23,004	$ (62,430)	$ (248,257)

Cash Flows from Operating Activities

Our net cash used in operating activities for the year ended December 31, 2024 decreased by $45.3 million from the year ended December 31, 2023, largely as a result of lower operating expenses and increased cost reimbursement received from AbbVie under our ABBV-RGX-314 collaboration in 2024. We expect to continue to incur regular net cash outflows from operations for the foreseeable future as we continue the development and advancement of our product candidates and other research programs.

For the year ended December 31, 2024, our net cash used in operating activities of $173.1 million consisted of a net loss of $227.1 million, offset by adjustments for non-cash items of $48.4 million and favorable changes in operating assets and liabilities of $5.5 million. Adjustments for non-cash items primarily consisted of stock-based compensation expense of $38.5 million and depreciation and amortization expense of $16.2 million, partially offset by realized gains on investments, credit recoveries and the accretion of discounts on marketable debt securities during the period. The changes in operating assets and liabilities include a decrease in total accounts receivable of $9.7 million, which was driven largely by a decrease in Zolgensma royalties receivable and the full collection of amounts due under our settlement agreement with Abeona, for which we recorded a $5.0 million credit recovery during the period. The changes in operating assets and liabilities also include a total decrease in prepaid expenses and other current assets of $12.0 million, which was driven primarily by decreases in prepaid fees to CROs and net cost reimbursement due from AbbVie under our ABBV-RGX-314 collaboration. The favorable changes in operating assets and liabilities were partially offset by a decrease in accrued expenses and other current liabilities of $12.1 million, which was driven primarily by decreases in accruals for external research and development services and sublicense and royalties due to licensors. Other changes in operating working capital occurred in the normal course of business.

For the year ended December 31, 2023, our net cash used in operating activities of $218.4 million consisted of a net loss of $263.5 million and unfavorable changes in operating assets and liabilities of $10.9 million, offset by adjustments for non-cash items of $56.0 million. The changes in operating assets and liabilities include an increase in other current assets of $10.5 million, which was driven primarily by an increase in net cost reimbursement due from AbbVie under our ABBV-RGX-314 collaboration. Other changes in operating working capital occurred in the normal course of business. Adjustments for non-cash items primarily consisted of stock-based compensation expense of $40.3 million and depreciation and amortization expense of $17.3 million.

Cash Flows from Investing Activities

For the year ended December 31, 2024, our net cash provided by investing activities consisted of $290.2 million in maturities of marketable debt securities and $5.8 million in proceeds received from uniQure upon the achievement of milestones associated with their acquisition of Corlieve, offset by $190.1 million used to purchase marketable debt securities and $2.4 million used to purchase property and equipment.

For the year ended December 31, 2023, our net cash provided by investing activities consisted of $285.5 million in maturities of marketable debt securities and $2.0 million in proceeds received from uniQure upon the achievement of milestones associated with their acquisition of Corlieve, offset by $86.6 million used to purchase marketable debt securities and $10.0 million used to purchase property and equipment.

Cash Flows from Financing Activities

For the year ended December 31, 2024, our net cash provided by financing activities primarily consisted of $131.1 million in net proceeds received from the public offering of common stock and pre-funded warrants completed in March 2024, net of underwriting discounts and commissions and other offering expenses paid during the period, and $2.7 million in proceeds received from the exercise of stock options and issuance of common stock under our employee stock purchase plan. Our net cash provided by financing activities was partially offset by $39.9 million of Zolgensma royalties paid, net of imputed interest, under our royalty purchase agreement with HCR.

For the year ended December 31, 2023, our net cash used in financing activities primarily consisted of $42.3 million of Zolgensma royalties paid, net of imputed interest, under our royalty purchase agreement with HCR. Our net cash used in financing activities was partially offset by $4.9 million in net proceeds received from a private placement of our common stock in July 2023 and $3.3 million in proceeds received from the exercise of stock options and issuance of common stock under our employee stock purchase plan.

Additional Capital Requirements

Our material capital requirements from known contractual and other obligations primarily relate to vendor service contracts and purchase commitments, in-license agreements, operating lease agreements and our Zolgensma royalty purchase agreement with HCR.

In the normal course of business, we enter into services agreements with CROs, CMOs and other third parties. Generally, these agreements provide for termination upon notice, with specified amounts due upon termination based on the timing of termination and the terms of the agreement. The amounts and timing of payments under these agreements are uncertain and contingent upon the initiation and completion of the services to be provided.

Obligations to licensors under our in-license agreements may include sublicense fees, milestones fees, royalties and reimbursement of patent maintenance costs. Sublicense fees are payable to licensors when we sublicense underlying intellectual property to third parties; the fees are based on a percentage of the license fees we receive from sublicensees. Milestone fees are payable to licensors upon our future achievement of certain development, regulatory and commercial milestones. Royalties are payable to licensors based on a percentage of net sales of licensed products. Patent maintenance costs are payable to licensors as reimbursement for the cost of maintaining licensed patents. Due to the contingent nature of the payments, the amounts and timing of payments to licensors under our in-license agreements are uncertain and may fluctuate significantly from period to period.

In March 2022, we entered into a letter agreement (the Penn Letter Agreement) with The Trustees of the University of Pennsylvania (Penn) to buy out our obligation to pay sublicense fees under our license agreement with Penn (the Penn License). Pursuant to the letter agreement, we are obligated to pay Penn a total of $12.0 million to satisfy any other past or future obligations to pay sublicense fees under the Penn License, which is payable in four equal annual installments of $3.0 million beginning in March 2023. We are no longer obligated to pay sublicense fees to Penn under the license agreement, but remain obligated to pay Penn royalties on net sales of licensed products, milestone fees and reimbursement of certain patent maintenance costs in accordance with the Penn License. As of December 31, 2024, we had $6.0 million remaining payable to Penn under the Penn Letter Agreement, in addition to other amounts payable Penn under the Penn License.

We have entered into a number of long-term operating leases for office, laboratory and manufacturing space in Rockville, Maryland, Washington, D.C. and New York, New York, as well as a number of laboratory and other equipment leases. As of December 31, 2024, we had recorded total lease liabilities of $82.0 million under our operating leases. Please refer to Note 6, "Leases" to the accompanying consolidated financial statements for further information regarding our lease commitments.

Under the terms of our royalty purchase agreement with HCR, our future Zolgensma royalties, less amounts payable by us to certain licensors, will be payable to HCR up to a specified capped amount. As of December 31, 2024, the total amount of future Zolgensma royalties to be paid to HCR under the agreement was $95.6 million. We have no obligation to repay any amounts to HCR if total future Zolgensma royalty payments are not sufficient to repay these amounts.

Future Funding Requirements

We have incurred cumulative losses since our inception and had an accumulated deficit of $932.1 million as of December 31, 2024. Our transition to recurring profitability is dependent upon achieving a level of revenues adequate to support our cost structure, which depends heavily on the successful development, approval and commercialization of our product candidates. We do not expect to achieve such revenues, and expect to continue to incur losses, for at least the next several years. We expect to continue to incur significant research and development and general and administrative expenses for the foreseeable future as we continue the development of, and seek regulatory approval for, our product candidates. Subject to obtaining regulatory approval for our product candidates, we expect to incur significant commercialization expenses for product sales, marketing, manufacturing and distribution. Additionally, we expect to continue to incur capital expenditures associated with building out additional laboratory and manufacturing capacity to further support the development of our product candidates and potential commercialization efforts. As a result, we will need significant additional capital to fund our operations, which we may obtain through one or more equity offerings, debt financings or other third-party funding, including potential strategic alliances and licensing or collaboration arrangements.

Our future capital requirements will depend on many factors, including:

•the timing of enrollment, commencement and completion of our clinical trials;

•the results of our clinical trials;

•the results of our preclinical studies for our product candidates and any subsequent clinical trials;

•the scope, progress, results and costs of drug discovery, laboratory testing, preclinical development and clinical trials for our product candidates;

•the costs associated with building out additional laboratory and manufacturing capacity;

•the costs, timing and outcome of regulatory review of our product candidates;

•the impact of any government-imposed tariffs on cost of goods and services, particularly related to partnered product candidates;

•the costs of future product sales, medical affairs, marketing, manufacturing and distribution activities for any of our product candidates for which we receive marketing approval;

•revenue, if any, received from commercial sales of our products, should any of our product candidates receive marketing approval;

•revenue received from commercial sales of Zolgensma and the timing and amount of Zolgensma royalties paid to HCR under our royalty purchase agreement;

•revenue received from other commercial sales of our licensees' and collaborators' products, should any of their product candidates receive marketing approval, and other revenue received under our licensing agreements and collaborations;

•the costs of preparing, filing and prosecuting patent applications, maintaining and enforcing our intellectual property rights and defending any intellectual property-related claims;

•our current licensing agreements or collaborations remaining in effect, including the AbbVie Collaboration Agreement relating to ABBV-RGX-314 and the Nippon Shinyaku Collaboration Agreement relating to RGX-121 and RGX-111, and our ability to timely achieve any milestones set forth in such agreements or collaborations;

•our ability to establish and maintain additional licensing agreements or collaborations on favorable terms, if at all; and

•the extent to which we acquire or in-license other product candidates and technologies.

The issuance of additional securities, whether equity or debt, by us, including through our at-the-market program, or the possibility of such issuance, may cause the market price of our common stock to decline. Adequate additional financing may not be available to us on acceptable terms, or at all. We also could be required to seek funds through arrangements with partners or otherwise that may require us to relinquish rights to our intellectual property, our product candidates or otherwise agree to terms unfavorable to us.

Off-Balance Sheet Arrangements

We did not have any off-balance sheet arrangements during the periods presented, and we do not currently have, any off-balance sheet arrangements, as defined in the rules and regulations of the SEC.

ITEM 7A. *QUANTITATIVE AND QUALITATIVE DISCLOSURES ABOUT MARKET RISK*

Interest Rate Risk

We are exposed to market risk related to changes in interest rates. Our primary exposure to interest rate risk results from the cash equivalents and marketable securities in our investment portfolio. Our primary objectives in managing our investment portfolio are to preserve principal, maintain proper liquidity to meet operating needs and maximize yields. At any time, significant changes in interest rates can affect the fair value of the investment portfolio and its interest earnings. Currently, we do not hedge these interest rate exposures. As of December 31, 2024 and 2023, we had cash, cash equivalents and marketable securities of $244.9 million and $314.1 million, respectively. Our cash equivalents and marketable securities as of December 31, 2024 consisted of money market mutual funds, U.S. government and agency securities, certificates of deposit and corporate bonds. If market interest rates were to increase immediately and uniformly by 100 basis points, or one percentage point, from levels at December 31, 2024, we estimate that the increase would have resulted in a hypothetical decline of $0.8 million in the net fair value of our interest-sensitive securities as of December 31, 2024. A similar increase in market interest rates as of December 31, 2023 would have resulted in an estimated hypothetical decline of $1.5 million in the net fair value of our interest-sensitive securities as of December 31, 2023.

Foreign Currency Exchange Rate Risk

We are exposed to foreign currency exchange rate risk as a result of entering into transactions denominated in currencies other than U.S. dollars, primarily including euros and British pounds. All foreign currency transactions settle on the applicable spot exchange basis at the time such payments are made. Accordingly, an adverse movement in foreign exchange rates between the U.S. dollar and the aforementioned currencies could impact our results of operations and cash flows. Currently, we do not hedge these foreign currency exchange rate exposures. The effect of a hypothetical 10% change in foreign currency exchange rates applicable to our business would not materially harm our business, financial condition or results of operations.

ITEM 8. *FINANCIAL STATEMENTS AND SUPPLEMENTARY DATA*

The financial statements and related financial statement schedules required to be filed are listed in the Index to Consolidated Financial Statements and are incorporated herein.

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

None.

ITEM 9A. *CONTROLS AND PROCEDURES*

Conclusion Regarding the Effectiveness of Disclosure Controls and Procedures

Our management, with the participation of our Chief Executive Officer and our Chief Financial Officer, evaluated the effectiveness of our disclosure controls and procedures as of December 31, 2024. The term "disclosure controls and procedures," as defined in Rules 13a-15(e) and 15d-15(e) under the Securities Exchange Act of 1934, as amended (the Exchange Act), means controls and other procedures of a company that are designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is recorded, processed, summarized and reported within the time periods specified in the SEC's rules and forms. Disclosure controls and procedures include, without limitation, controls and procedures designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is accumulated and communicated to the company's management, including its principal executive and principal financial officers, as appropriate to allow timely decisions regarding required disclosure.

Based on this evaluation, our Chief Executive Officer and Chief Financial Officer concluded that, as of December 31, 2024, our disclosure controls and procedures were effective at a reasonable assurance level.

Management's Report on Internal Control over Financial Reporting

Our management is responsible for establishing and maintaining adequate internal control over financial reporting. Internal control over financial reporting is defined in Rules 13a-15(f) and 15d-15(f) promulgated under the Exchange Act as a process designed by, or under the supervision of, our principal executive and principal financial officers and effected by our Board of Directors, management and other personnel, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles and includes those policies and procedures that:

- pertain to the maintenance of records that in reasonable detail accurately and fairly reflect the transactions and dispositions of our assets;
- provide reasonable assurance that transactions are recorded as necessary to permit preparation of financial statements in accordance with generally accepted accounting principles, and that our receipts and expenditures are being made only in accordance with authorizations of our management and directors; and
- provide reasonable assurance regarding prevention or timely detection of unauthorized acquisition, use or disposition of our assets that could have a material effect on the financial statements.

Under the supervision and with the participation of management, including our principal executive and financial officers, we assessed our internal control over financial reporting as of December 31, 2024, based on criteria for effective internal control over financial reporting established in *Internal Control — Integrated Framework* (2013), issued by the Committee of Sponsoring Organizations of the Treadway Commission (COSO). In our management's opinion, we have maintained effective internal control over financial reporting as of December 31, 2024, based on criteria established in the COSO 2013 framework.

The effectiveness of our internal control over financial reporting as of December 31, 2024 has been audited by PricewaterhouseCoopers LLP, our independent registered public accounting firm, as stated in their report which accompanies our audited consolidated financial statements appearing elsewhere in this Annual Report on Form 10-K.

Changes in Internal Control over Financial Reporting

There were no changes in our internal control over financial reporting (as defined in Rules 13a-15(f) and 15d-15(f) under the Exchange Act) that occurred during the quarter ended December 31, 2024 that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

Limitations on the Effectiveness of Controls

Control systems, no matter how well conceived and operated, are designed to provide a reasonable, but not an absolute, level of assurance that the objectives of the control system are met. Further, the design of a control system must reflect the fact that there are resource constraints, and the benefits of controls must be considered relative to their costs. Because of the inherent limitations in all control systems, no evaluation of controls can provide absolute assurance that all control issues and instances of fraud, if any, have been detected. Because of the inherent limitations in any control system, misstatements due to error or fraud may occur and not be detected.

ITEM 9B. ***OTHER INFORMATION***

Rule 10b5-1 Trading Plans

The adoption or termination of contracts, instructions or written plans for the purchase or sale of our securities by our Section 16 officers and directors for the three months ended December 31, 2024, each of which is intended to satisfy the affirmative defense of Rule 10b5-1(c) (Rule 10b5-1 Plan), were as follows:

Name	Title	Action	Date Adopted	Expiration Date	Rule 10b5-1 Trading Plan Provides for Purchase/Sale	Aggregate # of Securities to be Purchased/Sold (b)
David C. Stump	Director	Adoption	11/21/2024	6/1/2026	Sale	37,500
Stephen Pakola, M.D.	Executive Vice President, Chief Medical Officer	Adoption	12/20/2024	12/31/2025	Sale	260,368
Entities affiliated with Allan M. Fox (a)	Director	Adoption	12/21/2024	3/31/2027	Sale	1,441,231
Patrick J. Christmas	Executive Vice President, Chief Legal Officer	Adoption	12/26/2024	1/4/2027	Sale	235,400

(a)Entities affiliated with Allan M. Fox, a director of the Company, include AMF Family Gift Trust LLC, AMF Gift Trust LLC, HBF Gift Trust LLC and Quaker Gray LLC (collectively, the Fox LLCs). Mr. Fox maintains voting and investment control over the shares held by the Fox LLCs in his capacity as manager of the Fox LLCs.
(b)The aggregate number of shares in this column includes shares that may be forfeited or withheld to satisfy exercise price and tax withholding obligations at the time of vesting.

Other than as described above, during the three months ended December 31, 2024, none of our directors or Section 16 reporting officers adopted or terminated any Rule 10b5-1 trading arrangement or non-Rule 10b5-1 trading arrangement (as such terms are defined in Item 408 of the SEC's Regulation S-K).

ITEM 9C. ***DISCLOSURE REGARDING FOREIGN JURISDICTIONS THAT PREVENT INSPECTIONS***

Not applicable.

PART III

ITEM 10. *DIRECTORS, EXECUTIVE OFFICERS AND CORPORATE GOVERNANCE*

The information required by this item will be included in our proxy statement for the 2025 annual meeting of stockholders to be filed with the SEC within 120 days of the fiscal year ended December 31, 2024 (2025 Proxy Statement) under the headings "Election of Directors," "Information about our Executive Officers" and "Corporate Governance" and is incorporated herein by reference.

We maintain a code of business conduct and ethics that qualifies as a "code of ethics" under Item 406 of the SEC's Regulation S-K and applies to each of our directors, officers and employees, including our principal executive officer, principal financial officer, principal accounting officer and controller, or persons performing similar functions. The code of business conduct and ethics is available in the corporate governance section of our corporate website at www.regenxbio.com. Any amendment or waiver of the "code of ethics" provisions of the code of business conduct and ethics for an executive officer or director may be granted only by our Board of Directors or a committee thereof and must be timely disclosed as required by applicable law. We intend to satisfy the disclosure requirements regarding any such amendment or waiver applicable to any principal executive officer, principal financial officer, principal accounting officer or controller, or persons performing similar functions, in a current report filed with the SEC on Form 8-K or on our corporate website at www.regenxbio.com.

ITEM 11. *EXECUTIVE COMPENSATION*

The information required by this item will be included in our 2025 Proxy Statement under the headings "Corporate Governance," "Director Compensation" and "Executive Compensation" and is incorporated herein by reference.

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

The information required by this item will be included in our 2025 Proxy Statement under the headings "Executive Compensation" and "Security Ownership of Certain Beneficial Owners and Management" and is incorporated herein by reference.

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

The information required by this item will be included in our 2025 Proxy Statement under the headings "Certain Relationships and Related Party Transactions" and "Corporate Governance" and is incorporated herein by reference.

ITEM 14. *PRINCIPAL ACCOUNTANT FEES AND SERVICES*

The information required by this item will be included in our 2025 Proxy Statement under the heading "Ratification of Appointment of Independent Registered Public Accounting Firm" and is incorporated herein by reference.

PART IV

ITEM 15. *EXHIBITS, FINANCIAL STATEMENT SCHEDULES*

(a)The following documents are filed as part of, or incorporated by reference into, this Annual Report on Form 10-K:

1.*Financial Statements.* See Index to Consolidated Financial Statements under Item 8 of this Annual Report on Form 10-K.

2.*Financial Statement Schedules.* All schedules have been omitted because the information required to be presented in them is not applicable or is shown in the financial statements or related notes.

3.*Exhibits.* We have filed, or incorporated into this Annual Report on Form 10-K by reference, the exhibits listed on the accompanying Exhibit Index immediately following the financial statements in this Annual Report on Form 10-K.

(b)*Exhibits.* See Item 15(a)(3) above.

(c)*Financial Statement Schedules.* See Item 15(a)(2) above.

ITEM 16. *FORM 10-K SUMMARY*

Not applicable.

REGENXBIO INC.
INDEX TO CONSOLIDATED FINANCIAL STATEMENTS

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Report of Independent Registered Public Accounting Firm

To the Board of Directors and Stockholders of REGENXBIO Inc.

Opinions on the Financial Statements and Internal Control over Financial Reporting

We have audited the accompanying consolidated balance sheets of REGENXBIO Inc. and its subsidiaries (the "Company") as of December 31, 2024 and 2023, and the related consolidated statements of operations and comprehensive loss, of stockholders' equity and of cash flows for each of the three years in the period ended December 31, 2024, including the related notes (collectively referred to as the "consolidated financial statements"). We also have audited the Company's internal control over financial reporting as of December 31, 2024, based on criteria established in *Internal Control - Integrated Framework* (2013) issued by the Committee of Sponsoring Organizations of the Treadway Commission (COSO).

In our opinion, the consolidated financial statements referred to above present fairly, in all material respects, the financial position of the Company as of December 31, 2024 and 2023, and the results of its operations and its cash flows for each of the three years in the period ended December 31, 2024 in conformity with accounting principles generally accepted in the United States of America. Also in our opinion, the Company maintained, in all material respects, effective internal control over financial reporting as of December 31, 2024, based on criteria established in *Internal Control - Integrated Framework* (2013) issued by the COSO.

Basis for Opinions

The Company's management is responsible for these consolidated financial statements, for maintaining effective internal control over financial reporting, and for its assessment of the effectiveness of internal control over financial reporting, included in Management's Report on Internal Control over Financial Reporting appearing under Item 9A. Our responsibility is to express opinions on the Company's consolidated financial statements and on the Company's internal control over financial reporting 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 audits to obtain reasonable assurance about whether the consolidated financial statements are free of material misstatement, whether due to error or fraud, and whether effective internal control over financial reporting was maintained in all material respects.

Our audits of the consolidated financial statements included performing procedures to assess the risks of material misstatement of the consolidated financial statements, whether due to error or fraud, and performing procedures that respond to those risks. Such procedures included examining, on a test basis, evidence regarding the amounts and disclosures in the consolidated financial statements. Our audits also included evaluating the accounting principles used and significant estimates made by management, as well as evaluating the overall presentation of the consolidated financial statements. Our audit of internal control over financial reporting included obtaining an understanding of internal control over financial reporting, assessing the risk that a material weakness exists, and testing and evaluating the design and operating effectiveness of internal control based on the assessed risk. Our audits also included performing such other procedures as we considered necessary in the circumstances. We believe that our audits provide a reasonable basis for our opinions.

Definition and Limitations of Internal Control over Financial Reporting

A company's internal control over financial reporting is a process designed to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles. A company's internal control over financial reporting includes those policies and procedures that (i) pertain to the maintenance of records that, in reasonable detail, accurately and fairly reflect the transactions and dispositions of the assets of the company; (ii) provide reasonable assurance that transactions are recorded as necessary to permit preparation of financial statements in accordance with generally accepted accounting principles, and that receipts and expenditures of the company are being made only in accordance with authorizations of management and directors of the company; and (iii) provide reasonable assurance regarding prevention or timely detection of unauthorized acquisition, use, or disposition of the company's assets that could have a material effect on the financial statements.

Because of its inherent limitations, internal control over financial reporting may not prevent or detect misstatements. Also, projections of any evaluation of effectiveness to future periods are subject to the risk that controls may become inadequate because of changes in conditions, or that the degree of compliance with the policies or procedures may deteriorate.

Critical Audit Matters

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

Revenue Recognition – Royalty Revenue

As described in Notes 2 and 10 to the consolidated financial statements, the Company recognizes royalty revenue on sales of licensed products. The Company's consolidated royalty revenue was $81.5 million for the year ended December 31, 2024.

The principal consideration for our determination that performing procedures relating to royalty revenue recognition is a critical audit matter is a high degree of auditor effort in performing procedures related to the Company's revenue recognition.

Addressing the matter involved performing procedures and evaluating audit evidence in connection with forming our overall opinion on the consolidated financial statements. These procedures included testing the effectiveness of controls over the revenue recognition process. These procedures also included, among others, (i) obtaining and inspecting third party royalty reports and the related cash settlement, where applicable, (ii) testing the completeness and accuracy of data provided by management, and (iii) evaluating the financial statement presentation and related disclosures.

/s/ PricewaterhouseCoopers LLP

Washington, District of Columbia
March 13, 2025

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

REGENXBIO INC.
CONSOLIDATED BALANCE SHEETS
(in thousands, except per share data)

	As of December 31,	
	2024	2023
Assets		
Current assets		
Cash and cash equivalents	$ 57,526	$ 34,522
Marketable securities	177,161	240,736
Accounts receivable (net of allowance of $4,587 as of December 31, 2023)	20,473	24,790
Prepaid expenses	9,067	14,520
Other current assets	13,774	20,403
Total current assets	278,001	334,971
Marketable securities	10,179	38,871
Accounts receivable	474	701
Property and equipment, net	117,589	132,103
Operating lease right-of-use assets	53,716	60,487
Restricted cash	2,030	2,030
Other assets	4,000	4,807
Total assets	$ 465,989	$ 573,970
Liabilities and Stockholders' Equity		
Current liabilities		
Accounts payable	$ 22,798	$ 22,786
Accrued expenses and other current liabilities	38,070	49,703
Deferred revenue	115	148
Operating lease liabilities	7,902	7,068
Liability related to sale of future royalties	34,309	50,567
Total current liabilities	103,194	130,272
Operating lease liabilities	74,131	82,222
Liability related to sale of future royalties	25,378	43,485
Other liabilities	3,635	6,249
Total liabilities	206,338	262,228
Commitments and contingencies (Note 8)		
Stockholders' equity		
Preferred stock; $0.0001 par value; 10,000 shares authorized, no shares issued and outstanding at December 31, 2024 and 2023	—	—
Common stock; $0.0001 par value; 100,000 shares authorized at December 31, 2024 and 2023; 49,549 and 44,046 shares issued and outstanding at December 31, 2024 and 2023, respectively	5	4
Additional paid-in capital	1,192,536	1,021,214
Accumulated other comprehensive loss	(741)	(4,429)
Accumulated deficit	(932,149)	(705,047)
Total stockholders' equity	259,651	311,742
Total liabilities and stockholders' equity	$ 465,989	$ 573,970

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

REGENXBIO INC.
CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS
(in thousands, except per share data)

	Years Ended December 31,		
	2024	2023	2022
Revenues			
License and royalty revenue	$ 83,328	$ 90,242	$ 112,724
Total revenues	83,328	90,242	112,724
Operating Expenses			
Cost of revenues	33,567	37,213	54,545
Research and development	208,522	232,266	242,453
General and administrative	76,619	88,494	85,281
Credit losses (recoveries)	(5,000)	—	—
Impairment of long-lived assets	2,101	—	—
Other operating expenses (income)	865	397	(6,679)
Total operating expenses	316,674	358,370	375,600
Loss from operations	(233,346)	(268,128)	(262,876)
Other Income (Expense)			
Interest income from licensing	174	25	342
Investment income	18,729	11,319	5,383
Interest expense	(12,659)	(6,862)	(23,254)
Total other income (expense)	6,244	4,482	(17,529)
Loss before income taxes	(227,102)	(263,646)	(280,405)
Income Tax Benefit	—	152	84
Net loss	$ (227,102)	$ (263,494)	$ (280,321)
Other Comprehensive Income (Loss)			
Unrealized gain (loss) on available-for-sale securities, net	3,688	10,972	(12,832)
Total other comprehensive income (loss)	3,688	10,972	(12,832)
Comprehensive loss	$ (223,414)	$ (252,522)	$ (293,153)
Net loss per share, basic and diluted	$ (4.59)	$ (6.02)	$ (6.50)
Weighted-average common shares outstanding, basic and diluted	49,509	43,734	43,152

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

REGENXBIO INC.
CONSOLIDATED STATEMENTS OF STOCKHOLDERS' EQUITY
(in thousands)

	Common Stock Shares	Common Stock Amount	Additional Paid-in Capital	Accumulated Other Comprehensive Loss	Accumulated Deficit	Total Stockholders' Equity
Balances at December 31, 2021	42,831	$ 4	$ 928,095	$ (2,569)	$ (161,232)	$ 764,298
Vesting of restricted stock units, net of tax	60	—	(284)	—	—	(284)
Exercise of stock options, net of tax	332	—	2,804	—	—	2,804
Issuance of common stock under employee stock purchase plan	76	—	1,742	—	—	1,742
Stock-based compensation expense	—	—	40,788	—	—	40,788
Unrealized loss on available-for-sale securities, net	—	—	—	(12,832)	—	(12,832)
Net loss	—	—	—	—	(280,321)	(280,321)
Balances at December 31, 2022	43,299	4	973,145	(15,401)	(441,553)	516,195
Vesting of restricted stock units, net of tax	164	—	(419)	—	—	(419)
Exercise of stock options, net of tax	223	—	1,521	—	—	1,521
Issuance of common stock under employee stock purchase plan	103	—	1,826	—	—	1,826
Issuance of common stock upon private placement, net of transaction costs of $126	257	—	4,874	—	—	4,874
Stock-based compensation expense	—	—	40,267	—	—	40,267
Unrealized gain on available-for-sale securities, net	—	—	—	10,972	—	10,972
Net loss	—	—	—	—	(263,494)	(263,494)
Balances at December 31, 2023	44,046	4	1,021,214	(4,429)	(705,047)	311,742
Vesting of restricted stock units, net of tax	345	—	(910)	—	—	(910)
Exercise of stock options, net of tax	291	—	1,512	—	—	1,512
Issuance of common stock under employee stock purchase plan	105	—	1,191	—	—	1,191
Issuance of common stock and pre-funded warrants upon public offering, net of transaction costs of $534	4,565	1	131,066	—	—	131,067
Exercise of pre-funded warrants	197	—	—	—	—	—
Stock-based compensation expense	—	—	38,463	—	—	38,463
Unrealized gain on available-for-sale securities, net	—	—	—	3,688	—	3,688
Net loss	—	—	—	—	(227,102)	(227,102)
Balances at December 31, 2024	49,549	$ 5	$ 1,192,536	$ (741)	$ (932,149)	$ 259,651

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

REGENXBIO INC.
CONSOLIDATED STATEMENTS OF CASH FLOWS
(in thousands)

	Years Ended December 31,		
	2024	2023	2022
Cash flows from operating activities			
Net loss	$ (227,102)	$ (263,494)	$ (280,321)
Adjustments to reconcile net loss to net cash used in operating activities			
Stock-based compensation expense	38,463	40,267	40,788
Depreciation and amortization	16,215	17,320	12,909
Provision for credit losses (recoveries)	(5,000)	—	—
Net amortization of premiums (accretion of discounts) on marketable debt securities	(4,144)	687	4,712
Net realized loss (gain) on investments	(6,616)	(2,205)	79
Impairment of long-lived assets	2,101	—	—
Non-cash interest expense	6,101	(414)	137
Other non-cash adjustments	1,324	319	231
Changes in operating assets and liabilities			
Accounts receivable	9,707	3,935	4,822
Prepaid expenses	5,453	(620)	4,852
Other current assets	6,511	(10,546)	247
Operating lease right-of-use assets	6,196	5,761	4,450
Other assets	807	1,590	31
Accounts payable	(292)	(2,791)	18,790
Accrued expenses and other current liabilities	(12,124)	2,452	(25,616)
Deferred revenue	(33)	(1,512)	(1,165)
Operating lease liabilities	(8,083)	(6,641)	(544)
Other liabilities	(2,609)	(2,515)	8,110
Net cash used in operating activities	(173,125)	(218,407)	(207,488)
Cash flows from investing activities			
Purchases of marketable debt securities	(190,139)	(86,564)	(184,875)
Maturities of marketable debt securities	290,238	285,492	203,146
Sales of equity securities	5,783	1,975	524
Purchases of property and equipment	(2,436)	(9,960)	(30,724)
Net cash provided by (used in) investing activities	103,446	190,943	(11,929)
Cash flows from financing activities			
Proceeds from exercise of stock options	1,512	1,521	2,804
Taxes paid related to net settlement of stock-based awards	(910)	(419)	(284)
Proceeds from issuance of common stock under employee stock purchase plan	1,191	1,826	1,742
Proceeds from public offering of common stock and pre-funded warrants, net of issuance costs	131,067	—	—
Proceeds from private placement of common stock, net of issuance costs	—	4,874	—
Offering expenses related to at-the-market offering programs	(323)	(470)	—
Repayments under liability related to sale of future royalties, net of imputed interest	(39,854)	(42,298)	(33,102)
Net cash provided by (used in) financing activities	92,683	(34,966)	(28,840)
Net increase (decrease) in cash and cash equivalents and restricted cash	23,004	(62,430)	(248,257)
Cash and cash equivalents and restricted cash			
Beginning of period	36,552	98,982	347,239
End of period	$ 59,556	$ 36,552	$ 98,982
Supplemental cash flow information			
Cash paid (received) for income taxes	$ (72)	$ (142)	$ 11,812
Cash paid for imputed interest under liability related to sale of future royalties	$ 6,559	$ 7,276	$ 23,117

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

1. Nature of Business

REGENXBIO Inc. (the Company) is a clinical-stage biotechnology company seeking to improve lives through the curative potential of gene therapy. The Company's investigational gene therapies use adeno-associated virus (AAV) vectors from its proprietary gene delivery platform (NAV Technology Platform). The NAV® Technology Platform consists of exclusive rights to a large portfolio of proprietary AAV vectors. The Company has developed a broad pipeline of gene therapy product candidates using the NAV Technology Platform as a one-time treatment to address an array of diseases. In addition to its internal product development efforts, the Company also selectively licenses the NAV Technology Platform and other intellectual property rights to other leading biotechnology and pharmaceutical companies (NAV Technology Licensees). As of December 31, 2024, the NAV Technology Platform was being applied by NAV Technology Licensees in one commercial product, Zolgensma®, and in the preclinical and clinical development of a number of other licensed products. Additionally, the Company has licensed intellectual property rights to collaborators for the joint development and commercialization of certain product candidates. The Company was formed in 2008 in the State of Delaware and is headquartered in Rockville, Maryland.

Liquidity

In January 2025, the Company and Nippon Shinyaku Co., Ltd. (Nippon Shinyaku) entered into a collaboration and license agreement (the Nippon Shinyaku Collaboration Agreement) for the joint development and commercialization of RGX-121 for the treatment of Mucopolysaccharidosis Type II (MPS II) and RGX-111 for the treatment of Mucopolysaccharidosis Type I (MPS I). Pursuant the Nippon Shinyaku Collaboration Agreement, the Company will receive an up-front payment of $110.0 million following the effective date of the agreement and is eligible to receive up to $700.0 million upon the achievement of specified development and sales-based milestones. The Nippon Shinyaku Collaboration Agreement became effective in March 2025. Please refer to Note 10 for further information on the Nippon Shinyaku Collaboration Agreement.

The Company has incurred cumulative losses since inception and as of December 31, 2024, had generated an accumulated deficit of $932.1 million. The Company's ability to transition to recurring profitability is dependent upon achieving a level of revenues adequate to support its cost structure, which depends heavily on the successful development, approval and commercialization of its product candidates. The Company may never achieve recurring profitability and, unless and until it does, will continue to need to raise additional capital through equity offerings, licensing and collaboration arrangements, or other non-dilutive financings. There is no assurance that the Company will be able to raise sufficient capital or obtain financing on favorable terms, or at all. The Company’s ability to continue as a going concern may be impacted by the development and potential commercialization of its product candidates and its ability to raise additional capital to fund its operations. If the Company is unable to raise capital sufficient to meet its working capital needs in the future, it may be forced to delay expenditures, reduce the scope of its development activities or make other changes to its operating plans.

As of December 31, 2024, the Company had cash, cash equivalents and marketable securities of $244.9 million. Management believes that its cash, cash equivalents and marketable securities as of December 31, 2024, along with the up-front payment of $110.0 million expected to be received under the Nippon Shinyaku Collaboration Agreement, is sufficient to fund operations for at least the next 12 months from the date these consolidated financial statements were issued.

2. Summary of Significant Accounting Policies

Basis of Presentation and Principles of Consolidation

The accompanying consolidated financial statements have been prepared in conformity with accounting principles generally accepted in the United States of America (GAAP) and include the accounts of the Company and its wholly owned subsidiaries. All intercompany balances and transactions have been eliminated in consolidation.

Foreign Currency Transactions

The functional currency of the Company and its consolidated subsidiaries is the U.S. dollar. Transaction gains and losses that arise from exchange rate fluctuations on transactions denominated in currencies other than the U.S. dollar are included in results of operations as incurred. During the years ended December 31, 2024, 2023 and 2022, the Company recorded aggregate net foreign currency transaction losses of $0.9 million, $0.3 million and $0.7 million, respectively, which are included in other operating expenses (income) in the consolidated statements of operations and comprehensive loss.

Use of Estimates

The preparation of the consolidated financial statements in conformity with GAAP requires management to make estimates and assumptions that affect the reported amounts of assets, liabilities, revenues and expenses and related disclosure of contingent assets and liabilities for the periods presented. Management bases its estimates on historical experience and various other factors that it believes are reasonable under the circumstances, the results of which form the basis for making judgments about the carrying value of assets and liabilities, and other reported amounts, that are not readily apparent from other sources. Actual results may differ materially from these estimates. Estimates are used in the following areas, among others: license and royalty revenue, the allowance for credit losses, accrued research and development expenses and other accrued liabilities, stock-based compensation expense, interest expense under the liability related to the sale of future royalties, income taxes and fair value measurements.

Cash and Cash Equivalents

The Company considers all highly liquid investments purchased with original maturities of 90 days or less at acquisition to be cash equivalents.

Restricted Cash

Restricted cash consists of deposits held at financial institutions that are used to collateralize irrevocable letters of credit required under the Company's lease agreements and certain other agreements with third parties. The following table provides a reconciliation of cash and cash equivalents and restricted cash as reported on the consolidated balance sheets to the total of these amounts as reported at the end of the period in the consolidated statements of cash flows (in thousands):

	As of December 31,		
	2024	2023	2022
Cash and cash equivalents	$ 57,526	$ 34,522	$ 96,952
Restricted cash	2,030	2,030	2,030
Total cash and cash equivalents and restricted cash	$ 59,556	$ 36,552	$ 98,982

Marketable Securities

Marketable securities consist of available-for-sale debt securities and are carried at fair value. Marketable debt securities with remaining maturity dates exceeding 12 months which are not intended to be sold prior to maturity for use in current operations are classified as non-current assets. Unrealized gains and losses on available-for-sale debt securities, net of any related tax effects, are excluded from results of operations and are included in other comprehensive income (loss) and reported as a separate component of stockholders' equity until realized. The Company uses the aggregate portfolio approach to release the tax effects of unrealized gains and losses on available-for-sale debt securities in accumulated other comprehensive loss. Purchase premiums and discounts on marketable debt securities are amortized or accreted into the cost basis over the life of the related security as adjustments to the yield using the effective-interest method. Interest income is recognized when earned. Realized gains and losses from the sale or maturity of marketable securities are based on the specific identification method and are included in results of operations as investment income.

At each reporting date, the Company evaluates available-for-sale debt securities which have an amortized cost basis in excess of the fair value of the security to determine if the unrealized loss or any potential credit losses should be recognized in results of operations. If the Company does not have the intent and ability to hold the security until recovery of the unrealized loss, the difference between the fair value and amortized cost basis of the security is charged to results of operations resulting in a new amortized cost basis of the security. If the Company has the intent and ability to hold the security until recovery of the unrealized loss, the security is evaluated for potential credit losses. If a credit loss is deemed to exist, the credit loss is recognized in results of operations and an allowance for credit losses is recorded against the amortized cost basis of the security. In determining whether a credit loss exists related to impaired available-for-sale debt securities, the Company considers, among other factors, the extent of the unrealized loss relative to the amortized cost basis, the credit rating of the issuer and any recent changes thereto, current and expected future economic conditions, and any adverse events or other changes in circumstances that have occurred which may indicate a potential credit loss. The Company did not record an allowance for credit losses on its available-for-sale debt securities as of December 31, 2024 or 2023.

Accounts Receivable

Accounts receivable primarily consist of consideration due to the Company resulting from its license agreements with customers. Accounts receivable include amounts invoiced to licensees as well as rights to consideration which have not yet been invoiced, including unbilled royalties, and for which payment is conditional solely upon the passage of time. If a licensee elects to terminate a license prior to the end of the license term, the licensed intellectual property is returned to the Company and any accounts

receivable from the licensee which are not contractually payable to the Company are charged off as a reduction of license revenue in the period of the termination. Accounts receivable which are not expected to be received by the Company within 12 months from the reporting date are stated net of a discount to present value and recorded as non-current assets on the consolidated balance sheets. The present value discount is recognized as a reduction of revenue in the period in which the accounts receivable are initially recorded and is accreted as interest income from licensing over the term of the receivables.

Accounts receivable are stated net of an allowance for credit losses, if deemed necessary based on the Company's evaluation of collectability and potential credit losses. Management assesses the collectability of its accounts receivable using the specific identification of account balances, and considers the credit quality and financial condition of its significant customers, historical information regarding credit losses and the Company's evaluation of current and expected future economic conditions. If necessary, an allowance for credit losses is recorded against accounts receivable such that the carrying value of accounts receivable reflects the net amount expected to be collected. Accounts receivable balances are written off against the allowance for credit losses when the potential for collectability is considered remote. Please refer to Note 10 for further information regarding the allowance for credit losses related to accounts receivable.

Concentrations of Credit Risk and Off-balance Sheet Risk

Cash and cash equivalents, marketable debt securities and accounts receivable are financial instruments that are potentially subject to concentrations of credit risk. The Company's cash and cash equivalents are deposited in accounts at multiple financial institutions, and amounts may exceed federally insured limits. The Company believes it is not exposed to significant credit risk due to the financial strength of the depository institutions in which the cash and cash equivalents are held. The Company's marketable debt securities consist of investment grade securities and may be subject to concentrations of credit risk. The Company has adopted an investment policy which limits potential concentrations of investments and establishes minimum acceptable credit ratings, thereby reducing credit risk exposure. As of December 31, 2024, the Company believes that it is not exposed to significant credit risk related to accounts receivable due to the credit quality and history of collections from its significant customers, and the Company is unaware of any concentrations of credit risk related to accounts receivable from significant customers with deteriorated credit quality. The Company has no financial instruments with off-balance sheet risk of loss.

The following table summarizes those customers who represented at least 10% of revenues or total net accounts receivable for the periods presented:

	Revenues Years Ended December 31,			Accounts Receivable, Net As of December 31,	
	2024	2023	2022	2024	2023
Customer A	98%	95%	90%	97%	95%

Leases

The Company accounts for its lease arrangements in accordance with Accounting Standards Codification (ASC) 842, *Leases* (ASC 842). Under ASC 842, the Company classifies its leases as either finance or operating leases based on the principle of whether or not the lease is effectively a financed purchase by the Company. Lease classification is evaluated at the inception of the lease agreement. Regardless of classification, the Company records a right-of-use asset and a lease liability for all leases with a term greater than 12 months. All of the Company's leases are classified as operating leases. Operating lease expense is recognized on a straight-line basis over the term of the lease, with the exception of variable lease expenses which are recognized as incurred.

The Company identifies leases in its contracts if the contract conveys the right to control the use of identified property, plant or equipment for a period of time in exchange for consideration. The Company does not allocate lease consideration between lease and nonlease components and records a lease liability equal to the present value of the remaining fixed consideration under the lease. The interest rates implicit in the Company's leases are generally not readily determinable. Accordingly, the Company uses its estimated incremental borrowing rate at the commencement date of the lease to determine the present value discount of the lease liability. The Company estimates its incremental borrowing rate for each lease based on an evaluation of its expected credit rating and the prevailing market rates for collateralized debt in a similar economic environment with similar payment terms and maturity dates commensurate with the term of the lease. The right-of-use asset for each lease is equal to the lease liability, adjusted for unamortized initial direct costs and lease incentives and prepaid or accrued rent. Initial direct costs of entering into a lease are included in the right-of-use asset and amortized as lease expense over the term of the lease. Lease incentives, such as tenant improvement allowances, are recorded as a reduction of the right-of-use asset and amortized as a reduction of lease expense over the term of the lease. The Company excludes options to extend or terminate leases from the calculation of the lease liability unless it is reasonably certain the option will be exercised.

The Company evaluates its right-of-use assets for impairment in accordance with its policy for long-lived assets. To the extent an impairment of a right-of-use asset is recognized, the remaining carrying value of the asset is subsequently amortized as lease expense on a straight-line basis from the date of impairment to the earlier of the end of the right-of-use asset's useful life or the end of the lease term.

The Company determines the classification of subleases at the inception of the sublease, as well as whether the Company has been relieved of its primary obligation under the original lease. All of the Company's subleases are classified as operating leases and, in each case, the Company has not been relieved of its primary obligation under the original lease and continues to account for the original lease as it did prior to the commencement of the sublease. Sublease income is recognized on a straight-line basis over the term of the sublease as a reduction of the related lease expense of the original lease. Initial direct costs of entering into a sublease are deferred and amortized on a straight-line basis over the term of the sublease as a reduction of sublease income.

Property and Equipment

Property and equipment is stated at cost less accumulated depreciation and amortization. Maintenance and repairs that do not improve or extend the lives of the respective assets are expensed to operations as incurred. Upon disposal, the related cost and accumulated depreciation is removed from the accounts and any resulting gain or loss is included in the results of operations. Depreciation and amortization is calculated using the straight-line method over the estimated useful lives of the assets, which are as follows:

	Estimated Useful Life
Computer equipment and software	3 years
Furniture and fixtures	5 years
Laboratory and manufacturing equipment	5 to 15 years
Leasehold improvements	Shorter of lease term or estimated useful life

Cloud Computing Arrangements

The Company capitalizes certain costs associated with the implementation of cloud computing arrangements that are accounted for as service contracts. Once implementation activities are substantially complete and the cloud-based application is ready for its intended use, capitalization ceases and amounts capitalized are amortized on a straight-line basis over the term of the hosting arrangement. Capitalized implementation costs for cloud-based applications and associated amortization are classified on the consolidated balance sheets and statements of operations and comprehensive loss in the same manner as the costs of the associated hosting arrangement. As of December 31, 2024 and 2023, the Company had recorded capitalized costs, net of amounts amortized, of less than $0.1 million and $1.0 million, respectively, related to the implementation of cloud-based software applications, which were included in prepaid expenses and other assets on the consolidated balance sheets. Amortization of capitalized implementation costs for cloud-based applications recorded for the years ended December 31, 2024, 2023 and 2022 was $1.0 million, $1.4 million and $1.3 million, respectively, and was included in general and administrative expenses in the consolidated statements of operations and comprehensive loss.

Impairment of Long-lived Assets

The Company's long-lived assets consist primarily of property and equipment and operating lease right-of-use assets. The Company evaluates its long-lived assets for impairment when events or changes in circumstances indicate the carrying value of the assets may not be recoverable. Recoverability is measured by comparison of the book values of the assets to estimated 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 by the amount by which the book value of the assets exceed their fair value, which is measured based on the projected discounted future net cash flows arising from the assets. Please refer to Note 5 and Note 6 for further information on impairment of long-lived assets.

Non-marketable Equity Securities

Non-marketable equity securities consist of equity investments in other entities in which the Company's ownership interest is below 20% and the Company does not have significant influence over the operations of the entity, or for which the equity securities are not common stock or in-substance common stock. The Company's non-marketable equity securities do not have readily determinable fair values and are measured at cost less impairment, adjusted for observable price changes for identical or similar investments of the same issuer. Please refer to Note 4 for further information on non-marketable equity securities.

Fair Value Measurements

The Company is required to disclose information on all assets and liabilities reported at fair value that enables an assessment of the inputs used in determining the reported fair values. ASC 820, *Fair Value Measurements and Disclosures*, establishes a hierarchy of inputs used in measuring fair value that maximizes the use of observable inputs and minimizes the use of unobservable inputs by requiring that the observable inputs be used when available. 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. The fair value hierarchy applies only to the valuation inputs used in determining the reported fair value of the investments and is not a measure of the investment credit quality. The three levels of the fair value hierarchy are described below:

•Level 1—Valuations based on unadjusted quoted prices in active markets for identical assets or liabilities that the Company has the ability to access at the measurement date.

•Level 2—Valuations based on quoted prices for similar assets or liabilities in markets that are not active or for which all significant inputs are observable, either directly or indirectly.

•Level 3—Valuations that require inputs that reflect the Company's own assumptions that are both significant to the fair value measurement and unobservable.

To the extent that valuation is based on models or inputs that are less observable or 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 assets and liabilities categorized in Level 3. The level within the fair value hierarchy of an asset or liability measured at fair value is based on the lowest level of any input that is significant to the fair value measurement. The fair values of the Company's Level 2 financial instruments are based on quoted market prices or broker or dealer quotations for similar assets. These investments are initially valued at the transaction price and subsequently valued utilizing third-party pricing providers or other market observable data. Please refer to Note 4 for further information on the Company's fair value measurements.

Liability Related to Sale of Future Royalties

As discussed in Note 7, the Company recorded a liability for the net proceeds received from the sale of its Zolgensma royalty payments to entities managed by Healthcare Royalty Management, LLC (collectively, HCR). The liability is accounted for as debt since the return to HCR is explicitly capped under the royalty purchase agreement, and is amortized over the estimated life of the arrangement using the effective interest method. The total amount of royalty payments received by HCR under the agreement, less the net proceeds received by the Company, is recorded as interest expense over the life of the arrangement. The Company estimates the effective interest rate based on its estimate of total royalty payments to be received by HCR under the agreement. The Company reassesses these estimates at each reporting date and adjusts the effective interest rate and amortization of the liability on a prospective basis as necessary.

Due to its continuing involvement in the underlying license agreement with Novartis Gene Therapies, Inc. (formerly AveXis, Inc.) (Novartis Gene Therapies), the Company continues to recognize royalty revenue on net sales of Zolgensma and records the royalty payments to HCR as a reduction of the liability when paid. As such payments are made to HCR, the balance of the liability will be effectively repaid over the life of the royalty purchase agreement. The portion of the liability related to the sale of future royalties which is expected to be amortized within 12 months of the reporting date is recorded as a current liability, with the remaining portion of the liability recorded as a non-current liability.

Pre-funded Warrants

Warrants are accounted for based on the specific terms of the warrant agreements. The Company's pre-funded warrants are indexed to the Company's common stock and meet the criteria to be classified as equity. Proceeds from the issuance of pre-funded warrants are recorded within additional paid-in capital and are not subject to remeasurement. Please refer to Note 9 for further information regarding pre-funded warrants issued by the Company.

Revenue Recognition

The Company recognizes revenue in accordance with ASC 606, *Revenue from Contracts with Customers* (ASC 606). ASC 606 requires entities to recognize revenue when control of the promised goods or services is transferred to customers at an amount that reflects the consideration to which the entity expects to be entitled to in exchange for those goods or services. The following five steps

are performed to determine the appropriate revenue recognition for arrangements within the scope of ASC 606: (i) identify the 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 revenue when (or as) the entity satisfies the performance obligations.

The Company applies the five-step model to contracts that are within the scope of ASC 606 only when it is probable that the Company will collect the consideration it is entitled to in exchange for the goods or services it transfers to the customer. At contract inception, for contracts within the scope of ASC 606, the Company assesses the goods or services promised within each contract and determines those that are performance obligations and whether each promised good or service is distinct. The Company then recognizes as revenue the amount of the transaction price that is allocated to respective performance obligations when (or as) the respective performance obligations are satisfied.

The Company evaluates its contracts with customers for the presence of significant financing components. If a significant financing component is identified in a contract and provides a financing benefit to the customer, the transaction price for the contract is adjusted to account for the financing portion of the arrangement, which is recognized as interest income over the financing term using the effective interest method. In determining the appropriate interest rates for significant financing components, the Company evaluates the credit profile of the customer and prevailing market interest rates and selects an interest rate in which it believes would be charged to the customer in a separate financing arrangement over a similar financing term.

License and Royalty Revenue

The Company licenses its NAV Technology Platform and other intellectual property rights to other biotechnology and pharmaceutical companies, including collaborators for the joint development and commercialization of its product candidates. The terms of the licenses vary, and licenses may be exclusive or non-exclusive and may be sublicensable by the licensee. Licenses may grant intellectual property rights for purposes of internal and preclinical research and development only, or may include the rights, or options to obtain future rights, to commercialize drug therapies for specific diseases using the Company's NAV Technology Platform and other licensed rights. License agreements generally have a term at least equal to the life of the underlying patents, but are terminable at the option of the licensee. Consideration payable to the Company under its license agreements may include: (i) up-front and annual fees, (ii) milestone payments based on the achievement of certain development and sales-based milestones, (iii) sublicense fees, (iv) royalties on sales of licensed products, (v) fees for services related to the development of licensed products and (vi) other consideration payable upon optional goods and services purchased by licensees.

The Company's license agreements are accounted for as contracts with customers within the scope of ASC 606, with the exception of transactions for which the counterparty is determined not to be a customer. At the inception of each license agreement, the Company determines the contract term for purposes of applying the requirements of ASC 606. Licenses are generally terminable at the option of the licensee with advance notice to the Company. For each license granted, including licenses granted upon the exercise of license options, the Company evaluates these termination rights to determine whether a substantive termination penalty would be incurred by the licensee upon termination. If the licensee incurs a substantive termination penalty upon termination, the contract term for revenue recognition purposes is generally equal to the stated term of the license, which is the life of the underlying licensed patents. Alternatively, if the licensee does not incur a substantive termination penalty upon termination, the contract term for revenue recognition purposes may be shorter than the stated term of the license, in which case the termination rights may be accounted for as contract renewal options. The determination of whether a substantive termination penalty is associated with the termination rights requires significant judgment. In making this determination, the Company considers, among other things, the nature of the intellectual property rights that would be returned to the Company upon termination, including the exclusivity of the licensed rights and the stage of development of the licensed products, the payment terms, including the amount and timing of non-refundable or guaranteed payments, and the business purpose of the termination rights granted to the licensee. Generally, the most significant judgment in determining whether a substantive termination penalty exists relates to the amount of any up-front or guaranteed non-refundable payments relative to the amount of annual payments that may be avoided by the licensee upon termination of the license. The Company considers all of the facts and circumstances relevant to each license when making this determination.

Performance obligations under the Company's license agreements may include (i) the delivery of intellectual property licenses, (ii) options granted to licensees to acquire additional licenses, to the extent the options represent material rights to the licensee, and (iii) research and development services to be performed by the Company related to licensed products. License agreements may provide licensees with contract renewal options or options to acquire additional licenses, goods or other services. Options are evaluated at the inception of the license agreement to determine whether they provide material rights to the licensee. In making this determination, the Company considers whether the options are priced at an incremental discount to the standalone selling price for the underlying licenses, goods or services, in which case the option is considered to be a material right to the licensee and is accounted for as a separate performance obligation under the current license agreement. At the inception of each license agreement which contains

performance obligations for research and development services, the Company evaluates whether the license is distinct from the research and development services, which requires judgment. In making this determination, the Company considers, among other things, the stage of development of the licensed products and whether the research and development services will significantly impact further development of the licensed products. If it is determined that the license is not distinct from the research and development services, the license is combined with the research and development services into a single performance obligation.

The Company evaluates the transaction price of its license agreements at the inception of each agreement and at each reporting date. The transaction price includes the fixed consideration payable to the Company during the contract term, as well as any variable consideration to the extent that it is probable that a significant reversal of revenue will not occur in the future. Fixed consideration under the license agreements may include up-front and annual fees payable during the contract term and fees for development services performed by the Company. Variable consideration under the license agreements may include development and sales-based milestone payments, sublicense fees and royalties on sales of licensed products. Consideration contingent upon the exercise of options by a licensee is excluded from the transaction price and not accounted for as part of the license agreement until the option is exercised.

The transaction price for each license agreement is allocated to the underlying performance obligations based on their relative standalone selling prices and recognized as revenue when (or as) the performance obligations are satisfied. Consideration allocated to performance obligations for the delivery of an intellectual property license is recognized as revenue in full upon the delivery of the license to the licensee. Consideration allocated to performance obligations for license options is recognized as revenue in full upon the earlier of the option exercise or expiration. The exercise of a license option by a licensee is accounted for as a new license for revenue recognition purposes. Consideration allocated to performance obligations for research and development services is recognized as revenue as the services are performed by the Company.

Up-front and annual licenses fees payable to the Company over the contract term of each license are included in the transaction price, and the portion of this consideration allocated to the performance obligation for the delivery of the intellectual property license is recognized as revenue in full upon the delivery of the license to the licensee. If annual license fees are payable to the Company in periods beyond 12 months from the delivery of the license, a significant financing component is deemed to exist which provides a financing benefit to the licensee. If a significant financing component is identified, the Company adjusts the transaction price for the license to include only the present value of the annual license fees payable to the Company over the contract term. The discounted portion of the license fees is recognized as interest income from licensing over the financing period of the license.

Development milestone payments are payable to the Company upon the achievement of specified development milestones. At the inception of each license agreement that contains development milestone payments, the Company evaluates whether the milestones are considered probable of achievement and estimates the amount to be included in the transaction price using the most likely amount method. If it is probable that a significant revenue reversal will not occur in the future, milestone payments are included in the transaction price and recognized as revenue upon the delivery of the license. Milestone payments contingent on the achievement of development milestones that are not within the control of the Company or the licensee, such as regulatory approvals, are not considered probable of being achieved and are excluded from the transaction price until the milestone is achieved. At each reporting date, the Company re-evaluates the probability of achievement of each outstanding development milestone and, if necessary, adjusts the transaction price for any milestones for which the probability of achievement has changed due to current facts and circumstances. Any such adjustments are recorded on a cumulative catch-up basis and recognized as revenue in the period of the adjustment.

Royalties on sales of licensed products, sales-based milestone payments, including milestones payable upon first commercial sales of licensed products, and sublicense fees based on the receipt of certain fees by licensees from any sublicensees are excluded from the transaction price of each license and recognized as revenue in the period that the related sales or sublicenses occur, provided that the associated license has been delivered to the licensee.

Royalty revenue to date consists primarily of royalties on net sales of Zolgensma, which is a licensed product under the Company's license agreement with Novartis Gene Therapies, a wholly owned subsidiary of Novartis AG (Novartis), for the development and commercialization of treatments for spinal muscular atrophy (SMA). The Company recognizes royalty revenue from net sales of Zolgensma in the period in which the underlying products are sold by Novartis Gene Therapies, which in certain cases may require the Company to estimate royalty revenue for periods of net sales which have not yet been reported to the Company. Estimated royalties are reconciled to actual amounts reported in subsequent periods, and any differences are recognized as an adjustment to royalty revenue in the period the royalties are reported.

The Company receives payments from licensees based on the billing schedules established in each license agreement. Amounts recognized as revenue which have not yet been received from licensees, including unbilled royalties, are recorded as accounts receivable when the Company's rights to the consideration are conditional solely upon the passage of time. Amounts recognized as revenue which have not yet been received from licensees are recorded as contract assets when the Company's rights to the

consideration are not unconditional. Contract assets are recorded as other current assets on the consolidated balance sheets if the consideration is expected to be realized within 12 months from the reporting date, or as other assets if the consideration is expected to be realized in periods beyond 12 months from the reporting date. If a licensee elects to terminate a license prior to the end of the license term, the licensed intellectual property is returned to the Company and any consideration recorded as accounts receivable or contract assets which is not contractually payable by the licensee is charged off as a reduction of license revenue in the period of the termination. Amounts received by the Company prior to the delivery of underlying performance obligations are deferred and recognized as revenue upon the satisfaction of the performance obligations by the Company. Deferred revenue which is not expected to be recognized within 12 months from the reporting date is recorded as non-current on the consolidated balance sheets.

Collaborative Arrangements

The Company evaluates its agreements with collaboration partners to determine whether they are within the scope of ASC 808, *Collaborative Arrangements* (ASC 808). Such arrangements are within the scope of ASC 808 if they involve joint operating activities performed by parties that are both active participants in the activities and exposed to significant risks and rewards dependent on the commercial success of such activities. This evaluation is performed throughout the life of the arrangement based on any changes in the roles and responsibilities of the parties under the arrangement. For collaboration arrangements within the scope of ASC 808 that contain multiple elements, the Company identifies the various transactions with the counterparty and determines if any unit of account is more reflective of a transaction with a customer and therefore should be accounted for within the scope of ASC 606. For transactions that are accounted for pursuant to ASC 808, an appropriate method of recognition and presentation is determined and consistently applied. For transactions that are accounted for pursuant to ASC 606, the Company applies the five-step model as described in its revenue recognition policies.

For transactions accounted for as collaborative arrangements under ASC 808, payments to and from collaboration partners associated with multiple activities in a collaboration arrangement are classified based on the nature of each separate activity. Payments associated with development activities performed are recorded as research and development expense when owed to collaboration partners, or as a reduction of research and development expense when due from collaboration partners. Payments associated with commercialization activities performed are recorded as general and administrative expense when owed to collaboration partners, or as a reduction of general and administrative expense when due from collaboration partners. At the end of each reporting period, the Company records a net amount due to or from collaboration partners for activities performed by the parties under the collaboration.

Cost of Revenues

Cost of revenues consists primarily of sublicense fees and royalties on net sales of licensed products as specified in the Company's agreements with its licensors. Sublicense fees are based on a percentage of license fees received by the Company from licensees and are recognized in the period that the underlying revenue is recognized. Royalties are based on a percentage of net sales of licensed products by licensees and are recognized in the period that the underlying sales occur. Amounts which are payable to licensors in periods beyond 12 months from the reporting date are recorded as non-current liabilities on the consolidated balance sheets.

Research and Development Expenses

Research and development costs are expensed as incurred in performing research and development activities. Advance payments for goods or services related to research and development activities are deferred and expensed as the goods are delivered or the services are performed. Research and development costs include salaries, wages, benefits and other personnel costs, laboratory and facilities costs, allocated overhead costs, license and milestone fees, and costs of goods and services associated with preclinical research and clinical trial activities, associated manufacturing-related activities, regulatory activities and other related services performed by third parties. At the end of each reporting period, the Company compares payments made to third-party service providers to the estimated expenses incurred based on the services provided and progress toward completion of the research or development objectives. Such estimates are subject to change as additional information becomes available. Depending on the timing of payments to the service providers and the estimated expenses incurred, the Company may record net prepaid or accrued research and development expenses relating to these costs. Up-front fees incurred in obtaining technology licenses, as well as milestone payments to licensors, are charged to research and development expense as incurred if the technology licensed has no alternative future use.

Stock-based Compensation

The Company accounts for its stock-based compensation awards in accordance with ASC 718, *Compensation—Stock Compensation* (ASC 718). ASC 718 requires all stock-based awards to employees and nonemployees to be recognized as expense

based on the grant date fair value of the awards. The Company's stock-based awards include stock options and restricted stock units granted to employees and nonemployees and shares issued to employees under its employee stock purchase plan.

The Company's stock-based awards may be subject to either service or performance-based vesting conditions. Compensation expense related to awards with service-based vesting conditions is recognized on a straight-line basis based on the estimated grant date fair value over the requisite service period of the award, which is generally the vesting term. Compensation expense related to awards with performance-based vesting conditions is recognized based on the estimated grant date fair value over the requisite service period using the accelerated attribution method to the extent achievement of the performance condition is probable.

The Company has elected to not estimate forfeitures of stock-based awards and accounts for forfeitures as they occur.

The Company estimates the fair value of its stock option awards using the Black-Scholes option-pricing model, which requires the input of subjective assumptions, including (i) the fair value of the underlying common stock, (ii) the expected stock price volatility, (iii) the expected term of the award, (iv) the risk-free interest rate and (v) expected dividends. The Company estimates expected stock price volatility based on the historical volatility of its common stock over a period of time commensurate with the expected term of its stock option awards. Due to the lack of sufficient historical data, the Company estimates the expected term of its employee stock options using the "simplified" method, whereby the expected term equals the arithmetic average of the vesting term and the original contractual term of the option. For stock options granted to nonemployees, the Company uses the contractual term of the award rather than expected term to estimate the fair value of the award. The Company estimates the risk-free interest rates for periods within the expected term of its options based on the rates of U.S. Treasury securities with maturity dates commensurate with the expected term of the associated awards. The Company assumes a dividend yield of zero for its common stock as it has never paid dividends and does not expect to pay dividends for the foreseeable future.

The Company estimates the fair value of restricted stock units based on the fair value of the Company's common stock on the date of the grant.

Income Taxes

Income taxes are accounted for in accordance with ASC 740, *Income Taxes* (ASC 740), which provides for deferred taxes using an asset and liability approach. The Company recognizes deferred tax assets and liabilities for the expected future tax consequences of events that have been included in the consolidated financial statements or tax returns. Deferred tax assets and liabilities are determined based on the difference between the financial statement and tax bases of assets and liabilities using enacted tax rates in effect for the year in which the differences are expected to reverse. Valuation allowances are provided, if based upon the weight of available evidence, it is more likely than not that some or all of the deferred tax assets will not 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 recognizes interest and penalties related to uncertain tax positions in income tax expense. As of December 31, 2024 and 2023, the Company had no accrued interest or penalties related to uncertain tax positions and no amounts have been recognized in the Company's consolidated statements of operations and comprehensive loss.

Restructuring Expenses

The Company records costs and liabilities associated with exit and disposal activities in accordance with ASC 420, *Exit or Disposal Cost Obligations* (ASC 420) and ASC 712, *Compensation – Nonretirement Postemployment Benefits* (ASC 712). Such costs are based on estimates of fair value in the period liabilities are incurred. The Company evaluates and adjusts these costs as appropriate for changes in circumstances as additional information becomes available. Please refer to Note 14 for further information regarding restructuring expenses.

Net Loss Per Share

Basic net loss per share is calculated by dividing net loss applicable to common stockholders by the weighted-average common shares outstanding during the period, without consideration for common stock equivalents. Diluted net loss per share is calculated by adjusting the weighted-average common shares outstanding for the dilutive effect of common stock equivalents outstanding for the period, determined using the treasury-stock method. For purposes of computing both basic and diluted net loss per share, pre-funded

warrants are considered outstanding shares upon issuance because the underlying shares may be issued for nominal consideration and are exercisable after the original issuance date. Contingently convertible shares in which conversion is based on non-market-priced contingencies are excluded from the calculations of both basic and diluted net loss per share until the contingency has been fully met. For purposes of the diluted net loss per share calculation, common stock equivalents are excluded from the calculation of diluted net loss per share if their effect would be anti-dilutive.

Comprehensive Loss

Comprehensive loss includes net loss as well as unrealized gains and losses on available-for-sale debt securities, net of income tax effects and reclassification adjustments for realized gains and losses.

Recent Accounting Pronouncements

Recently Adopted Accounting Pronouncements

In November 2023, the Financial Accounting Standards Board (FASB) issued Accounting Standards Update (ASU) 2023-07, *Segment Reporting (Topic 280): Improvements to Reportable Segment Disclosures*, which enhances certain interim and annual disclosure requirements of reportable segment information, including information about significant segment expenses. Additionally, the standard requires entities with a single reportable segment to provide all disclosures required by ASC 280, *Segment Reporting*. The Company adopted this standard for annual reporting periods beginning January 1, 2024 and interim reporting periods beginning January 1, 2025, on a retrospective basis for all periods presented. Please refer to Note 17 for further information regarding reportable segment information.

Recent Accounting Pronouncements Not Yet Adopted

In December 2023, the FASB issued ASU 2023-09, *Income Taxes (Topic 740): Improvements to Income Tax Disclosures*, which enhances the disclosure of an entity's effective tax rate reconciliation and requires the disclosure of income taxes paid to be disaggregated by jurisdiction. The standard is effective for the Company beginning January 1, 2025, with early adoption permitted. Upon the adoption of this standard, the Company will modify its disclosures for income taxes, as applicable.

In November 2024, the FASB issued ASU 2024-03, *Income Statement – Reporting Comprehensive Income – Expense Disaggregation Disclosures (Subtopic 220-40): Disaggregation of Income Statement Expenses*. The new standard requires disclosures about specific types of expenses included in the expense captions presented on the face of the income statement as well as disclosures about selling expenses. The standard is effective for the Company for annual periods beginning January 1, 2027 and interim periods beginning January 1, 2028, with early adoption permitted. The standard may be applied either prospectively to financial statements issued for reporting periods after the effective date or retrospectively to any or all prior periods presented in the financial statements. The Company is evaluating the impact of this standard on its consolidated financial statements and related disclosures.

3. Marketable Securities

The following tables present a summary of the Company’s marketable securities, which consist solely of available-for-sale debt securities (in thousands):

	Amortized Cost	Unrealized Gains	Unrealized Losses	Fair Value
December 31, 2024				
U.S. government and agency securities	$ 44,281	$ 11	$ (77)	$ 44,215
Certificates of deposit	1,466	—	(4)	1,462
Corporate bonds	141,474	234	(45)	141,663
	$ 187,221	$ 245	$ (126)	$ 187,340

	Amortized Cost	Unrealized Gains	Unrealized Losses	Fair Value
December 31, 2023				
U.S. government and agency securities	$ 71,811	$ 6	$ (1,248)	$ 70,569
Certificates of deposit	6,572	—	(106)	6,466
Corporate bonds	204,793	143	(2,364)	202,572
	$ 283,176	$ 149	$ (3,718)	$ 279,607

As of December 31, 2024 and 2023, no available-for-sale debt securities had remaining maturities greater than two years. The amortized cost of marketable debt securities is adjusted for amortization of premiums and accretion of discounts to maturity, or to the earliest call date for callable debt securities purchased at a premium.

As of December 31, 2024 and 2023, the balance in accumulated other comprehensive loss consisted solely of unrealized gains and losses on available-for-sale debt securities, net of reclassification adjustments for realized gains and losses and income tax effects. Unrealized gain (loss) on available-for-sale securities, net, as presented in the consolidated statements of operations and comprehensive loss consisted of the following (in thousands):

	Years Ended December 31,		
	2024	2023	2022
Unrealized gain (loss) before reclassifications	$ 3,688	$ 10,972	$ (12,904)
Realized losses (gains) reclassified to investment income	—	—	72
Unrealized gain (loss) on available-for-sale securities, net	$ 3,688	$ 10,972	$ (12,832)

The following tables present the fair values and unrealized losses of available-for-sale debt securities held by the Company in an unrealized loss position for less than 12 months and 12 months or greater (in thousands):

	Less than 12 Months		12 Months or Greater		Total	
	Fair Value	Unrealized Losses	Fair Value	Unrealized Losses	Fair Value	Unrealized Losses
December 31, 2024						
U.S. government and agency securities	$ —	$ —	$ 22,917	$ (77)	$ 22,917	$ (77)
Certificates of deposit	—	—	1,221	(4)	1,221	(4)
Corporate bonds	21,317	(45)	—	—	21,317	(45)
	$ 21,317	$ (45)	$ 24,138	$ (81)	$ 45,455	$ (126)

	Less than 12 Months		12 Months or Greater		Total	
	Fair Value	Unrealized Losses	Fair Value	Unrealized Losses	Fair Value	Unrealized Losses
December 31, 2023						
U.S. government and agency securities	$ 12,877	$ (16)	$ 52,686	$ (1,232)	$ 65,563	$ (1,248)
Certificates of deposit	965	(2)	5,257	(104)	6,222	(106)
Corporate bonds	25,051	(48)	144,642	(2,316)	169,693	(2,364)
	$ 38,893	$ (66)	$ 202,585	$ (3,652)	$ 241,478	$ (3,718)

As of December 31, 2024, available-for-sale debt securities held by the Company which were in an unrealized loss position consisted of 22 investment grade security positions. The Company has the intent and ability to hold such securities until recovery, and based on the credit quality of the issuers and low severity of each unrealized loss position relative to its amortized cost basis, the Company did not identify any credit losses associated with its available-for-sale debt securities. The Company did not recognize any impairment or credit losses on available-for-sale debt securities during the years ended December 31, 2024, 2023 and 2022.

4. Fair Value Measurements

Financial instruments reported at fair value on a recurring basis include cash equivalents and marketable securities. The following tables present the fair value of cash equivalents and marketable securities in accordance with the fair value hierarchy discussed in Note 2 (in thousands):

	Level 1	Level 2	Level 3	Total
December 31, 2024				
Cash equivalents:				
Money market mutual funds	$ —	$ 43,895	$ —	$ 43,895
U.S. government and agency securities	—	2,498	—	2,498
Total cash equivalents	—	46,393	—	46,393
Marketable securities:				
U.S. government and agency securities	—	44,215	—	44,215
Certificates of deposit	—	1,462	—	1,462
Corporate bonds	—	141,663	—	141,663
Total marketable securities	—	187,340	—	187,340
Total cash equivalents and marketable securities	$ —	$ 233,733	$ —	$ 233,733

	Level 1	Level 2	Level 3	Total
December 31, 2023				
Cash equivalents:				
Money market mutual funds	$ —	$ 13,024	$ —	$ 13,024
Total cash equivalents	—	13,024	—	13,024
Marketable securities:				
U.S. government and agency securities	—	70,569	—	70,569
Certificates of deposit	—	6,466	—	6,466
Corporate bonds	—	202,572	—	202,572
Total marketable securities	—	279,607	—	279,607
Total cash equivalents and marketable securities	$ —	$ 292,631	$ —	$ 292,631

Management estimates that the carrying values of its current accounts receivable, other current assets, accounts payable, accrued expenses and other current liabilities approximate fair value due to the short-term nature of those instruments. Accounts receivable which contain non-current portions and certain non-current payables reported as other liabilities are recorded at their present values using a discount rate that is based on prevailing market rates on the date the amounts were initially recorded. Management does not believe there have been any significant changes in market conditions or credit quality that would cause the discount rates initially used to be materially different from those that would be used as of December 31, 2024 to determine the present value of these instruments. Accordingly, management estimates that the carrying values of its non-current accounts receivable and other liabilities approximate the fair value of those instruments. Management estimates that the carrying value of the liability related to the sale of future royalties approximates fair value. As discussed in Note 7, the carrying value of the liability related to the sale of future royalties is based on the Company's estimate of future royalties expected to be paid by the Company over the life of the arrangement, which are considered Level 3 inputs.

Long-lived assets, if determined to be impaired, are measured at fair value on a nonrecurring basis using Level 3 inputs. Please refer to Note 6 for further information on nonrecurring fair value measurements of long-lived assets during the years ended December 31, 2024, 2023 and 2022.

Non-marketable Equity Securities

Non-marketable equity securities are measured at cost less impairment, adjusted for observable price changes for identical or similar investments of the same issuer. The Company did not hold any non-marketable equity securities as of December 31, 2024 and 2023. No remeasurements or impairment losses were recorded on non-marketable equity securities during the years ended December 31, 2024, 2023 and 2022.

Prior to the acquisition of Corlieve Therapeutics SAS (Corlieve) by uniQure N.V. (uniQure) in July 2021, the Company held non-marketable equity securities of Corlieve which were originally acquired by the Company in June 2020 as consideration under a license and collaboration agreement with Corlieve. In connection with the acquisition of Corlieve by uniQure in July 2021, the Company received proceeds of €5.3 million ($6.1 million) from uniQure in exchange for its ownership interest in Corlieve, of which $5.6 million was received upon the closing of the acquisition and $0.5 million was received in August 2022 upon the expiration of a hold back period. As additional consideration, the Company became eligible to receive payments of up to €37.1 million from uniQure contingent upon the achievement of various development and regulatory milestones. During the years ended December 31, 2024 and 2023, the Company received €5.6 million and €1.9 million, respectively, in milestone payments from uniQure and recognized investment income of $6.6 million and $2.2 million, respectively, related to the achievement of the milestones during the period. As of December 31, 2024, there were €29.7 million ($31.0 million as of December 31, 2024) in remaining milestones which have not been paid or achieved and have not been recognized in the consolidated financial statements. Proceeds contingent upon the achievement of the remaining milestones will be recognized as investment income in the period in which any uncertainty regarding realization is substantially resolved, which may not occur until the achievement of the underlying milestones. It is at least reasonably possible that some or all of the proceeds contingent upon these milestones will not be realized by the Company.

5. Property and Equipment, Net

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

	As of December 31,	
	2024	2023
Laboratory and manufacturing equipment	$ 77,141	$ 75,632
Computer equipment and software	4,244	4,700
Furniture and fixtures	7,031	7,052
Leasehold improvements	101,465	101,927
Total property and equipment	189,881	189,311
Accumulated depreciation and amortization	(72,292)	(57,208)
Property and equipment, net	$ 117,589	$ 132,103

During the years ended December 31, 2024, 2023 and 2022, the Company recorded depreciation and amortization expense of $16.2 million, $17.3 million and $12.9 million, respectively.

In March 2024, the Company entered into an agreement to sublease its office facilities in New York, New York. In connection with the sublease, the Company recorded impairment of property and equipment of $0.7 million in the first quarter of 2024 related to furniture and fixtures and leasehold improvements located at the subleased facility. Please refer to Note 6 for further information regarding the sublease agreement and associated impairment of long-lived assets.

6. Leases

9804 Medical Center Drive

In November 2018, the Company entered into an operating lease, which has been amended from time to time, for approximately 186,000 square feet of office, laboratory and manufacturing space at 9804 Medical Center Drive in Rockville, Maryland (the 9804 Medical Center Drive Lease), which now serves as the Company's corporate, research and manufacturing headquarters. The initial construction of the building was performed by the landlord and the lease commenced in September 2020 upon delivery of the leased premises to the Company to make additional improvements to the building. Monthly payments under the lease began in September 2021 and escalate annually in accordance with the lease agreement. The lease expires in September 2036, subject to extension and termination options held by the Company. The Company has an option to extend the term of the lease for up to 10 additional years and an option to terminate the lease, with payment of an early termination fee, after 12 years from the delivery of the leased premises to the Company. As of December 31, 2024, the Company's extension and termination options under the 9804 Medical Center Drive Lease were excluded from the measurement of the right-of-use assets and lease liabilities as they were not reasonably certain of exercise. As required by the 9804 Medical Center Drive Lease, the Company has provided the landlord with an irrevocable letter of credit of $1.1 million which the landlord may draw upon in the event of any uncured default by the Company under the terms of the lease.

Pursuant to the 9804 Medical Center Drive Lease, the Company received a $19.5 million tenant improvement allowance from the landlord to perform improvements to the leased premises. The tenant improvement allowance was recorded as a reduction of the right-of-use assets for the lease and is amortized on a straight-line basis as a reduction of lease expense over the term of the lease. The

Company began occupation of a portion of the facility upon the completion of its construction in 2021. The remaining portion of the building, primarily associated with the manufacturing facility, was activated upon the completion of its construction in 2022. As of December 31, 2024, the Company had recorded property and equipment at cost of $132.6 million related to the buildout at 9804 Medical Center Drive, of which $131.6 million was placed in service and $1.0 million has not yet been placed in service.

As of December 31, 2024, the Company had recorded right-of-use assets of $42.9 million and lease liabilities of $69.0 million related to the 9804 Medical Center Drive Lease.

9712 Medical Center Drive

In March 2015, the Company entered into an operating lease for office space at 9712 Medical Center Drive in Rockville, Maryland (the 9712 Medical Center Drive Lease). The lease term commenced in April 2015, and monthly payments under the lease began in October 2015 and escalate annually in accordance with the lease agreement.

The 9712 Medical Center Drive Lease has been amended from time to time to include additional office and laboratory space at an adjacent building located at 9714 Medical Center Drive and extend the term of the lease, which expires in February 2027, subject to extension options held by the Company. The Company has an option to extend the term of the lease for three additional years, as well as an option to extend the lease term to be coterminous with the 9804 Medical Center Drive Lease, which expires in September 2036. As of December 31, 2024, the Company's extension options under the 9712 Medical Center Drive Lease were excluded from the measurement of the right-of-use assets and lease liabilities as they were not reasonably certain of exercise. The Company received a $0.4 million tenant improvement allowance from the landlord which was recorded as a reduction of the right-of-use assets for the lease and is amortized on a straight-line basis as a reduction of lease expense over the term of the lease.

As of December 31, 2024, the Company had recorded right-of-use assets of $3.2 million and lease liabilities of $3.6 million related to the 9712 Medical Center Drive Lease.

New York Lease and Sublease

In May 2016, the Company entered into an operating lease for office space in New York, New York (the New York Lease), which has since been amended to include additional office space and extend the term of the lease. The lease term commenced in July 2016 and expires in April 2027. The Company received a $0.7 million tenant improvement allowance from the landlord which was recorded as a reduction of the right-of-use assets for the lease and is amortized on a straight-line basis as a reduction of lease expense over the term of the lease. As required by the New York Lease, the Company has provided the landlord with an irrevocable letter of credit of $0.2 million which the landlord may draw upon in the event of any uncured default by the Company under the terms of the lease.

In March 2024, the Company entered into an agreement to sublease its office space under the New York Lease (the New York Sublease) to a third-party subtenant. The sublease term commenced in April 2024 and expires in April 2027 concurrent with the expiration of the New York Lease. Monthly payments under the New York Sublease commenced in July 2024 and escalate annually in accordance with the sublease agreement. As of December 31, 2024, total undiscounted future minimum lease payments to be received by the Company over the term of the New York Sublease were $1.2 million. The Company recognized sublease income of $0.3 million under the New York Sublease during the year ended December 31, 2024.

The New York Sublease is classified as an operating lease and the Company was not relieved of its primary obligation under the New York Lease. The Company continues to account for the New York Lease as it did prior to the commencement of the sublease.

As a result of the New York Sublease, the Company determined an impairment indicator was present as of March 31, 2024 related to the long-lived asset group subject to the sublease, which included the right-of-use asset under the New York Lease, leasehold improvements and other property and equipment allocable to the New York Sublease. The Company concluded the carrying value of the asset group as of March 31, 2024 was not recoverable, as it exceeded the sum of the estimated undiscounted cash flows to be generated by the assets over their remaining lives. The Company estimated the fair value of the asset group as of March 31, 2024 using a discounted cash flow method, which incorporated unobservable inputs including the net identifiable cash flows over the term of the New York Sublease and an estimated borrowing rate of a market participant subtenant. The estimated fair value of the asset group as of March 31, 2024 represents a Level 3 nonrecurring fair value measurement. The Company concluded the carrying value of the asset group of $3.4 million exceeded its estimated fair value of $1.3 million as of March 31, 2024. As such, the Company recognized impairment losses of $2.1 million during the year ended December 31, 2024 on the long-lived asset group associated with the New York Sublease. The impairment losses were allocated to the various assets within the long-lived asset group based on their

relative carrying values and consisted of $1.4 million recorded to the right-of-use assets and $0.7 million recorded to property and equipment. No material impairment losses on long-lived assets were recorded during the years ended December 31, 2023 and 2022.

As of December 31, 2024, the Company had recorded right-of-use assets of $0.6 million and lease liabilities of $2.2 million related to the New York Lease.

Other Leases

In October 2022, the Company entered into an operating lease for office space in Washington, D.C. (the DC Lease). The lease term commenced in October 2022 and expires in October 2027. The Company has an option to extend the term of the lease for five additional years. As of December 31, 2024, the Company's extension option under the DC Lease was excluded from the measurement of the right-of-use assets and lease liabilities as it was not reasonably certain of exercise. The Company recorded the right-of-use assets and lease liabilities related to the DC Lease upon its commencement in October 2022. As of December 31, 2024 the Company had recorded right-of-use assets of $4.0 million and lease liabilities of $4.0 million related to the DC Lease.

The Company leases additional office, laboratory and warehousing space, as well as laboratory and other equipment, under operating leases with various expiration dates through 2029, including leases which have been executed but have not yet commenced.

Operating Lease Information

All of the Company's leases are classified as operating leases. The following table summarizes the Company's lease costs and supplemental cash flow information related to its operating leases (in thousands):

	Years Ended December 31,		
	2024	2023	2022
Operating lease cost	$ 11,168	$ 11,107	$ 9,570
Variable lease cost	2,725	2,263	1,727
Sublease income	(319)	—	—
Total lease cost	$ 13,574	$ 13,370	$ 11,297
Cash paid for amounts included in operating lease liabilities	$ 12,969	$ 11,861	$ 5,697
Right-of-use assets acquired through operating lease liabilities	$ 826	$ 1,132	$ 8,662

Cash paid for amounts included in operating lease liabilities for the years ended December 31, 2024, 2023 and 2022 includes zero, $0.1 million and $1.3 million, respectively, received by the Company during the period under its tenant improvement allowances, which were deemed in-substance lease payments and included in the calculation of the lease liability. Short-term lease expense for the years ended December 31, 2024, 2023 and 2022 was not material and is included in operating lease cost in the table above. Variable lease cost under the Company's operating leases includes items such as common area maintenance, utilities, taxes and other charges.

The following table presents the weighted-average remaining lease term and weighted-average discount rate of the Company's operating leases:

	As of December 31,	
	2024	2023
Weighted-average remaining lease term (years)	10.3	11.0
Weighted-average discount rate	5.7%	5.7%

The following table presents a reconciliation of the undiscounted future minimum lease payments remaining under the Company's operating leases to the amounts reported as operating lease liabilities on the consolidated balance sheet as of December 31, 2024 (in thousands):

	As of December 31, 2024
Undiscounted future minimum lease payments:	
2025	$ 12,345
2026	12,885
2027	10,003
2028	7,940
2029	8,051
Thereafter	58,989
Total undiscounted future minimum lease payments	110,213
Amount representing imputed interest	(28,180)
Total operating lease liabilities	82,033
Current portion of operating lease liabilities	(7,902)
Operating lease liabilities, non-current	$ 74,131

The table above excludes future minimum lease payments for leases which were executed but had not yet commenced as of December 31, 2024, the total of which were not material.

7. Liability Related to Sale of Future Royalties

In December 2020, the Company entered into a royalty purchase agreement (the Royalty Purchase Agreement) with HCR. Under the Royalty Purchase Agreement, HCR purchased the Company's rights to a capped amount of Zolgensma royalty payments under the Company's license agreement (the Novartis License) with Novartis Gene Therapies, including $4.0 million of royalty payments received by the Company in the fourth quarter of 2020 (the Pledged Royalties). In consideration for these rights, HCR paid the Company $200.0 million (the Purchase Price), less $4.0 million representing the payment of the Pledged Royalties to HCR. Beginning upon the effective date of the Royalty Purchase Agreement, Zolgensma royalty payments, up to a specified threshold, shall be paid to HCR, net of upstream royalties payable by the Company to certain licensors in accordance with existing license agreements.

Pursuant to the Royalty Purchase Agreement, the total amount of royalty payments to be received by HCR was subject to an increasing cap (the Cap Amount) equal to (i) $260.0 million applicable for the period from the effective date of the Royalty Purchase Agreement through November 7, 2024 (the First Cap Amount), and (ii) $300.0 million applicable for the period from November 8, 2024 through the effective date of termination of the Novartis License (the Second Cap Amount). If, on or prior to the defined dates for each Cap Amount, the total amount of royalty payments received by HCR equals or exceeds the Cap Amount applicable to such date, the Royalty Purchase Agreement will automatically terminate and all rights to the Zolgensma royalty payments will revert back to the Company. The First Cap Amount was not achieved prior to November 7, 2024, therefore the Royalty Purchase Agreement will remain in effect until the achievement of the Second Cap Amount or the termination of the Novartis License, if earlier. The Company has no obligation to repay any amounts to HCR if total future Zolgensma royalty payments are not sufficient to achieve the applicable Cap Amount prior to the termination of the Novartis License.

The Company has a call option to repurchase its rights to the purchased royalties from HCR for a repurchase price equal to, as of the option exercise date, $300.0 million minus the total amount of royalty payments received by HCR.

The proceeds received from HCR of $196.0 million were recorded as a liability, net of transaction costs of $3.5 million, which is amortized over the estimated life of the arrangement using the effective interest method. In order to determine the amortization of the liability, the Company is required to estimate the total amount of future royalty payments to be received by HCR, subject to the Cap Amount, over the life of the arrangement. The total amount of royalty payments received by HCR under the Royalty Purchase Agreement, less the net proceeds received by the Company of $192.5 million, is recorded as interest expense over the life of the arrangement using the effective interest method. Due to its continuing involvement in the Novartis License, the Company continues to recognize royalty revenue on net sales of Zolgensma and records the royalty payments to HCR as a reduction of the liability when paid. As such payments are made to HCR, the balance of the liability will be effectively repaid over the life of the Royalty Purchase Agreement.

The Company estimates the effective interest rate used to record interest expense under the Royalty Purchase Agreement based on its estimate of future royalty payments to be received by HCR. At each reporting date, the Company reassesses its estimate of total future royalty payments to be received by HCR at the applicable Cap Amount, and prospectively adjusts the effective interest rate and amortization of the liability as necessary. Over the life of the arrangement, the actual effective interest rate will be affected by the amount and timing of the royalty payments received by HCR and changes in the Company's forecasted royalties. The estimated interest rate in effect as of December 31, 2024 and 2023 was 65.5% and 4.8%, respectively, which was based on the amortized balance of the liability and the estimated remaining royalties to be paid to HCR under the arrangement. This interest rate is subject to adjustments in the future based on actual royalties paid to HCR and changes in the royalty forecast. The interest rate increased during the year ended December 31, 2024 as a result of changes in the Company's forecasted royalties for territories outside the United States. As of December 31, 2024, the estimated effective interest rate over the life of the Royalty Purchase Agreement, taking into account actual royalties paid to date and the estimated remaining royalties to be paid under the arrangement, was 16.0%.

The following table presents the changes in the liability related to the sale of future royalties under the Royalty Purchase Agreement with HCR (in thousands):

	Liability Related to Sale of Future Royalties
Balance at December 31, 2022	$ 137,606
Zolgensma royalties paid to HCR	(49,574)
Interest expense recognized	6,020
Balance at December 31, 2023	94,052
Zolgensma royalties paid to HCR	(46,413)
Interest expense recognized	12,048
Balance at December 31, 2024	59,687
Current portion of liability related to sale of future royalties	(34,309)
Liability related to sale of future royalties, non-current	$ 25,378

8. Commitments and Contingencies

In-licensed Technology

The Company in-licenses intellectual property from third parties for technology and know-how used in its product candidates and development programs, some of which is further sublicensed to NAV Technology Licensees and collaboration partners. In-licenses may require the Company to make future payments relating to sublicense fees, milestone fees and royalties on future sales of licensed products. Additionally, the Company may be responsible for the cost of the maintenance of the intellectual property as incurred by its licensors. Up-front fees to obtain licensed technology, as well as associated milestone fees, are recorded as research and development expenses if the technology has no alternative future use. Sublicense fees are based on a specified percentage of license fees earned by the Company as a result of sublicensing the technology to third parties and are recorded as cost of revenues. Royalties due to licensors on sales of licensed products, including sales by NAV Technology Licensees, are recorded as cost of revenues. Patent maintenance costs are recorded as general and administrative expenses.

Please refer to Note 10 for information on licenses granted by the Company and collaboration agreements with third parties.

The Trustees of the University of Pennsylvania

In February 2009, the Company entered into a license agreement, which has been amended from time to time (as amended, the Penn License), with The Trustees of the University of Pennsylvania (together with the University of Pennsylvania, Penn) for exclusive, worldwide rights to certain patents owned by Penn underlying the Company's NAV Technology Platform, as well as exclusive rights to certain data, results and other information. Pursuant to the originally agreed upon Penn License, the Company was obligated to pay Penn royalties on net sales of licensed products and sublicense fees. Additionally, the Company was obligated to reimburse Penn for certain costs incurred related to the maintenance of the licensed patents.

In April 2019, the Penn License was amended to include exclusive license rights to certain patent rights and know-how, including research data and other information, relating to the treatment of late-infantile neuronal ceroid lipofuscinosis type 2 (CLN2) disease. In consideration for the additional licensed rights, and in addition to any consideration owed under the license prior to the amendment, the Company paid Penn an up-front fee and is obligated to pay milestone fees of up to $20.5 million upon the achievement of various development and sales-based milestones and additional royalties on net sales of licensed products for the

treatment of CLN2 disease. From the inception of the agreement through December 31, 2024, the Company had incurred $0.5 million for development milestones achieved, or deemed probable of achievement, under the Penn License.

In March 2022, the Company and Penn entered into a letter agreement (the Penn Letter Agreement) pursuant to which the Company will pay to Penn a total of $20.0 million, consisting of (i) $8.0 million paid in April 2022 to satisfy payment of any sublicense fees due or owed in the future under the Penn License as a result of the Company's collaboration and license agreement with AbbVie Global Enterprises Ltd., and (ii) $12.0 million to satisfy any other past or future obligations of the Company to pay sublicense fees under the Penn License, which is payable in four equal annual installments of $3.0 million beginning in March 2023. The Penn Letter Agreement amended the Penn License to remove the Company's obligations to pay sublicense fees under the license agreement. The Company remains obligated to pay Penn royalties on net sales of licensed products, milestone fees and reimbursement of certain patent maintenance costs in accordance with the Penn License.

The Company recognized a charge of $9.2 million as cost of revenues upon the execution of Penn Letter Agreement in March 2022, which consisted of $17.3 million representing the present value of the $20.0 million payable under the Penn Letter Agreement, less $8.1 million in sublicense fees previously recognized as expense by the Company in prior periods and accrued as liabilities prior to the effectiveness of the Penn Letter Agreement. The present value discount is accreted as interest expense over the contractual payment period using the effective interest method.

Expenses incurred by the Company related to the Penn License were recorded as follows (in thousands):

	Years Ended December 31,		
	2024	2023	2022
Cost of revenues	$ —	$ —	$ 9,242
Research and development	—	500	—
General and administrative	299	935	715
Interest expense	612	842	778
	$ 911	$ 2,277	$ 10,735

As of December 31, 2024, the Company had recorded $5.8 million payable under the Penn License, net of present value discount, of which $2.9 million was included in accrued expenses and other current liabilities, and $2.9 million was included in other liabilities on the consolidated balance sheet. As of December 31, 2023, the Company had recorded $8.2 million payable under the Penn License, net of present value discount, of which $2.6 million was included in accrued expenses and other current liabilities, and $5.6 million was included in other liabilities on the consolidated balance sheet.

GlaxoSmithKline

In March 2009, the Company entered into a license agreement, which was amended in April 2009 (as amended, the GSK License), with GlaxoSmithKline LLC (GSK) for exclusive, worldwide rights to certain patents underlying the Company's NAV Technology Platform which are owned by Penn and exclusively licensed to GSK. Pursuant to the GSK License, the Company is obligated to pay GSK royalties on net sales of licensed products and sublicense fees. Additionally, the Company is obligated to reimburse GSK for certain costs incurred related to the maintenance of the licensed patents. The Company was also obligated to pay $1.5 million to GSK upon the achievement of various milestones, all of which have been achieved and paid.

In connection with the execution of the Penn Letter Agreement in March 2022, the Company's royalty obligations under the GSK License were assigned by GSK to Penn. Beginning upon the effective date of the Penn Letter Agreement in March 2022, any royalties payable by the Company under the GSK License shall be paid to Penn rather than GSK. The Company remains obligated to pay GSK sublicense fees and reimbursement of certain patent maintenance costs in accordance with the GSK License.

Expenses incurred by the Company related to the GSK License were recorded as follows (in thousands):

	Years Ended December 31,		
	2024	2023	2022
Cost of revenues:			
Royalties on net sales of Zolgensma	$ 33,180	$ 37,043	$ 44,691
Other cost of revenues	337	170	612
Total cost of revenues	33,517	37,213	45,303
General and administrative	369	476	964
	$ 33,886	$ 37,689	$ 46,267

As of December 31, 2024, the Company had recorded $6.3 million payable under the GSK License, of which $6.2 million was included in accrued expenses and other current liabilities, and $0.1 million was included in other liabilities on the consolidated balance sheet. As of December 31, 2023, the Company had recorded $12.3 million payable under the GSK License, of which $12.2 million was included in accrued expenses and other current liabilities, and $0.1 million was included in other liabilities on the consolidated balance sheet.

The Company has been notified of a potential dispute with GSK over the amount of sublicense fees paid by the Company to GSK under the GSK License. GSK claims there has been a significant underpayment by the Company as they are entitled to a sublicense payment on all amounts received by the Company from sublicensees, including royalties, and not just amounts received for GSK's sublicensed patents. The Company disagrees with GSK's interpretation of the GSK License. The Company does not believe that a loss is probable, and no reasonable range of loss is estimable, related to this matter. No liabilities related to this matter have been recorded as of December 31, 2024.

Clearside Biomedical

In August 2019, the Company entered into an option and license agreement with Clearside Biomedical, Inc. (Clearside) pursuant to which the Company was granted an option to exclusively license the worldwide rights to certain patents related to Clearside's proprietary, in-office SCS Microinjector™ for the delivery of ABBV-RGX-314 to the suprachoroidal space to treat wet age-related macular degeneration (wet AMD), diabetic retinopathy (DR) and other diseases. The Company exercised its license option in October 2019, resulting in a payment of $1.6 million to Clearside payable under the license agreement. Additionally, the Company is obligated to pay milestone fees of up to $136.0 million upon the achievement of various development and sales-based milestones, as well as royalties on net sales of licensed products using the SCS Microinjector. Clearside is responsible for supplying the SCS Microinjector to the Company to support all preclinical, clinical and commercial needs. From the inception of the agreement through December 31, 2024, the Company had incurred $3.0 million for development milestones achieved, or deemed probable of achievement, under the agreement.

Other Licenses

In November 2014, the Company entered into a license agreement, which has been amended from time to time, with Regents of the University of Minnesota (Minnesota), for an exclusive license to Minnesota's interest in certain patent rights which are co-owned by Minnesota and the Company to commercialize products covered by the licensed patent rights in any country or territory in which a licensed patent has been issued and is unexpired, or a licensed patent application is pending. Pursuant to the license agreement, the Company is obligated to pay Minnesota annual maintenance fees, royalties on net sales, sublicense fees and fees upon the achievement of various milestones. Additionally, the Company is obligated to pay for certain costs incurred related to the maintenance of the licensed patents.

In August 2018, the Company entered into a license agreement with Emory University (Emory) for an exclusive license to Emory's interest in certain patent rights which are co-owned by Emory and the Company to commercialize products covered by the licensed patent rights in any country or territory. Pursuant to the license agreement, the Company is obligated to reimburse Emory for patent prosecution and maintenance expenses and pay Emory annual maintenance fees under certain circumstances, royalties on net sales, sublicense fees and fees upon the achievement of various milestones for the first licensed product.

In June 2022, the Company entered into a license agreement with Johns Hopkins University (JHU) for an exclusive license to JHU's interest in certain patent rights which are co-owned by JHU and the Company to commercialize products covered by the licensed patent rights in any country or territory. Pursuant to the license agreement, the Company paid JHU an upfront fee and is obligated to pay JHU royalties on net sales, minimum annual royalties, sublicense fees and fees upon the achievement of various milestones for the first two licensed products. Additionally, the Company is obligated to pay for certain costs incurred related to the maintenance of the licensed patents.

Other Funding Commitments

In the normal course of business, the Company enters into agreements with contract research organizations, contract manufacturing organizations and other third parties for services to be provided to the Company. Generally, these agreements provide for termination upon notice, with specified amounts due upon termination based on the timing of termination and the terms of the agreement. The actual amounts and timing of payments under these agreements are uncertain and contingent upon the initiation and completion of services to be provided to the Company.

Guarantees and Indemnifications

In the normal course of business, the Company enters into agreements that contain a variety of representations and provide for general indemnification. The Company's potential exposure under these agreements is unknown because it involves claims that may be made against the Company in the future. To date, the Company has not paid any claims or been required to defend any action related to its indemnification obligations. As of December 31, 2024 and 2023, the Company did not have any material indemnification claims that were probable or reasonably possible and consequently had not recorded any related liabilities.

Settlement Agreement

In the fourth quarter of 2022, the Company entered into a settlement agreement with a third party pursuant to which the Company released certain claims regarding infringement of the Company's intellectual property. In consideration for the release of claims made by the Company, the third party paid $7.5 million to the Company which was recorded as other operating income in the consolidated statements of operations and comprehensive loss for the year ended December 31, 2022.

9. Capitalization

As of December 31, 2024 and 2023, the authorized capital stock of the Company included 100,000,000 shares of common stock, par value $0.0001 per share, and 10,000,000 shares of preferred stock, par value $0.0001 per share. The Company's restated certificate of incorporation and bylaws contain the rights, preferences and privileges of the Company's stockholders and their respective shares.

Shares of common stock reserved for future issuance were as follows (in thousands):

	As of December 31,	
	2024	2023
Reserved for issuance under equity incentive plans	13,548	12,422
Reserved for issuance under employee stock purchase plan	913	1,018
Reserved for exercise of pre-funded warrants outstanding	1,325	—
	15,786	13,440

March 2024 Public Offering

In March 2024, the Company completed a public offering (March 2024 Offering) of 4,565,260 shares of its common stock at a price of $23.00 per share and 1,521,740 pre-funded warrants to purchase shares of its common stock at a price of $22.9999 per pre-funded warrant, which equaled the public offering price per share of the common stock less the $0.0001 exercise price of each pre-funded warrant. The aggregate net proceeds received by the Company from the offering were $131.1 million, net of underwriting discounts and commissions and offering expenses.

The rights and privileges of the pre-funded warrants issued under the March 2024 Offering are set forth in the warrant agreement between the Company and each of the respective warrant holders. The pre-funded warrants are exercisable at the option of the warrant holder at any time and do not expire. However, as set forth in the warrant agreements with each holder, the number of pre-funded warrants that may be exercised at any given time may be limited if, upon exercise, the warrant holder and any of its affiliates would beneficially own more than 9.99% of the Company's common stock, or have voting power of more than 9.99% of the Company's common stock. The limitation threshold may be increased or decreased by the warrant holder, with advance notice to the Company, to any other percentage not less than 4.99% nor in excess of 19.99%. Pre-funded warrants do not provide any of the rights or privileges provided by the Company's common stock, including any voting rights, until the pre-funded warrants are exercised and settled in underlying shares of common stock.

The Company evaluated the pre-funded warrants issued under the March 2024 Offering and concluded the warrants are indexed to the Company's common stock, meet the criteria to be classified as equity and are not subject to remeasurement. The proceeds received from the issuance of the pre-funded warrants were recorded as additional paid-in capital. The Company issued 197,000 shares of common stock upon the exercise of pre-funded warrants during the year ended December 31, 2024. As of December 31, 2024, 1,324,740 pre-funded warrants remained outstanding.

July 2023 Private Placement

In July 2023, the Company sold an aggregate of 257,466 shares of its common stock to Redmile Biopharma Investments III, L.P. (Redmile) at a purchase price of $19.42 per share, which was the closing price of the common stock on July 6, 2023 (the Private Placement). The Company received aggregate net proceeds from the Private Placement of $4.9 million, net of offering expenses. The Private Placement was conducted in reliance on the exemption from registration provided by Section 4(a)(2) of the Securities Act.

At-the-Market Offering Programs

In September 2023, the Company entered into an ATM Equity Offering℠ Sales Agreement with BofA Securities, Inc. (BofA) pursuant to which the Company may offer and sell shares of its common stock having an aggregate offering price of up to $150.0 million from time to time through BofA, acting as the Company's sales agent (the BofA ATM Program). The Company terminated the BofA ATM Program effective in November 2024. No shares of common stock were sold under the BofA ATM Program prior to its termination.

In December 2024, the Company entered into a Sales Agreement with Leerink Partners LLC (Leerink) pursuant to which the Company may offer and sell shares of its common stock having an aggregate offering price of up to $150.0 million from time to time through Leerink, acting as the Company's sales agent (the Leerink ATM Program). As of December 31, 2024, no shares of common stock had been sold under the Leerink ATM Program.

10. License and Collaboration Agreements

License and Royalty Revenue

As of December 31, 2024, the Company's NAV Technology Platform was being applied by NAV Technology Licensees in one commercial product, Zolgensma, and in the development of a number of other licensed products. Additionally, the Company has licensed intellectual property rights to collaborators for the joint development of certain product candidates. Consideration to the Company under its license agreements may include: (i) up-front and annual fees, (ii) milestone payments based on the achievement of certain development and sales-based milestones, (iii) sublicense fees, (iv) royalties on sales of licensed products, (v) fees for services related to the development of licensed products and (vi) other consideration payable upon optional goods and services purchased by licensees. Sublicense fees vary by license and range from a mid-single digit percentage to a low-double digit percentage of license fees received by licensees as a result of sublicenses. Royalties on net sales of commercialized products vary by license and range from a mid-single digit percentage to a low double-digit percentage of net sales by licensees.

License and royalty revenue consisted of the following (in thousands):

	Years Ended December 31,		
	2024	2023	2022
Zolgensma royalties	$ 81,505	$ 85,319	$ 101,919
Other license and royalty revenue	1,823	4,923	10,805
Total license and royalty revenue	$ 83,328	$ 90,242	$ 112,724

Outstanding development milestone payments are evaluated each reporting period and are only included in the transaction price of each license and recognized as license revenue to the extent the milestones are considered probable of achievement. Sales-based milestones are excluded from the transaction price of each license agreement and recognized as royalty revenue in the period of achievement. As of December 31, 2024, the Company's license agreements, excluding additional licenses that could be granted upon the exercise of options by licensees, contained unachieved milestones which could result in aggregate milestone payments to the Company of up to $1.51 billion, including (i) $524.9 million upon the commencement of various stages of clinical trials, (ii) $113.8 million upon the submission of regulatory approval filings or upon regulatory approval of licensed products, and (iii) $870.0 million upon the achievement of specified sales targets for licensed products, including milestones payable upon the first commercial sale of licensed products. To the extent the milestone payments are realized by the Company, the Company may be obligated to pay sublicense fees to licensors based on a specified percentage of the fees earned by the Company. The achievement of these milestones is highly dependent on the successful development and commercialization of licensed products and it is at least reasonably possible that some or all of the milestone fees will not be realized by the Company.

Changes in Accounts Receivable, Contract Assets and Deferred Revenue

The following table presents the balances of the Company's net accounts receivable, contract assets and deferred revenue, as well as other information regarding revenue recognized, during the periods presented (in thousands):

	Years Ended December 31,		
	2024	2023	2022
Accounts receivable, net, current and non-current:			
Beginning of period	$ 25,491	$ 29,586	$ 34,701
End of period	$ 20,947	$ 25,491	$ 29,586
Contract assets:			
Beginning of period	$ —	$ —	$ 1,074
End of period	$ 239	$ —	$ —
Deferred revenue:			
Beginning of period	$ 148	$ 1,829	$ 3,333
End of period	$ 115	$ 148	$ 1,829
Revenue recognized during the period from:			
Amounts included in deferred revenue at beginning of period	$ 148	$ 1,708	$ 3,333
Performance obligations satisfied in previous periods	$ 81,585	$ 87,152	$ 101,921

As of December 31, 2024, the Company had recorded deferred revenue of $0.1 million which represents consideration received or unconditionally due from licensees for performance obligations that have not yet been satisfied by the Company. Unsatisfied performance obligations as of December 31, 2024 consisted of development services to be performed by the Company related to licensed products, which will be satisfied as the services are performed. As of December 31, 2024, the aggregate transaction price of the Company's license agreements allocated to performance obligations not yet satisfied, or partially satisfied, was $1.1 million, which is expected to be satisfied over a period of approximately two years.

Revenue recognized from performance obligations satisfied in previous periods, as presented in the table above, was primarily attributable to Zolgensma royalties and changes in the transaction prices of the Company's license agreements. Changes in transaction prices were primarily attributable to development milestones achieved or deemed probable of achievement during the periods which were previously not considered probable of achievement, resulting in a cumulative catch-up adjustment to revenue. Revenue recognized during the years ended December 31, 2024, 2023 and 2022 included zero, $2.0 million and zero, respectively, in cumulative catch-up adjustments for changes in the probability of achievement of development milestones.

Accounts Receivable, Contract Assets and the Allowance for Credit Losses

Accounts receivable, net consisted of the following (in thousands):

	As of December 31,	
	2024	2023
Current accounts receivable:		
Billed to customers	$ 65	$ 265
Unbilled Zolgensma royalties	20,106	24,128
Due from Abeona, net of present value discount	—	4,587
Other unbilled	302	397
Allowance for credit losses	—	(4,587)
Current accounts receivable, net	20,473	24,790
Non-current accounts receivable:		
Other unbilled	474	701
Non-current accounts receivable, net	474	701
Total accounts receivable, net	$ 20,947	$ 25,491

The following table presents the changes in the allowance for credit losses related to accounts receivable and contract assets for the years ended December 31, 2024 and 2023 (in thousands):

	Allowance for Credit Losses	
	Accounts Receivable	Contract Assets
Balance at December 31, 2022	$ 4,152	$ —
Changes in present value discount of receivables	435	—
Balance at December 31, 2023	4,587	—
Changes in present value discount of receivables	413	—
Credit recoveries	(5,000)	—
Balance at December 31, 2024	$ —	$ —

The Company's allowance for credit losses during the years ended December 31, 2024, 2023 and 2022 was related solely to accounts receivable from Abeona Therapeutics Inc. (Abeona). Please refer to the section below, "Settlement Agreement with Abeona Therapeutics", for further information regarding amounts due from Abeona and the associated allowance for credit losses.

Zolgensma License with Novartis Gene Therapies

In March 2014, the Company entered into an exclusive license agreement (as amended, the Novartis License) with Novartis Gene Therapies. Under the Novartis License, the Company granted Novartis Gene Therapies an exclusive, worldwide commercial license, with rights to sublicense, to the NAV Technology Platform, as well as other certain rights, for the treatment of SMA in humans by *in vivo* gene therapy.

In consideration for the rights granted under the license, Novartis Gene Therapies paid the Company (i) an up-front fee of $2.0 million upon the execution of the agreement in 2014, (ii) license fees totaling $180.0 million upon the amendment of the agreement in January 2018 and the subsequent acquisition of AveXis, Inc. (now Novartis Gene Therapies) by Novartis in May 2018, (iii) total cumulative payments of $12.3 million upon the achievement of various development milestones, and (iv) a sales-based milestone payment of $80.0 million upon the achievement of $1.0 billion in cumulative net sales of Zolgensma in the third quarter of 2020. In addition to the consideration above, Novartis Gene Therapies is obligated to pay to the Company fixed annual fees, royalties on net sales of licensed products and a percentage of any sublicense fees received by Novartis Gene Therapies from sublicensees for the licensed intellectual property rights. Royalties are payable by Novartis Gene Therapies at a mid-single to low double-digit percentage of net sales of licensed products using the NAV AAV9 vector, and a low double-digit percentage of net sales of licensed products using a licensed vector other than NAV AAV9, and are subject to reduction in specified circumstances.

In 2019, Novartis Gene Therapies launched commercial sales of Zolgensma, a licensed product under the Novartis License. In accordance with the Novartis License, the Company receives royalties on net sales of Zolgensma. All development and sales-based milestones under the Novartis License have been achieved and there are no further milestone payments payable to the Company under the license agreement.

The Company recognized the following amounts under the Novartis License (in thousands):

	Years Ended December 31,		
	2024	2023	2022
Zolgensma royalties	$ 81,505	$ 85,319	$ 101,919
Other license revenue	28	—	31
Total license and royalty revenue	$ 81,533	$ 85,319	$ 101,950
Interest income from licensing	$ 123	$ 29	$ 92

As of December 31, 2024 and 2023, the Company had recorded total accounts receivable of $20.4 million and $24.3 million, respectively, from Novartis Gene Therapies under the Novartis License, which consisted primarily of Zolgensma royalties receivable. The Zolgensma royalties receivable recorded as of December 31, 2024 included $14.4 million expected to be paid to HCR in accordance with the Royalty Purchase Agreement discussed in Note 7.

Settlement Agreement with Abeona Therapeutics

In November 2021, the Company entered into a settlement agreement and mutual release with Abeona (the Settlement Agreement) related to claims associated with a license agreement between the parties which was terminated in May 2020. The Settlement Agreement resolved all arbitration and legal proceedings and mutually released each party from any and all claims under the terminated license agreement. Pursuant to the Settlement Agreement, Abeona paid the Company a total of $30.0 million as follows: (i) $20.0 million paid in November 2021, (ii) $5.0 million paid in November 2022, and (iii) $5.0 million paid in November 2024. As of December 31, 2024, all amounts due from Abeona under the Settlement Agreement had been paid in full and no further amounts were due to the Company from Abeona.

As of December 31, 2023, the Company had recorded accounts receivable of $4.6 million associated with the remaining amounts due from Abeona under the Settlement Agreement. The receivable of $4.6 million as of December 31, 2023 consisted of the $5.0 million payment due by November 2024, net of discount to present value. The Company assessed the credit risk of the receivable from Abeona as of December 31, 2023. Based on its evaluation of Abeona's credit profile and financial condition, and its expectations regarding Abeona's future cash flows and ability to satisfy the contractual obligations of the Settlement Agreement, the Company recorded an allowance for credit losses of $4.6 million as of December 31, 2023 related to the accounts receivable due from Abeona. Prior to collection, the present value discount of the Abeona receivable was accreted as interest income from licensing through the contractual due date using the effective interest method. The Company elected to record increases in the allowance for credit losses associated with the accretion of the present value discount as a reduction of the associated interest income, resulting in no interest income recognized during the periods related to the accretion of the present value discount. The Company collected the final payment of $5.0 million due from Abeona under the Settlement Agreement upon its contractual due date in November 2024. As a result of the full collection of the receivable, the Company recorded a credit recovery of $5.0 million during the year ended December 31, 2024 against the associated allowance for credit losses. No credit losses or recoveries were recorded on the Abeona receivable during the years ended December 31, 2023 and 2022.

Collaboration Agreements

AbbVie Collaboration and License Agreement

In September 2021, the Company entered into a collaboration and license agreement with AbbVie Global Enterprises Ltd. (AbbVie), a subsidiary of AbbVie Inc., to jointly develop and commercialize ABBV-RGX-314, the Company's product candidate for the treatment of wet AMD, DR and other chronic retinal diseases (the AbbVie Collaboration Agreement). The AbbVie Collaboration Agreement became effective in November 2021.

Pursuant to the AbbVie Collaboration Agreement, the Company granted AbbVie a co-exclusive license to develop and commercialize ABBV-RGX-314 in the United States and an exclusive license to develop and commercialize ABBV-RGX-314 outside the United States. The Company and AbbVie will collaborate to develop ABBV-RGX-314 in the United States, and AbbVie will be responsible for the development of ABBV-RGX-314 in specified markets outside the United States. Through December 31, 2022, the Company was responsible for the development expenses related to certain ongoing clinical trials of ABBV-RGX-314 and the parties shared the additional development expenses related to ABBV-RGX-314. Beginning on January 1, 2023, AbbVie became responsible for the majority of all ABBV-RGX-314 development expenses.

The Company will lead the manufacturing of ABBV-RGX-314 for clinical development and U.S. commercial supply, and AbbVie will lead the manufacturing of ABBV-RGX-314 for commercial supply outside the United States. Manufacturing expenses will be allocated between the parties in accordance with the terms of the AbbVie Collaboration Agreement and supply agreements determined in accordance with the agreement. If requested by AbbVie, the Company will manufacture up to a specified portion of ABBV-RGX-314 for commercial supply outside the United States at a price specified in the agreement. AbbVie will lead the commercialization of ABBV-RGX-314 globally, and the Company will participate in U.S. commercialization efforts as provided under a commercialization plan determined in accordance with the agreement. The Company and AbbVie will share equally in the net profits and net losses associated with the commercialization of ABBV-RGX-314 in the United States. Outside the United States, AbbVie will be responsible, at its sole cost, for the commercialization of ABBV-RGX-314.

In consideration for the rights granted under the AbbVie Collaboration Agreement, AbbVie paid the Company an up-front fee of $370.0 million upon the effective date of the agreement in November 2021 and is required to pay to the Company up to $1.38 billion upon the achievement of specified development and sales-based milestones, of which $562.5 million are based on development milestones and $820.0 million are sales-based milestones. AbbVie is also required to pay to the Company tiered royalties on net sales of ABBV-RGX-314 outside the United States at percentages in the mid-teens to low twenties, subject to specified offsets and reductions.

The AbbVie Collaboration Agreement contains provisions for termination, including termination for convenience by AbbVie. Contemporaneously with entering into the AbbVie Collaboration Agreement, the Company entered into a sublicense agreement with AbbVie (the AbbVie Sublicense Agreement) pursuant to which the Company granted AbbVie an exclusive sublicense to exploit licensed products in connection with the AbbVie Collaboration Agreement under specified patents licensed to the Company by Penn. The AbbVie Sublicense Agreement became effective contemporaneously with the AbbVie Collaboration Agreement in November 2021 and is coterminous with the AbbVie Collaboration Agreement.

The Company evaluated its various commitments under the AbbVie Collaboration Agreement and identified the following distinct units of account: (i) delivery of an intellectual property license for the rights to develop and commercialize ABBV-RGX-314 globally, (ii) development, manufacturing and commercialization activities for ABBV-RGX-314 in the United States, and (iii) manufacturing of commercial supply for sales of ABBV-RGX-314 outside the United States, if requested by AbbVie. In determining the distinct units of account, the Company concluded that the license granted to AbbVie to develop and commercialize ABBV-RGX-314 is distinct from the other goods and services promised under the agreement, as AbbVie can benefit from the license on a standalone basis and, based on the stage of development of ABBV-RGX-314, the underlying licensed products and know-how are not expected to be significantly modified as a result of other goods and services promised under the agreement.

For each of the distinct units of account identified under the AbbVie Collaboration Agreement, the Company determined whether the transactions should be accounted for as a contract with a customer within the scope of ASC 606 or as a collaborative arrangement within the scope of ASC 808. The Company concluded that the units of account for the delivery of the functional intellectual property license and the manufacturing of commercial supply for sales of ABBV-RGX-314 outside the United States should be accounted for as revenue under ASC 606, as AbbVie is deemed to be a customer for these transactions. The Company concluded that the unit of account for development, manufacturing and commercialization activities for ABBV-RGX-314 in the United States should be accounted for as a collaborative arrangement under ASC 808, as these represent joint operating activities for which the Company and AbbVie are both active participants and exposed to significant risks and rewards dependent on the commercial success of such activities.

The Company applied the requirements of ASC 606 to the AbbVie Collaboration Agreement for the units of account in which AbbVie was deemed to be a customer. The Company determined that there is only one material performance obligation under the agreement for the delivery of the intellectual property license to develop and commercialize ABBV-RGX-314 globally. The intellectual property licensed to AbbVie includes the rights to certain patents, data, know-how and other rights developed and owned by the Company, as well as other intellectual property rights exclusively licensed by the Company from various third parties. The Company evaluated options granted to AbbVie under the agreement and determined that the options do not represent material rights, and therefore are not considered separate performance obligations under the current contract. Specifically, the Company concluded that the option granted to AbbVie to purchase commercial supply of ABBV-RGX-314 from the Company for a portion of sales outside the United States does not convey a material right, as the option is not priced at an incremental discount to the standalone selling price of the underlying goods and services. Additionally, the Company identified various promises under the AbbVie Collaboration Agreement which were determined to be immaterial in the context of the contract and will not be accounted for as separate performance obligations.

As of December 31, 2024 and 2023, the transaction price of the AbbVie Collaboration Agreement was $370.0 million, which consisted solely of the up-front payment received from AbbVie in November 2021. The $370.0 million transaction price was fully recognized as revenue upon the delivery of the license to AbbVie in November 2021. Variable consideration under the AbbVie Collaboration Agreement, which has been excluded from the transaction price, includes $562.5 million in payments for development milestones that have not yet been achieved and were not considered probable of achievement. Additionally, the transaction price excludes sales-based milestone payments of $820.0 million and royalties on net sales of ABBV-RGX-314 outside the United States. Development milestones will be added to the transaction price and recognized as revenue upon achievement, or if deemed probable of achievement. In accordance with the sale- or usage-based royalty exception under ASC 606, royalties on net sales and sales-based milestones will be recognized as revenue in the period the underlying sales occur or milestones are achieved. There were no changes in the transaction price of the AbbVie Collaboration Agreement during the years ended December 31, 2024, 2023 and 2022.

The Company applied the requirements of ASC 808 to the AbbVie Collaboration Agreement for the units of account which were deemed to be a collaborative arrangement. Both the Company and AbbVie will perform various activities related to the development, manufacturing and commercialization of ABBV-RGX-314 in the United States. Development costs are shared between the parties in accordance with the terms of the AbbVie Collaboration Agreement, and the parties will share equally in the net profits and losses derived from sales of ABBV-RGX-314 in the United States. The Company accounts for payments to and from AbbVie for the sharing of development and commercialization costs in accordance with its accounting policy for collaborative arrangements. Amounts owed to AbbVie for the Company's share of development costs or commercialization costs incurred by AbbVie are recorded as research and development expense or general and administrative expense, respectively, in the period the costs are incurred.

Amounts owed to the Company for AbbVie's share of development costs or commercialization costs incurred by the Company are recorded as a reduction of research and development expense or general and administrative expense, respectively, in the period the costs are incurred. At the end of each reporting period, the Company records a net amount due to or from AbbVie as a result of the cost-sharing arrangement. As of December 31, 2024 and 2023, the Company had recorded $11.3 million and $17.7 million, respectively, due from AbbVie for net reimbursement of costs incurred for activities performed under AbbVie Collaboration Agreement, which was included in other current assets on the consolidated balance sheets.

The Company recognized the following amounts under the AbbVie Collaboration Agreement (in thousands):

	Years Ended December 31,		
	2024	2023	2022
Net cost reimbursement to (from) AbbVie included in:			
Research and development expense	$ (78,316)	$ (74,209)	$ (19,294)
General and administrative expense	1,866	768	1,531
Total net cost reimbursement to (from) AbbVie	$ (76,450)	$ (73,441)	$ (17,763)

Nippon Shinyaku Collaboration and License Agreement

In January 2025, the Company entered into a collaboration and license agreement with Nippon Shinyaku for the development and commercialization of RGX-121, the Company's product candidate for the treatment of MPS II, and RGX-111, the Company's product candidate for the treatment of MPS I (the Nippon Shinyaku Collaboration Agreement). The Nippon Shinyaku Collaboration Agreement became effective in March 2025.

Pursuant to the Nippon Shinyaku Collaboration Agreement, the Company granted Nippon Shinyaku a license to develop and exclusively commercialize RGX-121 and RGX-111 in the United States and certain countries in Asia. The Company is responsible for the development of RGX-121 and RGX-111 in the United States, and Nippon Shinyaku is responsible for development in licensed territories outside the United States. The Company is responsible for the manufacturing of RGX-121 and RGX-111 for clinical development and commercial supply, and manufacturing expenses will be allocated between the parties in accordance with the terms of the Nippon Shinyaku Collaboration Agreement and supply agreements determined in accordance with the agreement. Nippon Shinyaku will be responsible, at its sole cost, for the commercialization of RGX-121 and RGX-111 in the licensed territories. The Company reserves the right to develop and commercialize RGX-121 and RGX-111 in countries outside the licensed territories.

In consideration for the rights granted and services to be performed under the Nippon Shinyaku Collaboration Agreement, Nippon Shinyaku is required to pay an up-front fee of $110.0 million to the Company following the effective date of the agreement, and the Company is eligible to receive up to $700.0 million from Nippon Shinyaku upon the achievement of specified development and sales-based milestones, of which $40.0 million are based on development milestones and $660.0 million are sales-based milestones. The Company is also eligible to receive double-digit royalties on net sales of RGX-121 and RGX-111 by Nippon Shinyaku, subject to specified offsets and reductions. The Company retains all rights to, and any proceeds related to the sale of, any priority review vouchers that may be issued upon the potential approvals of RGX-121 and RGX-111.

11. Stock-based Compensation

In September 2014, the Board of Directors adopted the 2014 Stock Plan (the 2014 Plan). In June 2015, the Board of Directors adopted the 2015 Equity Incentive Plan (the 2015 Plan), which became effective upon the Company's initial public offering in September 2015. The 2015 Plan replaced the 2014 Plan, and as of the effective date of the 2015 Plan, no further awards may be issued under the 2014 Plan. Any options or awards outstanding under the 2014 Plan as of the effective date of the 2015 Plan remained outstanding and effective. The number of authorized shares under the 2015 Plan automatically increases annually on the first business day of each fiscal year, by the lesser of (i) 4% of the total number of shares of common stock outstanding on December 31 of the prior year, or (ii) a number of common shares determined by the Board of Directors. As of December 31, 2024, the total number of shares of common stock authorized for issuance under the 2015 Plan and the 2014 Plan was 19,118,989, of which 1,512,367 remained available for future grants under the 2015 Plan. An additional 1,981,975 shares were authorized for issuance under the 2015 Plan effective in January 2025.

The 2014 Plan and 2015 Plan provide for the issuance of stock options, stock appreciation rights, restricted and unrestricted stock and unit awards, and performance cash awards to employees, members of the Board of Directors and consultants of the Company. Since the inception of the plans, the Company has issued only stock options and restricted stock units under the plans. Stock options under the 2014 Plan and 2015 Plan generally expire 10 years following the date of grant. Options typically vest over a four-year period, but vesting provisions can vary by award based on the discretion of the Board of Directors. Certain stock option

awards granted by the Company may include performance conditions that must be achieved in order for vesting to occur. Stock options under the 2014 Plan and 2015 Plan have an exercise price at least equal to the estimated fair value of the Company's common stock on the date of grant. Restricted stock units typically vest over a four-year period, but vesting provisions can vary by award based on the discretion of the Board of Directors. Upon vesting, restricted stock units are settled in common stock of the Company.

Shares of common stock underlying awards previously issued under the 2014 Plan and 2015 Plan which are reacquired by the Company, withheld by the Company in payment of the purchase price, exercise price or withholding taxes, expired, cancelled due to forfeiture or otherwise terminated other than by exercise or settlement, are added to the number of shares of common stock available for issuance under the 2015 Plan. Shares available for issuance under the 2015 Plan may be either authorized but unissued shares of the Company's common stock or common stock reacquired by the Company and held in treasury. The 2015 Plan expires in June 2025, 10 years from the date it was adopted by the Board of Directors, unless earlier terminated.

Stock-based Compensation Expense

The Company's stock-based compensation expense by award type was as follows (in thousands):

	Years Ended December 31,		
	2024	2023	2022
Stock options	$ 23,518	$ 28,863	$ 34,444
Restricted stock units	14,455	10,748	5,569
Employee stock purchase plan	490	656	775
	$ 38,463	$ 40,267	$ 40,788

As of December 31, 2024, the Company had $59.7 million of unrecognized stock-based compensation expense related to stock options, restricted stock units and the 2015 Employee Stock Purchase Plan (the 2015 ESPP), which is expected to be recognized over a weighted-average period of 2.3 years.

The Company recorded aggregate stock-based compensation expense in the consolidated statements of operations and comprehensive loss as follows (in thousands):

	Years Ended December 31,		
	2024	2023	2022
Research and development	$ 17,985	$ 20,568	$ 21,368
General and administrative	20,478	19,699	19,420
	$ 38,463	$ 40,267	$ 40,788

Stock Options

The following table summarizes stock option activity under the 2014 Plan and 2015 Plan (in thousands, except per share data):

	Shares	Weighted-average Exercise Price	Weighted-average Remaining Contractual Life (Years)	Aggregate Intrinsic Value (a)
Outstanding at December 31, 2023	8,581	$ 32.62	6.2	$ 7,011
Granted	2,310	$ 16.11		
Exercised	(291)	$ 5.20		
Cancelled or forfeited	(606)	$ 34.73		
Outstanding at December 31, 2024	9,994	$ 29.48	5.9	$ 240
Exercisable at December 31, 2024	6,837	$ 34.20	4.8	$ 240
Vested and expected to vest at December 31, 2024	9,994	$ 29.48	5.9	$ 240

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

The weighted-average grant date fair value per share of options granted during the years ended December 31, 2024, 2023 and 2022 was $9.98, $13.66 and $19.43, respectively. During the years ended December 31, 2024, 2023 and 2022, the total number of stock options exercised was 290,753, 222,935 and 331,912, respectively, resulting in total proceeds of $1.5 million, $1.5 million and $2.8 million, respectively. The total intrinsic value of options exercised during the years ended December 31, 2024, 2023 and 2022 was $3.1 million, $2.9 million and $6.9 million, respectively.

The fair values of options granted were estimated at each grant date using the Black-Scholes valuation model with the following weighted-average assumptions:

	Years Ended December 31,		
	2024	2023	2022
Expected volatility	64%	65%	66%
Expected term (years)	6.0	6.0	6.0
Risk-free interest rate	4.0%	3.9%	1.7%
Expected dividend yield	0.0%	0.0%	0.0%

Restricted Stock Units

The following table summarizes restricted stock unit activity under the 2015 Plan (in thousands, except per share data):

	Shares		Weighted-average Grant Date Fair Value
Unvested balance at December 31, 2023	1,309	$	25.89
Granted	1,217	$	16.07
Vested	(395)	$	27.17
Forfeited	(89)	$	22.15
Unvested balance at December 31, 2024	2,042	$	19.95

The total intrinsic value of restricted stock units vested during the years ended December 31, 2024, 2023 and 2022 was $6.7 million, $3.9 million and $2.2 million, respectively.

Employee Stock Purchase Plan

In June 2015, the Board of Directors adopted the 2015 ESPP, which became effective upon the Company's initial public offering in September 2015. The number of authorized shares reserved for issuance under the 2015 ESPP automatically increases on the first business day of each fiscal year by the lesser of (i) 1% of the shares of common stock outstanding on the last business day of the prior fiscal year; or (ii) the number of shares determined by the Board of Directors. Unless otherwise determined by the administrator of the 2015 ESPP, two offering periods of six months' duration will begin each year on January 1 and July 1. As of December 31, 2024, the total number of shares of common stock authorized for issuance under the 2015 ESPP was 1,426,994, of which 912,964 remained available for future issuance. During the years ended December 31, 2024, 2023 and 2022, 105,400, 103,388 and 75,733 shares of common stock, respectively, were issued under the 2015 ESPP.

12. Retirement Plan

The Company sponsors a defined-contribution retirement plan under Section 401(k) of the Internal Revenue Code (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. The Company matches employee deferrals up to a specified percentage of eligible compensation. For the years ended December 31, 2024, 2023 and 2022, the Company incurred expenses of $3.1 million, $3.3 million and $3.0 million, respectively, for matching contributions to the 401(k) Plan.

13. Income Taxes

The components of loss before income taxes were as follows (in thousands):

	Years Ended December 31,		
	2024	2023	2022
United States	$ (227,022)	$ (263,574)	$ (280,341)
Foreign	(80)	(72)	(64)
Total loss before income taxes	$ (227,102)	$ (263,646)	$ (280,405)

The components of the provision for income tax expense (benefit) were as follows (in thousands):

	Years Ended December 31,		
	2024	2023	2022
Current:			
Federal	$ —	$ —	$ 3
State	—	(152)	(87)
Foreign	—	—	—
Total current	—	(152)	(84)
Deferred:			
Federal	—	—	—
State	—	—	—
Foreign	—	—	—
Total deferred	—	—	—
Total income tax expense (benefit)	$ —	$ (152)	$ (84)

Beginning in 2022, the Tax Cuts and Jobs Act of 2017 (the TCJA) eliminated the option to deduct research and development expenses currently and requires taxpayers to amortize such costs over a period of five years for expenses incurred in the United States and a period of 15 years for expenses incurred outside the United States. This provision of the TCJA resulted in deferred tax assets of $122.1 million and $107.3 million as of December 31, 2024 and 2023, respectively, related to capitalized research and development expenses, net of amounts amortized to date. There was no material impact to the Company's current or deferred tax provision or operating cash flows during the years ended December 31, 2024, 2023 and 2022 as a result of this provision of the TCJA given the Company incurred net operating losses (NOLs) during the periods and has recorded a full valuation allowance against its deferred tax assets.

The Inflation Reduction Act (the IRA) was enacted in August 2022 and contains revenue-raising provisions to include a book-income alternative minimum tax and an excise tax on stock buybacks, among other provisions. Based on the thresholds established by the IRA and a review of the Company’s transactions, the enactments of the IRA did not have an impact on the Company’s income tax provision for the years ended December 31, 2024, 2023 and 2022.

The Organization for Economic Co-operation and Development (OECD) has introduced BEPS Pillar 2 rules that impose a global minimum tax rate of 15%. Numerous countries have enacted corresponding legislation that is effective beginning January 1, 2024. These rules generally apply to multinational companies with consolidated revenue of at least €750.0 million in at least two of the four preceding fiscal years. Based on the revenue thresholds established in the BEPS Pillar 2 rules, these changes do not have an impact on the Company’s income tax provision for the year ended December 31, 2024.

The following table presents a reconciliation of income tax expense (benefit) computed at the statutory federal income tax rate of 21% to income tax expense (benefit) reported in the consolidated statements of operations and comprehensive loss (in thousands):

	Years Ended December 31,		
	2024	2023	2022
Federal income tax benefit at statutory rate	$ (47,692)	$ (55,366)	$ (58,885)
State income tax benefit, net of federal tax effect	(10,715)	(14,330)	(22,743)
Research and development credits	(9,617)	(14,435)	(16,844)
Stock-based compensation	3,171	3,348	3,216
Executive compensation	2,633	2,894	1,839
Other non-deductible expenses and reconciling items	(162)	569	241
Change in corporate tax rates	3,503	4,860	(3,636)
Change in valuation allowance	58,879	72,308	96,728
Total income tax expense (benefit)	$ —	$ (152)	$ (84)

The significant components of the Company's net deferred tax assets were as follows (in thousands):

	As of December 31,	
	2024	2023
Deferred tax assets:		
Net operating loss carryforwards	$ 105,468	$ 61,929
Research and development tax credits	87,030	77,178
Stock-based compensation	22,687	22,405
Lease liabilities	22,278	24,671
Liability related sale of future royalties	12,148	22,482
Capitalized research and development costs	122,082	107,302
Accruals and other	5,366	7,785
Total deferred tax assets before valuation allowance	377,059	323,752
Valuation allowance	(338,352)	(280,455)
Total deferred tax assets	38,707	43,297
Deferred tax liabilities:		
Right-of-use assets	(14,293)	(16,523)
Depreciation and amortization	(24,414)	(26,774)
Total deferred tax liabilities	(38,707)	(43,297)
Net deferred tax assets	$ —	$ —

The Company evaluated the positive and negative evidence bearing upon the realizability of its deferred tax assets as of December 31, 2024 and 2023. Based on the Company's history of operating losses, and other relevant facts and circumstances, the Company concluded that it was more likely than not that the benefit of its deferred tax assets will not be realized. Accordingly, the Company provided a full valuation allowance for its net deferred tax assets as of December 31, 2024 and 2023. The valuation allowance increased by $57.9 million and $69.3 million during the years ended December 31, 2024 and 2023, respectively. The increases in the valuation allowance during the years ended December 31, 2024 and 2023 were due primarily to research and development expenses incurred during the periods which were capitalized for tax purposes, and federal and state NOLs and research and development tax credits generated during the periods.

The following table presents the Company's U.S. federal and state NOL and tax credit carryforwards, net of unrecognized tax benefits, which may be available to offset future income tax liabilities (in thousands):

	As of December 31, 2024	Expiration Date (if not utilized)
U.S. federal NOL carryforwards	$ 373,599	Indefinite
U.S. federal tax credit carryforwards	$ 86,473	Various dates between 2037 and 2044
U.S. state NOL carryforwards	$ 273,319	Indefinite
U.S. state NOL carryforwards	$ 135,591	Various dates between 2029 and 2044
U.S. state tax credit carryforwards	$ 750	Various dates between 2029 and 2031

As of December 31, 2024, the Company had U.S. federal and state research and development tax credit carryforwards of approximately $87.2 million, net of unrecognized tax benefits of $0.1 million, which may be available to reduce future income tax liabilities. The calculation of these credits requires assumptions to be made by the Company to estimate qualified research expenses.

The Company conducts formal studies to document the qualified activities and expenses used to calculate these credits, however a portion of these credits may be subject to future examinations which have not yet occurred, the results of which may result in an adjustment to the Company's credit carryforwards. The Company accounts for uncertain tax positions in accordance with the requirements of ASC 740, and recognizes the tax benefit of tax positions to the extent that the benefit will more likely than not be realized. As of December 31, 2024 and 2023, the Company had total unrecognized tax benefits of $0.1 million and $0.1 million, respectively, which were reserved against its research and development tax credit carryforwards as uncertain tax positions. Further, a full valuation allowance has been provided against the net credit carryforwards and, if an adjustment is required upon the completion of the study, this adjustment would be offset by an adjustment to the deferred tax asset established for the research and development credit carryforwards and the valuation allowance. If these unrecognized tax benefits were to be recognized, the impact would be offset by an adjustment to the valuation allowance, resulting in no impact on the Company's effective tax rate. The Company expects that all of its unrecognized tax benefits as of December 31, 2024 will reverse in the next 12 months due to the expiration of the associated statute of limitations.

Under the provisions of the Internal Revenue Code, the Company's NOL and tax credit carryforwards are subject to review and possible adjustment by the Internal Revenue Service and state tax authorities. NOL and tax credit carryforwards may be subject to an annual limitation in the event of certain cumulative changes in the ownership interest of significant stockholders over a three-year period in excess of 50%, as defined under Sections 382 and 383 of the Internal Revenue Code, respectively, as well as similar state provisions. This could limit the amount of tax attributes that can be utilized annually to offset future taxable income or tax liabilities. The amount of the annual limitation is determined based on the value of the Company immediately prior to the ownership change. Subsequent ownership changes may further affect the limitation in future years. The Company has completed several financings since its inception which may have resulted in a change in control as defined by Sections 382 and 383 of the Internal Revenue Code, or could result in a change in control in the future.

The Company and its subsidiaries file income tax returns in the United States, at the federal level and in various states, and in foreign jurisdictions. The U.S. federal and state income tax returns are generally subject to tax examinations for the tax years ended December 31, 2020 onward. To the extent the Company has tax attribute carryforwards, the tax years in which the attribute was generated may still be adjusted upon examination by the Internal Revenue Service or state tax authorities to the extent utilized in a future period.

14. Restructuring

In November 2023, the Company implemented a strategic pipeline prioritization and corporate restructuring designed to reduce operating expenses and prioritize the development of ABBV-RGX-314, RGX-202 for the treatment of Duchenne muscular dystrophy, and RGX-121, while pursuing strategic alternatives for the Company's other clinical stage programs. The restructuring included a reduction in workforce and other planned operating expenses, primarily in rare neurodegenerative disease development, early research and other general and administrative areas.

In connection with the restructuring, the Company implemented a reduction in workforce of approximately 15%, which was substantially completed in the fourth quarter of 2023. The Company recorded restructuring costs of $3.7 million during the year ended December 31, 2023, of which $3.0 million is included in research and development expense and $0.7 million is included in general administrative expense in the consolidated statements of operations and comprehensive loss. Restructuring costs primarily consisted of employee severance, continuing healthcare benefits and other employee-related costs. Restructuring costs associated with one-time termination benefits were recorded pursuant to ASC 420, while restructuring costs associated with ongoing benefit arrangements were recorded pursuant to ASC 712. During the year ended December 31, 2024, the Company recorded reductions in the restructuring liability of $0.4 million associated with changes in total estimated restructuring costs. As of December 31, 2024, all of the restructuring costs had been paid by the Company and no restructuring liability was recorded.

The following table presents the changes in the Company's restructuring liability (in thousands):

	Restructuring Liability
Balance at December 31, 2022	$ —
Restructuring charges	3,731
Cash payments	(1,925)
Balance at December 31, 2023	1,806
Cash payments	(1,370)
Other adjustments	(436)
Balance at December 31, 2024	$ —

15. Related Party Transactions

From 2016 until June 2022, the Company was a party to professional services agreements with FOXKISER LLP (FOXKISER), an affiliate of certain stockholders of the Company and an affiliate of a member of the Company's Board of Directors, pursuant to which the Company paid a fixed monthly fee in consideration for certain strategic services provided by FOXKISER. The agreement with FOXKISER was terminated effective June 2022. Expenses incurred under the agreement with FOXKISER for the year ended December 31, 2022 were $2.4 million and were recorded as research and development expenses in the consolidated statements of operations and comprehensive loss. No expenses under the agreement with FOXKISER were incurred during the years ended December 31, 2024 and 2023.

16. Net Loss Per Share

Since the Company incurred net losses for the years ended December 31, 2024, 2023 and 2022, common stock equivalents were excluded from the calculation of diluted net loss per share for such periods as their effect would be anti-dilutive. Accordingly, basic and diluted net loss per share were the same for such periods. The weighted-average number of common shares outstanding used in the basic and diluted net loss per share calculations includes the weighted-average effect of pre-funded warrants to purchase shares of the Company's common stock, as the pre-funded warrants are exercisable at any time for nominal cash consideration. The following potentially dilutive common stock equivalents outstanding at the end of the period were excluded from the computations of weighted-average diluted common shares for the periods indicated as their effects would be anti-dilutive (in thousands):

	Years Ended December 31,		
	2024	2023	2022
Stock options issued and outstanding	9,994	8,581	7,668
Unvested restricted stock units outstanding	2,042	1,309	613
Employee stock purchase plan	47	27	30
	12,083	9,917	8,311

17. Segment and Geographical Information

Operating segments are identified as components of an enterprise about which separate discrete financial information is available for evaluation by the chief operating decision maker (CODM), or decision-making group, in making decisions on how to allocate resources and assess performance. The Company's CODM, its Chief Executive Officer, views the Company's operations and manages the business as one operating segment focused on the development and commercialization of gene therapies to treat an array of diseases. The determination of a single operating segment is consistent with the consolidated financial information regularly provided to the CODM.

The CODM reviews and evaluates consolidated net income (loss) for purposes of assessing performance, making operating decisions and allocating resources. The CODM uses net income (loss) to assess performance versus operating budgets and in the preparation of near-term and long-range operating plans to inform decisions on resource and capital allocation. The CODM reviews consolidated cash, cash equivalents and marketable securities as a measure of segment assets. As of December 31, 2024 and 2023, the Company's cash, cash equivalents and marketable securities were $244.9 million and $314.1 million, respectively.

The following table presents information about the Company's segment revenues, significant segment expenses regularly provided to the CODM, other segment items and consolidated net income (loss) (in thousands):

	Years Ended December 31,		
	2024	2023	2022
Revenues	$ 83,328	$ 90,242	$ 112,724
Less:			
Cost of revenues	33,567	37,213	54,545
Research and development expense			
Personnel	65,950	73,356	67,466
Direct development and support (a)	97,951	110,337	131,397
Facilities	11,410	11,653	10,284
Stock-based compensation	17,985	20,568	21,368
Depreciation and amortization	15,226	16,352	11,938
Total research and development expense	208,522	232,266	242,453
General and administrative expense			
Personnel	20,816	22,304	21,270
Other general and administrative (b)	29,480	41,609	40,340
Facilities	4,856	3,914	3,280
Stock-based compensation	20,478	19,699	19,420
Depreciation and amortization	989	968	971
Total general and administrative expense	76,619	88,494	85,281
Other segment items (c)	8,278	4,237	(10,766)
Net loss	$ (227,102)	$ (263,494)	$ (280,321)

(a)Direct development and support includes external goods and services for the development of product candidates and early-stage research activities, laboratory costs, consulting, development cost reimbursement to and from collaborators and other expenses in support of research and development activities.

(b)Other general and administrative expenses include professional and administrative services, consulting, commercial cost reimbursement to and from collaborators and other corporate overhead expenses.

(c)Other segment items include credit losses (recoveries), impairment of long-lived assets, other operating expenses (income), interest income from licensing, investment income, interest expense and income tax benefit.

The Company’s interest income during the years ended December 31, 2024, 2023 and 2022 included interest income from licensing as presented in the consolidated statements of operations and comprehensive loss, as well as interest income from investments of $12.1 million, $9.1 million and $5.5 million, respectively, which is included within investment income in the consolidated statements of operations and comprehensive loss.

The Company’s revenues consist of license and royalty revenue. For the year ended December 31, 2024, 36%, 10% and 10% of the Company’s revenues were attributed to the United States, Germany and United Arab Emirates, respectively, and no other countries accounted for 10% or more of the Company’s revenues. For the year ended December 31, 2023, 32% of the Company’s revenues were attributed to the United States and no other countries accounted for 10% or more of the Company’s revenues. For the year ended December 31, 2022, 35% and 10% of the Company’s revenues were attributed to the United States and Germany, respectively, and no other countries accounted for 10% or more of the Company’s revenues. The country of origin for license revenue is determined based on the country of domicile of the licensee. The country of origin for royalty revenue is determined based on the location of the underlying net sales of licensed products.

The substantial majority of the Company’s assets reside in the United States.

18. Supplemental Disclosures

Other Current Assets

Other current assets consisted of the following (in thousands):

	As of December 31,	
	2024	2023
Net cost reimbursement due from collaborators	$ 11,304	$ 17,745
Accrued interest on investments	1,094	1,551
Other	1,376	1,107
	$ 13,774	$ 20,403

Accrued Expenses and Other Current Liabilities

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

	As of December 31,	
	2024	2023
Accrued personnel costs	$ 17,607	$ 18,146
Accrued external research and development expenses	8,998	13,762
Accrued sublicense fees and royalties	8,658	14,234
Accrued external general and administrative expenses	2,002	2,717
Accrued purchases of property and equipment	156	386
Other accrued expenses and current liabilities	649	458
	$ 38,070	$ 49,703

Supplemental Disclosures of Non-cash Investing and Financing Activities

Purchases of property and equipment included in accounts payable and accrued expenses and other current liabilities as of December 31, 2024 were $0.3 million, a net decrease of $0.1 million from December 31, 2023. Purchases of property and equipment included in accounts payable and accrued expenses and other current liabilities as of December 31, 2023 were $0.4 million, a net decrease of $2.1 million from December 31, 2022. Purchases of property and equipment included in accounts payable and accrued expenses and other current liabilities as of December 31, 2022 were $2.5 million, a net decrease of $7.5 million from December 31, 2021.

Offering expenses for at-the-market offering programs included in accounts payable and accrued expenses and other liabilities as of December 31, 2024 and 2023 were $0.2 million and less than $0.1 million, respectively. No such amounts were recorded as of December 31, 2022. During the year ended December 31, 2024, the Company derecognized $0.6 million in deferred offering costs upon the termination of the BofA ATM Program in November 2024.

EXHIBIT INDEX

Exhibit Number	Description	Incorporated by Reference			Filed or Furnished Herewith
		Form	Exhibit Number	Filing Date	
1.1	Sales Agreement dated December 9, 2024 between the Registrant and Leerink Partners LLC	8-K	1.1	12/9/24	
3.1	Restated Certificate of Incorporation	8-K	3.1	6/7/21	
3.2	Amended and Restated Bylaws	8-K	3.2	9/22/15	
4.1	Specimen stock certificate evidencing the shares of common stock	S-1	4.1	8/17/15	
4.2	Description of Securities				X
4.3	Form of Pre-funded Warrant	8-K	4.1	3/11/24	
10.1	Form of Indemnity Agreement for directors and officers	S-1	10.1	8/17/15	
10.2*	2014 Stock Plan, as amended	S-1	10.2	8/17/15	
10.3*	2015 Equity Incentive Plan	S-1/A	10.3	9/15/15	
10.4*	Form of Restricted Stock Unit Award Agreement for the 2015 Equity Incentive Plan	10-K	10.4	3/1/21	
10.5*	Form of Stock Option Award Agreement for the 2015 Equity Incentive Plan	10-K	10.5	3/1/21	
10.6*	2015 Employee Stock Purchase Plan	S-1/A	10.4	9/8/15	
10.7*	Employment Agreement effective as of July 1, 2024 between the Registrant and Curran Simpson	10-Q	10.1	8/1/24	
10.8*	Form of Employment Agreement for Executive Vice Presidents	10-K	10.9	2/27/24	
10.9*	Consulting and Employment Separation Agreement effective as of July 1, 2024 between the Registration and Kenneth T. Mills	10-Q	10.2	8/1/24	
10.10*	Employment Separation Agreement effective as of September 21, 2024 between the Registrant and Vittal Vasista	10-Q	10.1	11/6/24	
10.11*	Consulting Agreement effective as of September 21, 2024 between the Registrant and Vittal Vasista	10-Q	10.2	11/6/24	
10.12*	Compensation Program for Non-Employee Directors	10-Q	10.1	8/3/22	
10.13*	Management Cash Incentive Plan	S-1	10.29	8/17/15	
10.14†	License Agreement effective February 24, 2009 between the Registrant and The Trustees of the University of Pennsylvania	S-1/A	10.9	9/15/15	
10.15†	First Amendment to License Agreement dated March 6, 2009 between the Registrant and The Trustees of the University of Pennsylvania	S-1	10.10	8/17/15	
10.16†	Second Amendment to License Agreement effective September 9, 2014 between the Registrant and The Trustees of the University of Pennsylvania	S-1/A	10.11	9/15/15	
10.17†	Third Amendment to License Agreement effective April 29, 2016 between the Registrant and The Trustees of the University of Pennsylvania	10-Q/A	10.36	12/23/16	
10.18†	Fourth Amendment to License Agreement effective April 4, 2019 between the Registrant and The Trustees of the University of Pennsylvania	10-Q	10.2	5/7/19	

Exhibit Number	Description	Incorporated by Reference			Filed or Furnished Herewith
		Form	Exhibit Number	Filing Date	
10.19†	Fifth Amendment to License Agreement effective September 11, 2020 between the Registrant and The Trustees of the University of Pennsylvania	10-Q	10.2	11/4/20	
10.20†	Letter Agreement dated March 21, 2022 between the Company and the Trustees of the University of Pennsylvania	10-Q	10.1	5/4/22	
10.21†	License Agreement dated March 6, 2009 between the Registrant and SmithKline Beecham Corporation d/b/a GlaxoSmithKline	S-1/A	10.12	9/15/15	
10.22	Amendment to License Agreement dated April 15, 2009 between the Registrant and SmithKline Beecham Corporation d/b/a GlaxoSmithKline	S-1	10.13	8/17/15	
10.23†	License Agreement dated March 21, 2014 between the Registrant and AveXis, Inc.	S-1/A	10.16	9/15/15	
10.24†	First Amendment to License Agreement dated January 8, 2018 between the Registrant and AveXis, Inc.	10-K	10.24	3/6/18	
10.25†	Collaboration and License Agreement dated September 10, 2021 between the Registrant and AbbVie Global Enterprises Ltd.	10-Q	10.1	11/2/21	
10.26	Lease dated March 6, 2015 between the Registrant and BMR-Medical Center Drive LLC	S-1	10.26	8/17/15	
10.27	First Amendment to Lease dated September 30, 2015 between the Registrant and BMR-Medical Center Drive LLC	10-K	10.31	3/3/16	
10.28	Second Amendment to Lease dated November 23, 2015 between the Registrant and BMR-Medical Center Drive LLC	10-K	10.32	3/3/16	
10.29	Third Amendment to Lease dated July 21, 2017 between the Registrant and BMR-Medical Center Drive LLC	10-Q	10.1	8/8/17	
10.30	Fourth Amendment to Lease dated April 20, 2018 between the Registrant and BMR-Medical Center Drive LLC	10-Q	10.1	5/8/18	
10.31	Fifth Amendment to Lease dated October 30, 2020 between the Registrant and ARE-Maryland No. 45, LLC, as successor in interest to BMR-Medical Center Drive LLC	10-Q	10.3	11/4/20	
10.32	Lease dated November 1, 2018 between the Registrant and ARE-Maryland No. 24, LLC	10-Q	10.1	11/7/18	
10.33	Letter Agreement to Lease dated April 12, 2019 between the Registrant and ARE-Maryland No. 24, LLC	10-Q	10.3	5/7/19	
10.34	First Amendment to Lease dated April 23, 2019 between the Registrant and ARE-Maryland No. 24, LLC	10-Q	10.4	5/7/19	

Exhibit Number	Description	Incorporated by Reference			Filed or Furnished Herewith
		Form	Exhibit Number	Filing Date	
10.35	Second Amendment to Lease dated November 4, 2019 between the Registrant and ARE-Maryland No. 24, LLC	10-Q	10.1	11/5/19	
10.36	Third Amendment to Lease dated October 30, 2020 between the Registrant and ARE-Maryland No. 24, LLC	10-Q	10.4	11/4/20	
10.37†	Royalty Purchase Agreement dated December 22, 2020 between the Registrant and entities managed by Healthcare Royalty Management, LLC	10-K	10.42	3/1/21	
19.1	Insider Trading Policy				X
21.1	Subsidiaries of the Registrant				X
23.1	Consent of PricewaterhouseCoopers LLP, independent registered accounting firm				X
31.1	Certification of the Chief Executive Officer, as required by Section 302 of the Sarbanes-Oxley Act of 2002				X
31.2	Certification of the Chief Financial Officer as required by Section 302 of the Sarbanes-Oxley Act of 2002				X
32.1	Certifications of the Chief Executive Officer and Chief Financial Officer as required by 18 U.S.C. 1350				X
97.1	Compensation Clawback Policy	10-K	97.1	2/27/24	
101	The following materials from the Registrant's Annual Report on Form 10-K for the fiscal year ended December 31, 2024 formatted in Inline XBRL (eXtensible Business Reporting Language): (i) Consolidated Balance Sheets (ii) Consolidated Statements of Operations and Comprehensive Loss (iii) Consolidated Statements of Stockholders' Equity (iv) Consolidated Statements of Cash Flows (v) Notes to Consolidated Financial Statements				X
104	The cover page from the Registrant's Annual Report on Form 10-K for the fiscal year ended December 31, 2024 formatted in Inline XBRL (included in Exhibit 101)				

* Management contract or compensatory plan or arrangement.
† Portions of this exhibit have been omitted.

The certifications attached as Exhibit 32.1 that accompany this Annual Report on Form 10-K are not deemed filed with the SEC and are not to be incorporated by reference into any filing of REGENXBIO Inc. under the Securities Act or the Exchange Act, whether made before or after the date of this Annual Report on Form 10-K, irrespective of any general incorporation language contained in such filing.

SIGNATURES

Pursuant to the requirements of Section 13 and 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 on March 13, 2025.

REGENXBIO INC.

By: /s/ Curran Simpson
Curran Simpson
President and Chief Executive Officer

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/ Curran Simpson Curran Simpson	President, Chief Executive Officer and Director (Principal Executive Officer)	March 13, 2025
/s/ Mitchell Chan Mitchell Chan	Chief Financial Officer (Principal Financial and Accounting Officer)	March 13, 2025
/s/ Kenneth T. Mills Kenneth T. Mills	Chairman of the Board of Directors	March 13, 2025
/s/ Jean Bennett Jean Bennett	Director	March 13, 2025
/s/ Allan M. Fox Allan M. Fox	Director	March 13, 2025
/s/ Alexandra Glucksmann Alexandra Glucksmann	Director	March 13, 2025
/s/ A.N. “Jerry” Karabelas A.N. “Jerry” Karabelas	Director	March 13, 2025
/s/ George Migausky George Migausky	Director	March 13, 2025
/s/ David C. Stump David C. Stump	Director	March 13, 2025
/s/ Daniel Tassé Daniel Tassé	Director	March 13, 2025
/s/ Jennifer Zachary Jennifer Zachary	Director	March 13, 2025