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UNITED STATES
SECURITIES AND EXCHANGE COMMISSION

Washington, D.C. 20549

FORM 10-K

(Mark One)

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

For the fiscal year ended December 31, 2020

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)

9600 Blackwell Road, Suite 210
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. ☒

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, 2020, the last business day of the registrant’s most recently completed second quarter, was $977,613,237.

As of February 25, 2021, there were 42,498,483 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 2021 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, 2020, are incorporated by reference into Part III of this Annual Report on Form 10-K.

REGENXBIO INC.

Form 10-K

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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 "believe," "may," "will," "estimate," "continue," "anticipate," "assume," "design," "intend," "expect," "could," "plan," "potential," "predict," "seek," "should," "would" or by variations of such words or by similar expressions. We have based these forward-looking statements on our current expectations and assumptions and analyses made by us 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:

- the impact of the COVID-19 pandemic on our business, operations and preclinical and clinical development timelines and plans;
- the ability to obtain and maintain regulatory approval of our product candidates and the labeling for any approved products;
- the timing of enrollment, commencement and completion and the success of our 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 ability to establish and maintain development partnerships;
- our expectations regarding our expenses and revenue;
- our expectations regarding the outcome of legal proceedings, including our arbitration with Abeona Therapeutics Inc. regarding license fees that have not been paid to us and our ability to recover such unpaid fees;
- our expectations regarding regulatory developments in the United States and foreign countries; 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 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 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.

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 gene therapy product candidates 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 gene therapy product candidates 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 vectors, including AAV7, AAV8, AAV9, AAVrh10 and more than 100 other novel AAV vectors (NAV Vectors). We believe this platform forms a strong foundation for our current programs and with our ongoing research and development, we expect to continue to expand the platform.

We have developed a broad pipeline of gene therapy programs using our NAV Technology Platform to address genetic diseases through two modalities: AAV-mediated antibody delivery and monogenic gene replacement. The AAV-mediated antibody delivery modality is designed to treat serious and chronic diseases by delivering the genes necessary for the sustained production of therapeutic antibodies *in vivo*. Our monogenic gene replacement approach builds upon the well-understood mechanism of replacing a dysfunctional or missing gene with a functional copy of the gene in order to enable sustained production of necessary proteins.

Gene Therapy Using NAV Vectors for AAV-Mediated Antibody Delivery

Our product candidate RGX-314 consists of the NAV AAV8 vector designed to deliver a gene encoding a therapeutic antibody fragment which inhibits vascular endothelial growth factor (VEGF). RGX-314 is being developed as a novel, single-administration gene therapy for the treatment of wet age-related macular degeneration (wet AMD), diabetic retinopathy (DR), and other additional chronic retinal conditions which cause total or partial vision loss for millions of patients in the United States, Europe and Japan. We are advancing two separate routes of administration of RGX-314 to the eye, through a standardized subretinal delivery procedure as well as by delivery to the suprachoroidal space using the SCS Microinjector™ licensed from Clearside Biomedical, Inc.

In January 2021, we announced that the pivotal program for RGX-314 using the subretinal delivery approach is active. The pivotal program is expected to support a Biologics Licensing Application (BLA) filing in 2024. We plan to conduct two randomized, well-controlled clinical trials to evaluate the efficacy and safety of RGX-314 in patients with wet AMD, enrolling approximately 700 patients total. In September 2020, we announced that the first patient had been dosed in the Phase II trial of the suprachoroidal delivery of RGX-314 using the SCS Microinjector for the treatment of wet AMD. In addition, we announced in December 2020 that the first patient had been dosed in the Phase II trial of the suprachoroidal delivery of RGX-314 for the treatment of DR.

We are developing a gene therapy candidate for the treatment of Hereditary Angioedema (HAE), which consists of the NAV AAV8 vector designed to deliver a gene encoding a therapeutic antibody against plasma kallikrein. Plasma kallikrein is a key protein of the plasma contact pathway which is left unregulated in patients with HAE. We expect to provide a program update in 2021.

We have also established a research program in partnership with Neurimmune AG, a leading clinical-stage Swiss biotechnology company, to discover and develop novel gene therapies using NAV Vectors to deliver antibodies against targets implicated in chronic neurodegenerative diseases, including tauopathies and alpha-synucleinopathies. We expect to provide a program update in 2021.

Gene therapy using NAV Vectors for Monogenic Gene Replacement

We are developing gene therapy product candidates using a monogenic gene replacement approach, in which we use a NAV Vector designed to deliver a functional copy of a gene in order to enable sustained production of necessary proteins.

In January 2021, we announced the development of RGX-202, a potential one-time gene therapy for the treatment of Duchenne Muscular Dystrophy (DMD), which is designed to use the NAV AAV8 vector to deliver a novel microdystrophin transgene which includes an extended coding region of the C-Terminal (CT) domain found in naturally occurring dystrophin, as well as other fundamental improvements. We expect to submit an investigational new drug application (IND) to the U.S. Food and Drug Administration (FDA) in mid-2021.

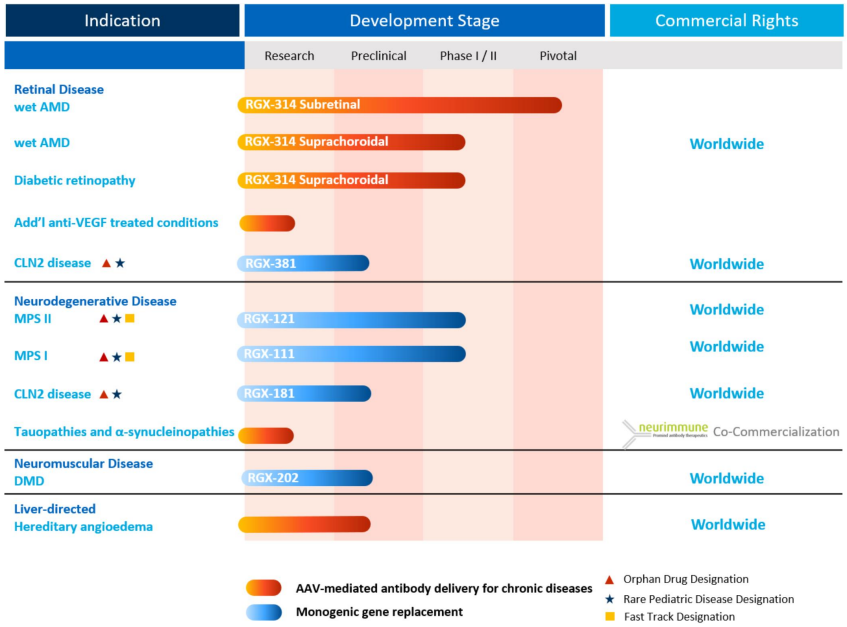
We have several NAV AAV9-based gene therapy programs that are designed to address the unmet neurological symptoms of severe genetic lysosomal storage diseases. These rare diseases include Mucopolysaccharidosis Type II (MPS II), Mucopolysaccharidosis Type I (MPS I) and late infantile neuronal ceroid lipofuscinosis type II (CLN2 disease). MPS II is caused by

deficiency of the *IDS* gene, which encodes the iduronate-2-sulfatase (I2S) enzyme; MPS I is caused by deficiency of the *IDUA* gene, which encodes the α-l-iduronidase (IDUA) enzyme; and CLN2 disease is caused by deficiency of the *TPP1* gene, which encodes the tripeptidyl peptidase 1 (TPP1) enzyme. Each of these enzymes are responsible for breakdown of cellular waste products; accumulation of waste products can ultimately result in cell, tissue, and organ dysfunction, and patients with severe forms of these diseases exhibit significant cognitive decline. Our product candidates for these diseases are:

- RGX-121 for the treatment of MPS II. A Phase I/II trial of RGX-121 in patients under the age of 5 years old is ongoing. In September 2020, we announced the expansion of the RGX-121 program into a second Phase I/II trial of RGX-121 in pediatric patients over the age of 5 years old.
- RGX-111 for the treatment of MPS I. We have initiated a Phase I/II clinical trial of RGX-111, and in December 2020, announced that the first patient had been dosed in the trial.
- RGX-181 for the treatment of CLN2 disease. We plan to submit an IND for the central nervous system (CNS) delivery of RGX-181 in the first quarter of 2021, and initiate enrollment in a Phase I/II trial in the first half of 2021.
- RGX-381 for the ocular manifestations of CLN2 disease. We expect to submit an IND, or foreign equivalent, for the subretinal delivery of RGX-381 in the first half of 2021.

In addition to the lead product candidates described above, we have also funded, and plan to continue to fund, preclinical research on potential product candidates that may become part of our internal product development pipeline. We will continue to seek partnerships with innovative academic institutions and biotechnology companies to develop novel NAV gene therapy product candidates.

Our internal pipeline is shown below.



We are an industry leader in AAV production and manufacturing, with deep in-house knowledge of vector characterization and strength in technical operations. We have robust suspension cell culture-based production capabilities, with well-integrated process optimization to enable scale and quality of product alongside our network of leading contract manufacturing organizations (CMOs). We are constructing a new current Good Manufacturing Practice (cGMP) production facility, to be located in our new corporate headquarters in Rockville, Maryland. This facility is expected to support future clinical and commercial production of gene therapies, allowing for production of NAV Technology-based vectors at scales up to 2,000 liters and is designed to meet regulatory requirements for clinical and commercial material supply in the jurisdictions in which we expect to develop and commercialize our product candidates. The cGMP production facility is expected to be operational starting in the first half of 2022, and will complement our current external manufacturing capabilities, enabling a reliable supply of NAV Vectors from both internal and external sources.

In addition to our internal product development efforts, we also selectively sublicense our NAV Vectors to other biotechnology companies with disease-specific expertise, which we refer to as NAV Technology Licensees. As of December 31, 2020, our NAV Technology Platform was being applied in the preclinical and clinical development of more than 20 partnered product candidates by our NAV Technology Licensees. Our partnering strategy provides us the flexibility to sublicense development of treatments designed to address significant unmet medical needs, while remaining focused on our core programs and therapeutic areas internally. We believe that the broad applicability of our NAV Technology Platform and any clinical successes of the treatments utilizing NAV Vectors will create new internal and partnered pipeline opportunities. In May 2019, the FDA approved the first gene therapy that leverages our proprietary NAV Technology Platform, Novartis AG's Zolgensma® (onasemnogene abeparvovec-xioi; AVXS-101). Zolgensma was approved by the FDA as a one-time infusion for pediatric patients with spinal muscular atrophy (SMA) who are less than two years of age.

Our partnered product development program pipeline is shown below.

	Research		Preclinical		Phase I / II		Phase III / Approved	
	Indication	Licensee	Indication	Licensee	Indication	Licensee	Indication	Licensee
Liver / hematologic	Undisclosed	ultragenyx			Hemophilia A	Takeda		
					Hemophilia A	ultragenyx / BAYER		
					OTC Deficiency	ultragenyx		
					GSDIa	ultragenyx		
					Wilson Disease	ultragenyx		
Central nervous system	CDKL5 Deficiency	ultragenyx	Rett Syndrome	NOVARTIS	SMA Type II / III	NOVARTIS	SMA Type I*	zolgensma NOVARTIS
	Undisclosed	Prevail	ALS SOD1	NOVARTIS	Parkinson's w/ GBA & Neuronopathic Gaucher	Prevail	MPS IIIA	LYSOGENE SANOFI GENZYME
	TLE	CORLIEVE	Friedreich's ataxia	Pfizer	MPS IIIA	ESTEVE		
			FTD-GRN	Prevail				
			Synucleinopathies (GBA + α-Syn RNAi)	Prevail				
Cardiac / skeletal muscle					Danon Disease	rocket pharma	XLMTM	astellas
					Pompe Disease	astellas		

Our management team includes leaders who are experienced in building and operating innovative healthcare ventures and have expert knowledge in the development of AAV gene therapy, as well as in the disease areas we seek to address. We believe the strength of our team positions us to succeed in developing and bringing to market, independently or with our development partners, unique, best-in-class gene therapy treatments for a range of severe diseases with significant unmet medical needs.

Our Strategy

Our mission is to improve lives through the curative potential of gene therapy. We are seeking to develop, manufacture, commercialize and license product candidates across multiple therapeutic areas and target organs while continuing to expand our NAV Technology Platform. To achieve our mission, we are pursuing the following strategies:

- **Apply our proprietary, next generation AAV vector technology to develop *in vivo* gene therapies for patients.** We believe *in vivo* gene therapy is an ideal treatment paradigm to address the underlying biological cause of a disease. We believe our NAV Technology Platform is a significant advancement over earlier generations of AAV vectors in delivering genes to cells, with significant differentiating attributes, namely: higher expression and increased durability, broad and novel tissue selectivity, and improved manufacturability. These unique benefits have been demonstrated in our own clinical studies and animal models using our NAV Vectors, as well as third-party clinical trials and animal models using our NAV Vectors. The regulatory approval and commercial success of Novartis' Zolgensma, which uses the NAV AAV9 vector, further validates the potential of the NAV Technology Platform. We believe that our NAV Technology Platform, which underpins our internal development programs and the programs of our NAV Technology Licensees, will continue to enable us and our partners to develop best-in-class gene therapy candidates for a wide range of disease targets.
- **Rapidly advance our broad pipeline gene therapy programs using two modalities: AAV-mediated antibody delivery and monogenic gene replacement.** The AAV-mediated antibody delivery modality is designed to treat serious and chronic diseases which often involve frequent administration of therapeutic antibodies over the course of a patient's lifetime, by delivering the genes necessary to result in the sustained production of therapeutic antibodies *in vivo*. A single-administration gene therapy approach using NAV Vectors may provide improved treatment options for patients by reducing their treatment burden or enabling treatments in tissues where it is difficult to deliver sufficient amounts of therapeutic antibodies via traditional delivery methods. Our lead program, RGX-314, is based on this approach, delivering the gene encoding an antibody fragment that binds to VEGF. Similarly, we are developing AAV-mediated antibody delivery gene therapies for the treatment of HAE as well as neurodegenerative diseases such as tauopathies and alpha-synucleinopathies.

  Many genetic diseases are caused by a mutated or missing gene in specific cells which can affect the ability of the cell to correctly produce a specific protein. Our monogenic gene replacement approach builds upon the well-understood mechanism of using AAV vectors to replace dysfunctional or missing genes with a functional copy of the gene in order to enable sustained production of necessary proteins. We are using this approach to develop potential gene therapies for rare diseases such as DMD, MPS II, MPS I, and CLN2 disease each of which requires restoration of particular proteins.
- **Leverage advanced routes of administration to direct gene therapy treatments to specific tissues for efficient transduction and effective protein use at lower titers.** We believe that targeting tissues where diseases manifest is critical to impacting the course of the disease with our NAV gene therapy treatments. For example, we are currently evaluating two routes of administration for RGX-314 to effectively reach the retinal cells to produce the anti-VEGF antibody in the back of the eye without immune response. The subretinal delivery technique is an established route of delivery for gene therapy, with direct and broad transduction of the retina and minimal exposure to the vitreous and anterior segment of the eye. We have also licensed certain exclusive rights to the SCS Microinjector from Clearside Biomedical to deliver gene therapies to the suprachoroidal space, potentially providing a targeted, in-office, non-surgical approach to deliver NAV gene therapy treatments to the retina. Separately, we are seeking to address the CNS-specific manifestations of MPS II, MPS I and CLN2 disease by using advanced routes of administration for the delivery of our gene therapies.
- **Further establish REGENXBIO as an industry leader in gene therapy manufacturing, with significant advancements in internal capabilities and innovative developments.** We have deep in-house knowledge of AAV production and manufacturing, which provides us with the ability to scale production of our gene therapies while ensuring quality for patients. We maintain a strong network of well-known CMOs, and we are constructing our own production facility at our future headquarters in Rockville, Maryland. This facility, which we expect to be operational starting in the first half of 2022, will provide the capability for cGMP production at bioreactor scales up to 2,000 liters. Additionally, we have invested in innovative process development and analytical capabilities, and use an established robust suspension cell culture-based manufacturing process.

- **Strengthen the validation of our NAV Technology Platform through strategic in-licensing and sublicensing of new programs and the progress made by our external NAV Technology Platform Licensees.** Our NAV Technology Platform is currently being applied to more than 20 partnered product candidates in development across a broad range of therapeutic areas, including the FDA-approved Zolgensma. We believe that these programs further validate the versatility of NAV Vectors, and provide additional data that collectively drive the advancement of the AAV gene therapy space. This strategic sublicensing allows us to maintain our internal product development focus in our core disease indications and therapeutic areas while expanding the NAV gene therapy pipeline, developing a greater breadth of treatments for patients, providing additional technological and potential clinical proofs-of-concept for our NAV Technology Platform, and creating potential additional revenue.
- **Maintain and grow our extensive intellectual property portfolio.** We plan to leverage our intellectual property rights and substantial expertise in AAV gene therapy in order to develop and commercialize NAV gene therapy products. We have licensed exclusive rights to a broad portfolio of certain fundamental AAV gene therapy patents and patent applications, including more than 100 patents and patent applications worldwide covering our NAV Vectors, as well as sequences that are at least 95% identical to NAV capsid sequences. We also have composition of matter claims for AAV7, AAV8, AAV9 and AAVrh10, as well as methods for their manufacture and therapeutic uses. In securing these rights, we have focused on obtaining robust rights for the intellectual property that we believe will be most important in providing us with a competitive advantage with respect to AAV gene therapy treatments. We plan to continue to seek to protect and enhance the proprietary technology, inventions, and improvements that are commercially important to the development of our business.

The Broad Potential and Application of Gene Therapy

The concept of developing human therapies involving the delivery of external genes has existed for decades, driven by the arrival of recombinant technology and the early demonstrations by scientists of the ability to deliver and drive expression of external gene sequences in mammalian cells.

We believe that gene therapy has the potential to become an important class of treatment because it may offer the following benefits:

- **Ability to treat a broad range of diseases.** Given the availability of the sequence of the entire human genome, it could be possible to design gene therapy to express or effect expression of many human proteins whose presence, absence or activity causes disease. We believe gene therapy treatments can also be designed to enable the body to continuously produce therapeutic proteins or antibodies or be efficiently adapted to deliver different genome editing components to address the specific treatment needs of many disease targets.
- **Ability to target mechanisms that cannot be targeted effectively by existing drug classes.** Many proteins that play roles in disease cannot be targeted effectively with small molecules and therapeutic proteins. These limitations on small molecule and protein drugs may not apply to gene therapy, which we believe can be designed to target any gene in the genome.
- **Ability to create convenient treatment profiles.** Because gene therapies are designed to deliver a long-term effect with a single administration, a single gene delivered via gene therapy could potentially do the same work as administering conventional drugs over the course of many years.
- **Simplified discovery of treatment candidates.** Identification of small molecule and protein drug candidates typically requires screening a large number of potential candidates to find prospective leads. Identification of gene therapy candidates has the potential to be simpler and take considerably less time because it can involve relatively standard processes that can be applied in a similar fashion to many successive product candidates.
- **Delivery of new treatment modalities.** In addition to monogenic gene replacement and antibody delivery approaches, viral vectors may be utilized to deliver new and novel approaches to modifying a cell’s DNA, including gene editing constructs and RNA-based therapeutics.

Our Internal Pipeline

Gene Therapy Using NAV Vectors for AAV-Mediated Antibody Delivery

RGX-314 for the Treatment of Wet AMD, DR and Other Anti-VEGF Treated Conditions

We are developing RGX-314 for the treatment of wet AMD, DR and other anti-VEGF treated conditions. These diseases are characterized by loss of vision due to excess fluid accumulation from new blood vessel formation.

Wet AMD is a leading cause of total and partial vision loss, 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 the diagnosis rate will continue to increase with the growth of the 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 the leading cause of vision loss in the working-age population and affects approximately 8 million people in the United States. DR is a complication of diabetes and is a progressive retinopathy, the severity of which ranges from mild non-proliferative diabetic retinopathy to a more advanced proliferative diabetic retinopathy (PDR). The main causes of vision loss secondary to DR are the vision-threatening complications of PDR, marked by the growth of new abnormal blood vessels onto the surface of the retina and vitreous cavity causing severe vision loss and diabetic macular edema (DME) leading to visual impairment. DME can occur at any stage of DR as the blood vessels in the retina become increasingly fragile and leak fluid.

Anti-VEGF injection therapies are the standard of care in wet AMD and DR due to their ability to reduce fluid accumulation and, on average, improve or stabilize vision in the majority of patients. However, these therapies require repetitive and inconvenient intraocular injections, typically ranging from every four to twelve weeks in frequency, to maintain efficacy. Patients often experience vision loss with reduced frequency of treatment. In addition, patient compliance is a significant concern with anti-VEGF injection therapies due to a variety of factors, including inconvenience and discomfort associated with frequent injections in the eye.

RGX-314 is being developed as a novel, one-time treatment that consists of the NAV AAV8 vector encoding a gene for a monoclonal antibody fragment. The expressed protein is designed to neutralize VEGF activity, modifying the pathway for formation of new leaky blood vessels and retinal fluid accumulation. After delivery of RGX-314, we believe retinal cells will continue to produce the anti-VEGF protein.

We are currently evaluating two routes of administration for RGX-314 to effectively reach the retinal cells to produce the anti-VEGF antibody in the back of the eye. The subretinal delivery technique is an established route of delivery for gene therapy, with direct and broad transduction of the retina and minimal exposure to the vitreous and anterior segment of the eye. We have also licensed rights to the SCS Microinjector from Clearside Biomedical to deliver gene therapies to the suprachoroidal space. This targeted, in-office, non-surgical approach to delivery can potentially allow for widespread transgene expression in the retina without exposing the vitreous and the anterior segment of the eye.

Clinical Development of RGX-314 for the Treatment of Wet AMD

In January 2021, we announced that we completed an End of Phase 2 meeting with the U.S. Food and Drug Administration (FDA) to discuss the details of a pivotal program to support a BLA. We plan to conduct two randomized, well-controlled clinical trials to evaluate the efficacy and safety of RGX-314 in patients with wet AMD, enrolling approximately 700 patients total. We expect to submit a BLA based on these trials in 2024.

The first planned pivotal trial, ATMOSPHERE™, is active and patient enrollment is on-going. ATMOSPHERE is a multi-center, randomized, active-controlled trial to evaluate the efficacy and safety of a single-administration of RGX-314 versus standard of care in patients with wet AMD. The trial is designed to enroll approximately 300 patients at a 1:1:1 ratio across two RGX-314 dose arms ($6.4x10^{10}$ genome copies (GC)/eye and $1.3x10^{11}$ GC/eye delivered subretinally) and an active control arm of monthly intravitreal injections of ranibizumab (0.5 mg/eye). The primary endpoint of the trial is non-inferiority to ranibizumab based on change from baseline in Best Corrected Visual Acuity (BCVA) at 54 weeks. Secondary endpoints of the trial include safety and tolerability, change in central retinal thickness (CRT) and need for supplemental anti-VEGF injections.

The second planned pivotal trial is expected to be similar in design to ATMOSPHERE, with two RGX-314 dose arms versus an active control arm of monthly intravitreal aflibercept and the planned primary endpoint is non-inferiority to aflibercept based on the change from baseline in BCVA at one year. We plan to initiate this trial in the second half of 2021.

We initiated the pivotal program using cGMP material produced from our existing manufacturing process and plan to incorporate our scalable suspension cell culture manufacturing process to support future commercialization, upon completion of a bridging study and the pivotal trials. The bridging study is expected to initiate in the first half of 2021.

In February 2021, we announced additional positive data from the patients enrolled in the ongoing Phase I/II trial of RGX-314 for the treatment of wet AMD and its Long-Term Follow-Up study. As of January 22, 2021, RGX-314 continued to be generally well-tolerated across all dose cohorts. Durable treatment effect was observed in patients in Cohorts 4 and 5 at 1.5 years after administration of RGX-314, including stable visual acuity, decreased retinal thickness, and reductions in anti-VEGF injection burden. Long-term,

durable treatment effect was demonstrated in Cohort 3 over three years, including mean improvement in vision and stable retinal thickness, and reductions in anti-VEGF treatment burden.

In 2020, we also initiated a Phase II trial of RGX-314 for the treatment of wet AMD delivered suprachoroidally using the SCS Microinjector, a targeted, in-office route of administration. This trial, AAVIATE™, is a multi-center, open-label, randomized, active-controlled, dose-escalation trial that will evaluate the efficacy, safety and tolerability of suprachoroidal delivery of RGX-314. The trial is expected to enroll approximately 40 patients with severe wet AMD across two cohorts. Patients in each cohort will be randomized to receive RGX-314 versus monthly 0.5 mg ranibizumab intravitreal injection at a 3:1 ratio, and two dose levels of RGX-314 will be evaluated: $2.5x10^{11}$ GC/eye and $5x10^{11}$ GC/eye. The primary endpoint of the trial is mean change in vision in patients dosed with RGX-314, as measured by BCVA, at Week 40 from baseline, compared to patients receiving monthly injections of ranibizumab. Other endpoints include mean change in CRT and number of anti-VEGF intravitreal injections received following administration of RGX-314. We have completed enrollment of patients in Cohort 1 of the AAVIATE trial, and plan to report interim data from the first cohort in the third quarter of 2021. Enrollment of patients in Cohort 2 has begun and is expected to be complete in the second quarter of 2021.

Clinical Development of RGX-314 for the Treatment of DR

In December 2020, we announced the first patient dosed in ALTITUDE™, a Phase II multi-center, open label, randomized, controlled dose-escalation trial that will evaluate the efficacy, safety and tolerability of suprachoroidal delivery of RGX-314 in patients with DR. The trial is expected to enroll approximately 40 patients with DR across two cohorts. Patients will be randomized to receive RGX-314 versus observational control at a 3:1 ratio, and two dose levels of RGX-314 will be evaluated: $2.5x10^{11}$ GC/eye and $5.0x10^{11}$ GC/eye. The primary endpoint of the trial is the proportion of patients that improve in DR severity based on the Early Treatment Diabetic Retinopathy Study-Diabetic Retinopathy Severity Scale (ETDRS-DRSS) at 48 weeks. Other endpoints include safety and development of DR-related ocular complications. We expect to report initial data from this trial in 2021.

AAV-Mediated Antibody Delivery Research Program for the Treatment of HAE

We have expanded our AAV-mediated antibody gene therapy pipeline to include novel treatments for HAE. HAE is a chronic and severe disease that results from C1-inhibitor deficiency. HAE is characterized by recurring severe swelling (angioedema), most commonly in the face, airway, intestines and limbs. Antibodies to plasma kallikrein, a key protein left unregulated in patients with HAE, have been shown to reduce the swelling and pain associated with HAE. These antibodies, however, require frequent administration to reduce the occurrence of angioedema events.

Our HAE program is focused on developing a novel, one-time treatment utilizing the NAV AAV8 vector to deliver a gene encoding for a therapeutic antibody that targets and binds to plasma kallikrein. Following a single intravenous administration, the HAE product candidate is designed to allow liver cells to produce therapeutic antibodies that are secreted into the blood. In preclinical animal models, we have observed that NAV Vectors can express therapeutic antibodies that target and bind to plasma kallikrein.

Planned Clinical Development of Treatment of HAE

We plan to provide a program update in 2021.

AAV-Mediated Antibody Delivery Research Program for the Treatment of Neurodegenerative Diseases

We have established a research program in partnership with Neurimmune to discover and develop novel AAV gene therapies using NAV Vectors to deliver antibodies against targets implicated in chronic neurodegenerative diseases. Under the exclusive license, development and commercialization agreement, REGENXBIO and Neurimmune will jointly develop and commercialize novel therapies using AAV vectors to deliver human antibodies. We will focus on diseases associated with the accumulation and deposition of the microtubule-associated protein tau (tauopathies) and alpha-synuclein (alpha-synucleinopathies). Delivery of human antibodies using AAV vectors has the potential to provide sustained brain exposure of antibodies for the clearance of abnormal tau or alpha synuclein via a one-time CNS administration.

REGENXBIO and Neurimmune will be jointly responsible for the design and development of vectorized antibody therapies and will share associated development costs equally. Following an initial research phase, on a target-by-target basis, each party will have the option to continue as a co-development and co-commercialization partner in the collaboration or to elect to receive a phase-based worldwide royalty in lieu of continued development investment.

Planned Clinical Development of Treatment of Neurodegenerative Diseases

We expect to provide a program update in 2021.

Gene Therapy Using NAV Vectors for Monogenic Gene Replacement

RGX-202 for the Treatment of DMD

RGX-202 is our product candidate for the treatment of DMD, a rare genetic disorder caused by mutations in the gene responsible for making dystrophin, a protein involved in protecting 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.

DMD primarily affects males with approximately 1 in 3,500 to 1 in 5,000 boys affected worldwide. The absence of functional dystrophin protein in individuals with DMD results in cell damage during muscle contraction, leading to cell death, fibrosis, and inflammation in muscle tissues. Initial symptoms of DMD include muscle weakness that are often noticeable at an early age with diagnosis typically occurring by 5 years of age. Over time, individuals with DMD experience progressive muscle weakness and eventually lose the ability to walk. Respiratory and heart muscles are also affected, leading to difficulty breathing and the need for ventilator assistance, along with the development of cardiomyopathy.

There is presently no cure for DMD. Currently approved treatments either do not address the underlying cause of the disorder or are helpful only to a subset of patients with specific genetic mutations.

RGX-202 utilizes REGENXBIO's propriety NAV AAV8 vector that is designed to deliver a gene to muscle cells that encodes for a microdystrophin, a shortened and functional form of the dystrophin protein. The novel RGX-202 microdystrophin transgene includes coding regions that retain essential functional elements of naturally occurring dystrophin, including a unique CT domain for potential improved function. A well-characterized muscle-specific promoter (Spc5-12) is employed to direct expression of microdystrophin protein in skeletal and heart muscles. Additional RGX-202 features are designed to improve gene expression and reduce immunogenicity. Proof of concept data from preclinical studies of RGX-202 in the *mdx* mouse model of DMD has demonstrated broad and robust expression of microdystrophin in muscle, recruitment of key proteins to the muscle cells, improvements in muscle histology, as well as meaningful increases in muscle strength and function.

Planned Clinical Development of RGX-202

We expect to submit an IND for RGX-202 in mid-2021.

RGX-121 for the Treatment of MPS II

RGX-121 is our product candidate for the treatment of MPS II. MPS II, also known as Hunter syndrome, is a rare, X-linked recessive, or sex-linked, disease caused by a deficiency of the *IDS* gene which encodes the I2S enzyme. I2S is responsible for the breakdown of polysaccharides called heparan sulfate (HS) and dermatan sulfate (DS) in lysosomes, which are intracellular 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 CNS of MPS II patients, which has been shown to correlate with neurocognitive manifestations of the disorder. 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.

In 2006, the recombinant form of human I2S (Elaprase), an enzyme replacement therapy (ERT), was approved by the FDA for the treatment of MPS II and has subsequently been approved for use internationally. However, ERT does not treat CNS manifestations of MPS II since the enzyme cannot cross the blood-brain barrier. Specific treatment to address the neurological manifestations of MPS II and prevent or stabilize cognitive decline remains a significant unmet medical need. Overall, the limitations of ERT leave a significant unmet need for a method to safely achieve long-term enzyme reconstitution in the CNS for MPS II patients experiencing neurological complications.

RGX-121 is designed to use the 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 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 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 and fast track designation from the FDA for RGX-121.

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

A Phase I/II clinical trial of RGX-121 in patients with MPS II under the age of 5 years old is ongoing, to evaluate the safety and tolerability of RGX-121, as well as the effects of RGX-121 on biomarkers of I2S enzyme activity, neurocognitive development and other clinical measures. The Phase I/II trial is expected to enroll up to 15 patients across three dose levels: three patients in Cohort 1 at a dose level of $1.3x10^{10}$ genome copies per gram (GC/g) of brain mass, up to nine patients in Cohort 2 at a dose level of $6.5x10^{10}$ GC/g of brain mass, and three patients in Cohort 3 at a dose level of $2.0x10^{11}$ GC/g brain mass. All patients must have documented evidence of neurocognitive deficits due to MPS II or have a relative diagnosed with severe MPS II who has the same *IDS* mutation as the subject. The primary endpoint will be a safety assessment. The secondary and exploratory endpoints include the effect of RGX-121 on biomarkers of I2S activity in the cerebrospinal fluid (CSF), serum and urine, and effect of RGX-121 on neurocognitive deficits, as well as other clinical outcome measures.

As reported in February 2021, RGX-121 was well-tolerated in Cohorts 1 and 2 of the Phase I/II trial, and no drug-related SAEs were reported. Biomarker data from patients in both cohorts indicate encouraging signals of I2S enzyme activity in the CNS following one-time administration of RGX-121, with consistent reductions of HS and D2S6, a component of HS. Patients in Cohorts 1 and 2 also demonstrated continued neurocognitive development and evidence of I2S enzyme activity in plasma and urine following administration of RGX-121. We expect to continue to enroll patients in the Phase I/II trial and plan to report additional data in 2021. We expect to begin dosing patients in the third dose cohort in the first quarter of 2021.

In September 2020, we announced plans to initiate a second Phase I/II multicenter, open-label trial of RGX-121 for the treatment of pediatric patients with severe MPS II ages 5-18 years old. Up to six patients will be enrolled, and RGX-121 will be administered at a dose level of $6.5x10^{10}$ GC/g of brain mass. The trial is designed to evaluate the safety of a single administration of RGX-121, the effects of RGX-121 on biomarkers of I2S enzyme activity, and changes in cognitive function, adaptive behavior, daily function, and quality of life. We expect to begin dosing patients in this trial in the first half of 2021.

RGX-111 for the Treatment of MPS I

We are developing RGX-111 for the treatment of MPS I. MPS I is a rare autosomal recessive, or non-sex-linked, genetic 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 storage 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. Studies suggest that severe forms of MPS I represent between one-half and two-thirds of all MPS I patients.

The current standard of care for patients with an attenuated form of MPS I is a recombinant form of human IDUA (Aldurazyme). Given as a weekly intravenous infusion, this ERT 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.

The first disease modifying therapy developed for severe MPS I was bone marrow transplant (BMT). Though BMT 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 BMT is that cognitive decline continues for up to a year after

transplant before stabilizing, leaving permanent cognitive deficits. Overall, the limitations of BMT 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 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 for RGX-111.

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

In December 2020, we announced that the first patient was dosed in the Phase I/II clinical trial of RGX-111. The trial is a multi-center, open-label, dose escalation trial that will evaluate the safety, tolerability and pharmacodynamics of RGX-111 delivered to patients with MPS I via injection directly into the CSF. Up to five patients will be enrolled at two dose levels: $1.0x10^{10}$ GC/g of brain mass and $5.0x10^{10}$ GC/g of brain mass. The primary endpoint of this trial is safety and the secondary endpoints include the effect of RGX-111 on biomarkers of IDUA activity in the CSF, serum and urine, neurocognitive development and other outcome measures.

In addition, RGX-111 was administered to a patient with MPS I through an investigator-initiated study at CHOC Children's Hospital, following review and agreement by the FDA. The patient was dosed intracisternally with $1x10^{10}$ GC/g of brain mass of RGX-111.

RGX-181 and RGX-381 for the Treatment of CLN2 Disease

CLN2 disease is a form of Batten disease, a rare, pediatric-onset, autosomal recessive, neurodegenerative lysosomal storage disorder caused by mutations in the *TPP1* gene. Mutations in the *TPP1* gene and subsequent deficiency in TPP1 enzyme activity result in lysosomal accumulation of storage material and degeneration of tissues including the brain and retina. CLN2 disease is characterized by seizures, rapid deterioration of language and motor functions, cognitive decline, loss of vision and blindness, and premature death by mid-childhood. Onset of symptoms is generally between two to four years of age with initial features of recurrent seizures (epilepsy), language delay, and difficulty coordinating movements (ataxia).

CLN2 disease is estimated to occur in approximately 1 in 250,000 births worldwide. Based on global population, this equates to as many as 500 patients born each year worldwide.

There is currently no cure for CLN2 disease. Current treatment options include palliative care or ERT. In 2017, recombinant TPP1 (Brineura), an ERT, was approved by the FDA for the treatment of CLN2 disease. Brineura is administered into the lateral ventricles via an implanted device on a biweekly basis. While an improvement over palliative care in slowing disease progression, we believe frequent administration of ERT into the CNS, reliance on limited and specialized infusion centers, the need for and complications associated with a permanently implanted device, and lack of a treatment for the underlying genetic cause of CLN2 disease represent an area of significant unmet medical need.

RGX-181 is our product candidate for the treatment of CLN2 disease. It is designed to use the AAV9 vector to deliver the human *TPP1* gene to the CNS. Delivery of the gene that is deficient within cells in the CNS could provide a permanent source of secreted TPP1 enzyme, allowing for long-term cross-correction of cells throughout the CNS.

In August 2020, we announced RGX-381, a new program targeting the ocular manifestations of CLN2 disease. RGX-381 is designed to use the AAV9 vector to deliver the *TPP1* gene directly to the retina. We believe that one-time administration of RGX-381 could provide a durable source of TPP1 activity in the retina, thereby potentially preventing visual decline. There is currently no available treatment for ocular manifestations of CLN2 disease. Data from non-human primates demonstrate elevated and sustained levels of TPP1 in the vitreous following a single subretinal injection of RGX-381.

We have received orphan drug product designation and rare pediatric disease designation from the FDA for RGX-181 and RGX-381.

Planned Clinical Development of RGX-181 and RGX-381 for the Treatment of CLN2 Disease

We expect to submit an IND for a first-in-human trial of RGX-181 in the first quarter of 2021.

We expect to submit an IND, or foreign equivalent, for a first-in-human trial of RGX-381 in the first half of 2021.

Our Preclinical Programs

In addition to our lead product candidates, we have also funded, and plan to continue to fund, preclinical research on potential product candidates that may become part of our internal product development pipeline. We have partnered with a number of leading academic institutions and will continue to seek partnerships with innovative institutions to develop novel NAV gene therapy product candidates.

AAV Vector Production Platform

We believe that we have the internal capabilities and access to the resources necessary to enable us to successfully commercialize NAV gene therapy products following regulatory approval, if any, by developing scalable processes to manufacture such products efficiently and in commercial quantities.

We have invested significantly in our internal capabilities and infrastructure, including the establishment of our advanced manufacturing and analytics lab. The suspension-based manufacturing process platform has demonstrated robust scalability from bench-scale to 500 liter and 1,000 liter cGMP batches with yield and product purity consistent across all scales studied. Multiple AAV serotypes, program candidates, and scales have been manufactured with consistent yield and high purity. We anticipate scaling the manufacturing process to 2,000 liter stirred-tank bioreactors which we expect to be an industry-leading position for transient transfection processes. This flexibility in manufacturing scale is ideal for supporting a wide range of commercial supply requirements for our program candidates.

A comprehensive set of analytical methods has been developed to characterize the manufacturing process and NAV Vectors. We continue to expand and enhance internal analytical lab capabilities for improved quality, control, and to support program acceleration.

We believe our proprietary technology, process development and analytical capabilities, and process platform strategy in combination with a talented internal team of scientists and engineers with deep expertise in biologics processing and characterization will continue to deliver cutting-edge advancements in AAV manufacturing.

We are constructing a new cGMP production facility, to be located in our new corporate headquarters in Rockville, Maryland. This facility is expected to support clinical and commercial production of gene therapies starting in the first half of 2022. The cGMP facility is designed to produce NAV Vectors at scales up to 2,000 liters, manufacture bulk drug substance and final drug product, incorporate additional analytical and quality control lab capability and capacity, and meet regulatory requirements for clinical and commercial material. The cGMP production facility is expected to enhance control over production of high-quality product, increase supply capacity, speed adoption of advanced manufacturing technologies, and accelerate availability of clinical material. This strategic investment in internal manufacturing capability will complement our current external manufacturing capabilities, enabling a reliable supply of NAV Vectors from both internal and external sources.

We have agreements with multiple biologics CMOs for production of material under cGMP requirements to support our current and future clinical trials, as well as potential future commercialization of our product development programs. We select our CMOs based on capability, capacity and expertise, and we believe partnering with multiple CMOs provides us with flexibility and diversity in suppliers, as well as access to potential future capacity to accommodate the scale that may be required for future clinical trials and commercialization.

In 2018, we entered into a strategic partnership with FUJIFILM Diosynth Biotechnologies (FUJIFILM) for the manufacture of our lead product candidates, which will support late-stage clinical development and early commercialization. Under the terms of the agreement with FUJIFILM, we gain guaranteed capacity for the supply of NAV AAV drug substance manufactured under cGMP at large scale—up to 2,000 liters—for three years, with the option to extend the agreement for an additional three years. We believe FUJIFILM facilities are compliant with regulatory standards in support of the initiation of worldwide clinical trials for our lead product candidates.

In addition, we believe we have established a robust supply chain for our key raw materials to ensure both high quality standards and assurance of raw material supply as we advance our programs. We have established dual supply sources for critical raw materials

to minimize the potential for disruption of ongoing manufacturing activities. We believe our management team retains significant expertise in managing a diverse network of CMOs and suppliers and that this expertise will enable us to execute on our manufacturing strategy in connection with our external partners.

We believe we have the internal capabilities and access to the resources necessary to enable us to successfully commercialize our product candidates following regulatory approval, if any.

Proprietary Methods

We have obtained rights to all of the proprietary technology underlying our NAV Technology Platform through our Platform Licenses (described below) and our sponsored research agreements (SRAs), under which we have exclusively licensed rights to certain manufacturing-related patents and non-exclusively licensed rights to certain know-how owned or developed by The University of Pennsylvania (Penn). This intellectual property encompasses areas including scalable AAV production methods, methods of increasing the packaging yield of AAV and methods of purification of AAV vectors.

We have examined several methods of larger-scale manufacturing of AAV, which have been optimized to yield high titer and quality vectors. Further improvements to the efficiency and simplicity of the process may remain important to address future needs for commercial applications. Our production methods utilize linearly scalable unit operations, which produce robust yield and purity of the target vector.

Scientists at Penn discovered that in contrast to earlier generation AAV2, most NAV Vectors were released primarily into the medium of production cultures and not retained in the cell. Because these vectors are secreted directly into the media, we are able to efficiently deliver a product of high purity and with relatively high yield with less need for complicated purification steps. This method, for which we have licensed from Penn the exclusive patent rights, is high-yielding and versatile for the production of different NAV Vectors and has been demonstrated to scale into a cGMP setting with comparable yields and product quality. Our future process development activities will build upon this platform to target higher yield of vector without impacting the product purity profile.

Other Capabilities

We have prepared and characterized several proprietary HEK293 master cell banks and other components (plasmid DNA banks) required for clinical vector production. Our master cell banks and other components are being used by us and a subset of our NAV Technology Licensees for the production of NAV Vectors under cGMP for use in clinical trials.

Commercial Licenses to NAV Technology Licensees

We sublicense our NAV Technology Platform to select third parties in order to develop and bring to market NAV gene therapy for a range of severe diseases with significant unmet medical needs. Sublicensing 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 proofs-of-concept for our NAV Technology Platform, and creating potential additional revenue.

Each sublicense specifies the vector or vectors and disease indication or indications as well as whether the sublicense is exclusive or non-exclusive. In determining whether to sublicense, we first evaluate whether the disease indication is of interest to us, in which case we may develop a therapeutic for the disease indication internally using our NAV Technology Platform. If it is not, we consider such factors as the size of the potential market and unmet need; competition; licensee development history; and capabilities and licensee's ability to pay in evaluating whether to enter into a license agreement. As of December 31, 2020, our NAV Technology Platform was being applied in the development of more than 20 partnered product candidates, most under a license to specific NAV Vectors for specific indications.

Our license agreements include upfront and annual fees, milestone fees based on licensee candidate progression, and low-single to low-double digit royalties on sales. Such royalties are subject to customary reductions, such as if the licensee must obtain a license from a third party to avoid infringement of such third party's rights in order to exercise its rights under the license granted by us. We are obligated to make payments to our licensors with respect to the revenues we receive from our licensees for these sublicenses in accordance with the terms of our agreements with our licensors.

Gene Therapy Overview and History of Earlier Generation AAV

Historically, the primary challenge for gene therapy has been the safe and effective delivery of genes into cells. Genes are made of DNA, which is a large, highly charged molecule that is difficult to transport across a cell membrane and deliver to the nucleus, where it can be transcribed and translated into protein. The genetic material needs to be delivered efficiently and to the desired target tissues and cell types, which will vary depending on the disease to be treated. Based on this need, scientists have designed and developed a variety of gene vectors in order to facilitate gene delivery in cells.

To date, the study of gene vectors as treatments in humans has involved approaches with *in vivo* and *ex vivo* techniques using a variety of different gene vectors. Each approach presents different features and benefits for the treatment of a particular disease. *Ex vivo* gene therapy approaches generally are employed to target correction in blood and bone marrow. These methods typically involve harvesting and isolating a patient's own cells. Both the patient and cells undergo several preparatory steps to allow for modification of the cells by gene vectors. Ultimately, the modified cells are re-administered to the patient. *In vivo* gene therapy approaches involve directly administering (e.g., by infusion or injection) gene vectors into patients in order to reach desired cells in target tissues (e.g., liver, brain, eye, muscle, heart). These methods rely on a combination of the route of administration and the gene vectors themselves to facilitate the correction in the target tissues.

We focus on *in vivo* gene therapy. Among vectors available for *in vivo* gene therapy, viral vectors have been adopted with the greatest frequency because they have demonstrated the greatest efficiency in gene delivery to date. This efficiency exists because viral vectors are derived from naturally occurring viruses with normal life-cycles that rely on gene delivery of their own genomes. In other words, they are naturally optimized to deliver genes to cells. Many viral vectors have presented sub-optimal safety profiles for *in vivo* treatment in humans because the viruses from which they are derived are pathogenic (causing disease), immunogenic (causing immune response) or create genomic toxicity (delivering a gene to a place where it interrupts normal function). Vectors derived from adenovirus, herpes virus and retroviruses have been tested as *in vivo* viral vectors, and other technologies are evolving.

Vectors derived from AAV have among the best safety profiles for gene therapy given that AAVs are not known to be associated with disease in humans. The earlier generation AAV vectors were designed by scientists in the mid-1980s and the first clinical trials using AAV began in the mid-1990s. There were only a handful of AAV vectors available to scientists at the time of the first clinical trials because AAV vectors were designed based on the capsid (the protein shell of a virus that encloses the genetic material of the virus) of AAV viruses known to be in existence, and only six distinct serotypes (groups within a single species of microorganisms, such as bacteria or viruses, which share distinctive surface structures) had been discovered at that time. These earlier generation AAV vectors were shown to be limited in their application due to a variety of limitations and challenges, including:

- low or unmeasurable gene expression, meaning the delivered gene was enabling production of low or unmeasurable amounts of the therapeutic protein;
- short-term gene expression, meaning if gene expression was measurable, it was transient;
- limited tissue selectivity, meaning concentrated gene expression was not observed in the target organ; and
- high levels of immune response, meaning the body may neutralize the gene delivery vector with pre-existing antibodies or generate T-cells that inhibit the therapeutic effect.

Discovery of Next Generation AAV

In recognition of the limitations and challenges of earlier generation AAV vectors, an effort was undertaken in the early 2000s at Penn to discover other naturally occurring AAV sequences. The identification of such sequences was based on the observation that wild-type AAV (in contrast to recombinant AAV) can undergo a latent cycle in which the AAV genome stays within the cell, meaning the virus, including its capsid gene sequence, remains intact within the cell but does not reproduce. This allowed for identification of new sequences not by purifying viruses from tissues, but by searching for capsid gene sequences in a variety of tissues isolated from non-human primates and from humans, based on regions of the AAV capsid gene that did not vary between the known AAV vectors. By searching for capsid gene sequences in this manner, many more capsid protein sequences were discovered than would have been found by purifying viruses from tissues.

More than 100 new capsid sequences were identified by the process. The first few were initially designated AAV7, AAV8 and AAV9, after which, other sequences were identified by species from which it was isolated (e.g., "rh" indicating rhesus macaque) followed by a number (e.g., 10, for rh10). Early characterization of the initial discoveries of AAV7, AAV8, AAV9 and AAVrh10 suggested that these vectors may be significantly more efficient in various applications important for clinical translation than other previously known AAVs.

After patenting the next generation AAV vectors, Penn initiated a distribution program through a material-transfer process that enabled researchers to access the next generation AAV vectors for research use only, under specific restrictions. Thousands of custom reagents were sent to independent researchers, who began to characterize and validate the beneficial features of AAV vectors in animal models of disease. In 2010, the first clinical trials were conducted using the next generation AAV vectors and initial proof-of-concept and safety in humans was established from these trials. These clinical trials also produced longer-term efficacy results which reinforced our belief that these next generation vectors have beneficial properties not seen in the earlier generation AAV vectors.

We believe the next generation AAV vectors, which form the basis of our NAV Technology Platform, have many improved properties relative to earlier generation AAV vectors for development and commercialization of AAV treatments, including:

- higher gene transfer;
- longer-term gene expression;
- broad and novel tissue selectivity;
- lower immune response; and
- improved manufacturability.

Our Proprietary NAV Technology Platform for Gene Delivery

In 2009, we licensed rights to the next generation AAV vectors discovered at Penn. Our NAV Vectors form the foundation of our NAV Technology Platform. Our NAV Technology Platform has been used in a number of clinical trials conducted by us, our partners and third-party investigators.

We are developing therapeutics using NAV Vectors that contain genes which are synthesized to code for the expression of therapeutic proteins in target cells to correct the underlying causes of the diseases we seek to treat. Each product candidate is designed with a NAV Vector for a specific cell target and to express a specific protein. We incorporate proprietary modifications to both the AAV and the gene, which enhance properties such as potency, stability and tissue distribution. Our proprietary technology, including the use of vectors derived from novel sequences of AAV such as AAV7, AAV8, AAV9 and AAVrh10, are protected by more than 100 licensed patents and patent applications. The rights to our NAV Technology Platform provide our product candidates with what we believe to be a competitive advantage over product candidates developed with earlier generation AAV vectors due to the novel and beneficial properties of our NAV Vectors.

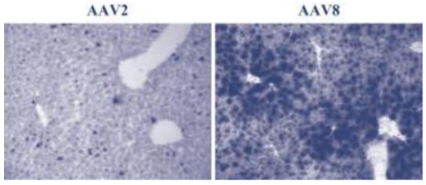
Key Potential Benefits of NAV Technology

The properties that make NAV Vectors unique from and potentially an improvement to earlier generation AAV vectors, as well as provide support that they are potentially best-in-class for development and commercialization of AAV treatments, are set forth below.

Higher Gene Transfer

NAV Vectors have been shown to generate higher levels of gene transfer in animals than earlier generation AAV vectors such as AAV2. In mouse livers, AAV8 produced levels of gene expression that were 10- to 100-fold higher than was achieved with AAV2. The figure below shows the contrast in the amount of gene expressed using the two vectors at the same dose.

AAV Transduction in Mouse Liver



In this experiment, the reporter gene LacZ, a gene which encodes a protein that turns a clear substrate blue in a specific medium, was included in the transgene sequence delivered by the vector so that cells expressing the transgene are stained blue, visually denoting expression level. It was possible to transduce the entire mouse liver and achieve long-term expression with AAV8. Higher gene expression creates the possibility of achieving therapeutic benefit in more diseases than was possible using earlier AAV vectors, as more therapeutic protein is generated with vectors that enable higher expression.

Longer-Term Gene Expression

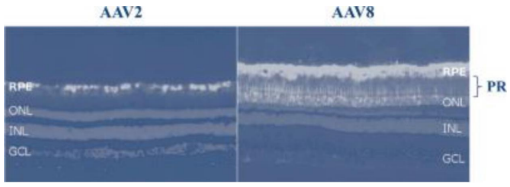
We believe the longer-term gene expression seen using NAV Vectors is due to more stable genomic persistence and reduced cellular immunity, which are a function of novel capsid structure and lower dosing required using NAV Vectors due to the greater gene expression discussed earlier herein. NAV Vectors have demonstrated stable expression in animals for over eight years. Moreover, AAV8 vectors have demonstrated stable expression for over eight years in clinical trials for hemophilia B patients.

Broad and Novel Tissue Selectivity

NAV Vectors also display high levels of tissue specificity. This property is important because it allows for development of therapeutics to target cells that earlier generation AAV vectors do not target or do not target well. AAV9 has emerged as a vector that enables efficient gene delivery when directly injected into the CNS. AAV9 has also demonstrated novel tissue selectivity for the CNS when delivered intravenously, resulting in efficient gene expression in the brain and spinal cord, and AAV9 can also be transported throughout the CNS, enabling broader delivery with a single injection. This was the first time a gene therapy vector was demonstrated to cross the blood-brain barrier, producing results in both small and large animals, including non-human primates. This route of administration has recently been used clinically by one of our NAV Technology Licensees to treat SMA Type I, which was approved by the FDA in 2019.

NAV Vectors have also shown novel properties in the eye when investigated for the treatment of acquired disease and inherited retinal degenerations. AAV8 expressing a fluorescent protein was administered by subretinal injection in the non-human primate eye in order to show gene expression in the retina itself, which contains the cell types to be treated. As is depicted in the graphic below, a cross-section of the non-human primate retina below showed more efficient gene delivery (as demonstrated by the much greater amount of the fluorescent protein expressed) with AAV8 as compared to AAV2 in the retinal pigment epithelium (RPE) and to the photoreceptor (PR) layer. The majority of genes associated with retinal degeneration are located in the RPE and PR layer. These genes influence the cell's development or function and are therefore critical to most inherited retinal degenerations.

AAV Transduction of Layers in the Non-Human Primate Eye(1)



(1) Science Translational Medicine: *Dosage Thresholds for AAV2 and AAV8 Photoreceptor Gene Therapy in Monkey*, Luk H. Vandenberghe, et al. (2011). Reprinted with permission from the American Association for the Advancement of Science.

Lower Immune Response

Lower immune response to the gene therapy vector used to deliver the transgene is important for longer-term gene expression, higher expression and higher potency. Data indicate that more than two thirds of certain human populations have a high level of neutralizing antibodies (NAbs) against the capsids of AAV1 and AAV2. This represents a major obstacle to the effective use of these earlier generation vectors due to the inhibition of gene delivery via particle neutralization in the circulation, as pre-existing antibodies neutralize the vector carrying the transgene before it can reach the target cells. Several studies have investigated the seroprevalence of neutralizing antibodies directed against AAV in humans. Although data can vary geographically, neutralizing antibodies against AAV1 and AAV2 are usually detected in 70% of individuals, while seroprevalence is reported to be 45% for AAV6 and AAV9 and less than 40% for AAV8. Shared amino acid sequences and common overall structure allow for antibody cross-reactivity between AAV serotypes and neutralizing antibodies recognizing virtually all serotypes can be found in most subjects.

T-cell responses to AAV vectors have also been studied in mice and nonhuman primates, in which high levels of T-cells specific to capsids of AAV2 were detected. AAV8, however, did not lead to activation of capsid-specific T-cells. In a recent clinical trial of an AAV8-based gene therapy for the treatment of hemophilia B, there was low liver toxicity from T-cells generated and reactive with AAV8. We believe this is likely due to differences in immunogenic capsid epitopes as well as the lower doses of AAV8 needed to be efficacious.

Improved Manufacturability

The manufacturing process for NAV Vectors can be designed to reduce the number of difficult processing steps required for the earlier AAV vectors, improving overall yield at larger scale. NAV Vectors are derived from naturally "fit" viruses, which are stable structures that efficiently assemble, in contrast to the earlier generation AAV vectors.

Platform License Agreements and Other Licenses

Platform Licenses

We have exclusively licensed many of our rights in our NAV Technology Platform from Penn and GlaxoSmithKline LLC (GSK), which together we refer to as our Platform Licenses. We currently use our NAV Technology Platform to develop treatments for retinal neurodegenerative and metabolic diseases. We also sublicense our NAV Technology Platform to third parties in order to develop and bring to market NAV Gene Therapy for a range of severe diseases with significant unmet medical needs outside of our core disease indications and therapeutic areas. For further information regarding our commercial sublicenses, please see "Commercial Licenses to NAV Technology Licensees" located elsewhere in this Annual Report on Form 10-K.

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 and September 2020. In February 2009, we also entered into an SRA 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 SRA (the 2013 SRA) with Penn in November 2013 which was funded entirely by our NAV Technology Licensee, Dimension Therapeutics, Inc. (since acquired by Ultragenyx Pharmaceutical Inc.) (Dimension). 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 previously 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. 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;
- 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;
- low-double digit to mid-teen digit percentages of sublicense fees we receive for the licensed intellectual property rights from sublicensees; 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-single digit royalty percentages on net sales of licensed products;
- 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.

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. However, if no patent ever issues from patent rights licensed from GSK, this license agreement will terminate a specified number of years after the first commercial sale of the first licensed product in any country. 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.

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. 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.

Intellectual Property

Our success depends in part on our ability to obtain and maintain intellectual property protection for our product candidates, core technologies and other know-how, to operate without infringing on the rights of others and to prevent others from infringing our rights. We strive to protect and enhance the proprietary technology, inventions, and improvements that are important to our business, including by seeking, maintaining and defending patent rights. We also rely on trade secrets relating to our technology platform and on know-how, continuing technological innovation and in-licensing opportunities to develop, strengthen and maintain our position in the field of gene therapy. Additionally, we intend to rely on regulatory protection afforded through orphan drug designations, data exclusivity and market exclusivity as well as patent term extensions, where available.

We anticipate that our patent portfolio will continue to expand as a result of our SRAs with academic institutions and our commercial licenses to NAV Technology Licensees. For further information regarding our commercial sublicenses, please see “Commercial Licenses to NAV Technology Licensees” located elsewhere in this Annual Report on Form 10-K.

Product Candidates

As of December 31, 2020, in addition to the patents related to our NAV Technology described below, our patent portfolio included a total of three issued U.S. patents, one issued European patent, one pending U.S. non-provisional patent application, five pending International Patent applications filed pursuant to the Patent Cooperation Treaty (PCTs) and 20 PCTs that have entered national stage relating to our product candidates, which are summarized below:

- *RGX-314*: Five PCTs that have entered national stage for which any issued U.S. or European patent would expire in 2037, 2038, 2039 or 2040, in each case without taking into account any possible patent term adjustment or extension;
- *RGX-202*: One pending PCT for which any issued U.S. or European patent would expire in 2040, without taking into account any possible patent term adjustment or extension;
- *RGX-111/RGX-121*: 11 PCTs that have entered national stage and one pending PCT for which any issued U.S. or European patents would expire in 2034, 2036, 2037, 2038 or 2039, in each case without taking into account any possible patent term adjustment or extension;
- *RGX-111*: Three issued U.S. patents and one issued European patent that will expire in 2034, in each case without taking into account any possible patent term extension;
- *RGX-181*: One pending U.S. non-provisional patent application for which any issued U.S. patent would expire in 2034, one PCT that has entered national stage for which any issued U.S. or European patent would expire in 2038 and one pending PCT for which any issued U.S. or European patent would expire in 2039, in each case without taking into account any possible patent term adjustment or extension;
- *RGX-381*: Two PCTs that have entered national stage for which any issued U.S. or European patent would expire in 2038 or 2040 and one pending PCT for which any U.S. or European patent would expire in 2039, in each case without taking into account any possible patent term adjustment or extension; and
- *AAV-Mediated Antibody Expression for the Treatment of HAE*: One PCT that has entered national stage for which any issued U.S. or European patent would expire in 2038 and one pending PCT for which any issued U.S. or European patent would expire in 2040, in each case without taking into account any possible patent term adjustment or extension.

NAV Technology

We have exclusively licensed rights relevant to our NAV Technology which includes novel recombinant AAV vectors AAV7, AAV8, AAV9, and AAVrh10, among others. Our licensed patent portfolio includes exclusive rights to more than 100 patents and patent applications worldwide relating to composition of matter patents and/or patent applications for our novel AAV vectors, as well as methods for their manufacture and therapeutic uses. We also possess substantial know-how and trade secrets relating to our NAV Technology. As of December 31, 2020, our licensed patent portfolio included 17 issued U.S. patents and five European patents relating to the AAV7, AAV8, AAV9 and AAVrh10 vectors and uses thereof. These patents have terms that will expire as late as 2026, not including patent term extensions.

Our licensed patent portfolio also includes composition of matter claims for novel AAV vectors having certain other capsids as well as AAV capsids that have an amino acid sequence at least 95% or at least 97% identical to the capsids of certain of the NAV Vectors.

Our patent portfolio also includes patents and patent applications owned or co-owned by us and exclusive rights to patents and patent applications relating to:

- therapeutic compositions and methods involving the foregoing AAV vectors further comprising certain transgenes that encode therapeutic products (including our vectored antibody portfolio), and their use in treating specified diseases;
- specific formulations or methods of delivery of the recombinant AAV vectors of interest for our in-house development programs;
- technology related to engineering AAV therapeutics including recombinant AAV vectors engineered to target conducting airway cells, methods of altering the targeting and cellular uptake efficiency of an AAV viral vector having a capsid containing an AAV9 cell surface binding domain, the design of recombinant AAV viral vectors that confer passive immunization to airborne pathogens (the aforementioned gene therapy systems can include the use of certain gene expression regulation technology; we have exclusively licensed the patents and patent applications relating to this technology), and recombinant AAV vectors having engineered capsids;
- methods of detecting an AAV nucleotide sequence useful in diagnostics; and
- methods of manufacture of recombinant AAV, including patents and applications directed to scalable AAV production methods, methods of increasing the packaging yield, transduction efficiency and gene transfer efficiency of an AAV, methods of assaying viral vectors, methods of purification of viral vectors, such as AAV vectors, and cultured host cell compositions encoding AAV capsid proteins used for the manufacture of recombinant AAV vectors.

Customers

Our revenues for the years ended December 31, 2020, 2019 and 2018 consisted solely of license and royalty revenue. One customer (Novartis Gene Therapies, Inc. (formerly AveXis, Inc.)) accounted for approximately 94% of our total revenues for the year ended December 31, 2020. Three customers (Novartis Gene Therapies and two other customers) accounted for approximately 92% of our total revenues for the year ended December 31, 2019. Two customers (Novartis Gene Therapies and Abeona Therapeutics Inc. (Abeona)) accounted for approximately 97% of our total revenues for the year ended December 31, 2018. 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.

Research and Development

We are building a research and development organization that includes extensive expertise in AAV gene therapy and related scientific disciplines. We operate cross-functionally and are led by an experienced research and development management team. We use rigorous project management techniques to assist us in making disciplined strategic research and development program decisions and to help limit the risk profile of our product pipeline. We also access relevant market information and key opinion leaders in creating target product profiles when appropriate, as we advance our programs towards commercialization. We engage third parties to conduct portions of our preclinical research. In addition, we are utilizing multiple clinical sites to conduct our clinical trials.

Competition

We are aware of a number of companies focused on developing gene therapies in various disease indications, including Adverum Biotechnologies, Inc., Amicus Therapeutics, Inc., Applied Genetic Technologies Corporation, BioMarin Pharmaceutical, Inc., bluebird bio, Inc., Homology Medicines, MeiraGTx Limited, Novartis AG, PTC Therapeutics, Inc., Roche, Sanofi Genzyme, Sarepta Therapeutics, Inc., Solid Biosciences, Inc., Voyager Therapeutics, 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), Regeneron Pharmaceuticals, Inc. (Eylea) and Novartis (Beovu). Companies with products in development

for the treatment of wet AMD and DR include, but may not be limited to, Adverum, Chengdu Kanghong Pharmaceutical Group, Graybug Vision, Inc., Kodiak Sciences, Inc., Opthea, Outlook Therapeutics, Inc. and Roche.

- **DR.** Currently marketed anti-VEGF competition for DR with DME include Roche/Genentech (Lucentis) and Regeneron (Eylea). Companies with products in development for the treatment of DR with DME include, but may not be limited to, Adverum, Graybug Vision, Kodiak Sciences, Novartis, Opthea and Roche. The principal marketed anti-VEGF competition for DR without DME is Roche/Genentech (Lucentis) and Regeneron (Eylea). Companies with products in development for the treatment of DR without DME include, but may not be limited to, Kodiak Sciences and Roche.
- **DMD.** There are currently two companies with marketed branded products to treat DMD. Sarepta products (Exondys, Vyondys) and PTC Therapeutics' products (Translarna, Emflaza) are only available in select geographies. There are three principal competitive gene therapy products in clinical development from Pfizer, Inc (PF-06939926), Sarepta/Roche (SRP-9001) and Solid Biosciences (SGT-001). Other companies with gene therapies in early development for DMD include, but may not limited to, Astellas Pharma Inc., Genethon and Ultragenyx.
- **MPS II.** The principal marketed competition for MPS II is a systemic enzyme replacement therapy, which is marketed by Takeda Pharmaceutical Company, Ltd. (Elaprase). Companies with products in development for the treatment of the neurological manifestations of MPS II include, but may not be limited to, Denali Therapeutics Inc., JCR Pharmaceuticals Co., Ltd. and Takeda.
- **MPS I.** There is one principal competitor with a marketed product for the treatment of MPS I, Sanofi Genzyme (Aldurazyme). Companies with products in development for the treatment of MPS I include, but may not be limited to, ArmaGen, Inc. and Orchard Therapeutics plc.
- **CLN2 Disease.** There is one principal competitor with a marketed product for the treatment of CLN2 disease, BioMarin (Brineura). Companies with products in development for the treatment of CLN2 disease include, but may not be limited to, Roche and Lexeo Therapeutics.
- **HAE.** There are two principal marketed competitors for the prophylactic treatment of HAE, including Takeda (Tahkzyro, Cinryze) and CLS Behring (Haegarda). BioCryst Pharmaceuticals, Inc. received US approval for its oral prophylactic treatment, Orladeyo, in December 2020; the product is currently under review in Europe and Japan. In addition, BioMarin and Intellia Therapeutics, Inc. have gene therapy programs in preclinical development for the treatment of HAE.

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, the level of generic competition and the 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. 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. 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.

One of the performance goals agreed to by the FDA under PDUFA is to review 90% of standard BLAs in 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. Two months are added to these time periods for new molecular entities. 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 reviewing 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. Also under the Fast Track program, the FDA may consider for review sections of the marketing application 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.

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 existing therapies. 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 durable modification of 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. Any product is eligible for priority review if it 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 and that provide meaningful therapeutic benefit over existing treatments may receive accelerated approval, which means that they may be approved on the basis of adequate and well-controlled clinical studies establishing that the product has an effect on a surrogate endpoint that is reasonably likely to predict a clinical benefit, or on the basis of an effect on a clinical endpoint other than survival or irreversible

morbidity. As a condition of approval, the FDA will require that a sponsor of a drug or biological product receiving accelerated approval perform adequate and well-controlled post-marketing clinical studies to confirm the clinical benefit of the medicine. 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. Fast Track designation, Breakthrough Therapy or RMAT designation, priority review and accelerated approval do not change the standards for approval. Rather, these programs are intended to expedite the development and approval process, but 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. We will rely, and expect to continue to rely, on third parties for the production of clinical and commercial quantities of any products that we may commercialize. Manufacturers of our products are required to comply with applicable requirements in the cGMP regulations, including quality control and quality assurance and maintenance of records and documentation. 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, the manufacturer is required to perform certain tests on each lot of the product before it is released for distribution. If the product is subject to official 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 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 implementation that are still being worked out by the FDA.

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 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, PPACA contains provisions that may reduce the profitability of drug products, including, for example, increasing the minimum rebates owed by manufacturers under the Medicaid Drug Rebate Program, extending the rebate program to individuals enrolled in Medicaid

managed care plans, addressing a new methodology by which rebates owed by manufacturers under the Medicaid Drug Rebate Program are calculated for drugs that are inhaled, infused, instilled, implanted or injected and establishing annual fees based on pharmaceutical companies' share of sales to federal health care programs. 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, an application for authorization of a clinical trial must be submitted to the competent regulatory authorities and a request for a related positive opinion must be submitted to the competent Ethics Committees in the EU Member States in which the clinical trial takes place, much like the FDA and the IRB, respectively. Once the clinical trial has been approved by the competent regulatory authorities and a positive opinion has been provided by the competent Ethics Committees in accordance with the EU and the EU Member State requirements, the corresponding clinical trial may proceed. The approval procedures and ethics committee involvement requirements vary to some extent among the EU Member States. Until the new EU Regulation on Clinical Trials (Reg. EU No. 536/2014) becomes applicable, trial sponsors must obtain individual approvals in every EU Member State where a trial site is located.

To obtain regulatory approval of a biological medicinal product under EU regulatory systems, 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 1394/2007/EC on advanced therapy medicinal products, read in combination with Directive 2001/83/EC of the European Parliament and of the Council, commonly known as the Community code on medicinal products and Regulation (EC) No 726/2004 of the European Parliament and of the Council of 31 March 2004 laying down Community procedures for the authorization and supervision of medicinal products for human and veterinary use and establishing the European Medicines Agency (the EMA), commonly referred to as the EMA Regulation. Regulation 1394/2007/EC lays down specific rules concerning the authorization, supervision and pharmacovigilance of gene therapy medicinal products, somatic cell therapy medicinal products and tissue engineered products. The EMA's Committee for Advanced Therapies (CAT) is responsible for assessing the quality, safety and efficacy of advanced therapy medicinal products (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 of the European Parliament and of the Council of 16 December 1999 on orphan medicinal products, a medicinal product may be designated as an orphan medicinal product if (1) it is intended for the diagnosis, prevention or treatment of a life-threatening or chronically debilitating condition; (2) 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 (3) 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 European Commission Regulation (EC) No. 847/2000 of 27 April 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).

Moreover, in the EU, the sole legal instrument at the EU level governing the pricing and reimbursement of medicinal products is Council 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.

Health Technology Assessment (HTA) of medicinal products is becoming an increasingly common part of the pricing and reimbursement procedures in some EU Member States. These countries include France, Germany, Ireland, Italy, and Sweden. The HTA

process in the EU Member States is governed by the national laws of these countries. 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.

In 2011, Directive 2011/24/EU was adopted at the EU level. This Directive concerns the application of patients' rights in cross-border healthcare. The Directive is intended to establish rules for facilitating access to safe and high-quality cross-border healthcare in the EU. It also provides for the establishment of a voluntary network of national authorities or bodies responsible for HTA in the individual EU Member States. The purpose of the network is to facilitate and support the exchange of scientific information concerning HTAs. This could lead to harmonization of the criteria taken into account in the conduct of HTA between EU Member States and in pricing and reimbursement decisions and negatively impact price in at least some EU Member States. On January 31, 2018, the European Commission adopted a new legislative proposal to amend Directive 2011/24/EU. The proposal aims at boosting the cooperation regarding HTA among the EU Member States. It covers new medicinal products and certain new medical devices. The proposal provides the possibility for EU Member States to use common HTA tools, methodologies and procedures across the EU and to perform joint clinical assessments. The proposal has not yet been adopted as new legislation. The proposed regulation was redrafted in 2019 and continues to be subject to discussion. It is expected that if it is adopted becomes effective, it will become applicable three years later. Following the date of application, a further three-year period is contemplated to allow for a phase-in approach for EU Member States to adapt to the new system.

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 United Kingdom (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. Therefore, the UK regulatory regime is currently similar to EU regulations, but under proposed legislation, the Medicines and Medical Devices Bill, the UK may adopt changed regulations that may diverge from the EU legislative regime for medicines, including their research, development and commercialization. For a two-year period, which started January 1, 2021, the UK has adopted transitional provisions that apply to the importation of medicines into the UK and rely on certain EMA marketing authorization application procedures.

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 February 25, 2021, we employed 306 full-time employees, of which 243 were engaged in research and development activities, including preclinical, manufacturing and clinical study related functions, and 63 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.

Diversity, Equity and Inclusion

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 are firmly committed to providing equal opportunity in all aspects of employment and aim for appropriate representation of gender, race and ethnicity at every level of our company. We have emphasized diversity, equity and inclusion as part of our company culture, as set out in our Code of Business Conduct and Ethics, and we are determined to support further progress in this area.

Health and Safety

We prioritize the health and safety of our employees and have implemented policies to minimize the spread of COVID-19 in our workplace, including a work-from-home policy for all employees who are not essential to be onsite. Employees working onsite have been regularly tested for COVID-19 and have been required to practice social distancing, wear masks and maintain contact tracing records. Additionally, we have provided employees with resources to help persevere through the pandemic, including work-from-home technology packages and personal protective equipment.

Available Information

Our principal offices are located at 9600 Blackwell Road, Suite 210, Rockville, MD 20850, and our telephone number is (240) 552-8181. Our website address is www.regenxbio.com. 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 materially adversely affect 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

- The COVID-19 pandemic may affect our business, operations and preclinical and clinical development timelines and plans.
- 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 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.
- Undesirable side effects may delay or prevent our product candidates and those of NAV Technology Licensees 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 normally 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.

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.
- 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.

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 development goals in the timeframes we announce and expect.
- 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, could limit our ability to market those products and decrease our ability to generate product revenue.
- Government price controls could restrict the amount that we are able to charge for any of our products, if approved.

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 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.
- Future acquisitions or strategic partnerships 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 designates an exclusive forum 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 business, operations and preclinical and clinical development timelines and plans could be adversely affected by the effects of the COVID-19 pandemic, including further resurgences or multiple waves of infections, and other public health crises.

The COVID-19 pandemic and other public health crises in regions where we have clinical trial sites or other business operations could have a material adverse effect on our business, operations and preclinical and clinical development timelines and plans, and could significantly constrain or disrupt the operations of third parties upon which we rely, including contract research organizations (CROs) and contract manufacturing organizations (CMOs).

In response to the COVID-19 pandemic, federal, state, local and foreign governments have put in place quarantines, executive orders, shelter-in-place orders, guidelines and other similar orders and restrictions intended to control the spread of the disease. Such orders and restrictions have resulted in business closures, work stoppages, delays, work-from-home policies, travel restrictions and cancellations of events, among other effects that could negatively impact productivity and disrupt our business and operations. Our offices, laboratories, clinical trial sites, prospective clinical trial sites, CROs, CMOs and other collaborators and partners are located in jurisdictions where such orders and restrictions have been enforced, and further resurgences or waves of infections may lead to similar orders and restrictions in the future. We have implemented a work-from-home policy for all employees who are not essential to be onsite, and we may take further actions that alter our operations, as may be required by federal, state or local authorities or which we determine are in the best interests of our employees. The increase in remote working may result in increased cybersecurity, privacy and fraud risks.

The COVID-19 pandemic has caused delays to our clinical trials and may further delay or prevent us from proceeding with our clinical trials. Our clinical trial site initiation and subject enrollment has been delayed, and may be further delayed or suspended, due to site closures, personnel turnover and prioritization of resources toward the COVID-19 pandemic. In addition, some subjects have been, and may continue to be, disinterested in participating in trials with regular follow-up visits during a pandemic, and some subjects have been, and may continue to be, unable or unwilling to comply with clinical trial protocols. Further, some clinical trial vendors have experienced significant delays in providing necessary equipment, supplies and services during the COVID-19 pandemic, and our ability to obtain clinical samples may be adversely affected. Our ability to conduct follow-up visits with treated subjects may be limited if travel or healthcare services are impeded. Similarly, our ability to recruit and retain principal investigators and other clinical trial personnel could be adversely affected.

The COVID-19 pandemic may impact our ability to procure resources, raw materials or components necessary for our research studies and preclinical and clinical development, and prices have escalated due to limited supplies of such resources, raw materials and components. For instance, our supply chain may be disrupted or our CMOs may be required to dedicate their facilities, personnel and resources to support vaccine production. Additionally, required inspections and reviews by regulatory authorities may be delayed due to a focus of resources on COVID-19 as well as continued travel and other restrictions. Significant delays in the timing and completion of our research studies and preclinical and clinical development would increase our costs and could adversely affect our ability to obtain marketing approval from regulatory authorities for the commercialization of our product candidates. Further, meetings with regulatory authorities that would be important in progressing our programs may be delayed or impeded in connection with the COVID-19 pandemic.

The construction of our new headquarters, including our current good manufacturing practice (cGMP) production facility, has been delayed from our original estimates, and may be delayed further, due to various government orders and restrictions relating to the COVID-19 pandemic. The potential impact of any such delay is unpredictable but may include significant additional costs and disruptions to our operations.

The spread of COVID-19 has caused a broad impact globally and may materially affect our business, financial condition and results of operations, as well as continue to increase the volatility and adversely affect the value of our common stock. While the full extent of the economic impact and duration of the COVID-19 pandemic may be difficult to assess or predict, the continuation of prolonged adverse economic conditions (including due to further resurgences or waves of COVID-19 infections) may reduce our ability to access capital and adversely affect our liquidity. In addition, if the business and operations of our licensees are adversely affected by the COVID-19 pandemic, our revenues could in turn be adversely affected.

Scientific and economic analyses of the COVID-19 pandemic, including the expected impact of vaccinations, continue to evolve and we will continue to monitor the situation closely. The ultimate impact of the COVID-19 pandemic and other public health crises is highly unpredictable and subject to change. We are not yet certain about the full extent of the potential impact of COVID-19

on our business, operations and preclinical and clinical development. To the extent COVID-19 adversely affects our business, financial condition and results of operations, as well as global economic conditions more generally, it may also heighten many of the other risk factors described in this Annual Report on Form 10-K.

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 adeno-associated virus (AAV) gene delivery platform (our NAV Technology Platform), and our future success depends on our and our licensees' successful development and commercialization of viable gene therapy product candidates. There can be no assurance that we or our 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 U.S. Food and Drug Administration (the FDA), the European Medicines Agency (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. 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.

Several of our lead product candidates are in the early stages of development and all of our lead 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 licensees under a license we grant of certain intellectual property related to our NAV Technology Platform (our NAV Technology Licensees), 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 NAV Technology Licensees 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 Biologics License Application (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, or 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 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. 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 product candidates for the treatment of certain diseases in which there is little clinical experience and we are using new endpoints or 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 determine 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 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.

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 our experiencing significant increases in costs 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 the safety and efficacy of the product candidates. 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 subject population, process for identifying subjects, design 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, including as a result of a serious adverse event or after an inspection of our clinical trial operations or trial sites;
- failure by us, any CROs we engage or any other third parties to adhere to clinical trial requirements;
- failure to perform in accordance with the FDA good clinical practice (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, 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 or allow our competitors to bring products to market before we do, which could impair our ability to successfully commercialize our product candidates and may harm our business, financial condition, results of operations and prospects.

Additionally, 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:

- be delayed in obtaining marketing 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 NAV Technology Licensees' 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 other trials using adenovirus vectors. While new recombinant vectors have been designed to reduce these side effects, gene therapy is still a relatively new approach to disease treatment and additional adverse side effects could develop. There also is the potential risk of delayed adverse events following exposure to gene therapy products due to persistent biologic activity of the genetic material or other components of products used to carry the genetic material. 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. 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. 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 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 will apply beginning in 2022 and provide 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 approval procedures in the United Kingdom (UK) for our product candidates may be uncertain 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.

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, 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.

Risks Related to Our Financial Position

We have incurred cumulative net losses and have had few profitable quarters since inception. We expect to incur losses for the foreseeable future and may never again achieve or maintain profitability.

Since inception, we have incurred cumulative net losses. We have historically financed our operations primarily through private and public offerings of our equity securities and licensing rights to our NAV Technology Platform. We have devoted substantially all of our efforts to licensing our NAV Technology Platform and to research and development, including preclinical and clinical development of our product candidates, as well as to building out our team. We expect that it could be several years, if ever, before we commercialize a product candidate. We license certain intellectual property related to our NAV Technology Platform to our NAV Technology Licensees. Our NAV Technology Licensees have multiple preclinical studies and clinical trials in progress. However, only one gene therapy product based on such licensing program, Novartis AG's Zolgensma, has been approved or commercialized. Other than revenue in connection with sales of Zolgensma, we expect to generate only limited revenue, if any, in the near term from our current NAV Technology Licensees and any future NAV Technology Licensees. We expect to continue to incur significant expenses and increasing operating losses for the foreseeable future. The net losses we incur may fluctuate significantly from quarter to quarter. We anticipate that our expenses will increase substantially if, and as, we:

- continue 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 profitable, we and our NAV Technology Licensees must develop and commercialize product candidates with significant market potential. This will require us and our NAV Technology Licensees 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 generate revenues that are sufficient to achieve profitability. If we do achieve profitability, we may not be able to sustain or increase profitability on a quarterly or annual basis. Our failure to become and remain profitable would decrease the value of our company and could impair our ability to raise capital, maintain our research and development efforts, expand our business or continue our operations. A decline in the value of our company also could cause you to lose all or part of your investment.

We may 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. 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 attractive terms, we would 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 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 other revenue, if any, received in connection with commercial sales of our NAV Technology Licensees' products, should any of their product candidates receive marketing approval;
- 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;
- 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.

Many of these factors are outside of our control. Identifying potential product candidates and conducting preclinical testing and clinical trials is a time-consuming, expensive and uncertain process that takes years to complete, and we may never generate the necessary data or results required to obtain regulatory and marketing approval and achieve product sales. In addition, our product candidates, if approved, may not achieve commercial success. Our product revenues, if any, and any commercial milestones or royalty payments under our licensing agreements, will be derived from or based on sales of products that may not be commercially available for many years, if at all. In addition, revenue from our NAV Technology Platform licensing is dependent in part on the clinical and commercial success of our licensing partners, including the commercialization of Zolgensma, and in part on maintaining our license agreements with our licensor partners, including GlaxoSmithKline LLC (GSK) and the University of Pennsylvania (Penn). Accordingly, we will need to continue to rely on additional financing to achieve our business objectives.

The issuance of additional securities, whether equity or debt, by us, 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, we have never generated revenue from sales of our product candidates.

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. 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 NAV Technology Licensees' products depends heavily on our, and our NAV Technology Licensees', 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 NAV Technology Licensees 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 NAV Technology Licensees' 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 NAV Technology Licensees' 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 anticipate incurring significant costs associated with commercializing any approved product candidate. Our expenses could increase beyond expectations if we are required by the FDA, the EMA or other regulatory authorities to perform clinical and other studies in addition to those that we currently anticipate. Even if we are able to generate revenues from sales of any of our product candidates or in connection with sales of any of our NAV Technology Licensees' 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 certain preclinical research and development activities and aspects of our clinical trials. If these third parties do not meet our deadlines or otherwise conduct the preclinical research and development activities and trials as required, our preclinical and clinical 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. Specifically, we 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, including our NAV Technology Licensees, 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 licensees or future collaborators, including our NAV Technology Licensees, may suffer adverse effects, including death;
- licensees or 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;
- 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;
- 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 GSK and Penn;
- 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;

- 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;
- 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, as applicable, under the license agreement or collaboration. 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.

For example, we are currently in arbitration with Abeona Therapeutics Inc. (Abeona) regarding a dispute under the License Agreement dated November 4, 2018 between the Company and Abeona, as amended on November 4, 2019 (the License Agreement), pursuant to which Abeona was required to make a payment of $8.0 million to us no later than April 1, 2020. Abeona failed to make this payment and we therefore delivered to Abeona a written demand for payment and breach notice in April 2020. Upon expiration of the applicable cure period under the License Agreement, which occurred on May 2, 2020, the License Agreement was terminated. Upon termination, all rights and licenses granted to Abeona under the License Agreement terminated and an additional $20.0 million fee that would have otherwise been due to us in November 2020 became payable within 15 days of the termination date. We have not received payment for any portion of the fees due from Abeona.

In May 2020, Abeona filed a claim in arbitration alleging that we breached certain responsibilities to communicate with Abeona regarding our prosecution of licensed patents under the License Agreement. We dispute Abeona's claim and have filed a counterclaim in arbitration for the $28.0 million total unpaid fees, plus interest, which accrues at a rate of 1.5% per month under the License Agreement. We have evaluated Abeona's credit profile and financial condition and have recorded an allowance for credit losses related to the accounts receivable due from Abeona. We may ultimately receive less than the full amount we believe we are owed, and the duration of the arbitration and the timing of payment from Abeona, if any, are unpredictable. Any such adverse result or delay in payment may have a material adverse effect on our business, financial condition, results of operations or cash flows.

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.

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. 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.

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 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 current good manufacturing practice (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 any of our vendors, contract laboratories or suppliers is found to be out of compliance with cGMP, we may experience delays or disruptions in manufacturing while we work with these third parties 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 and 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 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 regulatory 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 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 and therefore no product sales and marketing organization. To successfully commercialize any products that may result from our development programs, we will need to develop these capabilities, either on our own or with others. The establishment and development 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 collaborations regarding one or more of our product candidates with other entities to utilize their marketing and distribution capabilities, but we may be unable to enter into such agreements on favorable terms, if at all. If any current licensees or future licensees or collaborators do not commit sufficient resources to commercialize our products, 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 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.

Ethical, social and legal concerns about gene therapy could result in additional regulations restricting or prohibiting our products. 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;
- the limitation of our targeted patient population and other limitations or warnings contained in any FDA, European Commission, 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, 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 acceptance with respect to coverage and reimbursement. If coverage and reimbursement are not available, or are available only at limited levels, we may not be able to successfully commercialize 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 government payors develop their coverage and reimbursement policies. It is difficult to predict what the Centers for Medicare & Medicaid Services (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 no 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 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. At this time, we are unable to predict how these potential legislative changes 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 may 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.

Government price controls or other changes in pricing regulation could restrict the amount that we are able to charge for any of our product candidates, if approved, which would adversely affect our revenue and results of operations.

We expect that coverage and reimbursement of drugs and biologics may be increasingly restricted in the United States and internationally. The escalating cost of health care has led to increased pressure on the health care industry to reduce costs. In particular, pricing by biopharmaceutical companies recently has come under increased scrutiny and continues to be subject to intense political and public debate in the United States and abroad. Government and private third-party payors have proposed health care reforms and cost reductions of drugs and biologics. A number of federal and state proposals to control the cost of health care have been made in the United States. Specifically, there have been several recent U.S. Congressional inquiries and proposed federal and state bills designed to, among other things, bring more transparency to pricing, review the relationship between pricing and manufacturer patient programs and reform government program reimbursement methodologies. In some international markets, the government controls drug and biologic pricing, which can affect profitability.

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.

The success of our business depends upon our 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.

Additionally, because we have limited resources, we may forego or delay pursuit of opportunities with certain programs or product candidates or for indications that later prove to have greater commercial potential. 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.

We are highly dependent on members of our executive team, the loss of any of whose services may adversely impact the achievement of our objectives. While we have entered into employment agreements with each of our executive officers, any of them could leave our employment at any time, as all of our employees are "at will" employees. We currently do not have "key person" insurance on any of our employees. The loss of the services of one or more of our current employees, consultants and advisors might impede the achievement of our research, development, licensing and commercialization objectives.

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, which we believe is likely to continue. 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 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 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;
- the federal Health Insurance Portability and Accountability Act of 1996 (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, imprisonment, 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, such as the UK Bribery Act 2010. 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 NAV Technology Licensees, 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 NAV Technology Licensees, 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 NAV Technology Licensees', 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 internal computer systems, or those of our 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 internal computer systems and those of our current and any future collaborators and other contractors or consultants are vulnerable to damage from computer viruses, unauthorized access, natural disasters, terrorism, war and telecommunication and electrical failures. While we believe we have not experienced any system failure, accident or security breach to date that has had a material effect on our business, if such an event were to occur and cause interruptions in our operations, it could result in a material disruption of our business or financial operations, including our licensing and development programs. Unauthorized disclosure of sensitive or confidential patient or employee data, including personally identifiable information, whether through breach of computer 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.

Our computer systems or our business partners' computer systems may be vulnerable to service interruption or destruction, malicious intrusion and random attack. 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. Cyber-attacks could include the deployment of harmful malware, denial-of service, social engineering and other means to affect service reliability, threaten data confidentiality, integrity and availability and fraudulently obtain funds. Our business partners face similar risks, and a security breach of their systems could adversely affect our security posture. 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, 2020 and 2019 consisted solely of license and royalty revenue. One customer accounted for approximately 94% of our total revenues for the year ended December 31, 2020. Three customers accounted for approximately 92% of our total revenues for the year ended December 31, 2019. We expect future license and royalty revenue 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.

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 large 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 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. For example, we have filed a complaint for patent infringement against Sarepta Therapeutics, Inc. 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. Additionally, we have filed a complaint for patent infringement against Aldevron, LLC arising from its manufacture of cultured host cells containing certain recombinant nucleic acid molecules, which we believe are protected by another patent we licensed from Penn. Our litigations against Sarepta and Aldevron will have uncertain outcomes and may not result in the patent enforcement we desire.

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 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.

The agreements under which we currently license intellectual property or technology from or to third parties are complex, and certain provisions in such agreements may be susceptible to multiple interpretations. The resolution of any contract interpretation disagreement that may arise could narrow what we believe to be the scope of our rights to the relevant intellectual property or technology, 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.

For example, we have received correspondence from GSK and Penn questioning the amount of sublicense fees paid by us as well as the applicable Zolgensma royalty rate category under our license agreement with GSK. If the resolution of any potential dispute over such issues of interpretation is adverse to us, we could owe greater payments to GSK or Penn than we had previously expected, which could materially harm our financial condition.

As previously announced, on December 22, 2020, we entered into a royalty purchase agreement (the Purchase Agreement) with entities managed by Healthcare Royalty Management, LLC (collectively, HCR) providing for the acquisition by HCR of our interest in certain royalty payments based on net sales of Zolgensma. If it is determined that we owe greater royalty fees to GSK or Penn than we had previously expected based on our interpretation of the applicable license agreements, then pursuant to the Purchase Agreement, we would be obliged to compensate HCR such that their interest in certain royalty payments based on net sales of Zolgensma would not be reduced, which could materially harm our financial condition.

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. For example, under our license agreement with Minnesota, our rights are limited to those countries and territories, including the United States, in which a licensed patent has been issued and is unexpired or a licensed patent application is pending. 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.

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. 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.

We have registered trademarks with the USPTO, including for the marks "NAV" 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.

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 NAV Technology Licensees' 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 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 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 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 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. 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. Two cases involving diagnostic method claims and "gene patents" have recently been decided by the Supreme Court. 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.

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 NAV Technology Licensees' product candidates;
- our execution of any collaborative, licensing or similar arrangements, including with our NAV Technology Licensees, 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 failing 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 has allowed us, and could continue to allow us, to access capital in a timely manner. To the extent that we raise additional capital by issuing equity securities, 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 restated certificate of incorporation and amended and restated 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";
- 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 make, alter 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 designates the Court of Chancery of the State of Delaware as the exclusive forum 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 (1) any derivative action or proceeding brought on our behalf, (2) any action asserting a claim of breach of a fiduciary duty owed by any of our directors, officers or other employees to us or our stockholders, (3) any action asserting a claim arising pursuant to any provision of the Delaware General Corporation Law, our restated certificate of incorporation or our amended and restated bylaws or (4) 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 (i) 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 (ii) 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.

The forum selection clause 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 provision 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 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 2. ***PROPERTIES***

Our corporate headquarters are currently located in Rockville, Maryland. We occupy approximately 19,000 square feet of office space at this location under a lease that expires in September 2021. We also occupy approximately 73,000 square feet of office and laboratory space at other locations in Rockville, Maryland, and 15,000 square feet of office space in New York, New York, under leases that expire at various dates through 2027, some of which are renewable for additional years.

In November 2018, we entered into a lease agreement, as amended, for approximately 177,0000 square feet of office, laboratory and manufacturing space at a new facility being constructed in Rockville, Maryland, which will serve as our future corporate, research and manufacturing headquarters. Construction of the facility is ongoing and we expect to begin utilizing the new headquarters facility in the first half of 2021. The lease for the new facility expires in September 2036, subject to certain extension and termination options that we hold under the lease agreement.

We believe that our facilities, including our future corporate, research and manufacturing headquarters, will be adequate to meet our operating needs for the foreseeable future.

ITEM 3. ***LEGAL PROCEEDINGS***

For information regarding our legal proceedings with Abeona Therapeutics Inc., please refer to Note 10, “License and Royalty Revenue—Abeona Therapeutics Inc.,” to the accompanying audited consolidated financial statements.

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

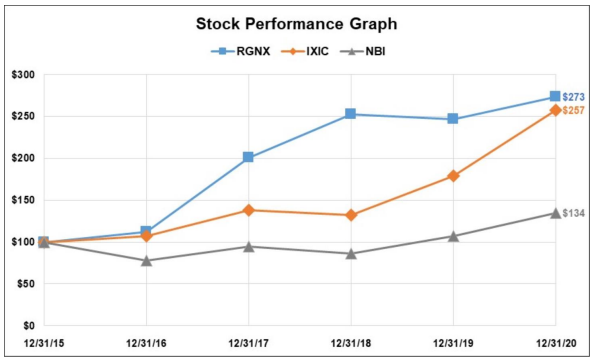
Our common stock is traded on The Nasdaq Global Select Market under the symbol "RGNX."

Stock Performance Graph

The graph set forth below compares the cumulative total stockholder return on our common stock between December 31, 2015 and December 31, 2020, with the cumulative total return of (a) the Nasdaq Composite Index (^IXIC) and (b) the Nasdaq Biotechnology Index (^NBI), over the same period. This graph assumes the investment of $100 on December 31, 2015 in our common stock, the Nasdaq Biotechnology Index and the Nasdaq Composite Index and assumes the reinvestment of dividends, if any.

The comparisons shown in the graph below are based upon historical data. 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.



Holders

As of February 25, 2021, there were six holders of record of our common stock. Because many shares of our common stock are held by brokers and other nominees on behalf of stockholders, we are unable to estimate the total number of beneficial holders represented by these record holders.

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. ***SELECTED FINANCIAL DATA***

The following selected consolidated financial data should be read in conjunction with "Management's Discussion and Analysis of Financial Condition and Results of Operations," the consolidated financial statements and related notes and other financial information included in this Annual Report on Form 10-K.

We derived the consolidated statements of operations data for the years ended December 31, 2020, 2019 and 2018 and the consolidated balance sheet data as of December 31, 2020 and 2019 from our audited consolidated financial statements, which are included elsewhere in this Annual Report on Form 10-K. We derived the consolidated statements of operations data for the years ended December 31, 2017 and 2016 and the consolidated balance sheet data as of December 31, 2018, 2017 and 2016 from our historical audited consolidated financial statements that are not included in this Annual Report on Form 10-K. Historical results are not necessarily indicative of the results to be expected in future periods.

	Years Ended December 31,				
	2020 (a)	2019 (a)	2018 (a)	2017	2016
	(in thousands, except per share data)				
Consolidated Statements of Operations Data:					
Total revenues	$ 154,567	$ 35,233	$ 218,505	$ 10,393	$ 4,589
Total operating expenses	273,800	184,231	130,405	86,278	69,929
Income (loss) from operations	(119,233)	(148,998)	88,100	(75,885)	(65,340)
Net income (loss)	(111,250)	(94,733)	99,937	(73,169)	(62,967)
Net income (loss) per share:					
Basic	$ (2.98)	$ (2.58)	$ 2.99	$ (2.45)	$ (2.38)
Diluted	$ (2.98)	$ (2.58)	$ 2.73	$ (2.45)	$ (2.38)
Weighted-average common shares outstanding:					
Basic	37,281	36,690	33,427	29,878	26,409
Diluted	37,281	36,690	36,648	29,878	26,409

(a) Effective January 1, 2018, we adopted Accounting Standards Update (ASU) 2014-09, *Revenue from Contracts with Customers* (Topic 606), which supersedes the revenue recognition requirements in Accounting Standards Codification (ASC) 605, *Revenue Recognition* (Topic 605). The selected financial data for the years ended December 31, 2020, 2019 and 2018 is presented in accordance with the requirements of Topic 606, while the selected financial data for the years ended December 31, 2017 and 2016 is presented in accordance with the requirements of Topic 605 and, accordingly, may not be comparable.

	December 31,				
	2020 (a)	2019 (a)	2018	2017	2016
	(in thousands)				
Consolidated Balance Sheet Data:					
Cash and cash equivalents	$ 338,426	$ 69,514	$ 75,561	$ 46,656	$ 24,840
Marketable securities	184,123	330,481	395,019	129,738	134,126
Working capital	449,750	311,356	315,737	153,560	83,702
Total assets	708,164	497,908	543,814	198,677	172,732
Non-current liabilities	248,964	14,035	12,790	1,211	1,326
Total liabilities	330,411	47,711	34,966	15,648	10,995
Common stock and additional paid-in capital	667,185	627,814	592,584	371,500	276,357
Total stockholders' equity	377,753	450,197	508,848	183,029	161,737

(a) Effective January 1, 2019, we adopted ASU 2016-02, *Leases* (Topic 842) which supersedes the lease accounting requirements in ASC 840, *Leases* (Topic 840). The selected financial data as of December 31, 2020 and 2019 is presented in accordance with the requirements of Topic 842, while the selected financial data as of December 31, 2018, 2017 and 2016 is presented in accordance with the requirements of Topic 840 and, accordingly, may not be comparable.

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, 2019, including a year-over-year comparison to the year ended December 31, 2018, 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, 2019, which we filed with the SEC on February 26, 2020.

Overview

We are a leading clinical-stage biotechnology company seeking to improve lives through the curative potential of gene therapy. Our gene therapy product candidates are designed to deliver genes to cells to address genetic defects or to enable cells in the body to produce therapeutic proteins that are intended to impact disease. Through a single administration, our gene therapy product candidates are designed to provide long-lasting effects, potentially significantly altering the course of disease and delivering improved patient outcomes.

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) to address genetic diseases through two modalities: AAV-mediated antibody delivery and monogenic gene replacement. The AAV-mediated antibody delivery modality is designed to treat serious and chronic diseases by delivering the genes necessary for the sustained production of therapeutic antibodies *in vivo*. Our monogenic gene replacement approach builds upon the well-understood mechanism of replacing a dysfunctional or missing gene with a functional copy of the gene in order to enable sustained production of necessary proteins.

Gene therapy using NAV Vectors for AAV-mediated antibody delivery

- **RGX-314:** We are developing RGX-314 as a novel, single-administration gene therapy for the treatment of wet age-related macular degeneration (wet AMD), diabetic retinopathy (DR), and other additional chronic retinal conditions which cause total or partial vision loss. We are advancing two separate routes of administration of RGX-314 to the eye, through a standardized subretinal delivery procedure as well as by delivery to the suprachoroidal space using the SCS Microinjector™ licensed from Clearside Biomedical, Inc.

  In January 2021, we announced that we completed an End of Phase 2 meeting with the U.S. Food and Drug Administration (FDA) to discuss the details of a pivotal program to support a Biologics License Application (BLA). The pivotal program is expected to support a Biologics License Application (BLA) filing in 2024. We plan to conduct two randomized, well-controlled clinical trials to evaluate the efficacy and safety of RGX-314 in patients with wet AMD, enrolling approximately 700 patients total. The first pivotal trial (ATMOSPHERE™) is active and enrolling patients. We plan to initiate the second pivotal trial in the second half of 2021.

  In February 2021, we announced additional positive data from the patients enrolled in the ongoing Phase I/II trial of RGX-314 for the treatment of wet AMD and its Long-Term Follow-Up study. As of January 22, 2021, RGX-314 continued to be generally well-tolerated across all dose cohorts. Durable treatment effect was observed in patients in Cohorts 4 and 5 at 1.5 years after administration of RGX-314, including stable visual acuity, decreased retinal thickness, and reductions in anti-VEGF injection burden. Long-term, durable treatment effect was demonstrated in Cohort 3 over three years, including mean improvement in vision and stable retinal thickness, and reductions in anti-VEGF treatment burden.

  In September 2020, we announced that the first patient had been dosed in AAVIATE™, a Phase II trial of the suprachoroidal delivery of RGX-314 using the SCS Microinjector for the treatment of wet AMD. In January 2021, we announced completion of enrollment in Cohort 1 of this trial, and we expect to report interim data from Cohort 1 in the third quarter of 2021. Enrollment of patients in Cohort 2 has begun and is expected to be complete in the second quarter of 2021.

  In addition, we announced in December 2020 that the first patient had been dosed in ALTITUDE™, a Phase II trial of the suprachoroidal delivery of RGX-314 for the treatment of DR. Patient enrollment continues and we expect to complete enrollment of patients in Cohort 1 in mid-2021. We plan to report initial data from this trial in 2021.

- **AAV-Mediated Antibody Expression for the Treatment of Hereditary Angioedema (HAE):** We are developing a novel, one-time treatment utilizing a NAV Vector to deliver a gene encoding for a therapeutic antibody that targets and binds to plasma kallikrein, a key protein left unregulated in patients with HAE. HAE is a chronic and severe disease characterized by recurring severe swelling (angioedema), most commonly in the face, airway, intestines and limbs. We expect to provide a program update in 2021.
- **AAV-Mediated Antibody Expression for the Treatment of Neurodegenerative Diseases:** We have established a research program in partnership with Neurimmune AG (Neurimmune) to jointly develop and commercialize novel gene therapies using NAV Vectors to deliver human antibodies for chronic neurodegenerative diseases, with an initial focus on diseases associated with the accumulation and deposition of the microtubule-associated protein tau (tauopathies) and alpha-synuclein (alpha-synucleinopathies). We expect to provide a program update in 2021.

Gene therapy programs for the potential treatment of rare monogenic diseases

- **RGX-202:** We are developing RGX-202 for the treatment of Duchenne Muscular Dystrophy (DMD), a severe, progressive, degenerative muscle disease caused by mutations in the gene which encodes dystrophin, a protein involved in muscle cell structure and function. Without functional dystrophin protein, muscles throughout the body degenerate and become weak. We expect to submit an Investigational New Drug (IND) application for this program in mid-2021.
- **RGX-121:** We are developing RGX-121 for the treatment of the neurological symptoms of Mucopolysaccharidosis Type II (MPS II), a severe genetic lysosomal storage disease caused by deficiency of iduronate-2-sulfatase (I2S), an enzyme that is responsible for breakdown of cellular waste products. We are conducting a Phase I/II trial of RGX-121 in patients with MPS II under the age of 5 years old. As reported in February 2021, RGX-121 was well-tolerated in Cohorts 1 and 2 of the Phase I/II trial, and no drug-related SAEs were reported. Biomarker data from patients in both cohorts indicated encouraging signals of I2S enzyme activity in the CNS following one-time administration of RGX-121, with consistent reductions of HS and D2S6, a component of HS. Patients in Cohorts 1 and 2 also demonstrated continued neurocognitive development and evidence of I2S enzyme activity in plasma and urine following administration of RGX-121. We expect to begin dosing patients in the third dose cohort in the first quarter of 2021.

  In addition, we plan to initiate a second Phase I/II trial of RGX-121 for the treatment of pediatric patients with MPS II ages 5-18 years old. We expect to begin dosing patients in this trial in the first half of 2021.
- **RGX-111:** We are developing RGX-111 for the treatment of the neurological symptoms of Mucopolysaccharidosis Type I (MPS I), a severe genetic lysosomal storage disease caused by deficiency of α-l-iduronidase (IDUA), an enzyme required for breakdown of cellular waste products. We have initiated a Phase I/II clinical trial for RGX-111.
- **RGX-181:** We are developing RGX-181 for the treatment of late-infantile neuronal ceroid lipofuscinosis type 2 (CLN2) disease, one of the most common forms of Batten disease, caused by mutations in the tripeptidyl peptidase 1 (TPP1) gene. We expect to submit an IND for the CNS delivery of RGX-181 in the first quarter of 2021, and plan to initiate enrollment in a Phase I/II trial in the first half of 2021.
- **RGX-381:** We are developing RGX-381 for the treatment of ocular manifestations of CLN2 disease. We plan to submit an IND, or foreign equivalent, for the subretinal delivery of RGX-381 in the first half of 2021.

In addition to our lead product candidates described above, we have also funded, and plan to continue to fund, preclinical research on potential product candidate programs that may become part of our internal product development pipeline. We have partnered with a number of leading academic institutions and will continue to seek partnerships with innovative institutions to develop novel NAV gene therapy product candidates.

Overview of Our NAV Technology Platform

In addition to our internal product development efforts, we also selectively license the NAV Technology Platform to other leading biotechnology and pharmaceutical companies, which we refer to as NAV Technology Licensees. As of December 31, 2020, our NAV Technology Platform was being applied in one FDA approved product (Zolgensma®), and the preclinical and clinical development of more than 20 partnered programs in total. 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.

Impact of COVID-19

We are actively monitoring the impact of the COVID-19 pandemic on our business, results of operations and financial condition. Our offices, laboratories, clinical trial sites, prospective clinical trial sites, contract research organizations (CROs), contract manufacturing organizations (CMOs) and other collaborators and partners are located in jurisdictions where quarantines, executive orders, shelter-in-place orders, guidelines, and other similar orders and restrictions intended to control the spread of the disease have been put in place by governmental authorities. We have implemented a work-from-home policy for all employees who are not essential to be onsite, and we may take further actions that alter our operations, as may be required by federal, state or local authorities or which we determine are in the best interests of our employees.

The COVID-19 pandemic has caused delays to our clinical trials and may further delay or prevent us from proceeding with our clinical trials. Our other business initiatives, such as preclinical development and manufacturing operations, may also be affected by the COVID-19 pandemic. For example, the construction of our new headquarters, including our current good manufacturing practice production facility, has been delayed from our original estimates, and may be delayed further, due to various government orders and restrictions relating to the COVID-19 pandemic. In addition, if the business and operations of our licensees are adversely affected by the COVID-19 pandemic, our revenues could in turn be adversely affected. We are proactively taking measures to mitigate or reduce any adverse impact of the COVID-19 pandemic on the progress of our clinical trials and other business initiatives.

Our results of operations for the year ended December 31, 2020 were not significantly impacted by the COVID-19 pandemic. However, the full extent to which the COVID-19 pandemic will directly or indirectly impact our business, results of operations and financial condition in the future is unknown at this time and will depend on future developments that are highly unpredictable. Please refer to the “Risk Factors” section of this Annual Report on Form 10-K for further discussion of the risks we face as a result of the COVID-19 pandemic.

Financial Overview

Revenues

Our revenues to date primarily consist of license and royalty revenue resulting from the licensing of our NAV Technology Platform. 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 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 the NAV Technology Platform. 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) option fees to acquire additional licenses, (iii) milestone payments based on the achievement of certain development and sales-based milestones by licensees, (iv) sublicense fees and (v) royalties on sales of licensed products.

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.

Future license and royalty revenues are dependent on the successful development and commercialization of licensed products by our licensees, 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 2 to our audited consolidated financial statements appearing elsewhere in this Annual Report on Form 10-K for a description of segment and geographical information regarding our revenues.

Operating Expenses

Our operating expenses consist primarily of cost of revenues, research and development expenses and general and administrative expenses. Personnel costs including salaries, 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.

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, including sublicense fees, milestone payments and royalties on net sales of licensed products. Sublicense fees are based on a percentage of license fees received by us from NAV Technology Licensees and are recognized in the period that the underlying license revenue is recognized. Milestone payments are payable to licensors upon the achievement of specified milestones by NAV Technology Licensees and are recognized in the period the milestone is achieved or deemed probable of achievement. Royalties are based on a percentage of net sales of licensed products by NAV Technology 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 expense primarily consists of:

- salaries and personnel-related costs, including benefits, stock-based compensation and travel, for our scientific personnel 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
- allocated facility-related costs, depreciation expense and other overhead.

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

We plan to increase our research and development expenses for the foreseeable future as we continue development of our product candidates. Our current and planned research and development activities include the following:

- a Phase I/II clinical trial and associated long-term follow-up study to evaluate the safety and efficacy of the subretinal delivery of RGX-314 for the treatment of wet AMD;
- pivotal trials (ATMOSPHERE and one additional pivotal trial) to evaluate the safety and efficacy of the subretinal delivery of RGX-314 for the treatment of wet AMD;
- Phase II clinical trials to evaluate the safety and efficacy of the suprachoroidal delivery of RGX-314 using the SCS Microinjector for the treatment of wet AMD (AAVIATE) and DR (ALTITUDE);
- preclinical research and development and a planned clinical trial for RGX-202 for the treatment of DMD;

- Phase I/II clinical trials to evaluate the safety and efficacy of RGX-121 for the treatment of MPS II;
- a Phase I/II clinical trial to evaluate the safety and efficacy of RGX-111 for the treatment of MPS I;
- preclinical research and development and a planned Phase I/II clinical trial for RGX-181 for the treatment of CLN2 disease;
- preclinical research and development and a planned clinical trial for RGX-381 for the treatment of ocular manifestations of CLN2 disease;
- preclinical research and development for potential product candidates to treat HAE;
- preclinical research and development for potential product candidates to treat neurodegenerative diseases, including tauopathies and alpha-synucleinopathies, under our collaboration with Neurimmune;
- completion of a long-term follow-up study for patients dosed in the Phase I/II clinical trial for RGX-501, which has been discontinued, for the treatment of homozygous familial hypercholesterolemia (HoFH);
- preclinical research and development for potential product candidates addressing other diseases across a range of therapeutics areas;
- continued investment in advanced manufacturing analytics and process development activities; and
- continued acquisition and manufacture of clinical trial materials in support of our anticipated clinical trials.

The following table summarizes our research and development expenses incurred during the years ended December 31, 2020, 2019 and 2018 (in thousands):

	Years Ended December 31,		
	2020	2019	2018
Direct Expenses			
RGX-314	$ 24,083	$ 20,713	$ 6,580
RGX-202	14,073	—	—
RGX-121	10,322	3,999	4,235
RGX-111	3,324	3,028	3,130
RGX-181 and RGX-381	7,463	7,602	4,399
Other product candidates	4,674	4,823	10,849
Total direct expenses	63,939	40,165	29,193
Unallocated Expenses			
Platform and new technologies	24,936	21,083	12,431
Personnel-related	63,531	50,164	34,275
Facilities and depreciation expense	12,488	9,511	5,816
Other unallocated	1,400	3,262	2,158
Total unallocated expenses	102,355	84,020	54,680
Total research and development	$ 166,294	$ 124,185	$ 83,873

Direct expenses related to RGX-202 and RGX-381 are included in platform and new technologies for all periods through December 31, 2019. Direct expenses related to RGX-181 are included in platform and new technologies for all periods through June 30, 2018. Direct expenses related to RGX-501 are included in other product candidates for all periods presented. We have discontinued internal clinical development of RGX-501. Planned future costs related to RGX-501 primarily relate to the completion of a long-term follow-up study for patients dosed to date.

Platform and new technologies include direct costs not identifiable with a specific lead product candidate, including costs associated with our research and development platform, process development, manufacturing analytics and early research and development for prospective product candidates and new technologies. We typically utilize our employee and infrastructure resources across our development programs. We do not allocate personnel and other internal costs, such as facilities and other overhead costs, to specific product candidates or development programs.

General and Administrative Expense

Our general and administrative expense consists primarily of salaries and personnel-related costs, including employee travel, benefits 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 and administrative support functions. Other general and administrative expenses include facility-related and overhead costs not otherwise allocated to research and development expense, professional fees for accounting, legal and advisory services, expenses associated with obtaining and maintaining patents, insurance costs, costs of our information systems and other commercial and general corporate activities. We expect that our general and administrative expense will continue to increase as we continue to develop, and potentially commercialize, our product candidates.

Other Income

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 as well as interest income accrued on unpaid balances due from licensees.

Investment Income

Investment income consists of interest income earned and gains and losses realized from our cash equivalents and marketable securities, as well as unrealized gains and losses on 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 and equity securities.

Interest Expense

Interest expense consists of non-cash interest imputed on the liability related to the sale of future Zolgensma royalties to entities managed by Healthcare Royalty Management, LLC (collectively, HCR). Non-cash 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.

Critical Accounting Policies and Significant Judgments 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 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* (Topic 606). Topic 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 Topic 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 Topic 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 Topic 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 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. 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) option fees to acquire additional licenses, (iii) milestone payments based on the achievement of certain development and sales-based milestones by licensees, (iv) sublicense fees and (v) royalties on sales of licensed products.

Our license agreements are accounted for as contracts with customers within the scope of Topic 606. At the inception of each license agreement, we determine the contract term for purposes of applying the requirements of Topic 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. At the inception of each license agreement which contains options for the licensee to acquire additional licenses, or contract renewal options, we evaluate the options to determine whether they provide material rights to the licensee. In making this determination, we consider whether the options are priced at a discount to the standalone selling price for the underlying licenses. If an option is priced at a discount to the standalone selling price for the underlying license, 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 if 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 includes up-front and annual fees payable during the contract term. Variable consideration under the license agreements includes 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 that is 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 by licensees. 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 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. Sales-based milestone payments related to net sales of Zolgensma are recognized as royalty revenue in the period in which the milestone is achieved.

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.

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 and other vendors in connection with preclinical development and clinical studies;
- contract manufacturing organizations 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 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 granted to employees and nonemployees, restricted stock units granted to employees 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 to employees and nonemployees 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 to employees and nonemployees 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 to 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. We do not have sufficient historical and implied volatility data for our common stock necessary to estimate the expected the volatility of our common stock over a period of time commensurate with the expected term of our stock option awards. As a result, we estimate expected volatility based on the historical volatility of both our common stock and the common stock of a selected peer group of similar publicly traded companies for which sufficient historical volatility data is available. Due to the lack of historical volatility data for our common stock, we have historically placed a higher weight on the historical volatility of the selected peer group in estimating expected volatility and have increased the weight placed on the historical volatility of its common stock as more historical trading data has become available. We compute the historical volatility data using the daily closing prices for the selected companies' shares during a period equivalent to the expected term of the stock option awards. For the purpose of identifying the selected peer group companies, we consider characteristics such as enterprise value, risk profiles, position within the industry and length of historical share price information. We plan to continue using historical peer group volatility data as an input to estimate expected volatility until a sufficient amount of historical volatility data for our common stock becomes available. 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, due to our lack of sufficient historical data. 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.

Non-cash 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 non-cash 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

As of December 31, 2020, we had federal net operating loss (NOL) carryforwards of $44.7 million, U.S. state NOL carryforwards of $79.7 million and federal and state research and development credit carryforwards of $48.0 million (net of unrecognized tax benefits of $0.1 million) which may be available to offset future income tax liabilities. A portion of our NOL and tax credit carryforwards as of December 31, 2020 may be carried forward indefinitely, with the remaining portion expiring at various dates between 2034 and 2040.

Under the provisions of the Internal Revenue Code, the 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 our value immediately prior to the ownership change. Subsequent ownership changes may further affect the limitation in future years. We have completed several financings since our 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.

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.

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, we believe that it is more likely than not that the benefit of our deferred tax assets will not be realized. Accordingly, we have provided a full valuation allowance for our net deferred tax assets as of December 31, 2020 and 2019.

Recent Accounting Pronouncements

See Note 2 “Recent Accounting Pronouncements” in the notes to the consolidated financial statements appearing elsewhere in this Annual Report on Form 10-K for a full description of accounting pronouncements which we have recently adopted and the impact to our financial statements upon adoption.

Results of Operations

Our consolidated results of operations were as follows:

	Years Ended December 31,			Change	
	2020	2019	2018	2020 vs. 2019	2019 vs. 2018
			(in thousands)		
Revenues					
License and royalty revenue	$ 154,567	$ 35,233	$ 218,505	$ 119,334	$ (183,272)
Total revenues	154,567	35,233	218,505	119,334	(183,272)
Operating Expenses					
Cost of revenues	35,714	8,241	9,640	27,473	(1,399)
Research and development	166,294	124,185	83,873	42,109	40,312
General and administrative	63,817	51,815	36,850	12,002	14,965
Provision for credit losses and other	7,975	(10)	42	7,985	(52)
Total operating expenses	273,800	184,231	130,405	89,569	53,826
Income (loss) from operations	(119,233)	(148,998)	88,100	29,765	(237,098)
Other Income					
Interest income from licensing	4,271	2,951	8,946	1,320	(5,995)
Investment income	9,723	48,559	7,070	(38,836)	41,489
Interest expense	(771)	—	—	(771)	—
Total other income	13,223	51,510	16,016	(38,287)	35,494
Income (loss) before income taxes	(106,010)	(97,488)	104,116	(8,522)	(201,604)
Income Tax Benefit (Expense)	(5,240)	2,755	(4,179)	(7,995)	6,934
Net income (loss)	$ (111,250)	$ (94,733)	$ 99,937	$ (16,517)	$ (194,670)

Comparison of the Years Ended December 31, 2020 and 2019

License and Royalty Revenue. License and royalty revenue increased by $119.3 million, from $35.2 million for the year ended December 31, 2019 to $154.6 million for the year ended December 31, 2020. The increase was primarily attributable to the following:

- an increase of $40.8 million in Zolgensma royalty revenue, from $20.8 million in 2019 to $61.6 million in 2020, as commercial sales of Zolgensma did not commence until the second quarter of 2019; and
- an $80.0 million milestone payment recognized as revenue upon the achievement of $1.0 billion in cumulative net sales of Zolgensma in the third quarter of 2020. Upon the achievement of this milestone, there are no further development or sales-based milestones remaining under the associated license agreement with Novartis Gene Therapies for the development and commercialization of treatments for SMA.

The decrease in license and royalty revenue from 2018 to 2019 was primarily attributable to non-recurring license revenue recognized in 2018 of $176.1 million under our license agreement with Novartis Gene Therapies for the development and commercialization of treatments for SMA, and $35.6 million under our November 2018 license agreement with Abeona Therapeutics Inc. (Abeona). The decrease was partially offset by $20.8 million of Zolgensma royalty revenue recognized in 2019, as commercial sales of Zolgensma did not commence until the second quarter of 2019.

Research and Development Expense. Research and development expenses increased by $42.1 million, from $124.2 million for the year ended December 31, 2019 to $166.3 million for the year ended December 31, 2020. The increase was primarily attributable to the following:

- an increase of $13.2 million for personnel-related costs as a result of increased headcount of research and development personnel, including a $3.2 million increase in stock-based compensation expense;
- an increase of $12.2 million for external costs associated with manufacturing-related services to support the ongoing development of our product candidates and process development activities;
- an increase of $11.3 million for external costs associated with clinical trial and regulatory activities for our lead product candidates;
- an increase of $4.5 million for laboratory costs and facilities used by research and development personnel, including a $0.8 million increase in depreciation expense allocated to research and development functions; and
- an increase of $2.5 million for external costs associated with preclinical studies and other early-stage research and development.

General and Administrative Expense. General and administrative expenses increased by $12.0 million, from $51.8 million for the year ended December 31, 2019 to $63.8 million for the year ended December 31, 2020. The increase was primarily attributable to the following:

- an increase of $8.8 million for professional services, primarily related to commercial consulting and legal services; and
- an increase of $2.9 million for personnel-related costs as a result of increased headcount of general and administrative personnel, including a $1.8 million increase in stock-based compensation expense.

Provision for Credit Losses and Other. Provision for credit losses and other increased by $8.0 million during the year ended December 31, 2020 as compared to the year ended December 31, 2019. The increase was primarily attributable to a provision for credit losses of $7.7 million recognized in 2020 related to our accounts receivable from Abeona. As of December 31, 2020, we had recorded gross accounts receivable from Abeona of $30.1 million and a related allowance for credit losses of $7.7 million. For further information regarding the provision for credit losses, refer to Note 10, “License and Royalty Revenue—Abeona Therapeutics Inc.” to the accompanying consolidated financial statements.

Investment Income. Investment income decreased by $38.8 million, from $48.6 million for the year ended December 31, 2019 to $9.7 million for the year ended December 31, 2020. The decrease was primarily attributable to net gains of $37.8 million recognized in 2019 related to our marketable equity securities of Prevail Therapeutics Inc. (Prevail), as compared to net gains of $4.8 million recognized in 2020 related to these securities. We acquired the securities as consideration for a license to the NAV Technology Platform granted to Prevail in August 2017. Prevail completed its initial public offering (IPO) in June 2019, prior to which the securities were accounted for as non-marketable equity securities without a readily determinable fair value and had a carrying value of $0.4 million. Upon Prevail’s IPO in June 2019, the securities were reclassified to marketable securities and subsequently measured at fair value at each reporting date. As of December 31, 2020, we had sold all of our Prevail securities. The change in investment income

also includes a decrease of $6.0 million in interest income in 2020 as a result of reduced investments in marketable debt securities during the period.

Income Tax Benefit (Expense). Income tax expense increased by $8.0 million, from income tax benefit of $2.8 million for the year ended December 31, 2019 to income tax expense of $5.2 million for the year ended December 31, 2020. The change was primarily attributable to an increase in U.S. state income taxes, largely driven by the sale of our Zolgensma royalties to HCR in 2020. The royalty purchase agreement was treated as a sale for income tax purposes, resulting in an unfavorable temporary difference in taxable income of $184.1 million in 2020 related to the transaction. The taxable income from the royalty sale resulted in current income tax expense in various states where our NOL carryforwards were insufficient to absorb the full amount of our taxable income for the period. Taxable income in 2020 was further increased by gains realized on the sale of our Prevail securities during the period.

Liquidity and Capital Resources

Sources of Liquidity

As of December 31, 2020, we had cash, cash equivalents and marketable securities of $522.5 million, which were primarily derived from the sale of our common stock, license and royalty revenue and the monetization of our Zolgensma royalty stream. We expect that our cash, cash equivalents and marketable securities as of December 31, 2020, will enable us to fund our operating expenses and capital expenditure requirements 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 December 2020, we entered into a royalty purchase agreement with HCR to monetize a portion of our Zolgensma royalty stream. Under the agreement, we received a gross up-front payment of $200.0 million from HCR in December 2020. In exchange, HCR received the rights to a capped amount of Zolgensma royalty payments under our license agreement with Novartis Gene Therapies, including a portion of the royalty payments we received in the fourth quarter of 2020. The aggregate net proceeds received from the sale were $192.5 million, net of a $4.0 million deduction from the purchase price as payment for the pledged royalties we received in the fourth quarter of 2020 and other transaction costs payable by us.
- Novartis Gene Therapies launched commercial sales of Zolgensma in the second quarter of 2019, upon which the we began recognizing royalty revenue on net sales of the licensed product. In accordance with the license agreement, Novartis Gene Therapies was obligated to pay a sales-based milestone fee of $80.0 million to us upon the achievement of $1.0 billion in cumulative net sales of licensed products. Novartis Gene Therapies achieved cumulative net sales of Zolgensma of $1.0 billion in third quarter of 2020 and we received payment of the $80.0 million milestone fee in October 2020. Upon the achievement of this milestone, there are no further development or sales-based milestones remaining under the associated license agreement with Novartis Gene Therapies for the development and commercialization of treatments for SMA.
- In August 2018, we completed a public offering of 3,105,000 shares of our common stock (inclusive of 405,000 shares pursuant to the full exercise by the underwriters of their option to purchase additional shares) at a price of $65.00 per share. The aggregate net proceeds from the offering, inclusive of the underwriters' option exercise, were $189.1 million, net of underwriting discounts and commissions and offering expenses payable by us.

In addition to the sources of liquidity listed above, in January 2021, we completed a public offering of 4,899,000 shares of our common stock (inclusive of 639,000 shares pursuant to the full exercise by the underwriters of their option to purchase additional shares) at a price of $47.00 per share. The aggregate net proceeds from the offering, inclusive of the underwriters' option exercise, were $216.1 million, net of underwriting discounts and commissions and offering expenses payable by us.

We intend to devote the majority of our current capital to clinical development, seeking regulatory approval of our product candidates and capital expenditures to build out additional office, laboratory and manufacturing capacity, including the buildout of our future corporate, manufacturing and research headquarters at 9804 Medical Center Drive in Rockville, Maryland. 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. Furthermore, given the continuing uncertainty and volatile market and economic conditions caused by the COVID-19 pandemic, as well as potential for further effects due to a resurgence in COVID-19 infections, we will continue to monitor the nature and extent of the impact of the COVID-19 pandemic on our liquidity and capital resources.

Cash Flows

	Years Ended December 31,		
	2020	2019	2018
	(in thousands)		
Net cash provided by (used in) operating activities	$ (54,061)	$ (107,705)	$ 104,648
Net cash provided by (used in) investing activities	122,759	93,559	(279,358)
Net cash provided by financing activities	200,214	8,376	204,443
Net increase (decrease) in cash and cash equivalents and restricted cash	$ 268,912	$ (5,770)	$ 29,733

Cash Flows from Operating Activities

Our net cash used in operating activities for the year ended December 31, 2020 decreased by $53.6 million from the year ended December 31, 2019. The decrease was driven largely by an increase in revenues of $119.3 million in 2020, primarily attributable to Zolgensma royalties and the sales-based milestone payment of $80.0 million related to net sales of Zolgensma. The decrease was partially offset by an increase in operating expenses of $89.6 million in 2020, primarily attributable to increased employee headcount and external research and development expenses. We expect to continue to incur net cash out outflows from operations for the foreseeable future as we continue the development and advancement of our lead product candidates and other research programs.

For the year ended December 31, 2020, our net cash used in operating activities of $54.1 million consisted of a net loss of $111.3 million, offset by $43.0 million in adjustments for non-cash items and changes in working capital of $14.2 million. Adjustments for non-cash items primarily consisted of stock-based compensation expenses of $31.9 million, depreciation and amortization expense of $8.4 million and a provision for credit losses of $7.7 million related to our accounts receivable from Abeona. The changes in working capital include a $17.4 million increase in accrued expenses and other current liabilities which was largely driven by increases in sublicense fees payable to licensors, accrued external research and development expenses, income taxes payable and accrued personnel costs. The changes in working capital were partially offset by an increase in accounts receivable of $9.9 million which was largely driven by an increase in Zolgensma royalties receivable as of December 31, 2020. Other changes in working capital were incurred in the normal course of business, primarily as a result of differences in the timing of payments to personnel and external service providers and the period in which such costs are incurred.

For the year ended December 31, 2019, our net cash used in operating activities of $107.7 million consisted of a net loss of $94.7 million, $7.6 million in adjustments for non-cash items and changes in working capital of $5.3 million. Adjustments for non-cash items primarily consisted of net gains on our marketable equity securities of Prevail of $37.8 million, imputed interest earned from our license agreements of $3.0 million and net accretion of discounts on marketable debt securities of $1.2 million, and were partially offset by stock-based compensation expenses of $26.9 million and depreciation and amortization expense of $7.2 million. The changes in working capital include an increase in accounts receivable of $8.6 million, which was largely driven by royalties on net sales of Zolgensma for the fourth quarter of 2019 recorded as accounts receivable as of December 31, 2019. Other changes in working capital were incurred in the normal course of business, primarily as a result of differences in the timing of payments to personnel and external service providers and the period in which such costs are incurred.

Cash Flows from Investing Activities

For the year ended December 31, 2020, net cash provided by investing activities consisted of $272.7 million in sales and maturities of marketable securities, offset by $123.0 million to purchase marketable securities and $26.9 million to purchase property and equipment. The majority of our capital expenditures in 2020 were related to the build out of our future corporate, manufacturing and research headquarters at 9804 Medical Center Drive in Rockville, Maryland. We expect capital expenditures to continue to increase in 2021 as a result of the continued build out of this facility. Total remaining capital expenditures related to the build out of the facility at 9804 Medical Center Drive, net of amounts to be reimbursed by the landlord under our tenant improvement allowance, are expected to be in the middle to upper double-digit millions (USD) and are expected to be incurred into 2022. However, the actual amount and timing of these capital expenditures are uncertain and may differ materially from our current estimates.

For the year ended December 31, 2019, net cash provided by investing activities consisted of $296.0 million in sales and maturities of marketable securities, offset by $190.7 million to purchase marketable securities and $11.7 million to purchase property and equipment.

Cash Flows from Financing Activities

For the year ended December 31, 2020, net cash provided by financing activities consisted of $192.8 million in net proceeds received from the sale of future Zolgensma royalties to HCR, net of transaction costs we paid during the period, and $7.4 million in proceeds received from the exercise of stock options and issuance of common stock under our employee stock purchase plan.

For the year ended December 31, 2019, net cash provided by financing activities consisted of $8.4 million in proceeds received from the exercise of stock options and issuance of common stock under our employee stock purchase plan.

Future Funding Requirements

We have incurred cumulative losses since our inception and had an accumulated deficit of $289.1 million as of December 31, 2020. 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 that our research and development and general and administrative expenses will continue to increase 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 our capital expenditures will continue to increase due to costs associated with building out additional office, laboratory and manufacturing capacity to further support the development of our product candidates and potential commercialization efforts, particularly with respect to the build out of our facility at 9804 Medical Center Drive as discussed above. 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 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 other revenue, if any, received in connection with commercial sales of our NAV Technology Licensees' products, should any of their product candidates receive marketing approval;

- 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;
- 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.

Many of these factors are outside of our control. Identifying potential product candidates and conducting preclinical testing and clinical trials is a time-consuming, expensive and uncertain process that takes years to complete, and we may never generate the necessary data or results required to obtain regulatory and marketing approval and achieve product sales. In addition, our product candidates, if approved, may not achieve commercial success. Our product revenues, if any, and any commercial milestones or royalty payments under our licensing agreements, will be derived from or based on sales of products, the majority of which may not be commercially available for many years, if at all. In addition, revenue from our NAV Technology Platform sublicensing is dependent in part on the clinical and commercial success of our licensing partners. Accordingly, we will need to continue to rely on additional financing to achieve our business objectives.

The issuance of additional securities, whether equity or debt, by us, 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.

Contractual Obligations, Commitments and Contingencies

Our principal commitments include obligations under vendor contracts to provide research services and other purchase commitments with our vendors. In the normal course of business, we enter into services agreements with contract research organizations, contract manufacturing organizations 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 actual amounts and timing of payments under these agreements are uncertain and contingent upon the initiation and completion of the services to be provided. These amounts are not fixed and determinable and therefore are not included in the table below.

Our commitments also include obligations to our licensors under our in-license agreements, which may include sublicense fees, milestones fees, royalties and reimbursement of patent maintenance costs. Sublicense fees are due to the licensors when we sublicense underlying intellectual property to third parties; the fees are based on a percentage of the license fees we receive from the sublicensees. Based on license fees we have received from sublicensees or recorded as accounts receivable as of December 31, 2020, we had accrued $2.5 million of sublicense fees payable to our licensors, of which $2.0 million was expected to be paid within 12 months and $0.5 million was expected to be paid in periods beyond 12 months. The actual amount of sublicense fees payable in future periods could differ materially if new licenses are granted to sublicensees, existing licenses are terminated by sublicensees or if certain other contingent consideration, such as milestone payments, is received from sublicensees in the future. Milestone fees are payable by us upon our future achievement of certain development and regulatory milestones. Royalties are payable by us based on a percentage of net sales of licensed products. Maintenance costs are reimbursements to the licensors for maintaining licensed patents. Amounts due to our licensors in future periods are not fixed and determinable and therefore are not included in the table below.

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, 2020, the total amount of future Zolgensma royalties to be paid to HCR under the agreement was $256.0 million if paid by November 7, 2024, or $296.0 million if paid after that date. We have no obligation to repay any amounts to HCR if total future Zolgensma royalty payments are not sufficient to repay these amounts. The amount and timing of these royalty payments are not fixed and determinable and therefore are not included in the table below.

We have entered into a number of long-term operating leases for office and laboratory space in Rockville, Maryland and New York, New York, as well as a number of laboratory and other equipment leases. The table below includes the future minimum lease payments under our lease agreements.

The following table summarizes our contractual obligations as of December 31, 2020, excluding the items discussed above related to vendor contracts, purchase commitments, license commitments and royalty sale obligations:

	Total	2021	2022 and 2023	2024 and 2025	2026 and Thereafter
			(in thousands)		
Future minimum lease payments	$ 131,739	$ 4,155	$ 14,692	$ 19,744	$ 93,148
Total contractual obligations	$ 131,739	$ 4,155	$ 14,692	$ 19,744	$ 93,148

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, 2020 and 2019, we had cash, cash equivalents and marketable securities of $522.5 million and $400.0 million, respectively. Our cash equivalents and marketable securities as of December 31, 2020 consisted of money market mutual funds, U.S. government and federal agency securities, certificates of deposit, corporate bonds and municipal securities. If market interest rates were to increase immediately and uniformly by 100 basis points, or one percentage point, from levels at December 31, 2020, we estimate that the increase in interest rates would have resulted in a hypothetical decline of $1.0 million in the net fair value of our interest-sensitive securities.

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, British pounds, Canadian dollars and Japanese yen. 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, 2020. 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, 2020, 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, 2020, 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, 2020, based on criteria established in the COSO 2013 framework.

The effectiveness of our internal control over financial reporting as of December 31, 2020 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, 2020 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***

None.

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 2021 annual meeting of stockholders to be filed with the SEC within 120 days of the fiscal year ended December 31, 2020 (2021 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 2021 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 2021 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 2021 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 2021 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

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, 2020 and 2019, and the related consolidated statements of operations and comprehensive income (loss), of stockholders' equity, and of cash flows for each of the three years in the period ended December 31, 2020, 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, 2020, 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, 2020 and 2019, and the results of its operations and its cash flows for each of the three years in the period ended December 31, 2020 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, 2020, based on criteria established in *Internal Control - Integrated Framework* (2013) issued by the COSO.

Change in Accounting Principle

As discussed in Note 2 to the consolidated financial statements, the Company changed the manner in which it accounts for leases in 2019.

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.

License revenue- Substantive Termination Penalties

As described in Notes 2 and 10 to the consolidated financial statements, at the inception of each license agreement, management determines the contract term for purposes of applying the requirements of generally accepted accounting policies relevant to revenue recognition. 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, management 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, management 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. Management considers all of the facts and circumstances relevant to each license when making this determination. License revenue makes up a portion of the Company's consolidated license and royalty revenue of $154.6 million for the year ended December 31, 2020.

The principal considerations for our determination that performing procedures relating to license revenue, specifically substantive termination penalties, is a critical audit matter are the significant judgment by management in determining whether each license granted, including licenses granted upon the exercise of license options, had substantive termination penalties used to determine the contract term for revenue recognition. This in turn led to significant auditor judgment, subjectivity and effort in performing procedures to evaluate the audit evidence obtained relating to management's determination of the existence of a substantive termination penalty in license revenue agreements.

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 relating to the revenue recognition process, including controls over the determination of the existence of a substantive termination penalty in license revenue agreements. These procedures also included, among others, evaluating and testing, for a sample of license revenue contracts, management's process for determining whether a licensee incurs a substantive termination penalty upon termination, which included evaluating (i) the nature of the license, (ii) the payment terms, (iii) the business purpose of contract terms that include termination rights, and (iv) the impact of contract cancellation on other performance obligations, if any, in the contract.

/s/ PricewaterhouseCoopers LLP

Arlington, Virginia
March 1, 2021

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,	
	2020	2019
Assets		
Current assets		
Cash and cash equivalents	$ 338,426	$ 69,514
Marketable securities	137,314	226,696
Accounts receivable (net of allowance of $7,678 as of December 31, 2020)	42,999	38,148
Prepaid expenses	10,505	6,475
Other current assets	1,953	4,199
Total current assets	531,197	345,032
Marketable securities	46,809	103,785
Accounts receivable	3,267	4,155
Property and equipment, net	56,467	28,973
Operating lease right-of-use assets	63,815	10,078
Restricted cash	1,330	1,330
Other assets	5,279	4,555
Total assets	$ 708,164	$ 497,908
Liabilities and Stockholders' Equity		
Current liabilities		
Accounts payable	$ 10,622	$ 6,409
Accrued expenses and other current liabilities	49,082	24,846
Deferred revenue	449	—
Operating lease liabilities	2,500	2,421
Liability related to sale of future royalties	18,794	—
Total current liabilities	81,447	33,676
Deferred revenue	3,783	3,333
Operating lease liabilities	70,153	8,874
Liability related to sale of future royalties	174,504	—
Other liabilities	524	1,828
Total liabilities	330,411	47,711
Commitments and contingencies (Note 8)		
Stockholders' equity		
Preferred stock; $0.0001 par value; 10,000 shares authorized, and no shares issued and outstanding at December 31, 2020 and December 31, 2019	—	—
Common stock; $0.0001 par value; 100,000 shares authorized at December 31, 2020 and December 31, 2019; 37,476 and 36,992 shares issued and outstanding at December 31, 2020 and December 31, 2019, respectively	4	4
Additional paid-in capital	667,181	627,810
Accumulated other comprehensive income (loss)	(360)	205
Accumulated deficit	(289,072)	(177,822)
Total stockholders' equity	377,753	450,197
Total liabilities and stockholders' equity	$ 708,164	$ 497,908

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

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

	Years Ended December 31,		
	2020	2019	2018
Revenues			
License and royalty revenue	$ 154,567	$ 35,233	$ 218,505
Total revenues	154,567	35,233	218,505
Operating Expenses			
Cost of revenues	35,714	8,241	9,640
Research and development	166,294	124,185	83,873
General and administrative	63,817	51,815	36,850
Provision for credit losses and other	7,975	(10)	42
Total operating expenses	273,800	184,231	130,405
Income (loss) from operations	(119,233)	(148,998)	88,100
Other Income			
Interest income from licensing	4,271	2,951	8,946
Investment income	9,723	48,559	7,070
Interest expense	(771)	—	—
Total other income	13,223	51,510	16,016
Income (loss) before income taxes	(106,010)	(97,488)	104,116
Income Tax Benefit (Expense)	(5,240)	2,755	(4,179)
Net income (loss)	$ (111,250)	$ (94,733)	$ 99,937
Other Comprehensive Income (Loss)			
Unrealized gain (loss) on available-for-sale securities, net	(565)	885	(5)
Total other comprehensive income (loss)	(565)	885	(5)
Comprehensive income (loss)	$ (111,815)	$ (93,848)	$ 99,932
Net income (loss) per share:			
Basic	$ (2.98)	$ (2.58)	$ 2.99
Diluted	$ (2.98)	$ (2.58)	$ 2.73
Weighted-average common shares outstanding:			
Basic	37,281	36,690	33,427
Diluted	37,281	36,690	36,648

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

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

	Common Stock		Additional Paid-in Capital	Accumulated Other Comprehensive Income (Loss)	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount				
Balances at December 31, 2017	31,295	$ 3	$ 371,497	$ (715)	$ (187,756)	$ 183,029
Adoption of ASU 2014-09 (Topic 606)	—	—	—	—	4,803	4,803
Issuance of common stock upon public offering, net of transaction costs of $12,728	3,105	1	189,096	—	—	189,097
Exercise of stock options	1,683	—	14,499	—	—	14,499
Issuance of common stock under employee stock purchase plan	37	—	847	—	—	847
Stock-based compensation expense	—	—	16,641	—	—	16,641
Unrealized loss on available-for-sale securities, net	—	—	—	(5)	—	(5)
Net income	—	—	—	—	99,937	99,937
Balances at December 31, 2018	36,120	4	592,580	(720)	(83,016)	508,848
Adoption of ASU 2016-02 (Topic 842)	—	—	—	—	(33)	(33)
Adoption of ASU 2018-02	—	—	—	40	(40)	—
Vesting of restricted stock units	40	—	—	—	—	—
Exercise of stock options	796	—	7,062	—	—	7,062
Issuance of common stock under employee stock purchase plan	36	—	1,314	—	—	1,314
Stock-based compensation expense	—	—	26,854	—	—	26,854
Unrealized gain on available-for-sale securities, net	—	—	—	885	—	885
Net loss	—	—	—	—	(94,733)	(94,733)
Balances at December 31, 2019	36,992	4	627,810	205	(177,822)	450,197
Exercise of stock options	428	—	5,623	—	—	5,623
Issuance of common stock under employee stock purchase plan	55	—	1,799	—	—	1,799
Stock-based compensation expense	—	—	31,949	—	—	31,949
Unrealized loss on available-for-sale securities, net	—	—	—	(565)	—	(565)
Net loss	—	—	—	—	(111,250)	(111,250)
Balances at December 31, 2020	37,476	$ 4	$ 667,181	$ (360)	$ (289,072)	$ 377,753

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,		
	2020	2019	2018
Cash flows from operating activities			
Net income (loss)	$ (111,250)	$ (94,733)	$ 99,937
Adjustments to reconcile net income (loss) to net cash provided by (used in) operating activities			
Stock-based compensation expense	31,949	26,854	16,641
Depreciation and amortization	8,407	7,152	3,982
Provision for credit losses	7,678	—	—
Net amortization of premiums (accretion of discounts) on marketable securities	1,083	(1,196)	755
Net realized and unrealized losses (gains) on marketable securities	(4,918)	(37,774)	39
Imputed interest income from licensing	(2,163)	(2,951)	(8,946)
Non-cash interest expense	771	—	—
Other non-cash adjustments	163	268	14
Changes in operating assets and liabilities			
Accounts receivable	(9,898)	(8,622)	(16,803)
Prepaid expenses	(4,030)	(973)	(400)
Other current assets	2,341	(499)	(2,069)
Operating lease right-of-use assets	3,219	2,431	—
Other assets	399	(2,694)	(1,453)
Accounts payable	3,873	1,528	(218)
Accrued expenses and other current liabilities	17,400	6,882	7,582
Deferred revenue	—	(600)	3,933
Operating lease liabilities	2,139	(2,255)	—
Deferred rent	—	—	(32)
Other liabilities	(1,224)	(523)	1,686
Net cash provided by (used in) operating activities	(54,061)	(107,705)	104,648
Cash flows from investing activities			
Purchases of marketable debt securities	(123,041)	(190,735)	(445,829)
Maturities of marketable debt securities	233,468	289,994	179,749
Sales of marketable debt securities	2,287	—	—
Sales of marketable equity securities	36,914	6,020	—
Purchases of property and equipment	(26,869)	(11,720)	(13,278)
Net cash provided by (used in) investing activities	122,759	93,559	(279,358)
Cash flows from financing activities			
Proceeds from exercise of stock options	5,623	7,062	14,499
Proceeds from issuance of common stock under employee stock purchase plan	1,799	1,314	847
Proceeds from public offerings of common stock, net of underwriting discounts and commissions	—	—	189,716
Issuance costs for public offerings of common stock	—	—	(619)
Proceeds from sale of future royalties	196,000	—	—
Transaction costs for sale of future royalties	(3,208)	—	—
Net cash provided by financing activities	200,214	8,376	204,443
Net increase (decrease) in cash and cash equivalents and restricted cash	268,912	(5,770)	29,733
Cash and cash equivalents and restricted cash			
Beginning of period	70,844	76,614	46,881
End of period	$ 339,756	$ 70,844	$ 76,614
Supplemental cash flow information			
Cash paid (received) for income taxes	$ (191)	$ 904	$ 3,443
Supplemental disclosures of non-cash investing and financing activities			
Additions to property and equipment through accounts payable and accrued expenses	$ 6,812	$ 1,572	$ —
Non-cash additions to property and equipment through tenant improvement allowance	$ 2,263	$ —	$ —
Assets acquired under financing lease obligation	$ —	$ —	$ 5,854
Non-cash consideration received for licenses granted	$ 1,123	$ —	$ —
Transaction costs for sale of future royalties in accounts payable and accrued expenses	$ 265	$ —	$ —

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

REGENXBIO INC.
NOTES TO 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 proprietary adeno-associated virus (AAV) gene delivery platform (NAV Technology Platform) consists of exclusive rights to over 100 novel AAV vectors, including AAV7, AAV8, AAV9 and AAVrh10. The NAV® Technology Platform is being applied by the Company, as well as by third-party licensees (NAV Technology Licensees), in the development of a broad pipeline of product candidates in multiple therapeutic areas and in one commercially available product, Zolgensma®, which is marketed by a NAV Technology Licensee. The Company was formed in 2008 in the State of Delaware and is headquartered in Rockville, Maryland.

As of December 31, 2020, the Company had generated an accumulated deficit of $289.1 million since inception. As the Company has incurred cumulative losses since inception, transition to recurring profitability is dependent upon achieving a level of revenues adequate to support the Company's 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, the Company will continue to need to raise additional capital, to the extent possible. As of December 31, 2020, the Company had cash, cash equivalents and marketable securities of $522.5 million, which management believes 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.

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 on 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. Significant 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, income taxes and the fair value of financial instruments.

The Company is actively monitoring the impact of the COVID-19 pandemic on its business, results of operations and financial condition. The full extent to which the COVID-19 pandemic will directly or indirectly impact the Company's business, results of operations and financial condition in the future is unknown at this time and will depend on future developments that are highly unpredictable. The most significant estimates affecting the Company's consolidated financial statements that may be impacted by the COVID-19 pandemic are related to the Company's assessment of credit losses on accounts receivable, contract assets and available-for-sale debt securities.

Reclassifications

Certain amounts reported in prior periods have been reclassified to conform to current period financial statement presentation. These reclassifications are not material and have no effect on previously reported financial position, results of operations and cash flows.

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 its operations and manages its business as one operating segment.

The Company's revenues consist of license and royalty revenue. For the year ended December 31, 2020, 80% of the Company's revenues were attributed to the U.S. and no other countries were attributed 10% or more of the Company's revenues. For the year ended December 31, 2019, 90% of the Company's revenues were attributed to the U.S. and no other countries were attributed 10% or more of the Company's revenues. For the year ended December 31, 2018, 99% of the Company's revenue were attributed to the U.S. 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 U.S.

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 includes money market mutual funds used to collateralize irrevocable letters of credit as required by the Company's lease agreements. 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):

	December 31, 2020	December 31, 2019	December 31, 2018
Cash and cash equivalents	$ 338,426	$ 69,514	$ 75,561
Restricted cash	1,330	1,330	1,053
Total cash and cash equivalents and restricted cash	$ 339,756	$ 70,844	$ 76,614

Marketable Securities

Marketable securities consist of available-for-sale debt securities and equity 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. Marketable equity securities are classified as 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 income (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. Unrealized gains and losses on marketable equity securities are included in results of operations as investment income. 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, 2020 or 2019.

Accounts Receivable

Accounts receivable primarily consist of consideration due to the Company resulting from its license agreements with NAV Technology Licensees. 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. With the exception of accounts receivable from Abeona Therapeutics Inc. (Abeona), as discussed further in Note 10, 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			Accounts Receivable, Net	
	Years Ended December 31,			December 31,	
	2020	2019	2018	2020	2019
Customer A	94%	69%	81%	44%	28%
Customer B	*	*	16%	48%	62%
Customer C	*	13%	*	*	*
Customer D	*	10%	*	*	*

* Represented less than 10%

Leases

Effective January 1, 2019, the Company adopted Accounting Standards Update (ASU) 2016-02, *Leases* (Topic 842) which supersedes the lease accounting requirements in Accounting Standards Codification (ASC) 840, *Leases* (Topic 840). Please refer to Recent Accounting Pronouncements below for additional information on the adoption of Topic 842 and the associated impact to the Company's consolidated financial statements.

Under Topic 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 as of December 31, 2020 and 2019 have been 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 improvements 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.

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

Impairment of Long-lived 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. No impairment losses on long-lived assets were recorded during the years ended December 31, 2020, 2019 and 2018.

Non-marketable Equity Securities

The Company's 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 of Financial Instruments

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 instruments categorized in Level 3. A financial instrument's level within the fair value hierarchy is based on the lowest level of any input that is significant to the fair value measurement. The fair values of the Company's Level 2 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 fair value measurement of the Company's financial instruments.

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 non-cash 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.), 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.

Revenue Recognition

The Company recognizes revenue in accordance with ASC 606, *Revenue from Contracts with Customers* (Topic 606). Topic 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 Topic 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 Topic 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 Topic 606, the Company assesses the goods or services promised within each contract and determine 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 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 the Company's NAV Technology Platform. 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) option fees to acquire additional licenses, (iii) milestone payments based on the achievement of certain development and sales-based milestones by licensees, (iv) sublicense fees and (v) royalties on sales of licensed products.

The Company's license agreements are accounted for as contracts with customers within the scope of Topic 606. At the inception of each license agreement, the Company determines the contract term for purposes of applying the requirements of Topic 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. At the inception of each license agreement which contains options for the licensee to acquire additional licenses, or contract renewal options, the Company evaluates the options to determine whether they provide material rights to the licensee. In making this determination, the Company considers whether the options are priced at a discount to the standalone selling price for the underlying licenses. If an option is priced at a discount to the standalone selling price for the underlying license, 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 if 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 includes up-front and annual fees payable during the contract term. Variable consideration under the license agreements includes 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 that is 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 by licensees. 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 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, Inc. (formerly AveXis, Inc.) (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. Sales-based milestone payments related to net sales of Zolgensma are recognized as royalty revenue in the period in which the milestone is achieved.

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 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.

Cost of Revenues

Cost of revenues consists primarily of sublicense fees, milestone payments 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 NAV Technology Licensees and are recognized in the period that the underlying revenue is recognized. Milestone payments are payable to licensors upon the achievement of specified milestones by NAV Technology Licensees and are recognized in the period the milestone is achieved or deemed probable of achievement. Royalties are based on a percentage of net sales of licensed products by NAV Technology 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, 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.

Collaborative Arrangements

The Company evaluates its collaboration arrangements to determine whether they are within the scope of ASC 808, *Collaborative Arrangements* (Topic 808). Such arrangements are within the scope of Topic 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 arrangements that meet the criteria of a collaborative arrangement under Topic 808, 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 Topic 606. For transactions that are accounted for pursuant to Topic 808, an appropriate method of recognition and presentation is determined and consistently applied. For such transactions, amounts owed to collaboration partners for development activities performed are recognized as research and development expenses as incurred by the collaboration partner. Amounts owed to the Company from collaboration partners for development activities performed are recognized as a reduction of research and development expenses as incurred by the Company. For transactions that are accounted for pursuant to Topic 606, the Company applies the five-step model as described in its revenue recognition policies.

Stock-based Compensation

The Company accounts for its stock-based compensation awards in accordance with ASC 718, *Compensation—Stock Compensation*. 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 granted to employees and nonemployees, restricted stock units granted to employees 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 to employees and nonemployees 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 to employees and nonemployees 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 to account 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 does not have sufficient historical and implied volatility data for its common stock necessary to estimate the expected the volatility of its common stock over a period of time commensurate with the expected term of its stock option awards. As a result, the Company estimates expected volatility based on the historical volatility of both its common stock and the common stock of a selected peer group of similar publicly traded companies for which sufficient historical volatility data is available. Due to the lack of historical volatility data for its common stock, the Company has historically placed a higher weight on the historical volatility of the selected peer group in estimating expected volatility and has increased the weight placed on the historical volatility of its common stock as more historical trading data has become available. The Company computes the historical volatility data using the daily closing prices for the selected companies' shares during a period equivalent to the expected term of the stock option awards. For the purpose of identifying the selected peer group companies, the Company considers characteristics such as enterprise value, risk profiles, position within the industry and length of historical share price information. The Company plans to continue using historical peer group volatility data as an input to estimate expected volatility until a sufficient amount of historical volatility data for its common stock becomes available. 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 due to its lack of sufficient historical data. 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 recorded in accordance with ASC 740, *Income Taxes*, 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, 2020 and 2019, 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 income (loss).

Net Income (Loss) Per Share

Basic net income (loss) per share is calculated by dividing net income (loss) applicable to common stockholders by the weighted-average common shares outstanding during the period, without consideration for common stock equivalents. Diluted net income (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. Contingently convertible shares in which conversion is based on non-market-priced contingencies are excluded from the calculations of both basic and diluted net income (loss) per share until the contingency has been fully met. For purposes of the diluted net income (loss) per share calculation, common stock equivalents are excluded from the calculation of diluted net income (loss) per share if their effect would be anti-dilutive.

Comprehensive Income (Loss)

Comprehensive income (loss) includes net income (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 June 2016, the Financial Accounting Standards Board (FASB) issued ASU 2016-13, *Financial Instruments—Credit Losses (Topic 326): Measurement of Credit Losses on Financial Instruments*, which amends the accounting for credit losses for most financial assets and certain other instruments. The standard requires that entities holding financial assets that are not accounted for at fair value through net income be presented at the net amount expected to be collected by recording an allowance for credit losses. The allowance for credit losses will be a valuation account that will be deducted from the amortized cost basis of the financial asset to present the net carrying value at the amount expected to be collected on the financial asset. The standard also amends the impairment model for available-for-sale debt securities, requiring credit losses on impaired debt securities to be included in results of operations. The Company adopted this standard effective January 1, 2020 using a modified retrospective transition method, which requires a cumulative-effect adjustment, if any, to opening accumulated deficit on the adoption date. The adoption of this standard primarily impacts the Company's methodology used to assess credit losses on its accounts receivable, contract assets and available-for-sale debt securities. Based on the composition of the Company's accounts receivable, contract assets and available-for-sale debt securities, the adoption of this standard required no cumulative-effect adjustments and did not have a material impact on the Company's financial position or results of operations. Please refer to the significant accounting policies above for a description of the Company's accounting policies for accounts receivable and marketable securities upon the adoption of this standard.

In August 2018, the FASB issued ASU 2018-13, *Fair Value Measurement (Topic 820): Disclosure Framework—Changes to the Disclosure Requirements for Fair Value Measurement*, which modifies certain disclosure requirements regarding fair value measurements. The Company adopted this standard effective January 1, 2020. The adoption of this standard did not have a material impact on the Company's financial statement disclosures.

In August 2018, the FASB issued ASU 2018-15, *Intangibles—Goodwill and Other—Internal-Use Software (Subtopic 350-40): Customer's Accounting for Implementation Costs Incurred in a Cloud Computing Arrangement That Is a Service Contract*. The standard aligns the requirements for capitalizing implementation costs incurred in a hosting arrangement that is a service contract with the requirements for capitalizing implementation costs incurred to develop or obtain internal-use software (and hosting arrangements that include an internal-use software license). The Company adopted this standard effective January 1, 2020 on a prospective basis. The Company has various cloud-based software applications accounted for as service contracts, the most significant of which is the Company's enterprise resource planning (ERP) system for which implementation was in progress on the adoption date of this standard. The adoption of this standard resulted in the capitalization of certain costs during the year ended December 31, 2020 related to the implementation of the ERP system and other cloud-based software applications which would have been expensed as incurred prior to the adoption of this standard. The total amount of costs capitalized during the year ended December 31, 2020 as a result of the adoption of this standard was not material and the adoption of this standard did not have a material impact on the Company's financial position or results of operations.

In December 2019, the FASB issued ASU 2019-12, *Income Taxes (Topic 740)—Simplifying the Accounting for Income Taxes*, which simplifies the current accounting for income taxes. Among other changes, the standard removes the exception to the incremental approach for intraperiod tax allocation when there is a loss from continuing operations and income or a gain from other items such as other comprehensive income. The Company early adopted this standard effective January 1, 2020, with certain aspects of the standard applied using the modified retrospective transition method and other aspects of the standard applied on a prospective basis. The adoption of this standard required no cumulative-effect adjustments and did not have a material impact on the Company's financial position or results of operations.

In February 2016, the FASB issued ASU 2016-02, *Leases* (Topic 842) which supersedes the lease accounting requirements in ASC 840, *Leases* (Topic 840). Effective January 1, 2019, the Company adopted Topic 842 using the modified retrospective transition method. Under this method, the Company applied Topic 842 to all leases in effect as of, or entered into after, January 1, 2019 and recorded the cumulative impact of the adoption as an adjustment to its accumulated deficit on January 1, 2019. The Company's consolidated financial statements for periods ending after January 1, 2019 are presented in accordance with the requirements of Topic 842, while comparative prior period amounts have not been adjusted and continue to be reported in accordance with Topic 840. The cumulative impact of the adoption of Topic 842 resulted in an increase in accumulated deficit of less than $0.1 million on January 1, 2019. The adoption of Topic 842 did not have a material impact on the Company's results of operations for years ended December 31, 2020 and 2019, nor does the Company believe it will have a material impact on future results of operations based on its current leasing arrangements. Please refer to the significant accounting policies above for a description of the Company's lease accounting policies upon the adoption on Topic 842.

In February 2018, the FASB issued ASU 2018-02, *Income Statement—Reporting Comprehensive Income (Topic 220): Reclassification of Certain Tax Effects from Accumulated Other Comprehensive Income*, which amends the previous guidance on

comprehensive income to provide an option for an entity to reclassify the stranded tax effects of the Tax Cuts and Jobs Act of 2017 (the TCJA) that was signed into law in December 2017 from accumulated other comprehensive income directly to retained earnings. The stranded tax effects result from the remeasurement of deferred tax assets and liabilities which were originally recorded in comprehensive income but whose remeasurement is reflected in the income statement. The Company adopted this standard effective January 1, 2019, and upon adoption recorded a cumulative adjustment of less than $0.1 million to reclassify the stranded tax effects of unrealized gains and losses on available-for-sale securities from accumulated other comprehensive income (loss) to accumulated deficit. The adoption of this standard did not have a material impact on the Company's financial position or results of operations.

In May 2014, the FASB issued ASU 2014-09, *Revenue from Contracts with Customers* (Topic 606), which supersedes the revenue recognition requirements in ASC 605, *Revenue Recognition* (Topic 605). Effective January 1, 2018, the Company adopted Topic 606 using the modified retrospective transition method. Under this method, the Company applied Topic 606 to all contracts with customers which were not completed as of January 1, 2018 and recorded the cumulative impact of the adoption as an adjustment to its accumulated deficit on January 1, 2018. The Company recorded a net reduction in opening accumulated deficit of $4.8 million as of January 1, 2018 for the cumulative impact of adoption of Topic 606, which was primarily the result of accelerated recognition of license revenue related to annual license fees under Topic 606. Under Topic 605, annual license fees payable to the Company by licensees were recognized as license revenue annually when the amounts became fixed or determinable. Under Topic 606, the present value of aggregate annual license fees over the contract term of the license agreement are recognized as revenue upon the delivery of the license to the licensee. The impact of the accelerated recognition of license revenue upon adoption was partially offset by the accelerated recognition of licensing costs to the Company's licensors. The Company recognizes sublicense fees to its licensors in the period the underlying license revenue is recognized.

3. Marketable Securities

The following tables present a summary of the Company's marketable securities, which consist of available-for-sale debt securities and equity securities (in thousands):

	Amortized Cost	Unrealized Gains	Unrealized Losses	Fair Value
December 31, 2020				
U.S. government and federal agency securities	$ 12,782	$ 22	$ —	$ 12,804
Certificates of deposit	1,956	34	—	1,990
Corporate bonds	165,850	497	(55)	166,292
Municipal securities	3,035	2	—	3,037
	$ 183,623	$ 555	$ (55)	$ 184,123

	Amortized Cost / Cost	Unrealized Gains	Unrealized Losses	Fair Value
December 31, 2019				
U.S. government and federal agency securities	$ 62,637	$ 215	$ (5)	$ 62,847
Certificates of deposit	8,506	77	—	8,583
Corporate bonds	226,137	808	(29)	226,916
Equity securities	351	31,784	—	32,135
	$ 297,631	$ 32,884	$ (34)	$ 330,481

As of December 31, 2020 and 2019, no available-for-sale debt securities had remaining maturities greater than three 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, 2020 and 2019, the balance in the Company's accumulated other comprehensive income (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, presented in the statements of operations and comprehensive income (loss) consisted of the following (in thousands):

	Years Ended December 31,		
	2020	2019	2018
Unrealized gain (loss) before reclassifications	$ (426)	$ 1,392	$ (44)
Realized losses (gains) reclassified to investment income	(139)	(40)	39
Income tax expense	—	(467)	—
Unrealized gain (loss) on available-for-sale securities, net	$ (565)	$ 885	$ (5)

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, 2020						
Corporate bonds	$ 55,507	$ (55)	$ —	$ —	$ 55,507	$ (55)
	$ 55,507	$ (55)	$ —	$ —	$ 55,507	$ (55)

	Less than 12 Months		12 Months or Greater		Total	
	Fair Value	Unrealized Losses	Fair Value	Unrealized Losses	Fair Value	Unrealized Losses
December 31, 2019						
U.S. government and federal agency securities	$ 12,562	$ (5)	$ —	$ —	$ 12,562	$ (5)
Corporate bonds	48,556	(29)	—	—	48,556	(29)
	$ 61,118	$ (34)	$ —	$ —	$ 61,118	$ (34)

As of December 31, 2020, available-for-sale debt securities held by the Company which were in an unrealized loss position consisted of 17 investment grade security positions. The Company has the intent and ability to hold such securities until recovery, and due to 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, 2020, 2019 and 2018.

Marketable equity securities as of December 31, 2019 consisted solely of common stock of Prevail Therapeutics Inc. (Prevail). The Company acquired the securities as consideration for a license to the NAV Technology Platform granted to Prevail in August 2017. Prevail completed its initial public offering (IPO) in June 2019, prior to which the securities were accounted for as non-marketable equity securities without a readily determinable fair value and had a carrying value of $0.4 million. Upon Prevail's IPO in June 2019, the securities were reclassified to marketable securities and subsequently measured at fair value at each reporting date. During the year ended December 31, 2019, the Company recognized total net realized and unrealized gains of $37.8 million related to the Prevail securities. During the year ended December 31, 2020, the Company sold all of its remaining Prevail securities and recognized total net realized and unrealized gains of $4.8 million during the period.

4. Fair Value of Financial Instruments

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 hierarchy discussed in Note 2 (in thousands):

	Quoted prices in active markets (Level 1)	Significant other observable inputs (Level 2)	Significant unobservable inputs (Level 3)	Total
December 31, 2020				
Cash equivalents:				
Money market mutual funds	$ —	$ 96,307	$ —	$ 96,307
Total cash equivalents	—	96,307	—	96,307
Marketable securities:				
U.S. government and federal agency securities	—	12,804	—	12,804
Certificates of deposit	—	1,990	—	1,990
Corporate bonds	—	166,292	—	166,292
Municipal securities	—	3,037	—	3,037
Total marketable securities	—	184,123	—	184,123
Total cash equivalents and marketable securities	$ —	$ 280,430	$ —	$ 280,430

	Quoted prices in active markets (Level 1)	Significant other observable inputs (Level 2)	Significant unobservable inputs (Level 3)	Total
December 31, 2019				
Cash equivalents:				
Money market mutual funds	$ —	$ 56,058	$ —	$ 56,058
Total cash equivalents	—	56,058	—	56,058
Marketable securities:				
U.S. government and federal agency securities	—	62,847	—	62,847
Certificates of deposit	—	8,583	—	8,583
Corporate bonds	—	226,916	—	226,916
Equity securities	32,135	—	—	32,135
Total marketable securities	32,135	298,346	—	330,481
Total cash equivalents and marketable securities	$ 32,135	$ 354,404	$ —	$ 386,539

There were no transfers of financial instruments between levels of the fair value hierarchy during the years ended December 31, 2020 and 2019.

Management estimates that the carrying amounts of its current accounts receivable, accounts payable and 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 are recorded at their present values using a discount rate that is based on prevailing market rates and the credit profile of the licensee on the date the amounts are 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, 2020 to determine the present value of the receivables. Accordingly, management estimates that the carrying value of its non-current accounts receivable approximates the fair value of those instruments.

Non-marketable equity securities are measured at cost less impairment, adjusted for observable price changes for identical or similar investments of the same issuer. As of December 31, 2020, non-marketable equity securities had a carrying value of $1.1 million and were included in other assets on the consolidated balance sheet. The Company did not identify any observable price changes or changes in circumstances that would have had an adverse effect on the fair value of the securities as of December 31, 2020. The Company did not hold any non-marketable equity securities as of December 31, 2019. No remeasurements or impairment losses were recorded on non-marketable equity securities during the years ended December 31, 2020, 2019 and 2018.

5. Property and Equipment, Net

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

	December 31, 2020	December 31, 2019
Laboratory and manufacturing equipment	$ 26,306	$ 19,663
Computer equipment and software	3,764	2,545
Furniture and fixtures	4,114	2,188
Leasehold improvements	44,957	18,915
Total property and equipment	79,141	43,311
Accumulated depreciation and amortization	(22,674)	(14,338)
Property and equipment, net	$ 56,467	$ 28,973

During the years ended December 31, 2020, 2019 and 2018, the Company recorded depreciation and amortization expense of $8.4 million, $7.2 million and $4.0 million, respectively.

6. Leases

9804 Medical Center Drive

In November 2018, the Company entered into an operating lease, as amended from time to time, for approximately 177,000 square feet of office, laboratory and manufacturing facilities at a new building to be constructed at 9804 Medical Center Drive in Rockville, Maryland (the 9804 Medical Center Drive Lease). The new facility will serve as the Company's future corporate, research and manufacturing headquarters. The initial construction of the building was performed by the landlord, and the lease commenced in September 2020 upon the delivery of leased premises to the Company to make additional improvements to the building. Monthly payments under the lease begin 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 the option to extend the term of the lease for up to 10 additional years and the 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, 2020, the Company's extension and termination options under the 9804 Medical Center Drive Lease have been 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 lease agreement, 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 has been 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, 2020, the Company had unreimbursed amounts remaining under the tenant improvement allowance of $12.9 million, which were deemed in-substance lease payments and recorded as a reduction of the lease liability. As of December 31, 2020, the Company had recorded property and equipment of $36.3 million related to the buildout of the facility at 9804 Medical Center Drive, which have not yet been placed in service.

The Company recorded the right-of-use assets and lease liabilities related to the 9804 Medical Center Drive Lease upon its commencement in September 2020. As of December 31, 2020, the Company had recorded right-of-use assets of $50.1 million and lease liabilities of $57.8 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 9714 Medical Center Drive and extend the term of the lease. In October 2020, the 9712 Medical Center Drive Lease was amended to extend the lease term from September 2021 to February 2027, subject to extension options held by the Company. The October 2020 amendment resulted in a $7.2 million increase in the right-of-use assets and lease liabilities under the 9712 Medical Center Drive Lease. 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, 2020, the Company's extension options under the 9712 Medical Center Drive Lease have been 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 has been 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.

9600 Blackwell Road

In January 2016, the Company entered into an operating lease for its corporate headquarters at 9600 Blackwell Road in Rockville, Maryland (the 9600 Blackwell Road Lease). The lease term commenced in February 2016, and monthly payments under the lease began in September 2016 and escalate annually in accordance with the lease agreement. In November 2017, the 9600 Blackwell Road Lease was amended to include additional office space for the remainder of the lease term. The Company received a $0.8 million tenant improvement allowance from the landlord which has been 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.

In November 2020, the Company exercised its termination option under the 9600 Blackwell Road Lease. Upon exercise of the termination option, the Company was obligated to pay an early termination fee of $0.4 million and the lease term was reduced from September 2023 to September 2021. The exercise of the termination option in November 2020 resulted in a $0.7 million decrease in the right-of-use assets and lease liabilities under the 9600 Blackwell Road Lease.

400 Madison Avenue

In May 2016, the Company entered into an operating lease for office space at 400 Madison Avenue in New York, New York (the 400 Madison Lease). The lease term commenced in July 2016, and monthly payments under the lease began in October 2016 and escalate annually in accordance with the lease agreement. In May 2019, the 400 Madison Lease was amended to include additional office space and extend the lease term from October 2020 to April 2027. The May 2019 amendment resulted in a $5.2 million increase in the right-of-use assets and lease liabilities under the 400 Madison Lease. The Company received a $0.7 million tenant improvement allowance from the landlord which has been 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 lease agreement, 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.

Other Leases

The Company leases additional office and laboratory facilities, laboratory equipment and other equipment under operating leases with various expiration dates through 2028, 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,	
	2020	2019
Operating lease cost	$ 5,246	$ 3,040
Variable lease cost	1,104	666
Total lease cost	$ 6,350	$ 3,706
Cash paid (received) for amounts included in operating lease liabilities	$ (678)	$ 2,724
Right-of-use assets acquired through operating lease liabilities	$ 56,956	$ 5,114

Cash received for amounts included in operating lease liabilities for the year ended December 31, 2020 includes $5.0 million 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. Right-of-use assets acquired through operating lease liabilities for the years ended December 31, 2020 and 2019 include additions and reductions to right-of-use assets resulting from lease modifications and changes in lease term. Short-term lease expense for the years ended December 31, 2020 and 2019 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 weighted-average remaining lease term and weighted-average discount rate of the Company's operating leases were as follows:

	As of December 31, 2020
Weighted-average remaining lease term (years)	13.7
Weighted-average discount rate	5.6%

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, 2020 (in thousands):

	As of December 31, 2020
Undiscounted future minimum lease payments:	
2021	$ 4,155
2022	6,021
2023	8,671
2024	9,745
2025	9,999
Thereafter	93,148
Total undiscounted future minimum lease payments	$ 131,739
Amount representing imputed interest	(46,212)
Tenant improvement allowance not yet received	(12,874)
Total operating lease liabilities	72,653
Current portion of operating lease liabilities	(2,500)
Operating lease liabilities, non-current	$ 70,153

The table above excludes future minimum lease payments for leases which were executed but had not yet commenced as of December 31, 2020, the total of which were not material.

Rent expense under all leases, including additional rent charges for utilities, parking, property management, operating expenses and real estate taxes, was $2.8 million for the year ended December 31, 2018, which was recognized in accordance with Topic 840.

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 agreement, HCR purchased the Company's rights to a capped amount of Zolgensma royalty payments under the Company's license agreement 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 agreement, Zolgenmsa royalty payments will 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 under the agreement is subject to an increasing cap (the Cap Amount) equal to (i) $260.0 million applicable for the period from the effective date of the agreement through November 7, 2024, and (ii) $300.0 million applicable for the period from November 8, 2024 through the effective date of termination of the license agreement with Novartis Gene Therapies. 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 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; provided, however, that with

respect to a call option exercised on or before November 7, 2024, in the event that the then applicable Cap Amount minus the total amount of royalty payments received by HCR is less than $1.0 million, the repurchase price shall equal such difference.

The proceeds received from HCR of $196.0 million were recorded as a liability, net of transaction costs of $3.5 million, which will be 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 agreement, less the net proceeds received by the Company of $192.5 million, is recorded as non-cash interest expense over the life of the arrangement using the effective interest method. Due to its continuing involvement in the underlying license agreement with 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 Company estimates the effective interest rate used to record non-cash interest expense under the Royalty Purchase Agreement based on its estimate of future royalty payments to be received by HCR. As of December 31, 2020, the estimated effective interest rate under the agreement was 13.6%. 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. 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.

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):

	Year Ended December 31, 2020
Liability related to sale of future royalties, beginning balance	$ —
Proceeds from sale of future royalties	196,000
Deferred transaction costs	(3,473)
Non-cash interest expense	771
Liability related to sale of future royalties, ending balance	193,298
Current portion of liability related to sale of future royalties	(18,794)
Liability related to sale of future royalties, non-current	$ 174,504

8. Commitments and Contingencies

Licenses and Collaborations

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. 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 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 NAV Technology Licensees and are recorded as cost of revenues. Milestone fees are recorded as cost of revenues if the underlying milestone is achieved by a licensee, or as research and development expense if the underlying milestone is achieved by the Company as a result of the development of its product candidates and the technology has no alternative future use. 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.

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, 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 license agreement, the Company is obligated to pay Penn royalties on net sales of licensed products and sublicense fees. Additionally, the Company is obligated to reimburse Penn for certain costs incurred related to the maintenance of the licensed patents.

In April 2019, the Company amended its license from Penn 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. Additionally, the amendment modified the percentage of sublicense fees the Company is obligated to pay Penn on amounts received by the Company from third parties for the sublicensing of the licensed rights for the treatment of CLN2 disease.

Expenses incurred by the Company related to its license from Penn were recorded as follows (in thousands):

	Years Ended December 31,		
	2020	2019	2018
Cost of revenues	$ 39	$ —	$ (18)
Research and development	—	200	—
General and administrative	821	905	130
	$ 860	$ 1,105	$ 112

As of December 31, 2020 and 2019, the Company had recorded $0.1 million and $0.1 million, respectively, in expenses payable to Penn under the license agreement, which are included in accounts payable, accrued expenses and other current liabilities and other liabilities on the consolidated balance sheets.

GlaxoSmithKline LLC

In March 2009, the Company entered into a license agreement, which was amended in April 2009, 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 license agreement, 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 as of December 31, 2020.

Expenses incurred by the Company related to its license from GSK were recorded as follows (in thousands):

	Years Ended December 31,		
	2020	2019	2018
Cost of revenues:			
Royalties on net sales of Zolgensma	$ 26,278	$ 5,822	$ —
Other cost of revenues	9,398	2,419	9,407
Total cost of revenues	35,676	8,241	9,407
General and administrative	1,046	928	548
	$ 36,722	$ 9,169	$ 9,955

As of December 31, 2020 and 2019, the Company had recorded $13.1 million and $6.7 million, respectively, in expenses payable to GSK under the license agreement, which are included in accounts payable, accrued expenses and other current liabilities and other liabilities on the consolidated balance sheets.

Neurimmune AG

In July 2019, the Company entered into a collaboration and license agreement with Neurimmune AG (Neurimmune) pursuant to which the Company and Neurimmune will jointly develop and commercialize novel gene therapies using AAV vectors from the NAV Technology Platform to deliver human antibodies for chronic neurodegenerative diseases. The Company and Neurimmune will share all research and development costs for the first two years of the agreement, after which each party will have the option, on a target-by-target basis, to: (i) continue as a 50% partner in the collaboration; (ii) receive a phase-based worldwide royalty in lieu of continued development investment; or (iii) negotiate with the other party to lead the development and commercialization of the respective program. Unless the parties agree otherwise, upon the commercialization of any product candidates, if any, it is anticipated that profits and losses will be shared equally on a worldwide basis.

The Company determined that the collaboration and license agreement with Neurimmune is a collaborative arrangement within the scope of Topic 808, and that no unit of account under the arrangement should be accounted for as a transaction with a customer within the scope of Topic 606. In accordance with the Company's accounting policies for collaborative arrangements, if Neurimmune's development costs incurred under the collaboration exceed those incurred by the Company during a reporting period, the Company will recognize research and development expense and record a liability for the amount due to Neurimmune at the end of the period. Alternatively, if the Company's development costs incurred under the collaboration exceed those incurred by Neurimmune during a reporting period, the Company will recognize a reduction of research and development expenses and record an amount due from Neurimmune at the end of the period. During the years ended December 31, 2020 and 2019, the Company recognized net research and development expenses of $0.5 million and less than $0.1 million, respectively, under the collaboration and license agreement with Neurimmune.

Clearside Biomedical, Inc.

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 RGX-314 to the suprachoroidal space to treat wet age-related macular degeneration (wet AMD), diabetic neuropathy (DR) and other diseases. The Company exercised its license option in October 2019, resulting in a payment of $1.6 million to Clearside which was recognized as research and development expense upon exercise. 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, 2020, 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 was amended in November 2016, with Regents of the University of Minnesota (Minnesota), for an exclusive license under 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 under 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.

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, 2020 and 2019, the Company did not have any material indemnification claims that were probable or reasonably possible and consequently had not recorded any related liabilities.

9. Capitalization

As of December 31, 2020 and 2019, 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.

In August 2018, the Company completed a public offering of 3,105,000 shares of its common stock (inclusive of 405,000 shares pursuant to the full exercise by the underwriters of their option to purchase additional shares) at a price of $65.00 per share. The aggregate net proceeds received by the Company from the offering, inclusive of the underwriters' option exercise, were $189.1 million, net of underwriting discounts and commissions and offering expenses payable by the Company.

The Company's reserved shares of common stock for future issuance were as follows (in thousands):

	December 31, 2020	December 31, 2019
Reserved for issuance under equity incentive plans	8,659	7,607
Reserved for issuance under employee stock purchase plan	448	134
	9,107	7,741

In January 2021, the Company completed a public offering of 4,899,000 shares of its common stock (inclusive of 639,000 shares pursuant to the full exercise by the underwriters of their option to purchase additional shares) at a price of $47.00 per share. The aggregate net proceeds received by the Company from the offering, inclusive of the underwriters' option exercise, were $216.1 million, net of underwriting discounts and commissions and offering expenses payable by the Company.

10. License and Royalty Revenue

As of December 31, 2020, the Company's NAV Technology Platform was being applied by NAV Technology Licensees in one commercial product, Zolgensma, and in the development of more than 20 product candidates. Consideration to the Company under its license agreements may include: (i) up-front and annual fees, (ii) option fees to acquire additional licenses, (iii) milestone payments based on the achievement of certain development and sales-based milestones by licensees, (iv) sublicense fees and (v) royalties on sales of licensed products. 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.

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, 2020, 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 $213.1 million, including (i) $26.6 million upon the commencement of various stages of clinical trials, (iii) $26.0 million upon the submission of regulatory approval filings, (iii) $103.5 million upon the approval of commercial products by regulatory agencies and (iv) $57.0 million upon the achievement of specified sales targets for licensed products. To the extent the milestone payments are realized by the Company, the Company will be obligated to pay sublicense fees to licensors based on a specified percentage of the fees earned by the Company. The achievement of milestones by licensees 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 changes in 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,		
	2020	2019	2018
Accounts receivable, current and non-current:			
Balance, beginning of period	$ 42,303	$ 31,599	$ 5,850
Additions	158,682	39,203	231,154
Deductions	(154,719)	(28,499)	(205,405)
Balance, end of period	$ 46,266	$ 42,303	$ 31,599
Contract assets:			
Balance, beginning of period	$ —	$ 750	$ 350
Additions	350	1,000	3,000
Deductions	—	(1,750)	(2,600)
Balance, end of period	$ 350	$ —	$ 750
Deferred revenue, current and non-current:			
Balance, beginning of period	$ 3,333	$ 3,933	$ —
Additions	1,124	—	3,933
Deductions	(225)	(600)	—
Balance, end of period	$ 4,232	$ 3,333	$ 3,933
Revenue recognized during the period from:			
Amounts included in deferred revenue at beginning of period	$ —	$ 600	$ —
Performance obligations satisfied in previous periods	$ 146,772	$ 26,689	$ 5,348

Additions to accounts receivable during the periods presented consisted primarily of receivables recorded related to royalties on net sales of Zolgenmsa, new licenses granted by the Company and the achievement of milestones by licensees during the period, as well as interest income from licensing recognized during the period. Deductions to accounts receivable during the periods presented consisted primarily of amounts collected from licensees and increases in the allowance for credit losses, as discussed further below. Additions to contract assets during the periods presented consisted of development milestones deemed probable of achievement by licensees during the periods. Deductions to contract assets during the periods presented consisted of the achievement of such milestones and billing of the associated milestone payments by the Company.

As of December 31, 2020, the Company had recorded deferred revenue of $4.2 million which represents consideration received from licensees for performance obligations that have not yet been satisfied by the Company. Unsatisfied performance obligations consisted of (i) options granted to licensees that provide material rights to the licensee to acquire additional licenses from the Company, which will be satisfied upon the exercise or expiration of the options and (ii) research and development services to be performed by the Company related to licensed products, which will be satisfied as the research and development services are performed.

Revenue recognized from performance obligations satisfied in previous periods was primarily attributable to royalty and sublicense revenues as well as 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.

Accounts Receivable, Contract Assets and the Allowance for Credit Losses

Accounts receivable, net consisted of the following (in thousands):

	December 31, 2020	December 31, 2019
Current accounts receivable:		
Billed to customers	$ 30,573	$ 376
Unbilled	20,104	37,772
Allowance for credit losses	(7,678)	—
Current accounts receivable, net	42,999	38,148
Non-current accounts receivable:		
Unbilled	3,267	4,155
Allowance for credit losses	—	—
Non-current accounts receivable, net	3,267	4,155
Total accounts receivable, net	$ 46,266	$ 42,303

The following table presents the changes in the allowance for credit losses related to accounts receivable and contract assets for the year ended December 31, 2020 (in thousands):

	Accounts Receivable	Contract Assets
Balance at December 31, 2019	$ —	$ —
Provision for credit losses	7,678	—
Write-offs	—	—
Balance at December 31, 2020	$ 7,678	$ —

The Company's allowance for credit losses as of December 31, 2020 was related solely to accounts receivable from Abeona. Please refer to the section below, "Abeona Therapeutics Inc.", for further information regarding amounts due from Abeona and the associated allowance for credit losses. The Company's provision for credit losses for the year ended December 31, 2020 was $7.7 million and was related solely to changes in estimates regarding the allowance for credit losses associated with the accounts receivable from Abeona. No allowance for credit losses was recorded as of December 31, 2019, and no provision for credit losses was recorded for the years ended December 31, 2019 and 2018.

Novartis Gene Therapies

In March 2014, the Company entered into an exclusive license agreement, as amended, (the March 2014 License) with Novartis Gene Therapies (formerly AveXis, Inc.). Under the March 2014 License, the Company granted Novartis Gene Therapies an exclusive, worldwide commercial license, with rights to sublicense, to the NAV AAV9 vector for the treatment of SMA in humans by *in vivo* gene therapy. In consideration for the license, Novartis Gene Therapies paid the Company an up-front fee of $2.0 million, and is required to pay annual fees, development milestone payments of up to $12.3 million, mid-single to low double-digit royalties on net sales of licensed products, subject to reduction in specified circumstances, and a lower mid-double digit percentage of any sublicense fees Novartis Gene Therapies receives from sublicensees for the licensed intellectual property rights.

In January 2018, the Company and Novartis Gene Therapies amended the March 2014 License (the January 2018 Amendment). Under the January 2018 Amendment, the licensed intellectual property was expanded to include, in addition to the NAV AAV9 vector previously licensed, sublicenses to other third-party patents exclusively licensed by the Company and any other recombinant AAV vector in the Company's intellectual property portfolio during a period of 14 years from the effective date of the January 2018 Amendment, for the treatment of SMA in humans by *in vivo* gene therapy.

The January 2018 Amendment also modified the assignment provision of the March 2014 License. Under the amended assignment provision, Novartis Gene Therapies was permitted to transfer the March 2014 License without the Company's written consent in connection with a change of control of Novartis Gene Therapies, subject to certain conditions. Prior to the January 2018 Amendment, any assignment by Novartis Gene Therapies without the Company's prior written consent had been prohibited under the March 2014 License.

In consideration for the additional rights granted under the January 2018 Amendment, and in addition to any consideration owed under the original March 2014 License, Novartis Gene Therapies paid to the Company a fee of $80.0 million upon entry into the January 2018 Amendment. In addition, Novartis Gene Therapies was obligated to pay the Company (i) $30.0 million on the first

anniversary of the effective date of the January 2018 Amendment, (ii) $30.0 million on the second anniversary of the effective date of the January 2018 Amendment and (iii) potential sales-based milestone payments of up to $120.0 million. In the event of a change of control of Novartis Gene Therapies, to the extent that any fee described in (i) or (ii) above, or the first $40.0 million of sales-based milestone payments described in (iii) above, had not yet been paid to the Company, Novartis Gene Therapies was required to pay any such unpaid fee to the Company upon the change of control. For any product developed for the treatment of SMA using the NAV AAV9 vector, Novartis Gene Therapies will continue to be obligated to pay to the Company mid-single to low double-digit royalties on net sales as required by the March 2014 License, and for any product developed for the treatment of SMA using a licensed vector other than NAV AAV9, the Company will receive a low double-digit royalty on net sales.

In May 2018, AveXis, Inc. (now Novartis Gene Therapies) was acquired by Novartis, which qualified as a change of control under the January 2018 Amendment. Pursuant to the January 2018 Amendment, Novartis Gene Therapies paid the Company $100.0 million in accelerated license payments as a result of the change of control.

Novartis Gene Therapies launched commercial sales of Zolgensma, a licensed product under the March 2014 License, in the second quarter of 2019, upon which the Company began recognizing royalty revenue on net sales of the licensed product. In accordance with the license agreement, Novartis Gene Therapies was obligated to pay a sales-based milestone fee of $80.0 million to the Company upon the achievement of $1.0 billion in cumulative net sales of licensed products. Novartis Gene Therapies achieved cumulative net sales of Zolgensma of $1.0 billion in third quarter of 2020, upon which the Company recognized revenue of $80.0 million related to the sales-based milestone, and the Company received payment of the $80.0 million milestone fee in October 2020.

Accounting Analysis

The January 2018 Amendment was accounted for under Topic 606 as a modification of the license agreement resulting in a new and separate contract from the original March 2014 License for revenue recognition purposes. The Company determined that a substantive termination penalty is associated with Novartis Gene Therapies' termination rights under both the original March 2014 License and the January 2018 Amendment, and therefore the contract term for revenue recognition purposes is equal to the stated term of the amended license agreement. The only material performance obligation of the Company under the January 2018 Amendment was for the delivery of the modified license, which occurred upon the execution of the amendment in January 2018.

As of December 31, 2020, the transaction price of the original March 2014 License was $14.5 million. The transaction price included: (i) the up-front payment in March 2014 of $2.0 million, (ii) the present value of aggregate annual fees payable to the Company over the term of the license and (iii) $12.3 million of payments for development milestones achieved to date. The discounted portion of the annual fees represents the financing benefit provided to Novartis Gene Therapies and is recognized as interest income from licensing over the term of the license. Variable consideration under the original March 2014 License, which has been excluded from the transaction price, includes royalties on net sales of licensed products and any potential sublicense fees, if any, which will be recognized in the period of the underlying sales or sublicenses. The transaction price of the original March 2014 License increased by $3.5 million during the year ended December 31, 2020 as a result of development milestones achieved during the period which were previously excluded from the transaction price. As of December 31, 2020, all development milestones under the March 2014 License had been achieved and associated milestone payments had been received from Novartis Gene Therapies.

Upon its execution, the transaction price of the January 2018 Amendment was $132.1 million, which was fully recognized as license revenue upon the delivery of the modified license in January 2018. In May 2018, as a result of the acquisition of AveXis, Inc. (now Novartis Gene Therapies) by Novartis, the transaction price was increased by $40.0 million to account for the acceleration of the sale-based milestone which was previously excluded from the transaction price. The $40.0 million increase in the transaction price was recognized as license revenue upon the completion of the change of control in May 2018 since the amended license had been fully delivered by the Company. Additionally, due to the acceleration of the two $30.0 million payments originally due in January 2019 and January 2020, the Company recognized $6.1 million of interest income from licensing upon the completion of the change of control of AveXis, Inc., which represented the remaining present value discount on such payments as of the date of the change of control. As of December 31, 2020, the transaction price of the January 2018 Amendment was $172.1 million, which included: (i) the $80.0 million payment in January 2018, (ii) the present value, as of the date of the January 2018 Amendment, of the two $30.0 million payments originally due in January 2019 and January 2020 and (iii) the $40.0 million sales-based milestone which was accelerated upon the change of control in May 2018. Variable consideration under the January 2018 Amendment, which has been excluded from the transaction price, includes the $80.0 million sales-based milestone received in 2020 upon Novartis Gene Therapies' achievement of $1.0 billion in cumulative net sales of Zolgensma, royalties on net sales of licensed products and any potential sublicense fees, if any, which will be recognized in the period of the underlying sales or sublicenses. There were no increases in the transaction price of the January 2018 Amendment during the year ended December 31, 2020. As of December 31, 2020, all sales-based milestones under the January 2018 Amendment had been achieved and associated milestone payments had been received from Novartis Gene Therapies.

The Company recognized the following amounts under the March 2014 License with Novartis Gene Therapies (in thousands):

	Years Ended December 31,		
	2020	2019	2018
License revenue	$ 3,500	$ 3,500	$ 176,066
Royalties on net sales of Zolgensma	61,631	20,829	—
Achievement of sales-based milestone for Zolgensma	80,000	—	—
Total license and royalty revenue	$ 145,131	$ 24,329	$ 176,066
Interest income from licensing	$ 26	$ 29	$ 7,966

As of December 31, 2020, the Company had recorded total accounts receivable of $19.6 million from Novartis Gene Therapies under the March 2014 License, of which $19.4 million were included in current assets and $0.2 million were included in non-current assets. As of December 31, 2019, the Company had recorded total accounts receivable of $11.0 million from Novartis Gene Therapies under the March 2014 License, of which $10.8 million were included in current assets and $0.2 million were included in non-current assets. Current accounts receivable as of December 31, 2020 included unbilled Zolgensma royalties of $19.4 million, of which $9.2 million is expected to be paid to HCR in accordance with the Royalty Purchase Agreement discussed in Note 7.

Abeona Therapeutics Inc.

In November 2018, the Company entered into an exclusive license agreement, as amended, (the November 2018 License) with Abeona. Under the November 2018 License, the Company granted Abeona an exclusive, worldwide commercial license (subject to certain non-exclusive rights previously granted by the Company), with rights to sublicense, to the NAV AAV9 vector for the treatment of Mucopolysaccharidosis Type IIIA (MPS IIIA), also known as Sanfilippo Syndrome Type A, Mucopolysaccharidosis Type IIIB (MPS IIIB), also known as Sanfilippo Syndrome Type B, Neuronal Ceroid Lipfuscinosis-1 (CLN1 disease), also known as infantile Batten Disease, and Neuronal Ceroid Lipfuscinosis-3 (CLN3 disease), also known as juvenile Batten Disease, by *in vivo* gene therapy.

In November 2019, the November 2018 License was amended to increase the total amount of license fees payable to the Company by $1.0 million and modify the timing of the first required annual payment under the license agreement. In consideration for the license, as amended, Abeona was obligated to pay up-front and annual fees to the Company totaling up to $121.0 million, payable as follows: (i) $10.0 million upon the execution of the license agreement, (ii) $3.0 million within 12 months of the effective date of the license agreement, (iii) $8.0 million by April 1, 2020 and (iv) $100.0 million payable in five annual installments of $20.0 million beginning on the second anniversary of the effective date of the license agreement, which were subject to reduction should Abeona terminate some but not all of the licensed indications. Pursuant to the license agreement, any unpaid portion of the first $41.0 million of up-front and annual fees described above shall become payable upon termination of the license agreement. In the event of a change of control of Abeona, any remaining unpaid portion of the $121.0 million of up-front and annual fees described above shall become payable upon the change of control. Additionally, Abeona was obligated to pay the Company up to $60.0 million upon the achievement of specified sales-based milestones, low double-digit royalties on net sales of licensed products, subject to reduction in specified circumstances, and a lower mid-double-digit percentage of any sublicense fees Abeona receives from sublicensees for the licensed intellectual property rights.

Termination and Arbitration Proceedings

Pursuant to the November 2018 License, Abeona was required to pay a license fee of $8.0 million to the Company no later than April 1, 2020. Abeona failed to make this payment, and in April 2020, the Company delivered to Abeona a notice of its breach of the license agreement and written demand for payment. Upon expiration of the applicable cure period in May 2020, the license agreement was terminated. As a result of the termination, Abeona was required to pay a $20.0 million license fee to the Company within 15 days of the termination date, which otherwise would have been due to the Company in November 2020. As of February 25, 2021, the Company had not received any portion of the $28.0 million in license fees due from Abeona under the license agreement. Unpaid balances due under the November 2018 License accrue interest at 1.5% per month.

In May 2020, subsequent to the termination of the November 2018 License, Abeona filed a claim in arbitration alleging that the Company had breached certain responsibilities to communicate with Abeona regarding the Company's prosecution of licensed patents under the November 2018 License. The Company disputes Abeona's claim and filed a counterclaim in arbitration demanding payment of the $28.0 million of unpaid fees from Abeona, plus accrued interest. Based on its evaluation of the merits of Abeona's claims, the Company had not recorded any liabilities related these claims as of December 31, 2020, and the Company currently expects that its demand for payment in full will be upheld in arbitration. The Company intends to enforce the full collection of all amounts due from Abeona upon completion of arbitration, which is currently scheduled to occur in March 2021. However, the duration and outcome of arbitration and timing of payment from Abeona are unpredictable and uncertain at this time.

Accounting Analysis

The Company determined that the November 2018 License had an initial contract term of three years for revenue recognition purposes due to the lack of a substantive termination penalty for license periods beyond three years from the date of the license agreement. The annual payments which were due under the agreement beginning in November 2021 represented contract renewal options granted to Abeona for revenue recognition purposes, and therefore were not accounted for under the initial license agreement. The Company determined that the contract renewal options did not represent material rights granted to Abeona, and the only material performance obligations of the Company under the license agreement were for the delivery of the initial intellectual property licenses, which occurred upon the execution of the license agreement in November 2018.

Upon its execution, the transaction price of the November 2018 License was $35.6 million, which was fully recognized as license revenue upon the delivery of the licenses in November 2018. As a result of the November 2019 amendment to the license agreement, the transaction price was increased by $0.6 million to account for the modifications to the amount and timing of annual fees under the license. Upon its termination in May 2020, the transaction price of the November 2018 License, as amended, was $36.3 million, which included the following fixed consideration payable to the Company over contract term: (i) the $10.0 million payment in November 2018 and (ii) the present values, as of the date of the license agreement, of the $3.0 million payment due in November 2019, the $8.0 million payment due in April 2020 and the $20.0 million payment due in November 2020. The discounted portion of the annual payments represents the financing benefit provided to Abeona and was recognized as interest income from licensing over the financing term of the license, which ended upon the termination of the agreement in May 2020. Variable consideration under the license agreement, which was excluded from the transaction price, included the sales-based milestone payments of $60.0 million, as well as any potential royalties on sales of licensed products or sublicense fees, which would be recognized in the period of the underlying sales or sublicenses. The annual payments due under the agreement beginning in November 2021 represented contract renewal options granted to Abeona for revenue recognition purposes, and therefore were excluded from the transaction price.

During the years ended December 31, 2020, 2019 and 2018, the Company recognized license revenue of zero, $0.6 million and $35.6 million, respectively, and interest income from licensing of $3.8 million, $2.6 million and $0.4 million, respectively under the November 2018 License. Interest income from licensing recognized during the year ended December 31, 2020 includes $2.1 million of interest accrued on unpaid license fees recognized subsequent to the termination of the license agreement in May 2020, as discussed further below.

As of December 31, 2020, the Company had recorded gross accounts receivable of $30.1 million from Abeona under the November 2018 License, which consisted of the $8.0 million fee due April 1, 2020, the $20.0 million fee due within 15 days of the termination of the license agreement in May 2020 and accrued interest on the outstanding balances. While the Company currently expects its demand for payment in full will be upheld in arbitration, the Company assessed the collectability of the $30.1 million due from Abeona as of December 31, 2020 as it relates to credit risk. In performing this assessment, the Company evaluated Abeona's credit profile and financial condition, as well its expectations regarding Abeona's future cash flows and ability to satisfy this obligation upon the completion of arbitration in 2021. Additionally, the Company considered Abeona's continued failure to remit payment to the Company, as well as events which occurred during the year ended December 31, 2020 impacting Abeona's business and credit profile, specifically the departure of key members of Abeona's management and board of directors and subsequent decline in market capitalization. As a result of its evaluation, the Company recorded an allowance for credit losses of $7.7 million as of December 31, 2020 related to the accounts receivable due from Abeona. However, management intends to enforce the full collection of all amounts due from Abeona upon the completion of arbitration.

From the termination of the agreement in May 2020 to December 31, 2020, the Company recognized interest income from licensing of $2.1 million related to unpaid license fees from Abeona under the November 2018 License. In accordance with its interest accrual policy, the Company ceased the recognition of interest income accrued under the license agreement subsequent to the recognition of the allowance for credit losses in the third quarter of 2020, and will continue to maintain the accounts receivable from Abeona on non-accrual status unless and until such amounts are deemed to be collectable. However, management intends to enforce the full collection of all accrued interest contractually due from Abeona upon the completion of arbitration.

11. Stock-based Compensation

In September 2014, the Board of Directors adopted the 2014 Stock Plan (2014 Plan). In June 2015, the Board of Directors adopted the 2015 Equity Incentive Plan (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, 2020, the total number of shares of common stock authorized for issuance under the 2015 Plan and 2014 Plan was 12,412,917, of which 2,291,626 remained available for future grants under the 2015 Plan. In January 2021, the Board of Directors authorized an additional 1,499,037 shares to be issued under the 2015 Plan.

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 vest in accordance with the underlying award agreements and, upon vesting, 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,		
	2020	2019	2018
Stock options	$ 31,178	$ 25,964	$ 15,960
Restricted stock units	—	257	275
Employee stock purchase plan	771	633	406
	$ 31,949	$ 26,854	$ 16,641

As of December 31, 2020, the Company had $62.1 million of unrecognized stock-based compensation expense related to stock options and the 2015 Employee Stock Purchase Plan (the 2015 ESPP), which is expected to be recognized over a weighted-average period of 2.4 years.

The Company recorded aggregate stock-based compensation expense in the consolidated statements of operations and comprehensive income (loss) as follows (in thousands):

	Years Ended December 31,		
	2020	2019	2018
Research and development	$ 16,280	$ 13,031	$ 7,612
General and administrative	15,669	13,823	9,029
	$ 31,949	$ 26,854	$ 16,641

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, 2019	5,544	$ 28.79	7.5	$ 86,509
Granted	1,633	$ 37.84		
Exercised	(435)	$ 12.95		
Cancelled or forfeited	(381)	$ 45.04		
Outstanding at December 31, 2020	6,361	$ 31.21	7.2	$ 101,356
Exercisable at December 31, 2020	3,703	$ 24.27	6.2	$ 84,115
Vested and expected to vest at December 31, 2020	6,361	$ 31.21	7.2	$ 101,356

(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, 2020, 2019 and 2018 was $23.82, $31.19 and $27.49, respectively. During the years ended December 31, 2020, 2019 and 2018, the total number of stock options exercised was 434,534, 796,847 and 1,684,522, respectively, resulting in total proceeds of $5.6 million, $7.1 million and $14.5 million, respectively. The total intrinsic value of options exercised during the years ended December 31, 2020, 2019 and 2018 was $11.5 million, $30.8 million and $68.2 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,		
	2020	2019	2018
Expected volatility	71%	74%	75%
Expected term (in years)	6.0	6.1	6.0
Risk-free interest rate	1.4%	2.3%	2.6%
Expected dividend yield	0.0%	0.0%	0.0%

Restricted Stock Units

The Company had no unvested restricted stock units outstanding as of December 31, 2020 and 2019, nor at any point during the year ended December 31, 2020. No restricted stock units vested during the year ended December 31, 2020. The total intrinsic value of restricted stock units vested during the year ended December 31, 2019 was $1.8 million. No restricted stock units vested during the year ended December 31, 2018.

Employee Stock Purchase Plan

In June 2015, the Company's 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 Company's 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, 2020, the total number of shares of common stock authorized for issuance under the 2015 ESPP was 623,924, of which 448,011 remained available for future issuance. During the years ended December 31, 2020, 2019 and 2018, 55,499, 35,994 and 36,700 shares of common stock, respectively, were issued under the 2015 ESPP. In January 2021, the Board of Directors authorized an additional 374,759 shares to be 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, 2020, 2019 and 2018, the Company incurred expenses of $2.2 million, $1.8 million and $1.3 million, respectively, for matching contributions to the 401(k) Plan.

13. Income Taxes

The components of income (loss) before income taxes were as follows (in thousands):

	Years Ended December 31,		
	2020	2019	2018
United States	$ (105,960)	$ (97,435)	$ 104,181
Foreign	(50)	(53)	(65)
Total income (loss) before income taxes	$ (106,010)	$ (97,488)	$ 104,116

The components of the provision for income tax expense (benefit) were as follows (in thousands):

	Years Ended December 31,		
	2020	2019	2018
Current:			
Federal	$ —	$ —	$ —
State	5,240	(2,288)	4,179
Foreign	—	—	—
Total current	5,240	(2,288)	4,179
Deferred:			
Federal	—	(284)	—
State	—	(183)	—
Foreign	—	—	—
Total deferred	—	(467)	—
Total income tax expense (benefit)	$ 5,240	$ (2,755)	$ 4,179

In response to the COVID-19 pandemic, the Coronavirus Aid, Relief and Economic Security Act (the CARES Act) was signed into law in March 2020. The CARES Act (i) lifts certain deduction limitations originally imposed by the TCJA, (ii) allows corporate taxpayers to carryback net operating losses (NOLs) originating during 2018 through 2020 for up to five years, which was not previously allowed under the TCJA, (iii) eliminates the 80% of taxable income limitations on NOL utilization imposed by the TCJA, allowing corporate entities to fully utilize NOL carryforwards to offset taxable income in 2018, 2019 or 2020, and (iv) enacts various other changes to corporate taxation. Also included in the CARES Act was a change to the TCJA related to qualified improvement property, retroactively allowing for a 15-year recovery period and bonus depreciation. As a result of this change, the Company recorded current income tax benefit of $0.5 million for the year ended December 31, 2020 related to a reduction of state taxes associated with additional depreciation deductions allowed for the 2018 tax year. Overall, the enactment of the CARES Act, including the change for qualified improvement property, did not result in any material adjustments to the Company's income tax provision for the year ended December 31, 2020, or to the Company's net deferred tax assets as of December 31, 2020.

A reconciliation of income tax expense (benefit) computed at the statutory federal income tax rate of 21% to income tax expense (benefit) as reflected in the consolidated statements of operations and comprehensive income (loss) is as follows (in thousands):

	Years Ended December 31,		
	2020	2019	2018
Federal income tax expense (benefit) at statutory rate	$ (22,263)	$ (20,473)	$ 21,862
State income tax expense (benefit), net of federal tax effect	(11,495)	(15,323)	9,691
Research and development credits	(7,793)	(11,075)	(7,847)
Stock-based compensation	587	(2,134)	(6,493)
Other non-deductible expenses and reconciling items	886	144	139
Change in corporate tax rates	109	130	(729)
Change in valuation allowance	45,209	45,976	(12,444)
Total income tax expense (benefit)	$ 5,240	$ (2,755)	$ 4,179

The significant components of the Company's net deferred tax assets were as follows (in thousands):

	December 31, 2020	December 31, 2019
Deferred tax assets:		
Net operating loss carryforwards	$ 14,683	$ 53,841
Research and development tax credits	47,970	40,365
Stock-based compensation	15,644	10,224
Lease liabilities	21,961	3,916
Liability related sale of future royalties	62,089	—
Depreciation and amortization	—	26
Accruals and other	7,370	4,779
Total deferred tax assets before valuation allowance	169,717	113,151
Valuation allowance	(142,907)	(97,511)
Total deferred tax assets	26,810	15,640
Deferred tax liabilities:		
Unrealized gains on marketable securities	(163)	(11,344)
Right-of-use assets	(21,094)	(3,467)
Depreciation and amortization	(5,155)	—
Other	(398)	(829)
Total deferred tax liabilities	(26,810)	(15,640)
Net deferred tax assets	$ —	$ —

The Company evaluated the positive and negative evidence bearing upon the realizability of its deferred tax assets. Based on the Company's history of operating losses, including a three-year cumulative loss position as of December 31, 2020, the Company has concluded that it is 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, 2020 and 2019. The valuation allowance increased by $45.4 million and $46.0 million during the years ended December 31, 2020 and 2019, respectively. The increase in the valuation allowance during the year ended December 31, 2020 was due primarily to an increase in deferred tax assets resulting from the sale of the Company's Zolgensma royalty rights to HCR, which was treated as a sale for tax purposes, research and development credits generated during the period, stock-based compensation expense and realized gains on sales of marketable securities, the impact of which was partially offset by the utilization of federal and state NOLs during the period. The increase in the valuation allowance during the year ended December 31, 2019 was due primarily to federal and state NOLs and research and development credits generated during the period, stock-based compensation expense and other increases in deferred tax assets during the period, the impact of which was partially offset by unrealized gains on marketable securities.

As of December 31, 2020 and 2019, the Company had U.S. federal NOL carryforwards of approximately $44.7 million and $170.4 million, respectively, and U.S. state NOL carryforwards of approximately $79.7 million and $269.3 million, respectively, which may be available to offset future income tax liabilities. The Company's U.S. federal NOL carryforwards as of December 31, 2020 may be carried forward indefinitely. A portion of the Company's U.S. state NOL carryforwards as of December 31, 2020 may be carried forward indefinitely, with the remaining portion expiring at various dates between 2034 and 2040.

As of December 31, 2020 and 2019, the Company had U.S. federal and state research and development tax credit carryforwards of approximately $48.0 million and $40.4 million, respectively, net of unrecognized tax benefits of $0.1 million and $0.1 million, respectively, which may be available to reduce future income tax liabilities and expire at various dates between 2035 and 2040. 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 studies 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, 2020 and 2019, 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. No reserve for uncertain tax positions has been placed against qualified expenses for which a study has not been conducted. However, 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 does not expect that a significant portion of its unrecognized tax benefits will increase or decrease in the next 12 months as of December 31, 2020.

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, 2016 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. Related Party Transactions

FOXKISER LLP

Since 2016, the Company has been 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 pays a fixed monthly fee in consideration for certain strategic services provided by FOXKISER. Effective January 2019, the Company entered into a new professional services agreement with FOXKISER with similar terms and conditions as the previous agreements. The agreement was amended effective June 2019 to expand the scope of the services provided and increase the monthly fee. Effective August 2020, the agreement was further amended to extend the term of the agreement by two years through December 2022. The agreement may be terminated by either party with six months' advanced written notice. Expenses incurred under the agreements with FOXKISER for the years ended December 31, 2020, 2019 and 2018 were $4.8 million, $4.1 million and $2.1 million, respectively, and were recorded as research and development expenses in the consolidated statements of operations and comprehensive income (loss).

15. Net Income (Loss) Per Share

The computations of basic and diluted net income (loss) per share were as follows (in thousands, except per share data):

	Years Ended December 31,		
	2020	2019	2018
Basic net income (loss) per share:			
Net income (loss)	$ (111,250)	$ (94,733)	$ 99,937
Shares used in computation:			
Weighted-average common shares outstanding	37,281	36,690	33,427
Basic net income (loss) per share	$ (2.98)	$ (2.58)	$ 2.99
Diluted net income (loss) per share:			
Net income (loss)	$ (111,250)	$ (94,733)	$ 99,937
Shares used in computation:			
Weighted-average common shares outstanding	37,281	36,690	33,427
Stock options	—	—	3,186
Restricted stock units	—	—	32
Employee stock purchase plan	—	—	3
Weighted-average diluted common shares	37,281	36,690	36,648
Diluted net income (loss) per share	$ (2.98)	$ (2.58)	$ 2.73

For periods in which the Company incurred net losses, common stock equivalents were excluded from the calculation of diluted net loss per share as their effect would be anti-dilutive. Accordingly, basic and diluted net loss per share were the same for such periods. 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,		
	2020	2019	2018
Stock options issued and outstanding	6,361	5,544	928
Employee stock purchase plan	19	17	—
	6,380	5,561	928

16. Supplemental Disclosures

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

	December 31, 2020	December 31, 2019
Accrued personnel costs	$ 13,155	$ 10,903
Accrued sublicense fees and royalties	12,160	4,542
Accrued external research and development expenses	9,738	5,791
Accrued purchases of property and equipment	7,853	1,328
Accrued income taxes payable	3,135	—
Accrued external general and administrative expenses	2,865	2,053
Other accrued expenses and current liabilities	176	229
	$ 49,082	$ 24,846

17. Selected Quarterly Financial Information (Unaudited)

The following tables contain quarterly financial information for the years ended December 31, 2020 and 2019. Management believes that the following information reflects all normal recurring adjustments necessary for a fair statement of the information for the periods presented. The operating results for any quarter are not necessarily indicative of results for any future period. Amounts in the following table are in thousands, except per share data.

	Quarters Ended			
	March 31, 2020	June 30, 2020	September 30, 2020	December 31, 2020
Total revenues	$ 17,644	$ 16,566	$ 98,912	$ 21,445
Cost of revenues	3,409	4,684	17,364	10,257
Research and development expense	37,035	38,111	43,968	47,180
General and administrative expense	14,833	15,554	15,859	17,571
Total operating expenses	55,344	58,399	84,961	75,096
Net income (loss)	(40,038)	(33,762)	8,791	(46,241)
Net income (loss) per share:				
Basic	$ (1.08)	$ (0.91)	$ 0.24	$ (1.24)
Diluted	(1.08)	(0.91)	0.23	(1.24)

	Quarters Ended			
	March 31, 2019	June 30, 2019	September 30, 2019	December 31, 2019
Total revenues	$ 884	$ 7,881	$ 14,700	$ 11,768
Cost of revenues	29	1,927	2,494	3,791
Research and development expense	25,203	29,483	35,692	33,807
General and administrative expense	11,558	13,405	12,402	14,450
Total operating expenses	36,790	44,753	50,596	52,092
Net income (loss)	(32,228)	(1,457)	(34,584)	(26,464)
Net income (loss) per share:				
Basic	$ (0.89)	$ (0.04)	$ (0.94)	$ (0.72)
Diluted	(0.89)	(0.04)	(0.94)	(0.72)

EXHIBIT INDEX

Exhibit Number	Description	Incorporated by Reference			Filed or Furnished Herewith
		Form	Exhibit Number	Filing Date	
3.1	Restated Certificate of Incorporation	8-K	3.1	9/22/15	
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
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				X
10.5*	Form of Stock Option Award Agreement for the 2015 Equity Incentive Plan				X
10.6*	2015 Employee Stock Purchase Plan	S-1/A	10.4	9/8/15	
10.7*	Employment Agreement effective as of June 30, 2015 between the Registrant and Kenneth T. Mills	S-1	10.5	8/17/15	
10.8*	Employment Agreement effective as of June 30, 2015 between the Registrant and Vittal Vasista	S-1	10.7	8/17/15	
10.9*	Employment Agreement effective as of February 16, 2016 between the Registrant and Curran Simpson	10-K	10.34	3/3/16	
10.10*	Employment Agreement effective as of August 18, 2016 between the Registrant and Patrick J. Christmas	10-Q	10.37	11/9/16	
10.11*	Employment Agreement effective as of March 27, 2017 between the Registrant and Olivier Danos, Ph.D.	10-Q	10.1	5/9/17	
10.12*	Employment Agreement effective as of April 17, 2019 between the Registrant and Steve Pakola, M.D.	10-Q	10.1	5/7/19	
10.13*	Compensation Program for Non-Employee Directors	10-Q	10.1	11/4/20	
10.14*	Management Cash Incentive Plan	S-1	10.29	8/17/15	
10.15†	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	
10.16†	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	
10.17†	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	
10.18†	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	
10.19†	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	
10.20†	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	

Exhibit Number	Description	Incorporated by Reference			Filed or Furnished Herewith
		Form	Exhibit Number	Filing Date	
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†	License Agreement dated November 4, 2018 between the Registrant and Abeona Therapeutics Inc.	10-K	10.22	2/27/19	
10.26†	First Amendment to License Agreement dated November 4, 2019 between the Registrant and Abeona Therapeutics Inc.	10-K	10.23	2/26/20	
10.27	Lease dated March 6, 2015 between the Registrant and BMR-Medical Center Drive LLC	S-1	10.26	8/17/15	
10.28	First Amendment to Lease dated September 30, 2015 between the Registrant and BMR-Medical Center Drive LLC	10-K	10.31	3/3/16	
10.29	Second Amendment to Lease dated November 23, 2015 between the Registrant and BMR-Medical Center Drive LLC	10-K	10.32	3/3/16	
10.30	Third Amendment to Lease dated July 21, 2017 between the Registrant and BMR-Medical Center Drive LLC	10-Q	10.1	8/8/17	
10.31	Fourth Amendment to Lease dated April 20, 2018 between the Registrant and BMR-Medical Center Drive LLC	10-Q	10.1	5/8/18	
10.32	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	
10.33	Lease dated May 16, 2016 between the Registrant and 400 Madison Holdings, LLC	8-K	10.1	6/3/19	
10.34	First Amendment to Lease dated May 28, 2019 between the Registrant and DS400OWNER, LLC, as successor-in-interest to 400 Madison Holdings, LLC	8-K	10.2	6/3/19	
10.35	Lease dated January 28, 2016 between the Registrant and TNREF III 9600 Blackwell, LLC	10-K	10.33	3/3/16	
10.36	First Amendment to Lease dated November 3, 2017 between the Registrant and 9600 Blackwell II LLC, as successor in interest to TNREF III 9600 Blackwell, LLC	10-Q	10.1	11/8/17	
10.37	Lease dated November 1, 2018 between the Registrant and ARE-Maryland No. 24, LLC	10-Q	10.1	11/7/18	
10.38	Letter Agreement to Lease dated April 12, 2019 between the Registrant and ARE-Maryland No. 24, LLC	10-Q	10.3	5/7/19	
10.39	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	
10.40	Second Amendment to Lease dated November 4, 2019 between the Registrant and ARE-Maryland No. 24, LLC	10-Q	10.1	11/5/19	
10.41	Third Amendment to Lease dated October 30, 2020 between the Registrant and ARE-Maryland No. 24, LLC	10-Q	10.4	11/4/20	
10.42†	Royalty Purchase Agreement dated December 22, 2020 between the Registrant and entities managed by Healthcare Royalty Management, LLC				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
101	The following materials from the Registrant's Annual Report on Form 10-K for the fiscal year ended December 31, 2020 formatted in Inline XBRL (eXtensible Business Reporting Language): (i) Consolidated Balance Sheets (ii) Consolidated Statements of Operations and Comprehensive Income (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, 2020 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 1, 2021.

REGENXBIO INC.

By: /s/ Kenneth T. Mills
Kenneth T. Mills,
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/ Kenneth T. Mills Kenneth T. Mills	President, Chief Executive Officer and Director (Principal Executive Officer)	March 1, 2021
/s/ Vittal Vasista Vittal Vasista	Chief Financial Officer (Principal Financial and Accounting Officer)	March 1, 2021
/s/ Allan M. Fox Allan M. Fox	Chairman of the Board of Directors	March 1, 2021
/s/ Daniel J. Abdun-Nabi Daniel J. Abdun-Nabi	Director	March 1, 2021
/s/ Luke M. Beshar Luke M. Beshar	Director	March 1, 2021
/s/ Alexandra Glucksmann Alexandra Glucksmann	Director	March 1, 2021
/s/ A.N. “Jerry” Karabelas A.N. “Jerry” Karabelas	Director	March 1, 2021
/s/ David C. Stump David C. Stump	Director	March 1, 2021
/s/ Daniel Tassé Daniel Tassé	Director	March 1, 2021

EXHIBIT 4.2

DESCRIPTION OF REGISTRANT'S SECURITIES
REGISTERED PURSUANT TO SECTION 12 OF THE
SECURITIES EXCHANGE ACT OF 1934

REGENXBIO Inc. has one class of securities registered under Section 12 of the Securities Exchange Act of 1934, as amended: our common stock, par value $0.0001 per share. The following is a general summary of the terms of shares of our common stock. The description below does not include all of the terms of the shares of common stock and should be read together with our restated certificate of incorporation and amended and restated bylaws, each of which are incorporated by reference as an exhibit to the Annual Report on Form 10-K of which this Exhibit is a part. Unless the context otherwise requires, throughout this document, the words "REGENXBIO," "we," or "us" refer to REGENXBIO Inc.

Description of Common Stock

Authorized Capital Stock

Our authorized capital stock consists of 110,000,000 shares, with a par value of $0.0001 per share, of which 100,000,000 shares are designated as common stock and 10,000,000 shares are designated as preferred stock. The outstanding shares of our common stock are fully paid and non-assessable.

Dividend Rights

Subject to preferences that may apply to shares of preferred stock outstanding at the time, the holders of outstanding shares of our common stock are entitled to receive dividends out of assets legally available at the times and in the amounts that our board of directors may determine from time to time.

Preemptive and Conversion Rights

Holders of common stock have no preemptive or conversion rights or other subscription rights.

Redemption and Sinking Fund Rights

There are no redemption or sinking fund provisions applicable to our common stock.

Liquidation Rights

Upon our liquidation, dissolution or winding-up, the holders of common stock are entitled to share ratably in all assets remaining after payment of all liabilities and the liquidation preferences of any outstanding preferred stock.

Voting Rights

Each holder of common stock is entitled to one vote per share on all matters submitted to a vote of stockholders. Notwithstanding the previous sentence, unless otherwise provided by law holders of common stock are not entitled to vote on any amendment to our restated certificate of incorporation that relates solely to the terms of any preferred stock if the holders of such preferred stock are entitled to vote on such amendment.

We have not provided for cumulative voting in the election of directors.

The General Corporation Law of the State of Delaware, or the Delaware General Corporation Law, provides that holders of a class of stock will have the right to vote separately as a class on any proposal involving fundamental changes in the rights of the holders of that class of stock for proposals that adversely affect such holders.

Anti-Takeover Effects of Delaware Law and Our Restated Certificate of Incorporation and Amended and Restated Bylaws

Delaware law, our restated certificate of incorporation and our amended and restated bylaws contain provisions that could make it more difficult to effect an acquisition of us by means of a tender offer, proxy contest or otherwise, or to remove our incumbent officers and directors. It is possible that these provisions could make it more difficult to accomplish, or could deter, transactions that stockholders otherwise consider to be in our or their best interest, including transactions which provide for payment of a premium over the market price for our shares.

These provisions, summarized below, are intended to discourage coercive takeover practices and inadequate takeover bids. These provisions are also designed to encourage persons seeking to acquire control of us to first negotiate with our board of directors. We believe that the benefits of the increased protection of our potential ability to negotiate with the proponent of an unsolicited or unfriendly proposal to acquire or restructure us outweigh the disadvantages of discouraging these proposals because negotiation of these proposals could result in an improvement of their terms.

Delaware Business Combination Statute

We are subject to Section 203 of the Delaware General Corporation Law. Section 203 prohibits a publicly held Delaware corporation from engaging in a "business combination" with an "interested stockholder" for a period of three years following the time of the transaction in which the person or entity became an interested stockholder, unless:

- prior to that time, either the business combination or the transaction that resulted in the stockholder becoming an interested stockholder is approved by the board of directors of the corporation;
- upon consummation of the transaction that resulted in the stockholder becoming an interested stockholder, the interested stockholder owned at least 85% of the outstanding voting stock of the corporation, excluding for this purpose shares owned by persons who are directors and also officers of the corporation and by specified employee benefit plans; or
- at or after such time, the business combination is approved by the board of directors of the corporation and by the affirmative vote, and not by written consent, of at least 66 2/3% of the outstanding voting stock that is not owned by the interested stockholder.

For the purposes of Section 203, a "business combination" is broadly defined to include:

- any merger or consolidation involving the corporation and the interested stockholder;
- any sale, transfer, pledge or other disposition of 10% or more of the assets of the corporation involving the interested stockholder;
- any transaction that results in the issuance or transfer by the corporation of any stock of the corporation to the interested stockholder, subject to limited exceptions;
- any transaction involving the corporation that has the effect of increasing the proportionate share of the stock of any class or series of the corporation beneficially owned by the interested stockholder; or
- the receipt by the interested stockholder of the benefit of any loans, advances, guarantees, pledges or other financial benefits provided by or through the corporation.

An "interested stockholder" is a person who, together with affiliates and associates, owns or within the immediately preceding three years did own 15% or more of the corporation's voting stock.

Undesignated Preferred Stock

The ability of our board of directors, without action by the stockholders, to issue up to 10,000,000 shares of undesignated preferred stock with voting or other rights or preferences as designated by our board of directors could impede the success of any attempt to change control of us. The existence of authorized but unissued shares of preferred stock may have the effect of deferring hostile takeovers or delaying changes in control or management of our company.

Stockholder Action by Written Consent; Stockholder Meetings

Our restated certificate of incorporation and amended and restated bylaws eliminate the right of stockholders to act by written consent without a meeting. As a result, a holder controlling a majority of our capital stock would not be able to amend our bylaws or remove directors without holding a meeting of our stockholders called in accordance with our amended and restated bylaws. Our amended and restated bylaws provide that a special meeting of stockholders may be called only by our chairman of the board or president, or by a resolution adopted by a majority of our board of directors. These provisions might delay the ability of our stockholders to force consideration of a proposal or for stockholders controlling a majority of our capital stock to take any action, including the removal of directors.

Requirements for Advance Notification of Stockholder Nominations and Proposals

Our amended and restated bylaws establish advance notice procedures with respect to stockholder proposals to be brought before a stockholder meeting and the nomination of candidates for election as directors, other than nominations made by or at the direction of the board of directors or a committee of the board of directors. These provisions could have the effect of delaying until the next stockholder meeting stockholder actions that are favored by the holders of a majority of our outstanding voting securities.

Staggered Board

Our board of directors is divided into three classes. The directors in each class will serve for a three-year term, one class being elected each year by our stockholders. This system of electing and removing directors may discourage a third party from making a tender offer or otherwise attempting to obtain control of us, because it generally makes it more difficult for stockholders to replace a majority of the directors.

Removal of Directors

Our restated certificate of incorporation provides that no member of our board of directors may be removed from office by our stockholders except for cause and, in addition to any other vote required by law, upon the approval of holders of at least two-thirds of the total voting power of all of our outstanding voting stock then entitled to vote in the election of directors.

Board of Directors Vacancies

Our restated certificate of incorporation and amended and restated bylaws authorize our board of directors to fill vacant directorships. In addition, the number of directors constituting our board of directors is set only by resolution adopted by a majority vote of our entire board of directors. These provisions will prevent a stockholder from increasing the size of our board of directors and gaining control of our board of directors by filling the resulting vacancies with its own nominees.

Stockholders Not Entitled to Cumulative Voting

Our restated certificate of incorporation does not permit stockholders to cumulate their votes in the election of directors. Accordingly, the holders of a majority of the outstanding shares of our common stock entitled to vote in any election of directors can elect all of the directors standing for election, if they choose, other than any directors that holders of our preferred stock may be entitled to elect.

Amendment of Charter Provisions

The amendment of any of the above provisions, except for the provision making it possible for our board of directors to issue preferred stock, would require approval by holders of at least two-thirds of the total voting power of all of our outstanding voting stock.

The provisions of Delaware law, our restated certificate of incorporation and our amended and restated bylaws could have the effect of discouraging others from attempting hostile takeovers and, as a consequence, they may also inhibit temporary fluctuations in the market price of our common stock that often result from actual or rumored hostile takeover attempts. These provisions may also have the effect of preventing changes in the composition of our board and management. It is possible that these provisions could make it more difficult to accomplish transactions that stockholders may otherwise deem to be in their best interests.

Choice of Forum

Our restated certificate of incorporation provides that 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 exclusive forum for any derivative action or proceeding brought on our behalf; any action asserting a breach of fiduciary duty; any action asserting a claim against us arising pursuant to the Delaware General Corporation Law, our restated certificate of incorporation or amended and restated bylaws; or any action asserting a claim against us that is 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, or is a "foreign action" (as defined in our restated certificate of incorporation), in the name of any stockholder, such stockholder shall be deemed to have consented to (i) 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 (ii) 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. The enforceability of similar choice of forum provisions in other companies' certificates of incorporation has been challenged in legal proceedings, and it is possible that a court could find these types of provisions to be inapplicable or unenforceable.

Indemnification

Our restated certificate of incorporation includes provisions that limit the liability of our directors for monetary damages for breach of their fiduciary duty as directors, except for liability that cannot be eliminated under the Delaware General Corporation Law. Accordingly, our directors will not be personally liable for monetary damages for breach of their fiduciary duty as directors, except for liabilities:

- for any breach of the director's duty of loyalty to us or our stockholders;
- for acts or omissions not in good faith or which involve intentional misconduct or a knowing violation of law;
- for unlawful payments of dividends or unlawful stock repurchases or redemptions, as provided under Section 174 of the Delaware General Corporation Law; or
- for any transaction from which the director derived an improper personal benefit.

Any amendment or repeal of these provisions will require the approval of the holders of shares representing at least two-thirds of the shares entitled to vote in the election of directors, voting as one class.

Our restated certificate of incorporation and amended and restated bylaws also provide that we will indemnify our directors and officers to the fullest extent permitted by Delaware law. Our restated certificate of incorporation and amended and restated bylaws also permit us to purchase insurance on behalf of any officer, director, employee or other agent for any liability arising out of his or her actions as our officer, director, employee or agent, regardless of whether Delaware law would permit indemnification. We have entered into separate

indemnification agreements with our directors and officers that require us, among other things, to indemnify them against certain liabilities that may arise by reason of their status or service as directors and to advance their expenses incurred as a result of any proceeding against them as to which they could be indemnified. We believe that the limitation of liability provision in our restated certificate of incorporation and the indemnification agreements facilitate our ability to continue to attract and retain qualified individuals to serve as directors and officers. The limitation of liability and indemnification provisions in our restated certificate of incorporation and amended and restated bylaws may discourage stockholders from bringing a lawsuit against directors for breach of their fiduciary duties. They may also reduce the likelihood of derivative litigation against directors and officers, even though an action, if successful, might benefit us and our stockholders. A stockholder's investment may be harmed to the extent we pay the costs of settlement and damage awards against directors and officers pursuant to these indemnification provisions.

Stock Exchange Listing

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

Transfer Agent and Registrar

The transfer agent and registrar for our common stock is Computershare Trust Company, N.A.

EXHIBIT 10.4

REGENXBIO Inc.
2015 Equity Incentive Plan
Notice of Restricted Stock Unit Award

You have been granted units representing shares of Common Stock of REGENXBIO Inc. (the "Company") on the following terms:

Name of Recipient:	**[Participant Name]**
Total Number of Units Granted:	**[Number of Units]**
Date of Grant:	**[Grant Date]**
Vesting Schedule:	**[Vesting Schedule]**

You and the Company agree that these units are granted under and governed by the terms and conditions of the REGENXBIO Inc. 2015 Equity Incentive Plan (the "Plan") and the Restricted Stock Unit Award Agreement, both of which are attached to and made a part of this document.

You further agree that the Company may deliver and you shall accept by email all documents relating to the Plan or this award (including, without limitation, prospectuses required by the Securities and Exchange Commission) and all other documents that the Company is required to deliver to its security holders (including, without limitation, annual reports and proxy statements). You also agree that the Company may deliver these documents by posting them on a website maintained by the Company or by a third party under contract with the Company. If the Company posts these documents on a website, you will be notified by email. You acknowledge that you may incur costs in connection with electronic delivery, including the cost of accessing the internet and printing fees, and that an interruption of internet access may interfere with your ability to access the documents. This consent will remain in effect until you give the Company written notice that it should deliver paper documents.

You further agree to comply with the Company's Insider Trading Policy when selling shares of the Company's Common Stock.

Recipient:

[Participant Name]

REGENXBIO Inc.

By:
Name: **[Authorized Individual Name]**
Title: **[Authorized Individual Title]**

REGENXBIO Inc.
2015 Equity Incentive Plan

Restricted Stock Unit Award Agreement

Payment for Units	No payment is required for the units that you are receiving.
Vesting	The units vest as shown in the Notice of Stock Unit Award. No additional units vest after your Service has terminated for any reason.
Forfeiture	If your Service terminates for any reason, then your units will be forfeited to the extent that they have not vested before the termination date. This means that any units that have not vested under this Agreement will be cancelled immediately. You receive no payment for units that are forfeited. The Company determines when your Service terminates for this purpose.
Settlement of Units	Each unit will be settled promptly following the applicable Vesting Date. However, each unit must be settled no later than the March 15th of the calendar year following the calendar year in which it vests. At the time of settlement, you will receive one share of the Company's Common Stock for each vested unit.
"Permissible Trading Day"	"Permissible Trading Day" means a day that satisfies each of the following requirements: •The Nasdaq Global Select Market is open for trading on that day; •You are permitted to sell shares of the Company's Common Stock on that day without incurring liability under Section 16(b) of the Securities Exchange Act of 1934, as amended; •Either (a) you are not in possession of material non-public information that would make it illegal for you to sell shares of the Company's Common Stock on that day under Rule 10b-5 under the Securities and Exchange Commission or (b) Rule 10b5-1 under the Securities and Exchange Commission is applicable; •Under the Company's Insider Trading Policy, you are permitted to sell shares of the Company's Common Stock on that day; and •You are not prohibited from selling shares of the Company's Common Stock on that day by a written agreement between you and the Company or a third party.

Section 409A

This paragraph applies only if the Company determines that you are a "specified employee," as defined in the regulations under Section 409A of the Internal Revenue Code of 1986, as amended (the "Code"), at the time of your "separation from service," as defined in those regulations. If this paragraph applies, and any units subject to this award constitute nonqualified deferred compensation within the meaning of Section 409A of the Code, then any units that otherwise would have been settled during the first six months following your separation from service will instead be settled during the seventh month following your separation from service.

Nature of Units

Your units are mere bookkeeping entries. They represent only the Company's unfunded and unsecured promise to issue shares of Common Stock on a future date. As a holder of units, you have no rights other than the rights of a general creditor of the Company.

No Voting Rights or Dividends

Your units carry neither voting rights nor rights to receive dividends. You have no rights as a stockholder of the Company unless and until your units are settled by issuing shares of the Company's Common Stock.

Units Nontransferable

You may not sell, transfer, assign, pledge or otherwise dispose of any units. For instance, you may not use your units as security for a loan.

Withholding Taxes

Unless you elect at least 30 calendar days prior to the applicable Vesting Date, which election must be made on a Permissible Trading Day, to satisfy the applicable withholding taxes upon such Vesting Date ("Withholding Taxes") through any of the following means or by a combination of such means: (i) withholding from any compensation otherwise payable to you by the Company or an Affiliate; or (ii) tendering a cash payment to the Company (which may be in the form of a check, electronic wire transfer or other method permitted by the Company), then to the greatest extent permitted under the Plan and applicable law, the Company may elect in its sole discretion to satisfy applicable Withholding Taxes through either (a) the Company withholding the number of units necessary to satisfy any tax withholding obligation that arises in connection with the Award (a "net settlement"); or (b) the sale of a number of the shares subject to the Award and the remittance of the cash proceeds of such sale to the Company (a "same day sale"). If the Company elects the "same day sale" method, you authorize the Company to make payment from the cash proceeds of this sale directly to the appropriate taxing authorities in an amount equal to the Withholding Taxes. ***It is the Company's intent that the mandatory sale to cover Withholding Taxes imposed by the Company on the Participant in connection with the receipt of this Award comply with the requirements of Rule 10b5-1(c)(1)(i)(B) under the Exchange Act and be interpreted to comply with the requirements of Rule 10b5-1(c).***

If, for any reason, a "net settlement" or "same day sale" does not result in sufficient proceeds to satisfy the Withholding Taxes, the Company or an Affiliate may, in its sole discretion, satisfy any remaining portion of the Withholding Taxes using any other means permitted by law or under the terms of the Plan.

Notwithstanding any other provision of the Award or the Plan, if the recipient of an Award is subject to Section 16 of the Securities Exchange Act of 1934, as amended (pursuant to Rule 16a-2 thereunder), at the time that all or any portion of the Award becomes subject to tax of any kind (including, but not limited to, federal, state, local, or non-U.S. income or employment tax), then the Company shall satisfy such withholding obligations using the "net settlement" method described above.

Unless the tax withholding obligations of the Company and/or any Affiliate are satisfied, the Company shall have no obligation to deliver to you any Common Stock. Withholding Taxes shall be equal to the Company's required tax withholding obligations using the minimum statutory withholding rates for federal, state, local and foreign tax purposes, including payroll taxes, that are applicable to supplemental taxable income.

Restrictions on Resale	You agree not to sell any shares at a time when applicable laws, Company policies or an agreement between the Company and its underwriters prohibit a sale. This restriction will apply as long as your Service continues and for such period of time after the termination of your Service as the Company may specify.
Employment at Will	Your award or this Agreement does not give you the right to be retained by the Company or a subsidiary of the Company in any capacity. The Company and its subsidiaries reserve the right to terminate your Service at any time, with or without cause.
Adjustments	In the event of a stock split, a stock dividend or a similar change in Company stock, the number of your units will be adjusted accordingly, as the Company may determine pursuant to the Plan.

Beneficiary Designation

You may dispose of your units in a written beneficiary designation. A beneficiary designation must be filed with the Company on the proper form. It will be recognized only if it has been received at the Company's headquarters before your death. If you file no beneficiary designation or if none of your designated beneficiaries survives you, then your estate will receive any vested units that you hold at the time of your death.

Effect of Merger

If the Company is a party to a merger, consolidation or reorganization, then your units will be subject to the applicable provision of the Plan, provided that any action taken must either (a) preserve the exemption of your units from Section 409A of the Code or (b) comply with Section 409A of the Code.

Applicable Law

This Agreement will be interpreted and enforced under the laws of the State of Delaware (without regard to their choice-of-law provisions).

The Plan and Other Agreements

The text of the Plan is incorporated in this Agreement by reference.

The Plan, this Agreement and the Notice of Restricted Stock Unit Award constitute the entire understanding between you and the Company regarding this award. Any prior agreements, commitments or negotiations concerning this award are superseded. This Agreement may be amended only by another written agreement between the parties.

All capitalized terms used in this Agreement shall have the meanings assigned to them in this Agreement, the Notice of Restricted Stock Unit Award or the Plan.

For all purposes applicable to this award, "Service" means your continuous service as an Employee or Consultant.

BY SIGNING THE COVER SHEET OF THIS AGREEMENT, YOU AGREE TO ALL OF THE TERMS AND CONDITIONS DESCRIBED ABOVE AND IN THE PLAN.

REGENXBIO Inc.
2015 Equity Incentive Plan
Notice of Stock Option Grant

You have been granted the following option to purchase shares of the common stock of REGENXBIO Inc. (the "Company"):

Name of Optionee:	**[Participant Name]**
Total Number of Shares:	**[Number of Units]**
Type of Option:	**[Incentive Stock Option or Nonstatutory Stock Option]**
Exercise Price per Share:	**[Exercise Price]**
Date of Grant:	**[Grant Date]**
Vesting Commencement Date:	**[Vesting Commencement Date]**
Vesting Schedule:	**[Vesting Schedule]**
Expiration Date:	**[10th anniversary of Date of Grant]**. This option expires earlier if your Service terminates earlier, as described in the Stock Option Agreement, and may terminate earlier in connection with certain corporate transactions as described in Article 9 of the Plan.

You and the Company agree that this option is granted under and governed by the terms and conditions of the Company's 2015 Equity Incentive Plan (the "Plan") and the Stock Option Agreement, both of which are attached to, and made a part of, this document.

You further agree to accept by email all documents relating to the Plan or this option (including, without limitation, prospectuses required by the Securities and Exchange Commission) and all other documents that the Company is required to deliver to its security holders (including, without limitation, annual reports and proxy statements). You also agree that the Company may deliver these documents by posting them on a website maintained by the Company or by a third party under contract with the Company. If the Company posts these documents on a website, it will notify you by email.

You further agree to comply with the Company's *Insider Trading Policy* when selling shares of the Company's common stock.

Optionee

[Participant Name]

REGENXBIO Inc.

By: _____

Name: **[Authorized Individual Name]**

Title: **[Authorized Individual Title]**

REGENXBIO Inc.
2015 Equity Incentive Plan
Stock Option Agreement

Grant of Option	Subject to all of the terms and conditions set forth in the Notice of Stock Option Grant, this Stock Option Agreement (the "Agreement") and the Plan, the Company has granted you an option to purchase up to the total number of shares specified in the Notice of Stock Option Grant at the exercise price indicated in the Notice of Stock Option Grant. All capitalized terms used in this Agreement shall have the meanings assigned to them in this Agreement, the Notice of Stock Option Grant or the Plan. For all purposes applicable to this option, "Service" means your continuous service as an Employee, Outside Director or Consultant.
Tax Treatment	This option is intended to be an incentive stock option under Section 422 of the Code or a nonstatutory stock option, as provided in the Notice of Stock Option Grant. However, even if this option is designated as an incentive stock option in the Notice of Stock Option Grant, it shall be deemed to be a nonstatutory stock option to the extent it does not qualify as an incentive stock option under federal tax law, including under the $100,000 annual limitation under Section 422(d) of the Code.
Vesting	This option vests and becomes exercisable in accordance with the vesting schedule set forth in the Notice of Stock Option Grant. In no event will this option vest or become exercisable for additional shares after your Service has terminated for any reason.
Term	This option expires in any event at the close of business at Company headquarters on the day before the 10th anniversary of the Date of Grant, as shown in the Notice of Stock Option Grant. (This option will expire earlier if your Service terminates, as described below, and this option may be terminated earlier as provided in Article 9 of the Plan.)
Termination of Service	If your Service terminates for any reason, this option will expire immediately to the extent the option is unvested as of your termination date and does not vest as a result of your termination of Service. The Company determines when your Service terminates for all purposes of this option.
Regular Termination	If your Service terminates for any reason except death or total and permanent disability, then this option, to the extent vested as of your termination date, will expire at the close of business at Company headquarters on the date three months after your termination date.

Death

If you die before your Service terminates, then this option will expire at the close of business at Company headquarters on the date 12 months after the date of death.

Disability

If your Service terminates because of your total and permanent disability, then this option will expire at the close of business at Company headquarters on the date 12 months after your termination date.

For all purposes under this Agreement, "total and permanent disability" means that you are unable to engage in any substantial gainful activity by reason of any medically determinable physical or mental impairment which can be expected to result in death or which has lasted, or can be expected to last, for a continuous period of not less than one year.

Leaves of Absence and Part-Time Work

For purposes of this option, your Service does not terminate when you go on a military leave, a sick leave or another *bona fide* leave of absence, if the leave was approved by the Company in writing and if continued crediting of Service is required by applicable law, the Company's leave of absence policy, or the terms of your leave. However, your Service terminates when the approved leave ends, unless you immediately return to active work.

If you go on a leave of absence, then the vesting schedule specified in the Notice of Stock Option Grant may be adjusted in accordance with the Company's leave of absence policy or the terms of your leave. If you commence working on a part-time basis, the Company may adjust the vesting schedule so that the rate of vesting is commensurate with your reduced work schedule.

Notice Concerning Incentive Stock Option Treatment

Even if this option is designated as an incentive stock option in the Notice of Stock Option Grant, it ceases to qualify for favorable tax treatment as an incentive stock option to the extent that it is exercised: (a) more than three months after the date when you cease to be an Employee for any reason other than death or permanent and total disability (as defined in Section 22(e)(3) of the Code), (b) more than 12 months after the date when you cease to be an Employee by reason of permanent and total disability (as defined in Section 22(e)(3) of the Code) or (c) more than three months after the date when you have been on a leave of absence for three months, unless your reemployment rights following such leave were guaranteed by statute or by contract.

Restrictions on Exercise

The Company will not permit you to exercise this option if the issuance of shares at that time would violate any law or regulation.

Notice of Exercise

When you wish to exercise this option, you must notify the Company by filing the proper "Notice of Exercise" form at the address given on the form or, if the Company has designated a brokerage firm to administer the Plan, you must notify such brokerage firm in the manner such brokerage firm requires. Your notice must specify how many shares you wish to purchase. The notice will be effective when the Company receives it.

However, if you wish to exercise this option by executing a same-day sale (as described below), you must follow the instructions of the Company and the broker who will execute the sale.

If someone else wants to exercise this option after your death, that person must prove to the Company's satisfaction that he or she is entitled to do so.

You may only exercise your option for whole shares.

Form of Payment

When you submit your notice of exercise, you must include payment of the option exercise price for the shares that you are purchasing. To the extent permitted by applicable law, payment may be made in one (or a combination of two or more) of the following forms:

•By delivering to the Company your personal check, a cashier's check or a money order, or arranging for a wire transfer.

•By delivering to the Company certificates for shares of Company stock that you own, along with any forms needed to effect a transfer of those shares to the Company. The value of the shares, determined as of the effective date of the option exercise, will be applied to the option exercise price. Instead of surrendering shares of Company stock, you may attest to the ownership of those shares on a form provided by the Company and have the same number of shares subtracted from the option shares issued to you.

•By giving to a securities broker approved by the Company irrevocable directions to sell all or part of your option shares and to deliver to the Company, from the sale proceeds, an amount sufficient to pay the option exercise price and any withholding taxes. (The balance of the sale proceeds, if any, will be delivered to you.) The directions must be given in accordance with the instructions of the Company and the broker. This exercise method is sometimes called a "same-day sale."

Withholding Taxes

You will not be allowed to exercise this option unless you make arrangements acceptable to the Company to pay any withholding taxes that may be due as a result of the option exercise. These arrangements include payment in cash. With the Company's consent, these arrangements may also include (a) payment from the proceeds of the sale of shares through a Company-approved broker, (b) withholding shares of Company stock that otherwise would be issued to you when you exercise this option with a fair market value no greater than the minimum amount required to be withheld by law, (c) surrendering shares that you previously acquired with a fair market value no greater than the minimum amount required to be withheld by law, or (d) withholding cash from other compensation. The fair market value of withheld or surrendered shares, determined as of the date when taxes otherwise would have been withheld in cash, will be applied to the withholding taxes.

Restrictions on Resale	You agree not to sell any option shares at a time when applicable laws, Company policies or an agreement between the Company and its underwriters prohibit a sale. This restriction will apply as long as your Service continues and for such period of time after the termination of your Service as the Company may specify.
Transfer of Option	Prior to your death, only you may exercise this option. You cannot transfer or assign this option. For instance, you may not sell this option or use it as security for a loan. If you attempt to do any of these things, this option will immediately become invalid. You may, however, dispose of this option in your will or by means of a written beneficiary designation; provided, however, that your beneficiary or a representative of your estate acknowledges and agrees in writing in a form reasonably acceptable to the Company, to be bound by the provisions of this Agreement and the Plan as if such beneficiary of the estate were you. Regardless of any marital property settlement agreement, the Company is not obligated to honor a notice of exercise from your former spouse, nor is the Company obligated to recognize your former spouse's interest in your option in any other way.
Retention Rights	Your option or this Agreement does not give you the right to be retained by the Company, a Parent, Subsidiary, or an Affiliate in any capacity. The Company and its Parents, Subsidiaries, and Affiliates reserve the right to terminate your Service at any time, with or without cause.
Stockholder Rights	You, or your estate or heirs, have no rights as a stockholder of the Company until you have exercised this option by giving the required notice to the Company, paying the exercise price, and satisfying any applicable withholding taxes. No adjustments are made for dividends or other rights if the applicable record date occurs before you exercise this option, except as described in the Plan.
Recoupment Policy	This option, and the shares acquired upon exercise of this option, shall be subject to any Company recoupment policy in effect from time to time.
Adjustments	In the event of a stock split, a stock dividend or a similar change in Company stock, the number of shares covered by this option and the exercise price per share will be adjusted pursuant to the Plan.
Effect of Significant Corporate Transactions	If the Company is a party to a merger, consolidation, or certain change in control transactions, then this option will be subject to the applicable provisions of Article 9 of the Plan.

Applicable Law This Agreement will be interpreted and enforced under the laws of the State of Delaware (without regard to its choice-of-law provisions).

The Plan and Other Agreements The text of the Plan is incorporated in this Agreement by reference.

This Plan, this Agreement and the Notice of Stock Option Grant constitute the entire understanding between you and the Company regarding this option. Any prior agreements, commitments or negotiations concerning this option are superseded. This Agreement may be amended only by another written agreement between the parties.

BY SIGNING THE COVER SHEET OF THIS AGREEMENT, YOU AGREE TO ALL OF THE TERMS AND CONDITIONS DESCRIBED ABOVE AND IN THE PLAN.

EXHIBIT 10.42

Certain identified information has been excluded from this exhibit because such information both (i) is not material and (ii) would likely cause competitive harm if publicly disclosed. Excluded information is indicated with brackets and asterisks.

Royalty Purchase Agreement

Between

REGENXBIO Inc., as Seller

THE ENTITIES SET FORTH ON SCHEDULE 1.1 HERETO, as Purchaser, and

HCR COLLATERAL MANAGEMENT LLC, as Purchaser Representative

Dated as of December 22, 2020

List of Exhibits

List of Schedules

ROYALTY PURCHASE AGREEMENT, dated as of December 22, 2020 (this "*Agreement*"), between REGENXBIO INC., a Delaware corporation, as Seller ("*Seller*"), the entities set forth on Schedule 1.1, as Purchaser (collectively, "*Purchaser*"), and, solely in its capacity as representative of the Purchaser, HCR COLLATERAL MANAGEMENT LLC, a Delaware limited liability company ("*HCR Agent*").

INTRODUCTION

Seller is a party to that certain License Agreement, dated March 21, 2014, between Seller and AveXis, Inc. (formerly known as BioLife Cell Bank, Inc.) (the "*Licensee*"), as amended and otherwise modified by that certain First Amendment to License Agreement, dated as of January 8, 2018, between Seller and the Licensee (the "*First Amendment*") (as so amended and otherwise modified, and as may be further amended, amended and restated, supplemented or otherwise modified from time to time, the "*License Agreement*").

Seller desires to sell, transfer, assign and convey to Purchaser, and Purchaser desires to purchase, acquire and accept from Seller, all of Seller's right, title and interest in and to the Purchased Receivables (as defined below), for the consideration and on the terms and subject to the conditions set forth in this Agreement.

In consideration of the representations, warranties, covenants and agreements set forth herein and for good and valuable consideration, the receipt and adequacy of which are hereby acknowledged, Seller and Purchaser hereby agree as follows:

ARTICLE I

DEFINITIONS; INTERPRETATION

Section 1.1 Definitions. As used in this Agreement, the following terms shall have the following meanings:

"*Affiliate*" means, with respect to any Person, any other Person that directly, or indirectly through one or more intermediaries, Controls, or is Controlled by, or is under common Control with, such Person. For clarity, any reference to "Affiliates" of Licensee as of the date hereof shall include Novartis AG.

"*Applicable Multiple*" means, for each applicable time period specified below, the factor set forth below:

Time Period	Multiple
From the Closing Date through November 7, 2024	1.30
If the Threshold Date has not occurred on or before November 7, 2024, from November 8, 2024 through to the end of the Purchased Royalty Period	1.50

"*Applicable Withholding Certificate*" means, with respect to each Tax Purchaser and Purchaser Representative a valid, true and properly executed IRS Form W-9 (or any applicable successor form) stating the taxpayer identification number of such Tax Purchaser or Purchaser Representative, as applicable, and certifying under penalties of perjury that such Tax Purchaser or Purchaser Representative, as applicable, is a "United States Person" (within the meaning of Section 7701(a)(30) of the Code) and is exempt from United States federal backup withholding.

"*Base Escrow Agreement*" means that certain Master Escrow Agreement, substantially in the form of Exhibit F-1 attached hereto with such changes as may be agreed pursuant to Section 3.6, entered into by Purchaser and the Escrow Agent on or following the date hereof.

"*Bill of Sale and Assignment*" means that certain bill of sale and assignment, substantially in the form of Exhibit D attached hereto, entered into by Seller and Purchaser as of the date hereof.

"*Business Day*" means any day other than (a) a Saturday or Sunday or (b) a day on which banking institutions located in New York, New York are permitted or required by applicable Law to remain closed.

"*Call Price*" means, as of any date (a) the product of (i) the Purchase Price and (ii) 1.50 *minus* (b) the Total Net Amount; *provided, however*, that solely for the purpose of determining the Call Price with respect to a Call Notice delivered on or before November 7, 2024, in the event that the then applicable Threshold Amount *minus* the Total Net Amount is less than $1,000,000, the Call Price shall equal such difference.[1]

[1] Illustrative calculations of the Call Price as of November 7, 2024:

(A) Total Net Amount as of November 7, 2024 is $259,500,000. The then applicable Threshold Amount ($260,000,000) *minus* the Total Net Amount ($259,500,000) equals $500,000. Therefore, the Call Price equals $500,000.

(B) Total Net Amount as of November 7, 2024 is $258,500,000. The then applicable Threshold Amount ($260,000,000) *minus* the Total Net Amount ($258,500,000) equals $1,500,000. Because the difference is greater than $1,000,000, the Call Price is $41,500,000 ($300,000,000 (1.50 * Purchase Price) *minus* the Total Net Amount ($258,500,000)).

For the avoidance of doubt, the reduction in the Call Price in the foregoing proviso shall only apply and be available to Seller with respect to a Call Notice delivered on or before November 7, 2024, and, for a Call Notice delivered on any subsequent date, the Call Price shall equal (a) the product of (i) the Purchase Price and (ii) 1.50 *minus* (b) the Total Net Amount.

"*Code*" means the United States Internal Revenue Code of 1986.

"*Competitor*" has the meaning set forth on Schedule 8.5.

"*Connection Tax*" means any Tax to the extent that it would not be imposed but for (i) any connection of Purchaser with the jurisdiction of the applicable taxing authority (other than a connection arising solely from this Agreement or any transaction contemplated thereby) or (ii) any failure of Purchaser to provide any applicable documentation that is reasonably requested by the applicable withholding agent and that Purchaser is legally eligible to provide.

"*Consent*" means any consent, approval, license, permit, order, authorization, registration, filing or notice.

"*Contract*" means any contract, license, indenture, instrument or agreement.

"*Control*" and its derivatives mean the possession, directly or indirectly, of the power to direct or cause the direction of the management and policies of a Person, whether through the ownership of voting securities or other voting interests, by contract or otherwise.

"*Escrow Agent*" means Citizens Bank, N.A., as escrow agent.

"*Escrow Agreement*" means the Base Escrow Agreement, as supplemented by the Escrow Client Addendum.

"*Escrow Client Addendum*" means that certain Client Addendum, substantially in the form of Exhibit F-2 attached hereto with such changes as may be agreed pursuant to Section 3.6, entered into by Seller, Purchaser and the Escrow Agent on or following the date hereof

"*Excluded Assets*" means collectively:

(a) the Seller IP Assets;

(b) the Retained Rights;

(c) any and all Royalty Payments to the extent attributable to Net Sales of Zolgensma sold prior to July 1, 2020;

(d) any and all royalty payments payable by the Licensee pursuant to the License Agreement other than the portion of the Royalty Payments that constitute Purchased Receivables;

(e) any and all reimbursements, milestone payments, fees (including sublicense fees), indemnification, damages, awards, settlement payments or any other payments,

compensation or consideration of any kind pursuant to (i) Section 3.1, Section 3.2, Section 3.3, Section 3.5, Section 3.8 and Section 8.4 of the License Agreement and (ii) Section 1 of the First Amendment;

(f) the Retained Receivables; and

(g) any and all other rights of Seller to payment, compensation or consideration under or in respect of the License Agreement (other than (i) the Purchased Receivables, (ii) Proceeds payable to Seller in respect of unpaid Purchased Receivables and (iii) Proceeds payable to Seller as a result of actions taken by Seller in accordance with Sections 6.6 and 6.14 hereof that are to be shared with Purchaser in accordance with such Sections).

"*Governmental Entity*" means any government, regulatory or administrative agency or commission, or other governmental agency, authority, instrumentality or body, whether foreign, federal, state or local, including any applicable patent office, the United States Food and Drug Administration, the European Medicines Agency, the United States National Institutes of Health, or any other governmental authority in any country.

"*GSK*" means SmithKline Beecham Corporation, a Pennsylvania corporation doing business as GlaxoSmithKline.

"*GSK Agreement*" means the License Agreement, dated as of March 6, 2009, as amended on April 15, 2009, by and between Seller and GSK, and as may be further amended, amended and restated, supplemented or otherwise modified from time to time.

"*Judgment*" means any judgment, order, injunction or decree.

"*Knowledge of Seller*" or "*Knowledge*," when used with respect to Seller, means the actual knowledge of [****].

"*Law*" means any law, statute, rule, regulation, code, ordinance, treaty or order, whether domestic or foreign, and all applicable requirements, official directives, rules, consents, approvals and authorizations issued or promulgated by any Governmental Entity.

"*Losses*" means any and all losses, liabilities, expenses (including reasonable investigation costs and reasonable attorneys' fees and expenses in connection with any third party action, suit or proceeding) and damages, including for the avoidance of doubt, Lost Profits.

"*Lost Profits*" means all damages and losses in respect of the loss of expected returns due to the loss of Purchased Receivables due from Licensee, but excluding, for the avoidance of doubt, lost opportunity costs resulting from this investment being made.

"*Penn*" means The Trustees of the University of Pennsylvania.

"*Penn Agreement*" means the License Agreement, effective on February 24, 2009, as amended on March 6, 2009, September 9, 2014, April 29, 2016, April 4, 2019 and September 11, 2020, between Seller and Penn, and as may be further amended, amended and restated, supplemented or otherwise modified from time to time.

"*Person*" means any individual, firm, corporation, partnership, limited liability company, trust, joint venture, association, unincorporated organization, Governmental Entity or other entity or organization.

"*Proceeds*" means any amounts actually received by Seller from a Person (other than Purchaser or Purchaser Representative) as a result of any settlement or resolution of any actions, suits, proceedings, claims or disputes related to, and to the extent involving, the Receivables, except for any such amounts (a) that are required to be paid to the Licensee under Section 7.2.5 or the ReGenX Licensors under Section 7.2.6, in each case, of the License Agreement or (b) that are otherwise used to reimburse or indemnify the Licensee or the ReGenX Licensors for costs, expenses, legal fees or other fees relating to such actions, suits, proceedings, claims or disputes.

"*Purchased Receivables*" means, during the Purchased Royalty Period, 100% of all Receivables, it being understood and agreed that (i) once the Threshold Date or the Call Closing Date has occurred, Purchaser shall have no further interest in the Receivables and 100% of the Receivables shall constitute Retained Receivables and (ii) the portion of the Total Net Amount that exceeds the Threshold Amount shall belong to Seller and shall constitute Retained Receivables.

"*Purchased Royalty Period*" means the period beginning on (and including) July 1, 2020 and ending on the effective date of termination of the License Agreement pursuant to Article 6 of the License Agreement.

"*Purchaser Material Adverse Effect*" means any one or more of: (a) a material adverse effect on the ability of Purchaser or Purchaser Representative to consummate the transactions contemplated by the Transaction Documents and perform its obligations under the Transaction Documents and (b) a material adverse effect on (i) the validity or enforceability of the Transaction Documents against Purchaser or Purchaser Representative or (ii) the rights of Seller under the Transaction Documents.

"*Receivables*" means, (a) each Royalty Payment during the Purchased Royalty Period and (b) any interest on any amounts referred to in the immediately preceding clause (a) payable by the Licensee pursuant to Section 3.7 of the License Agreement.

"*Representatives*" means, collectively, with respect to any Person, the trustees, directors, board members, members, partners, managers, officers, employees, agents, advisors or other representatives (including attorneys, accountants, consultants, scientists and financial advisors) of such Person.

"*Required Royalty Party*" means (a) for any date prior to the earlier of the Threshold Date and the Call Closing Date, Purchaser and (b) on any date on or after the earlier of the Threshold Date or the Call Closing Date, Seller.

"*Responsible Employee of Seller*" means any employee of Seller referred to in the definition of "Knowledge of Seller" and any successor to such employee.

"*Retained Receivables*" means the portion of the Receivables that does not constitute Purchased Receivables.

"*Royalty Deductions*" means in respect of any Royalty Payments (a) any adjustments, modifications, offsets, credits, reductions or deductions to such Royalty Payments made pursuant to the License Agreement, (b) any royalties payable in respect to such Royalty Payments to GSK in accordance with Section 3.3 of the GSK Agreement at the rate specified in Section 4.10(k) and (c) to the extent not offset on account of amounts paid in respect of clause (b) or otherwise, any royalties payable in respect to such Royalty Payments to Penn in accordance with Section 3.2 of the Penn Agreement.

"*Royalty Payment*" means the running royalty payments payable by the Licensee to Licensor pursuant to Section 3.4.1 of the License Agreement in respect of Net Sales of Zolgensma sold, after giving effect to all Royalty Deductions applicable thereto and any adjustments required under Section 3.4.1.1 of the License Agreement. An illustrative calculation of the Royalty Payment and Royalty Deductions based on Net Sales of Zolgensma sold during the Calendar Quarter ended September 30, 2020 is attached as Schedule 1.2.

"*Royalty Reports*" means the reports setting forth the calculation of the royalties due to Seller that are required to be delivered by the Licensee pursuant to Section 3.6.1 of the License Agreement.

"*Seller IP Assets*" means, collectively, (a) subject to the interests of Penn and the other ReGenX Licensors therein, the Listed Patents, (b) any rights to develop, make, use, have made, import, export, sell, have sold and/or offer to sell any Licensed Products, and (c) any other intellectual property or other proprietary rights of any kind that are owned or held by, or licensed to, Seller.

"*Seller Material Adverse Effect*" means any one or more of: (a) a material adverse effect on the ability of Seller to consummate the transactions contemplated by the Transaction Documents and perform its obligations under the Transaction Documents, (b) a material adverse effect on (i) the validity or enforceability of the Transaction Documents against Seller or (ii) the rights of Purchaser under the Transaction Documents, (c) a material adverse effect on the rights of Seller under the License Agreement, (d) a material adverse effect on the value of the Purchased Receivables (including the timing, amount or duration thereof), (e) a material adverse effect on the ability to perfect the security interest granted to Purchaser pursuant to Section 2.4 or (f) a material adverse effect on the right or ability of Purchaser to receive the Purchased Receivables or any payment due to Purchaser hereunder as contemplated by this Agreement after the Closing.

"*Subsidiary*" means, as of any date, any corporation, limited liability company, partnership, association or other business entity (a) of which securities or other ownership interests representing more than 50% of the equity value or more than 50% of the ordinary voting power or, in the case of a partnership, more than 50% of the general partnership interests are, as of such date, owned, controlled or held or (b) that is, as of such date, otherwise Controlled, by Seller or one or more subsidiaries of Seller or by Seller and one or more subsidiaries of Seller.

"*Tax Purchaser*" means each entity that is included in Purchaser.

"*Threshold Amount*" means, as of any date, an amount equal to the product of (a) the Purchase Price and (b) the Applicable Multiple as of such date.

"*Threshold Date*" means the first date on which the Total Net Amount as of such date equals or exceeds the Threshold Amount. For purposes of determining the Threshold Date, and calculating the Total Net Amount, (a) Purchased Receivables attributable to Royalty Payments paid by the Licensee shall be deemed to have been remitted to Purchaser in equal installments on each day of the calendar quarter in respect of which such payment is made, and (b) all other amounts shall be deemed to have been remitted to Purchaser on the date on which such amounts actually were remitted to, or otherwise received by, Purchaser.

"*Total Net Amount*" means, as of any date, (a) the aggregate amount of all payments remitted to, or otherwise received by, Purchaser on or prior to such date pursuant to the Transaction Documents and the Licensee Instruction Letter (reduced, for the avoidance of doubt, by any amounts that Purchaser does not actually receive because such amounts are payable to any taxing authority, other than any withholding imposed in respect of Connection Taxes ("*Connection Tax Withholding*"), which Connection Tax Withholding shall be deemed to have been payments received by Purchaser), including (i) all payments in respect of Purchased Receivables pursuant to the Licensee Instruction Letter or Section 6.2, Section 6.4(b)(iii), and Section 6.14(c) (or otherwise), (ii) the aggregate amount of Proceeds that are remitted to, or otherwise received by, Purchaser pursuant to Section 6.6, (iii) the aggregate amount of consideration remitted to, or otherwise received by, Purchaser by virtue of its consent rights hereunder, (iv) the aggregate amount of all payments made by Seller pursuant to Section 7.1(a) (except to the extent such payments are paid to make Purchaser or any Purchaser Indemnified Party whole with respect to an out-of-pocket Loss incurred by Purchaser or such Purchaser Indemnified Party), and (v) the Q3 Royalty Adjustment (as defined below) *less* (b) (i) all overpayments of Royalty Payments required to be, and actually, reimbursed by Purchaser to Seller pursuant to Section 6.4(b)(ii) (or otherwise) on or prior to such date (but only to the extent that such overpayments have been included in the calculation of the Total Net Amount under the immediately preceding clause (a)), and (ii) the aggregate amount of all costs and expenses actually paid by Purchaser (and not actually reimbursed to Purchaser, whether by Seller or any other Person) on or prior to such date pursuant to Section 6.4(b), Section 6.6 and Section 6.14(d).

"*Transaction Documents*" means this Agreement, the Escrow Agreement, the Bill of Sale and Assignment, and the Licensee Instruction Letter.

"*Zolgensma*" means Zolgensma (onasemnogene abeparvovec-xioi).

Capitalized terms used in this Agreement and not otherwise defined herein shall have the respective meanings ascribed to them in the License Agreement. In the event a capitalized term used herein is defined in both this Agreement and the License Agreement, the meaning given to such term in this Agreement shall control.

Section 1.2 Certain Interpretations. Except where expressly stated otherwise in this Agreement, the following rules of interpretation apply to this Agreement:

(a) "include," "includes" and "including" shall be deemed to be followed by the words "without limitation";

(b) "hereof," "hereto," "herein" and "hereunder" and words of similar import when used in this Agreement refer to this Agreement as a whole and not to any particular provision of this Agreement;

(c) references to a Contract mean such Contract as from time to time amended, amended and restated, supplemented or otherwise modified, in each case, to the extent not prohibited by such Contract or this Agreement;

(d) references to a Person are also to its permitted successors and assigns;

(e) references to an "Article," "Section," "Exhibit" or "Schedule" refer to an Article or Section of, or an Exhibit or Schedule to, this Agreement;

(f) references to "$" or otherwise to dollar amounts refer to the lawful currency of the United States;

(g) references to a Law include any amendment or modification to such Law and any rules and regulations issued thereunder, whether such amendment or modification is made, or issuance of such rules and regulations occurs, before, on or after the date of this Agreement; and

(h) references to this "Agreement" shall include a reference to all Schedules and Exhibits attached to this Agreement (including the Schedule of Exceptions attached hereto as Exhibit C), all of which constitute a part of this Agreement and are incorporated herein for all purposes.

ARTICLE II

PURCHASE AND SALE OF PURCHASED RECEIVABLES

Section 2.1 Purchase and Sale of Purchased Receivables.

(a) *Purchase and Sale*. Upon the terms and subject to the conditions of this Agreement, at the Closing, Seller shall sell, transfer, assign and convey to Purchaser, and Purchaser shall purchase, acquire and accept from Seller, free and clear of all liens and encumbrances (other than those contemplated by this Agreement and the Escrow Agreement), all of Seller's right, title and interest in and to the Purchased Receivables. It is understood and agreed that Purchaser shall not, by purchase of the Purchased Receivables, acquire any assets or rights of Seller under, or relating to, the License Agreement other than those specified in the immediately preceding sentence.

(b) *Purchase Price*. The purchase price for the Purchased Receivables, net of any deduction for any withholding or other taxes, is $200,000,000 (the "*Purchase Price*").

Section 2.2 No Purchase or Sale of Excluded Assets. Notwithstanding anything to the contrary contained in this Agreement, Seller shall retain all its right, title and interest in and to, and there shall be excluded from the sale, transfer, assignment and conveyance to Purchaser under this Agreement, all Excluded Assets.

Section 2.3 No Obligations Transferred. Notwithstanding anything to the contrary contained in this Agreement, (a) the sale, transfer, assignment and conveyance to Purchaser of the Purchased Receivables pursuant to this Agreement shall not in any way subject Purchaser to, or transfer, affect or modify, any obligation or liability of Seller under the License Agreement and (b) Purchaser expressly does not assume or agree to become responsible for any obligation or liability of Seller whatsoever, whether presently in existence or arising or asserted hereafter, under the License Agreement, the GSK Agreement, the Penn Agreement or otherwise, except to the extent expressly contemplated by Section 6.4 or Section 6.9 (collectively, the "*Assumed Liabilities and Obligations*"). All such obligations and liabilities of Seller that are not transferred to Purchaser hereunder, as set forth in this Section 2.3 (collectively, the "*Excluded Liabilities and Obligations*") shall be retained by, and remain the obligations and liabilities of, Seller after the Closing. All such Excluded Liabilities and Obligations shall be satisfied by Seller, and in no event shall such Excluded Liabilities and Obligations be included in the amount of any Royalty Deductions.

Section 2.4 True Sale .

(a) Seller and Purchaser intend and agree that the sale, transfer, assignment and conveyance contemplated by this Agreement be, and is, a true, complete, absolute and irrevocable sale, transfer, assignment and conveyance by Seller to Purchaser of all of Seller's right, title and interest in and to the Purchased Receivables. Neither Seller nor Purchaser intends the transactions contemplated by this Agreement to be, or for any purpose characterized as, a loan from Purchaser to Seller or a pledge, a security interest, a financing transaction or a borrowing. Seller disclaims any beneficial ownership interest in the Purchased Receivables upon execution of this Agreement and each of Seller and Purchaser hereby waives, to the maximum extent permitted by applicable Law, any right to contest or otherwise assert that this Agreement does not constitute a true, complete, absolute and irrevocable sale, transfer, assignment and conveyance by Seller to Purchaser of all of Seller's right, title and interest in and to the Purchased Receivables under applicable Law, which waiver shall, to the maximum extent permitted by applicable Law, be enforceable against Seller in any bankruptcy or insolvency proceeding relating to Seller. Seller will treat the sale, transfer, assignment and conveyance of the Purchased Receivables as sales of "accounts" in accordance with the UCC (to the extent applicable) and Seller does hereby authorize Purchaser, on and after the Closing, to file such financing statements (and continuation statements with respect to such financing statements when applicable) (the "*Financing Statements*") naming Seller as the seller and Purchaser Representative as the agent for the purchaser of the Purchased Receivables as may be necessary to perfect such sale.

(b) In furtherance of the foregoing statement of the intent of Seller and Purchaser, and for the purposes of providing additional assurance to Purchaser in the event that, despite the intent of the parties hereto, the sale, transfer, assignment and conveyance contemplated by this Agreement is hereafter recharacterized as something other than a true sale or such sale will for any reason be ineffective or unenforceable as such, as determined in a judicial, administrative

or other proceeding (any of the foregoing being a "*Recharacterization*"), Seller does hereby grant to Purchaser and Purchaser Representative, as agent for Purchaser, as security for the payment of amounts to Purchaser equal to the Purchased Receivables as they become due and payable, a continuing security interest of first priority in and to all right, title and interest of Seller, in, to and under the Purchased Receivables and any "proceeds" (as such term is defined in the UCC) thereof, and Seller does hereby authorize Purchaser and Purchaser Representative, from and after the Closing, to file such financing statements (and continuation statements with respect to such financing statements when applicable) in such manner and such jurisdictions as are necessary or appropriate to perfect such security interest.

Section 2.5 Seller's Call Option.

(a) At any time on or prior to the Threshold Date, Seller shall have the right (the "*Call Option*"), exercisable upon [****] Business Days prior written notice to Purchaser (the "*Call Notice*"), to repurchase from Purchaser all, but not less than all, of Purchaser's right to, and interest in, the Purchased Receivables for a repurchase price equal to the Call Price. In order to exercise the Call Option, Seller shall deliver written notice to Purchaser of its election to so repurchase the Purchased Receivables and setting forth the proposed closing date (the "*Call Closing Date*"). Purchaser shall notify Seller within [****] Business Days of its calculation of the Total Net Amount received by Purchaser up to the date of calculation, which calculation shall be reasonably acceptable to Seller and which shall be used by Seller and Purchaser in connection with determining the Call Price.

(b) On the Call Closing Date, Seller shall repurchase from Purchaser the Purchased Receivables by payment of the Call Price made by wire transfer of immediately available funds to the account set forth on Exhibit B or such other account as Purchaser may designate in writing. Purchaser and Seller shall execute and deliver to the other party a bill of sale with respect to the Purchased Receivables substantially similar to the Bill of Sale reflecting the exercise of the Call Option.

ARTICLE III

CLOSING; DELIVERABLES

Section 3.1 Closing. The closing of the purchase and sale of the Purchased Receivables (the "*Closing*") shall take place at the offices of Covington & Burling LLP, The New York Times Building, 620 Eighth Avenue, New York, New York, 10018, at 10:00 a.m. New York City time on the date hereof, or at such other place, time and date as the parties hereto may mutually agree. The date on which the Closing occurs is referred to in this Agreement as the "*Closing Date*."

Section 3.2 Payment of Purchase Price. At the Closing, Purchaser shall deliver to Seller payment of an amount equal to the Purchase Price less the Q3 Royalty Adjustment by wire transfer of immediately available funds to the account set forth in Exhibit A and without any deduction for withholding or other taxes and without any other set off or deduction of any kind. The "Q3 2020 Royalty Adjustment" means $4,000,000.

Section 3.3 Closing Certificate. At the Closing, Seller shall deliver to Purchaser a certificate of an officer or other authorized signatory of Seller, dated the Closing Date, certifying as to the Seller's organizational documents and the attached resolutions adopted by the Board of Directors of Seller or a duly authorized committee thereof authorizing the execution and delivery by Seller of the Transaction Documents and the consummation by Seller of the transactions contemplated by the Transaction Documents.

Section 3.4 Bill of Sale and Assignment. At the Closing, Seller and Purchaser shall each deliver to the other party hereto a duly executed counterpart to the Bill of Sale and Assignment, evidencing the sale and assignment to Purchaser of the Purchased Receivables.

Section 3.5 Tax Forms. Prior to the Closing, each Tax Purchaser and Purchaser Representative shall have delivered to each of Seller and the Escrow Agent an Applicable Withholding Certificate.

Section 3.6 Escrow Agreement.

(a) On or promptly following the Closing, (i) Purchaser shall deliver to Seller a duly executed counterpart to the Base Escrow Agreement, (ii) each of Seller and Purchaser shall deliver to the other party hereto a duly executed counterpart to the Escrow Client Addendum and (iii) each party hereto shall receive a duly executed counterpart to the Escrow Agreement from the Escrow Agent. The Escrow Agreement shall be substantially in the form previously agreed between Seller and Purchaser set forth on Exhibit F-1 and Exhibit F-2 hereof, with only such changes as may be requested by the Escrow Agent and are reasonably acceptable to each of Seller and Purchaser. Seller and Purchaser shall work in good faith towards the prompt finalization of the Escrow Agreement with the Escrow Agent.

(b) Purchaser agrees (i) not to amend, waive or otherwise modify the Base Escrow Agreement in a manner that would adversely affect Seller without the consent of Seller; *provided* that Purchaser and Seller shall negotiate in good faith with respect to any such proposed amendment, waiver or modification and (ii) not to assign any of its right, title and interest in and to the Base Escrow Agreement other than to an Affiliate of Purchaser or the Purchaser Representative or in connection with, and to the extent of, any assignment permitted under this Agreement.

(c) At Seller's request, Purchaser agrees to provide Seller with reasonable access to any monthly statements or similar information relating to the escrow account that are provided by the Escrow Agent to Purchaser pursuant to the Escrow Agreement.

Section 3.7 Licensee Instruction Letter. Upon entry into the Escrow Agreement, Seller shall deliver to Licensee a duly executed letter of instruction, substantially in the form of Exhibit E attached hereto (the "*Licensee Instruction Letter*"), such delivery to be effected in accordance with Section 10.4 of the License Agreement and also by e-mail (with the Licensee Instruction Letter attached thereto as a PDF attachment) and such e-mail to include a request that Licensee confirm receipt thereof by e-mail.

ARTICLE IV

Seller's Representations and Warranties

Except as set forth on, or disclosed in, Exhibit C, Seller hereby represents and warrants to Purchaser and the Purchaser Representative that as of the time of the Closing on the date hereof:

Section 4.1 Existence. Seller is a corporation duly incorporated, validly existing and in good standing under the Laws of the State of Delaware. Seller has all corporate power and licenses, authorizations, consents and approvals required to carry on its business as now conducted and as proposed to be conducted in connection with the transactions contemplated by the Transaction Documents and the License Agreement, except, in each case, as would not reasonably be expected to have a Seller Material Adverse Effect.

Section 4.2 Authorization. Seller has the requisite corporate power and authority to execute, deliver and perform its obligations under the Transaction Documents and to consummate the transactions contemplated thereby. The execution, delivery and performance of the Transaction Documents, and the consummation of the transactions contemplated thereby, have been duly authorized by Seller.

Section 4.3 Enforceability. Each of the Transaction Documents has been duly executed and delivered by Seller, and constitutes a valid and binding obligation of Seller, enforceable against Seller in accordance with its terms, except as may be limited by general principles of equity (regardless of whether considered in a proceeding at law or in equity) and by applicable bankruptcy, insolvency, moratorium and other similar Laws of general application relating to or affecting creditors' rights generally.

Section 4.4 Absence of Conflicts. The execution, delivery and performance by Seller of the Transaction Documents and the consummation of the transactions contemplated thereby will not: (i) conflict with, or constitute a breach of or default under any provision of (a) the organizational documents of Seller, (b) any Law or Judgment applicable to Seller, (c) the License Agreement, the GSK Agreement or the Penn Agreement or (d) any Contract (other than the License Agreement, GSK Agreement and Penn Agreement) to which Seller is a party or by which Seller is bound, except, in the case of clauses (b) and (d), for such breaches or defaults that, individually or in the aggregate, would not reasonably be expected to result in a Seller Material Adverse Effect, (ii) result in the creation or imposition of any lien on the Purchased Receivables, except to the extent contemplated by the Transaction Documents and the Escrow Agreement, or (iii) give rise to any right of termination, cancellation or acceleration of any right of Seller as such right or obligation relates to the Purchased Receivables or the loss of any benefit relating to the Purchased Receivables.

Section 4.5 Consents. No Consent of any Governmental Entity or any other Person is required by or with respect to Seller in connection with the execution, delivery and performance by Seller of the Transaction Documents or the consummation of the transactions contemplated thereby, except for (a) the Licensee Instruction Letter, (b) the filing of the Financing Statements with the Secretary of State of the State of Delaware, (c) such Consents, the failure of which to be

obtained or made, would not reasonably be expected to result in a Seller Material Adverse Effect, and (d) such Consents as shall have been obtained on or prior to the date hereof.

Section 4.6 Litigation. No action, suit, proceeding or investigation before any Governmental Entity, court or arbitrator is pending, or, to the Knowledge of Seller, threatened, against Seller or its Subsidiaries that, individually or in the aggregate, would reasonably be expected to result in a Seller Material Adverse Effect.

Section 4.7 Compliance with Laws. Neither Seller nor any of its Subsidiaries has violated, is in violation of, has been given written notice that it has violated, and, to the Knowledge of Seller, neither Seller nor any of its Subsidiaries is under investigation with respect to its violation of, and has not been threatened to be charged with any violation of, any applicable Law or any Judgment of any Governmental Entity, court or arbitrator, which violation would reasonably be expected to result in a Seller Material Adverse Effect.

Section 4.8 Brokers' Fees. Other than the Escrow Agent, there is no investment banker, broker, finder, financial advisor or other Person who has been retained by or is authorized to act on behalf of Seller who is entitled to any fee or commission from Purchaser in connection with the transactions contemplated by this Agreement.

Section 4.9 License Agreement.

(a) *License Agreement; Royalty Reports; Material Notices*. Attached hereto as Schedule 4.9(a) are the following: (i) a true, correct and complete copy of the License Agreement; (ii) the Royalty Reports in respect of each Calendar Quarter ended on or prior to the date hereof that have been received by Seller prior to the date hereof (it being understood and agreed that all information in such Royalty Reports that does not relate to or involve the Royalty Payments has been redacted); (iii) the Development Progress Reports that have been received by Seller prior to the date hereof (it being understood and agreed that all information in such Development Progress Report that does not relate to or involve Zolgensma has been redacted) and (iv) all written notices delivered to the Licensee or its Affiliates by Seller, or by the Licensee or its Affiliates to Seller, pursuant to the License Agreement that could reasonably be expected to have an effect on the value of the Purchased Receivables in any material respect, in each case, since [****] (excluding pre-execution drafts and prior versions of the First Amendment and any accompanying or related correspondence).

(b) *Validity and Enforceability of License Agreement*. The License Agreement is a valid and binding obligation of Seller and, to the Knowledge of Seller, of the Licensee, enforceable against each of Seller and, to the Knowledge of Seller, the Licensee in accordance with its terms, except as may be limited by general principles of equity (regardless of whether considered in a proceeding at law or in equity) and by applicable bankruptcy, insolvency, moratorium and other similar Laws of general application relating to or affecting creditors' rights generally. Seller has not received any written notice or, to its Knowledge, other notice from the Licensee or its Affiliates challenging the validity or enforceability of the License Agreement or any obligation of the Licensee to pay the Royalty Payments thereunder.

(c) *No Waivers, Releases or Amendments*. Seller (i) has not granted any material written waiver or, to the Knowledge of Seller, any other material waiver, under the License Agreement, (ii) has not granted any waiver under the License Agreement related to, or involving, any Royalty Payments or any Royalty Deductions and (iii) has not released the Licensee, in whole or in part, from any of its material obligations under the License Agreement, except, in each case of the immediately foregoing clauses (i), (ii) and (iii), to the extent set forth in the License Agreement. Since [****], Seller has not received from the Licensee or its Affiliates any written proposal, and has not made any proposal to the Licensee or its Affiliates, to amend or waive any provision of the License Agreement.

(d) *No Termination, etc*. Seller has not (i) given the Licensee or its Affiliates any notice of termination of the License Agreement pursuant to Article 6 of the License Agreement (or otherwise) or (ii) received from the Licensee or its Affiliates any written notice of termination of the License Agreement pursuant to Article 6 of the License Agreement (or otherwise). To the Knowledge of Seller, no event has occurred that would give Seller a right to terminate the License Agreement pursuant to Article 6 of the License Agreement. Seller has not received any written notice or, to its Knowledge, other notice from the Licensee or its Affiliates expressing any intention or desire to terminate the License Agreement.

(e) *No Breaches*. Except, for the avoidance of doubt, as set forth on Exhibit C hereof, Seller has not breached any provision of the License Agreement in any material respect, and, to the Knowledge of Seller, the Licensee has not breached any provision of the License Agreement in any material respect.

(f) *Payments Made*. Seller has received from the Licensee each of the amounts specified in the section captioned "Quarterly Royalties in USD" in the Royalty Reports delivered by the Licensee in respect of each Calendar Quarter ended on or prior to the date hereof that have been received by Seller prior to the date hereof.

(g) *No Royalty Deductions*. Except for Royalty Deductions pursuant to Section 3.4 of the License Agreement, the Royalty Payments have not been and, to the Knowledge of Seller, are not, subject to any Royalty Deduction pursuant to clause (a) of the definition thereof. To the Knowledge of Seller, no event or condition exists that would permit the Licensee to claim any Royalty Deduction against payment of the Royalty Payments pursuant to clause (a) of the definition thereof, other than Royalty Deductions pursuant to Section 3.4 of the License Agreement. Except for Royalty Deductions pursuant to Section 3.4 of the License Agreement, Seller has not received any written notice or, to its Knowledge, any other notice from the Licensee or its Affiliates expressing an intention by the Licensee to take any Royalty Deductions or otherwise offset, credit against, reduce or deduct from the Receivables because of any amount owed or claimed owed from Seller to the Licensee. To the Knowledge of Seller, except as specified in the section captioned "Anti Stacking Calculation" in the Royalty Reports attached hereto as Schedule 4.9(a), there are no third party patents triggering a setoff right against the Royalty Payments.

(h) *No Assignments*. Seller has not consented to any assignment by the Licensee of, and, to the Knowledge of Seller, the Licensee has not assigned, the License Agreement or any part thereof. Except as contemplated by the Transaction Documents, Seller has

not assigned, in whole or in part, and has not granted any liens upon or security interests with respect to, the License Agreement or the Receivables.

(i) *No Indemnification Claims*. Seller has not given any notice to the Licensee or its Affiliates regarding any claims for indemnification under Section 8.4 of the License Agreement.

(j) *Examinations*. Seller has not initiated any review or audit pursuant to Section 3.6.5 of the License Agreement.

(k) *No Other Agreements*. Other than the License Agreement, there are no Contracts between Seller or any of its Affiliates, on the one hand, and the Licensee or any of its Affiliates (including Novartis AG), on the other hand, that (i) relate to Zolgensma or (ii) that would reasonably be expected to result in a Seller Material Adverse Effect.

(l) *Licensed Product*. Zolgensma is a Licensed Product.

(m) *No Disputes*. Except, for the avoidance of doubt, as set forth on Exhibit C hereof, Seller has not (i) received any written notice of any dispute from the Licensee or any of its Affiliates for resolution pursuant to Section 10.6 of the License Agreement or (ii) given any notice of any dispute to the Licensee for resolution pursuant to Section 10.6 of the License Agreement.

(n) *Sub-licenses*. Seller has not received from the Licensee or any of its Affiliates any written notice of any sub-license granted by the Licensee under the License Agreement, and, to the Knowledge of Seller, no executed sublicenses or other agreements have been entered into by Licensee or its Affiliates.

Section 4.10 GSK Agreement.

(a) *GSK Agreement; Reports; Material Notices*. Attached hereto as Schedule 4.10(a) are the following: (i) a true, correct and complete copy of the GSK Agreement; (ii) the reports delivered by Seller to GSK under Section 3.5.1 of the GSK Agreement and relating to Zolgensma in respect of each Calendar Quarter ended on or prior to the date hereof (it being understood and agreed that all information in such reports that does not relate to or involve the Royalty Payments has been redacted); and (iii) all material written notices delivered to GSK by Seller, or by GSK to Seller, pursuant to the GSK Agreement relating to or involving Zolgensma, in each case since [****].

(b) *Validity and Enforceability of GSK Agreement*. The GSK Agreement is a valid and binding obligation of Seller and, to the Knowledge of Seller, of GSK, enforceable against each of Seller and, to the Knowledge of Seller, GSK in accordance with its terms, except as may be limited by general principles of equity (regardless of whether considered in a proceeding at law or in equity) and by applicable bankruptcy, insolvency, moratorium and other similar Laws of general application relating to or affecting creditors' rights generally. Seller has not received any written notice from GSK challenging the validity or enforceability of the GSK Agreement, to the extent related to or involving Zolgensma.

(c) *No Waivers, Releases or Amendments*. In each case, to the extent related to or involving Zolgensma, Seller (i) has not granted any material written waiver or, to the Knowledge of Seller, any other material waiver, under the GSK Agreement, and (ii) has not released GSK, in whole or in part, from any of its material obligations under the GSK Agreement, except, in each case of the immediately foregoing clauses (i) and (ii), to the extent set forth in the GSK Agreement. Since [****], Seller has not received from GSK any written proposal, and has not made any proposal to GSK, to amend or waive any provision of the GSK Agreement, to the extent related to or involving Zolgensma.

(d) *No Termination, etc*. Seller has not (i) given GSK any notice of termination of the GSK Agreement pursuant to Article 6 of the GSK Agreement (or otherwise) or (ii) received from GSK any written notice of termination of the GSK Agreement pursuant to Article 6 of the GSK Agreement (or otherwise). To the Knowledge of Seller, no event has occurred that would give GSK a right to terminate the GSK Agreement pursuant to Article 6 of the GSK Agreement. Seller has not received any written notice from GSK expressing any intention or desire to terminate the GSK Agreement.

(e) *No Breaches*. Except, for the avoidance of doubt, as set forth on Exhibit C hereof, Seller has not breached any provision of the GSK Agreement in any material respect, and, to the Knowledge of Seller, GSK has not breached any provision of the GSK Agreement in any material respect, in each case, related to or involving Zolgensma.

(f) *Payments Made*. Seller has paid to GSK each of the amounts specified in the section captioned "Total Royalties Due" in the reports delivered to GSK under Section 3.5.1 of the GSK Agreement and related to or involving Zolgensma in respect of each Calendar Quarter ended on or prior to the date hereof that have been delivered by Seller to GSK prior to the date hereof.

(g) *No Assignments*. Seller has not consented to any assignment by GSK of, and, to the Knowledge of Seller, GSK has not assigned, the GSK Agreement or any part thereof. Seller has not assigned, in whole or in part, and has not granted any liens upon or security interests with respect to, the GSK Agreement, to the extent related to or involving Zolgensma.

(h) *No Indemnification Claims*. Seller has not given any notice to GSK regarding any claims for indemnification under Section 8.4 of the GSK Agreement, to the extent related to or involving Zolgensma.

(i) *Examinations*. GSK has not initiated any review or audit pursuant to Section 3.5.3 of the GSK Agreement.

(j) *No Disputes*. Except, for the avoidance of doubt, as set forth on Exhibit C hereof, Seller has not (i) received any written notice of any dispute from GSK for resolution pursuant to Section 10.5 of the GSK Agreement or (ii) given any notice of any dispute to GSK for resolution pursuant to Section 10.5 of the GSK Agreement, in each case, related to or involving Zolgensma.

(k) [****]

Section 4.11 Title to Purchased Receivables. Seller has good and valid title to the Purchased Receivables, free and clear of all liens and encumbrances (other than those contemplated by this Agreement and the Escrow Agreement). Upon payment of the amount specified in Section 3.2(a) by Purchaser and the filing of the Financing Statements with the Secretary of State of the State of Delaware, Purchaser will have acquired, subject to the terms and conditions set forth in this Agreement, good and valid title to the Purchased Receivables, free and clear of all liens and encumbrances (other than those contemplated by this Agreement and the Escrow Agreement). Except as contemplated by this Agreement and the Escrow Agreement, Seller is the sole Person with rights to receive the Purchased Receivables.

Section 4.12 Intellectual Property.

(a) *Listed Patents*. The issued patents and pending patent applications listed on Schedule 4.12(a) are referred to in this Agreement as the "*Listed Patents*." Schedule 4.12(a) specifies (x) with respect to each Listed Patent that is an issued patent, (i) the jurisdiction in which such Listed Patent has issued as a patent and (ii) the patent number of such Listed Patent and (y) with respect to each Listed Patent that is a pending patent application, (i) the jurisdiction in which such Listed Patent is pending and (ii) the patent application number of such Listed Patent.

(b) *No Litigation*.

Seller:

(i) has not received any written notice from the Licensee or its Affiliates to the effect that (A) the Licensee believes or (B) any other Person has asserted,

(ii) has not received any written notice from any other Person, and

(iii) otherwise has no Knowledge,

that there are any pending or threatened litigations, interferences, reexaminations, oppositions or like patent office proceedings involving any of the Listed Patents.

(c) *Ownership of the Listed Patents*.

Seller:

(i) has not received any written notice from the Licensee or its Affiliates to the effect that (A) the Licensee believes or (B) any other Person has asserted,

(ii) has not received any written notice from any other Person, and

(iii) otherwise has no Knowledge,

that (I) Penn is not the sole owner of the entire right, title and interest in any of the Listed Patents, free and clear of any encumbrances in the Field (other than (w) any interest of the other ReGenX Licensors, (x) any interest of Seller, (y) the License Agreement (and any encumbrances referred to therein or contemplated thereby) and (z) any encumbrances arising by operation of Law) or (II)

there are any facts that would preclude Penn from having clear title as the sole owner to any of the Listed Patents in the Field (other than as described in clauses (w), (x), (y) and (z) above).

(d) *Validity and Enforceability*.

Except, for the avoidance of doubt, as set forth on Exhibit C hereof, Seller:

(i) has not received any written notice from the Licensee or its Affiliates to the effect that (A) the Licensee believes or (B) any other Person has asserted,

(ii) has not received any written notice from any other Person, and

(iii) otherwise has no Knowledge,

that any of the issued Listed Patents are unenforceable or invalid.

(e) *Inventorship*.

Seller:

(i) has not received any written notice from the Licensee or its Affiliates to the effect that (A) the Licensee believes or (B) any other Person has asserted,

(ii) has not received any written notice from any other Person, and

(iii) otherwise has no Knowledge,

that there is a Person who is or claims to be an inventor under any of the Listed Patents who is not a named inventor thereof.

(f) *No Challenges*.

Seller:

(i) has not received any written notice from the Licensee or its Affiliates to the effect that (A) the Licensee believes or (B) any other Person has asserted,

(ii) has not received any written notice from any other Person, and

(iii) otherwise has no Knowledge,

of any claim by any Person asserting that the manufacture, importation, sale, offer for sale or use of Zolgensma infringes any Person’s patents or other intellectual property rights.

(g) *No Infringement*.

Seller:

(i) has not received any written notice from the Licensee or its Affiliates to the effect that (A) the Licensee believes or (B) any other Person has asserted,

(ii) has not received any written notice from any other Person, and

(iii) otherwise has no Knowledge,

that there is a Person who is engaging in or has engaged in any activity that infringes upon any of the Listed Patents in the Field. Except as set forth on Schedule 4.12(g), Seller has not obtained any non-infringement, freedom to operate, clearance or invalidity opinions from outside counsel regarding the Listed Patents or Zolgensma.

(h) *Maintenance, etc*. Seller has not received any written notice from the Licensee or any other Person to the effect that, and Seller otherwise has no Knowledge that, the Licensee has not paid, or caused to be paid, all required maintenance fees and like payments with respect to the issued Listed Patents. Seller has not received any written notice from the Licensee or its Affiliates to the effect that the Licensee believes, or that any other Person has asserted, that any of the Listed Patents have lapsed, expired or otherwise been terminated.

Section 4.13 Penn Agreement.

(a) *Penn Agreement; Reports; Material Notices*. Attached hereto as Schedule 4.13(a) are the following: (i) a true, correct and complete copy of the Penn Agreement; (ii) to the extent any such reports were delivered, any reports delivered by Seller to Penn under Section 4.1 of the Penn Agreement and relating to Zolgensma in respect of each Calendar Quarter ended on or prior to the date hereof (it being understood and agreed that all information in such reports that does not relate to or involve the Royalty Payments has been redacted); and (iii) all written notices relating to matters that could reasonably be expected to have, individually or in the aggregate, a Seller Material Adverse Effect, delivered to Penn by Seller, or by Penn to Seller, pursuant to Section 13.6 of the Penn Agreement relating to or involving Zolgensma.

(b) *Validity and Enforceability of Penn Agreement*. The Penn Agreement is a valid and binding obligation of Seller and, to the Knowledge of Seller, of Penn, enforceable against each of Seller and, to the Knowledge of Seller, Penn in accordance with its terms, except as may be limited by general principles of equity (regardless of whether considered in a proceeding at law or in equity) and by applicable bankruptcy, insolvency, moratorium and other similar Laws of general application relating to or affecting creditors' rights generally. Seller has not received any written notice from Penn challenging the validity or enforceability of the Penn Agreement, to the extent related to or involving Zolgensma.

(c) *No Waivers, Releases or Amendments*. In each case, to the extent related to or involving Zolgensma, Seller (i) has not granted any material written waiver or, to the Knowledge of Seller, any other material waiver, under the Penn Agreement, and (ii) has not released Penn, in whole or in part, from any of its material obligations under the Penn Agreement,

except, in each case of the immediately foregoing clauses (i) and (ii), to the extent set forth in the Penn Agreement. Since [****], Seller has not received from Penn any written proposal, and has not made any proposal to Penn, to amend or waive any provision of the Penn Agreement, to the extent related to or involving Zolgensma.

(d) *No Termination, etc*. Seller has not (i) given Penn any notice of termination of the Penn Agreement pursuant to Article 6 of the Penn Agreement (or otherwise) or (ii) received from Penn any written notice of termination of the Penn Agreement pursuant to Article 6 of the Penn Agreement (or otherwise). To the Knowledge of Seller, no event has occurred that would give Penn a right to terminate the Penn Agreement pursuant to Article 6 of the Penn Agreement (or otherwise). Seller has not received any written notice from Penn expressing any intention or desire to terminate the Penn Agreement.

(e) *No Breaches*. Except, for the avoidance of doubt, as set forth on Exhibit C hereof, Seller has not breached any provision of the Penn Agreement in any material respect, and, to the Knowledge of Seller, Penn has not breached any provision of the Penn Agreement in any material respect, in each case, related to or involving Zolgensma.

(f) *Payments Made*. Seller has paid to Penn any amounts required pursuant to the Penn Agreement related to or involving Zolgensma prior to the date hereof. As of the time of the Closing on the date hereof, no payments are owed by Seller to Penn pursuant to the Penn Agreement related to or involving Zolgensma.

(g) *No Assignments*. Seller has not consented to any assignment by Penn of, and, to the Knowledge of Seller, Penn has not assigned, the Penn Agreement or any part thereof. Seller has not assigned, in whole or in part, and has not granted any liens upon or security interests with respect to, the Penn Agreement, to the extent related to or involving Zolgensma.

(h) *Examinations*. Penn has not initiated any review or audit pursuant to Section 4.4 of the Penn Agreement.

(i) *No Disputes*. Except, for the avoidance of doubt, as set forth on Exhibit C hereof, Seller has not (i) received any written notice of any dispute from Penn for resolution pursuant to the Penn Agreement or (ii) given any notice of any dispute to Penn for resolution pursuant to the Penn Agreement, in each case related to or involving Zolgensma and in each case since [****].

Section 4.14 UCC Representations. Seller's exact legal name is, and for the prior five years has been, "REGENXBIO Inc." Seller is incorporated, and for the prior five years has been incorporated, in the State of Delaware.

Section 4.15 Taxes. No deduction or withholding for or on account of any tax has been made in respect of any payment under the License Agreement by the Licensee to Seller and Seller has not avoided any such deduction or withholding by claiming any exemption from, or reduction of, any withholding tax in respect of any such payments under any income tax treaty. There are no liens in respect of any Taxes on the Purchased Receivables.

ARTICLE V

Purchaser's Representations and Warranties

Each of Purchaser and Purchaser Representative hereby represent and warrant to Seller that as of the time of the Closing on the date hereof:

Section 5.1 Existence. Purchaser is a limited partnership duly organized, validly existing and in good standing under the Laws of the State of Delaware. Purchaser Representative is a limited liability company duly organized, validly existing and in good standing under the Laws of the State of Delaware. Each of Purchaser and Purchaser Representative has all power and licenses, authorizations, consents and approvals required to carry on its business as now conducted and as proposed to be conducted in connection with the transactions contemplated by the Transaction Documents, except, in each case, as would not reasonably be expected to have a Purchaser Material Adverse Effect.

Section 5.2 Authorization. Each of Purchaser and Purchaser Representative has the requisite organizational power and authority to execute, deliver and perform the Transaction Documents and to consummate the transactions contemplated thereby. The execution, delivery and performance of the Transaction Documents, and the consummation of the transactions contemplated thereby, have been duly authorized by each of Purchaser and Purchaser Representative.

Section 5.3 Enforceability. Each of the Transaction Documents has been duly executed and delivered by each of Purchaser and Purchaser Representative, and constitutes a valid and binding obligation of each of Purchaser and Purchaser Representative, enforceable against each of Purchaser and Purchaser Representative in accordance with its terms, except as may be limited by general principles of equity (regardless of whether considered in a proceeding at law or in equity) and by applicable bankruptcy, insolvency, moratorium and other similar Laws of general application relating to or affecting creditors' rights generally.

Section 5.4 Absence of Conflicts. The execution, delivery and performance by each of Purchaser and Purchaser Representative of the Transaction Documents and the consummation of the transactions contemplated thereby do not conflict with, constitute a breach of or default under any provision of (a) the organizational documents of each of Purchaser and Purchaser Representative, (b) any Law or Judgment applicable to each of Purchaser and Purchaser Representative or (c) any Contract to which Purchaser is a party or by which each of Purchaser and Purchaser Representative is bound, except, in the case of clauses (b) and (c), for such breaches or defaults that, individually or in the aggregate, would not reasonably be expected to result in a Purchaser Material Adverse Effect.

Section 5.5 Consents. No Consent of any Governmental Entity or any other Person is required by or with respect to each of Purchaser and Purchaser Representative in connection with the execution, delivery or performance by each of Purchaser and Purchaser Representative of the Transaction Documents or the consummation of the transactions contemplated thereby, except for (a) the filing of Financing Statements with the Secretary of State of the State of Delaware, (b) such Consents, the failure of which to be obtained or made, would not reasonably be expected to result

in a Purchaser Material Adverse Effect, and (c) such Consents as shall have been obtained on or prior to the date hereof.

Section 5.6 Litigation. No action, suit, proceeding or investigation before any Governmental Entity, court or arbitrator is pending, or, to the knowledge of each of Purchaser and Purchaser Representative, threatened, against either of Purchaser and Purchaser Representative that, individually or in the aggregate, would reasonably be expected to result in a Purchaser Material Adverse Effect.

Section 5.7 Compliance with Laws. Neither Purchaser nor Purchaser Representative has violated, is in violation of, has been given written notice that it has violated, and, to the knowledge of each of Purchaser and Purchaser Representative, neither Purchaser nor Purchaser Representative is under investigation with respect to its violation of, and has been threatened to be charged with any violation of, any applicable Law or any Judgment of any Governmental Entity, court or arbitrator, which violation would reasonably be expected to result in a Purchaser Material Adverse Effect.

Section 5.8 Brokers' Fees. Other than the Escrow Agent, there is no investment banker, broker, finder, financial advisor or other Person who has been retained by or is authorized to act on behalf of either of Purchaser and Purchaser Representative who is entitled to any fee or commission from Seller in connection with the transactions contemplated by this Agreement.

Section 5.9 Financing. Purchaser has sufficient cash on hand or binding and enforceable commitments to provide it with funds sufficient to satisfy its obligations to pay the Purchase Price. Purchaser has no reason to believe, nor has been provided with any notice (whether written or otherwise), that any of the Persons providing the commitments referred to above are unable or are not required or do not intend, for any reason, to satisfy their obligations under such commitments. Purchaser acknowledges that its obligations under the Transaction Documents are not contingent on obtaining financing.

Section 5.10 Tax Status. Each Tax Purchaser and the Purchaser Representative is a "United States person" as defined in section 7701(a)(30) of the Code.

ARTICLE VI

COVENANTS

Section 6.1 Performance of License Agreement, GSK Agreement and Penn Agreement. Seller agrees that it shall (i) not breach any of the License Agreement, GSK Agreement or Penn Agreement, in each case, in any respect material to the interests of Purchaser hereunder and (ii) use commercially reasonable efforts to cure any such breach by Seller of the License Agreement, GSK Agreement or Penn Agreement.

Section 6.2 Misdirected Payments; Offsets by the Licensee.

(a) *Payments to Purchaser*. If Seller shall, notwithstanding the provisions of the License Agreement, the Escrow Agreement and the Licensee Instruction Letter, receive from the Licensee, the Escrow Agent or any other Person any Purchased Receivables, Seller shall

promptly (and in any event no later than [****] Business Days) following the date on which a Responsible Employee of Seller becomes aware of the receipt by Seller of such Purchased Receivables, remit to Purchaser such Purchased Receivables. Notwithstanding the foregoing, and for the avoidance of doubt, it is understood and agreed that Seller shall not be required to remit any Receivables to Purchaser if the Threshold Date or the Call Closing Date has occurred.

(b) *Payments to Seller*. If Purchaser shall, notwithstanding the provisions of the License Agreement, the Escrow Agreement and the Licensee Instruction Letter, receive from the Licensee, the Escrow Agent or any other Person (i) any Royalty Payment that does not consist entirely of Purchased Receivables or (ii) any Excluded Asset (which, for the avoidance of doubt, in each case of clauses (i) and (ii), includes any portion of the Total Net Amount that exceeds the Threshold Amount), Purchaser shall promptly (and in any event no later than [****] Business Days) following the date any amount previously received by Purchaser is so identified or Purchaser otherwise becomes aware of its receipt thereof, remit to Seller (A) such Royalty Payment, or portion thereof, that does not constitute Purchased Receivables or (B) such Excluded Asset, as the case may be.

(c) *Offsets by the Licensee*. If the Licensee sets off against the Purchased Receivables any amount owing from Seller to the Licensee in respect of any right of the Licensee against Seller arising from or in connection with any matter other than the Purchased Receivables (such amount owing from Seller to the Licensee, the "*Seller Obligation*"), then Seller shall promptly (and in any event no later than [****] Business Days) following the date on which a Responsible Employee of Seller becomes aware of such set-off (including the amount thereof and the nature and extent of the Seller Obligation), pay to Purchaser the amount of such set-off. After Seller makes the payment referred to in the first sentence of this Section 6.2(c), Seller shall be entitled to, and Purchaser shall not be entitled to, any amounts recovered from the Licensee in respect of such set-off. Notwithstanding the foregoing, and for the avoidance of doubt, it is understood and agreed that Seller shall not be required to remit any Receivables to Purchaser if the Threshold Date or the Call Closing Date has occurred.

(d) *Remittances*. All remittances pursuant to this Section 6.2 shall be made (i) without set-off or deduction of any kind (except as required by applicable Law) and (ii) by wire transfer of immediately available funds to the account set forth in Exhibit A (if the payee is Seller) or Exhibit B (if the payee is Purchaser) or to such other account as the relevant payee may designate in writing (such designation to be made at least [****] Business Days prior to any such payment).

(e) *Payments Held In Trust*. Each party hereto agrees that it shall hold any amounts received by it to which the other party hereto is entitled under Section 6.2(a) or Section 6.2(b) in trust and agrees that it shall have no right, title or interest whatsoever in such amounts.

Section 6.3 Royalty Reports; Notices; Correspondence. Promptly (and in any event no later than [****] Business Days) following the receipt by Seller from the Licensee of (a) a Royalty Report or (b) any written notice or material written correspondence relating to, or involving, the Purchased Receivables (including notice of any Royalty Deductions) pursuant to the License Agreement or the GSK Agreement, Seller shall furnish a copy of such Royalty Report or such notice or correspondence to Purchaser (it being understood and agreed that all information in such Royalty Reports or such notices or correspondence that does not relate to or involve the Purchased

Receivables may be redacted by Seller before furnishing copies thereof to Purchaser). Except for the Licensee Instruction Letter and notices and correspondence required to be given or made by Seller (i) under the License Agreement or (ii) by applicable Law, Seller shall not send any notice or correspondence to the Licensee relating to, or involving, the Purchased Receivables, in each case, without the prior written consent of the Required Royalty Party (such consent not to be unreasonably withheld or delayed), unless the sending of such notice or correspondence would not reasonably be expected to adversely affect in any material respect the value of the Purchased Receivables. Seller shall, promptly (and in any event no later than [****] Business Days) following the delivery thereof by Seller to the Licensee, provide to Purchaser a copy of any material notice or material correspondence sent by Seller to the Licensee or GSK relating to, or involving, the Purchased Receivables, including any report sent by Seller to GSK under Section 3.5.1 of the GSK Agreement to the extent relating to Zolgensma (it being understood and agreed that all information in such notices or correspondence that does not relate to or involve the Purchased Receivables may be redacted by Seller before furnishing copies thereof to Purchaser).

Section 6.4 Examinations of Licensee Records and Books of Account.

(a) *Consultation*. Seller and Purchaser shall consult with each other regarding the timing, manner and conduct of any review or audit of the Licensee's records and books of account with respect to the Purchased Receivables pursuant to Section 3.6.5 of the License Agreement.

(b) *Examinations.*

(i) Seller may, and if requested in writing by the Required Royalty Party, shall, cause an independent firm of accountants to conduct a review or audit of the Licensee's records and books of account with respect to the Purchased Receivables pursuant to Section 3.6.5 of the License Agreement; *provided*, *however*, that the Required Royalty Party shall not be entitled to request such an examination (A) more frequently than [****] or (B) if such an examination would contravene the provisions of Section 3.6.5 of the License Agreement. All of the costs and expenses of any such review or audit (including the fees and expenses of any independent firm of accountants) that would otherwise be borne by Seller pursuant to the License Agreement shall instead be borne (as such costs and expenses are incurred) by Purchaser.

(ii) If, following the completion of such a review or audit (regardless of whether such examination was initiated as a result of a written request from the Required Royalty Party or initiated by Seller in the absence of any such request), the Licensee must be reimbursed for overpayment of Purchased Receivables, then Purchaser shall promptly (and in any event no later than [****] Business Days) following Seller's request pay such reimbursement amount to Seller. Notwithstanding anything to the contrary in the immediately preceding sentence, and for the avoidance of doubt, it is understood and agreed that Purchaser shall not be entitled to receive any such reimbursement amount if the Threshold Date or the Call Closing Date has occurred.

(iii) If, following the completion of such a review or audit (regardless of whether such examination was initiated as a result of a written request from the Required Royalty Party or initiated by Seller in the absence of any such request), the Licensee is required to make additional payments for underpayment of Purchased Receivables, then such payments received by Seller

from Licensee, after deduction (and reimbursement to Seller and Purchaser) of all costs and expenses (including the fees and expenses of any independent firm of accountants) borne by Seller and Purchaser in connection with such examination pursuant to the last sentence of Section 6.4(b)(i), shall be allocated between, and paid to, Purchaser (such payment, after giving effect to such deduction and reimbursement, is referred to in the immediately succeeding sentence as the "net payment"). Notwithstanding anything to the contrary in the immediately preceding sentence, and for the avoidance of doubt, it is understood and agreed that Purchaser shall not be entitled to receive any such net payment if the Threshold Date or the Call Closing Date has occurred.

Section 6.5 Amendment of License Agreement; Amendment of GSK Agreement; Amendment of Penn Agreement.

(a) *Amendment of License Agreement*. Seller shall provide Purchaser a copy of any proposed amendment, supplement, modification or waiver (each, a "*Modification*") of any provision of the License Agreement as soon as practicable (and in any event not less than [****] Business Days) prior to the date Seller proposes to execute such Modification. Seller shall not, without the prior written consent of the Required Royalty Party (such consent not to be unreasonably withheld or delayed), execute or agree to execute any proposed Modification of the License Agreement if such Modification would reasonably be expected to adversely affect in any material respect the value of the Purchased Receivables (it being understood and agreed that any proposed Modification to the provisions of the License Agreement governing the amount or calculation of the Purchased Receivables (including the Royalty Deductions) or the procedures for payment of the Purchased Receivables shall be deemed, for purposes of this Section 6.5, to have such an effect). Promptly (and in any event within [****] Business Days) following receipt by Seller of a fully executed Modification of the License Agreement, Seller shall furnish a copy of such Modification to Purchaser.

(b) *Amendment of GSK Agreement*. Seller shall not, without the prior written consent of the Required Royalty Party (such consent not to be unreasonably withheld or delayed), execute or agree to execute any proposed Modification to the GSK Agreement, to the extent such Modification would reasonably be expected to adversely affect in any material respect the value of the Purchased Receivables (it being understood and agreed that any proposed Modification to the provisions of the GSK Agreement governing the amount or calculation of the payments related to Zolgensma pursuant to Section 3.3 or the procedures for payment of such payments shall be deemed, for purposes of this Section 6.5, to have such an effect, and notwithstanding anything to the contrary in this Section 6.5, it shall be deemed reasonable for Purchaser to withhold its consent to any such proposed Modification to the extent adverse to Purchaser (as determined by Purchaser in its sole discretion) unless Seller has concurrently offered to Purchaser to amend this Agreement to eliminate such adverse effect).

(c) *Amendment of Penn Agreement*. Seller shall not, without the prior written consent of the Required Royalty Party (such consent not to be unreasonably withheld or delayed), execute or agree to execute any proposed Modification to the Penn Agreement, to the extent such Modification would reasonably be expected to adversely affect in any material respect the value of the Purchased Receivables (it being understood and agreed that any proposed Modification to the provisions of the Penn Agreement governing the amount or calculation of the payments related to Zolgensma or the procedures for payment of such payments shall be deemed, for purposes of

this Section 6.5, to have such an effect, and notwithstanding anything to the contrary in this Section 6.5, it shall be deemed reasonable for Purchaser to withhold its consent to any such proposed Modification to the extent adverse to Purchaser (as determined by Purchaser in its sole discretion) unless Seller has concurrently offered to Purchaser to amend this Agreement to eliminate such adverse effect).

Section 6.6 Enforcement of License Agreement.

(a) *Notice of Licensee's Breaches*. Promptly (and in any event within [****] Business Days) following a Responsible Employee of Seller becoming aware of a breach of the License Agreement by the Licensee that would reasonably be expected to adversely affect in any material respect the value of the Purchased Receivables, Seller shall provide notice of such breach to Purchaser.

(b) *Enforcement of License Agreement*. Seller and Purchaser shall consult with each other regarding any breach referred to in Section 6.6(a) and as to the timing, manner and conduct of any enforcement of the Licensee's obligations under the License Agreement relating thereto.

(i) *Enforcement*. Seller may, and if requested in writing by the Required Royalty Party within [****] Business Days after receipt of notice of such breach pursuant to Section 6.6(a), shall, proceed in consultation with the Required Royalty Party, to use commercially reasonable efforts to enforce compliance by the Licensee with the relevant provisions of the License Agreement and to use commercially reasonable efforts to exercise such rights and remedies relating to such breach as shall be available to Seller, whether under the License Agreement or by operation of applicable Law.

(ii) *Lead Counsel*. In connection with any enforcement of the Licensee's obligations under the License Agreement in respect of any breach referred to in Section 6.6(a) (regardless of whether such enforcement is initiated by Seller as a result of a written request from the Required Royalty Party or initiated by Seller in the absence of any such request), the lead counsel selected by Seller shall be such counsel as the Required Royalty Party shall recommend for such purpose (as long as such counsel is reasonably acceptable to Seller).

(c) *Allocation of Proceeds of Enforcement*. The Proceeds of any enforcement of the Licensee's obligations under the License Agreement in respect of any breach referred to in Section 6.6(a) (regardless of whether such enforcement is initiated by Seller as a result of a written request from the Required Royalty Party or initiated by Seller in the absence of any such request), after deduction (and reimbursement to Seller and Purchaser) of all costs and expenses (including attorneys' fees and expenses) incurred by Seller and Purchaser in connection with such enforcement pursuant to the first two sentences of Section 6.6(d) below, shall be paid to Seller and Purchaser in proportion to their respective interests in the Receivables (taking into account any variation in such interests over different time periods, if applicable) (such payment, after giving effect to such deduction and reimbursement, is referred to in the immediately succeeding sentence as the "net payment"). Notwithstanding anything to the contrary herein, and for the avoidance of doubt, it is understood and agreed that Purchaser shall not be entitled to receive any such net payment if the Threshold Date or the Call Closing Date has occurred.

(d) *Allocation of Costs of Enforcement.* All costs and expenses (including attorneys' fees and expenses) incurred by Seller in connection with any enforcement of the Licensee's obligations under the License Agreement in respect of any breach referred to in Section 6.6(a) shall be borne by Seller and Purchaser (as such costs and expenses are incurred) in proportion to their respective interests in the Receivables (taking into account any variation in such interests over different time periods, if applicable), including any retainers or advances required by the lead counsel selected pursuant to Section 6.6(b)(ii) for such enforcement (and that are incurred by Seller). Nothing contained herein shall limit Purchaser from retaining, at its sole cost, separate outside counsel who shall be permitted, where reasonably practicable, to consult with the lead counsel selected pursuant to Section 6.6(b)(ii) for such enforcement.

Section 6.7 Termination of License Agreement; Negotiation of Licenses.

(a) Promptly (and in any event within [****] Business Days) following a Responsible Employee of Seller becoming aware of the occurrence of any event that gives rise to a right on the part of Seller to terminate the License Agreement pursuant to Article 6 of the License Agreement, Seller shall provide notice of such occurrence to Purchaser and shall consult with Purchaser in determining whether or not to exercise Seller's right to terminate the License Agreement pursuant to Article 6 of the License Agreement. In any event, Seller shall not, without the prior written consent of the Required Royalty Party (such consent not to be unreasonably withheld or delayed), (i) exercise its right to terminate the License Agreement pursuant to Article 6 of the License Agreement or (ii) agree with the Licensee to terminate the License Agreement.

(b) Subject to Section 6.7(a), if the Licensee or Seller terminates or provides written notice of termination of the License Agreement in its entirety, or the License Agreement otherwise terminates in its entirety, then, to the extent permitted by the survival provisions of the License Agreement and any provisions of the GSK Agreement and Penn Agreement, Seller shall use commercially reasonable efforts, at Purchaser's reasonable request and sole cost and expense (including Purchaser's payment of Seller's reasonable attorney's fees, if any, in connection therewith), in consultation and cooperation with Purchaser, for a period of [****] days (or such shorter period as set forth in this Section 6.7) (the "*Initial Search Period*"), to locate, negotiate and secure a license of the Seller IP Assets with respect to the Licensed Product (any such license, a "*New Arrangement*"); *provided*, that the counterparty to such New Arrangement shall be reasonably acceptable to Seller, *provided, further*, that Purchaser shall have the right to consent in writing to any New Arrangement, which approval shall not be unreasonably withheld or delayed. Seller agrees to undertake in connection with any New Arrangement such obligations and liabilities, if any, as are comparable to the obligations and liabilities it currently has under the License Agreement; *provided*, that (i) in no event shall Seller have any obligation in connection with the New Arrangement to renegotiate the GSK Agreement or the Penn Agreement and (ii) such license shall include terms not materially more onerous to Seller than those contained in the License Agreement with respect to the obligations and costs imposed on Seller and not materially less favorable with respect to the rights and remedies of Seller. Seller shall not pay (or enter into any agreement to pay) any upfront costs, fees or expenses to a third party in connection with Seller's efforts to locate, negotiate and secure a New Arrangement ("*New Arrangement Expenses*") without the prior written consent of Purchaser. In no event shall Seller be required to incur any obligation of any kind with respect to, and any directions provided by Purchaser under this Section 6.7 shall not include any direction regarding, the prosecution, maintenance, enforcement or

defense of the Seller IP Assets. If Purchaser does not consent to such New Arrangement Expenses, Purchaser may, upon written notice to Seller, terminate the Initial Search Period. Following the expiration or termination of the Initial Search Period, Purchaser may, at its option and sole cost and expense, continue efforts to locate, negotiate and secure a New Arrangement; *provided*, that Seller shall have the right to consent in writing to any New Arrangement, which approval shall not be unreasonably withheld or delayed. Seller shall use commercially reasonable efforts, at Purchaser's request and sole cost and expense (including Purchaser's payment of Seller's reasonable attorney's fees, if any, in connection therewith) to provide cooperation and assistance to Purchaser in connection with Purchaser's efforts pursuant to the foregoing sentence. In the event Seller enters into a New Arrangement, references in this Agreement to the Purchased Receivables and the License Agreement shall be deemed to be references to any new purchased receivables and the new license agreement constructed under the New Arrangement, and references to Licensee shall be deemed to be references to the other party to such New Arrangement. Such New Arrangement shall also provide, for no additional consideration from Purchaser (other than, for clarity, the costs and expenses described in this Section 6.7(b)), that (i) Purchaser shall have the same rights as those acquired under the License Agreement pursuant to this Agreement and (ii) all payments and other consideration (including any upfront fees) thereunder (to the extent that such payments or other consideration would have constituted Royalty Payments under the License Agreement) be made by the other party to such New Arrangement directly to Purchaser subject to the Threshold Amount; *provided*, that all such payments and other consideration (including any upfront fees) made by the other party to such New Arrangement shall be deemed to be Royalties hereunder for purposes of determining the Termination Date, Threshold Date and Total Net Amount. All out-of-pocket third party expenses of Seller (including reasonable attorney's fees) incurred pursuant to this Section 6.7(b) shall be promptly reimbursed by Purchaser.

Section 6.8 Approval of Assignments of License Agreement.

(a) *Assignments by the Licensee*. Promptly (and in any event within [****] Business Days) following receipt by Seller of a written request from the Licensee for consent to assign the License Agreement (in whole or in part) pursuant to Section 10.2 of the License Agreement, Seller shall provide notice thereof to Purchaser. Seller and Purchaser shall consult with each other regarding whether to grant such consent, and Seller shall not grant or withhold such consent without the prior written consent of the Required Royalty Party (such consent of the Required Royalty Party not to be unreasonably withheld or delayed). Notwithstanding anything to the contrary contained in this Section 6.8, and for the avoidance of doubt, no consent of Purchaser shall be required in connection with any assignment by the Licensee to which Seller does not have a consent under the License Agreement, including an assignment by the Licensee to a wholly owned Affiliate or in connection with a Change of Control (subject to meeting the requirements in Section 10.2 of the License Agreement).

(b) *Assignments by Seller*. Seller may not assign the License Agreement (in whole or in part) without the prior written consent of the Required Royalty Party (such consent not to be unreasonably withheld or delayed); *provided*, that no such consent shall be required in connection with (i) any assignment, sale or transfer (in whole or in part) of Seller's right, title and interest in and to the Excluded Assets (including the Retained Receivables) or the delegation of any of Seller's duties with respect to the Excluded Assets (including the Retained Receivables), (ii) any assignment, sale or transfer of Seller's right, title and interest in and to all or substantially

all of the assets of Seller related to, or necessary to perform Seller's obligations in respect of, the License Agreement and (iii) any assignment to an Affiliate, in which case Seller shall remain responsible for the performance of this Agreement by such Affiliate.

(c) *Copies of Assignments*. Promptly (and in any event no later than [****] Business Days) following Seller's receipt of any fully executed assignment of the License Agreement by the Licensee or Seller, Seller shall furnish a copy of such assignment to Purchaser.

Section 6.9 Confidentiality.

(a) *Confidentiality*. Purchaser and Purchaser Representative shall keep confidential and not disclose to any Person (other than their Affiliates, actual or potential financing sources, investors or co-investors and permitted assigns, and their Representatives (each, a "*Permitted Recipient*"), and shall cause their Permitted Recipients to keep confidential and not disclose to any Person, any Confidential Information (as defined below). Purchaser and Purchaser Representative shall, and shall cause their Permitted Recipients to, use the Confidential Information solely in connection with Purchaser and Purchaser Agent's administration of the Transaction Documents (and not for any other purpose). The foregoing obligations shall continue until the later of (x) [****] and (y) the date of expiration of the last to expire of the Relevant Obligations (as defined below). "*Relevant Obligations*" means confidentiality obligations of Seller or any of its Affiliates under any agreement with a third party (including the License Agreement, the GSK Agreement and the Penn Agreement) to which any Confidential Information is subject.

(b) *Confidential Information*. "*Confidential Information*" means, collectively, all information (whether written or oral, or in electronic or other form, and whether furnished before, on or after the date of this Agreement) concerning, or relating in any way, directly or indirectly, to Seller, the License Agreement or the Receivables, including, (i) any license, sublicense, assignment, product development, royalty, sale, supply or other agreements (including, without limitation, this Agreement, the License Agreement, the GSK Agreement, the Penn Agreement) involving or relating in any way, directly or indirectly, to the Receivables or the intellectual property, compounds or products giving rise to the Receivables, whether or not such licenses, sub-licenses or other agreements currently exist, are executed after the date hereof, or have been previously terminated, and including all terms and conditions hereof and thereof and the identities of the parties thereto, (ii) any Royalty Reports, Modifications, assignments, notices, requests, correspondence or other information furnished pursuant to this Agreement (including this Article VI) and any other reports, data, information, materials, notices, correspondence or documents of any kind relating in any way, directly or indirectly, to this Agreement, the License Agreement, the GSK Agreement, the Penn Agreement, the Receivables or the intellectual property, compounds or products giving rise to the Receivables, and including reports, data, information, materials, notices, correspondence or documents of any kind delivered pursuant to or under this Agreement or any of the other agreements referred to in the immediately preceding clause (i), and (iii) any inventions, devices, improvements, formulations, discoveries, compositions, ingredients, patents, patent applications, know-how, processes, trial results, research, developments or any other intellectual property, trade secrets or information involving or relating in any way, directly or indirectly, to the Receivables or the compounds or products giving rise to the Receivables. Notwithstanding the foregoing, "*Confidential Information*" shall not include any information that

(A) was known by Purchaser at the time such information was disclosed to Purchaser or its Permitted Recipients in accordance herewith or in accordance with the Confidentiality Agreement (as defined below), as evidenced by its written records or other competent evidence; (B) was or becomes generally available to the public (other than as a result of a disclosure by Purchaser or its Permitted Recipients in violation of this Agreement or the Confidentiality Agreement); (C) becomes known to Purchaser or its Permitted Recipients on a non-confidential basis from a source other than Seller, its Affiliates and its and its Affiliates' Representatives (and without any breach of this Agreement or the Confidentiality Agreement by Purchaser or its Permitted Recipients); *provided*, that such source (i) had the right to disclose such information to Purchaser or its Permitted Recipients, as the case may be (without breaching any legal, contractual or fiduciary obligation to Seller or any of its Affiliates) and (ii) did not obtain such information directly or indirectly from, or on behalf of, Seller or any of its Affiliates or Representatives; or (D) is or has been independently developed by Purchaser or its Permitted Recipients without use of or reference to the Confidential Information, as evidenced by its written records or other competent evidence.

(c) *Permitted Disclosures*. In the event that Purchaser or any of its Permitted Recipients are requested by a governmental or regulatory authority or required by applicable Law, regulation or legal process (including the regulations of a stock exchange or governmental or regulatory authority or the order or ruling of a court, administrative agency or other government or regulatory body of competent jurisdiction) to disclose any Confidential Information, Purchaser shall promptly, to the extent permitted by Law, notify Seller in writing of such request or requirement so that Seller may seek an appropriate protective order or other appropriate remedy (and if Seller seeks such an order or other remedy, Purchaser will provide such cooperation, at Seller's sole expense, as Seller shall reasonably request). If no such protective order or other remedy is obtained and Purchaser or any of its Permitted Recipients are, in the view of their respective counsel (which may include their respective internal counsel), legally compelled to disclose Confidential Information, Purchaser or its Permitted Recipients, as the case may be, shall only disclose that portion of the Confidential Information that their respective counsel advises that Purchaser or its Permitted Recipients, as the case may be, are compelled to disclose and will exercise commercially reasonable efforts, at Seller's sole expense, to obtain reliable assurance that confidential treatment will be accorded to that portion of the Confidential Information that is being disclosed. In any event, Purchaser will not oppose action by Seller to obtain an appropriate protective order or other reliable assurance that confidential treatment will be accorded the Confidential Information. Notwithstanding the foregoing, notice to Seller shall not be required where disclosure is made (i) in response to a request by a governmental or regulatory authority having competent jurisdiction over Purchaser or its Permitted Recipients, as the case may be, or (ii) in connection with a routine examination by a regulatory examiner, where in each case, such request or examination does not expressly reference Seller, its Affiliates, the Receivables or this Agreement.

(d) *Financial Statements*. Notwithstanding anything herein to the contrary, nothing in this Section 6.9 shall be construed to restrict Purchaser from (i) including disclosure of the Purchase Price and the amount and nature of the Purchased Receivables in the footnotes to Purchaser's audited annual financial statements, to the extent so required by Purchaser's independent accountants, or including comparable disclosure in Purchaser's unaudited quarterly financial statements, or (ii) providing copies of such audited annual and unaudited quarterly financial statements to Purchaser's existing or prospective lenders or direct or indirect beneficial

owners, as long as such lenders or beneficial owners have agreed to be bound by the provisions of this Section 6.9 or are otherwise subject to reasonable restrictions of confidentiality.

(e) *Termination of Confidentiality Agreement*. Effective upon the date hereof, the Confidentiality Agreement, dated [****] (the "*Confidentiality Agreement*"), between Seller and Purchaser shall terminate and be of no further force or effect, and shall be superseded by the provisions of this Section 6.9.

(f) *Specific Enforcement*. Each of Purchaser and Purchaser Representative acknowledges and agrees that remedies at law may not be adequate to protect Seller against any actual or threatened breach of this Section 6.9 by Purchaser, Purchaser Representative, their Affiliates or their Affiliates' Representatives, and that Seller shall be entitled to seek specific performance and temporary and permanent injunctive relief or other equitable relief as a remedy for any such actual or threatened breach. Such remedy shall not be deemed to be the exclusive remedy for breach of this Section 6.9 but shall be in addition to all other rights and remedies available at law or equity to Seller.

Section 6.10 Public Announcements; Use of Names.

(a) Neither party hereto shall, and each party hereto shall instruct its Affiliates and its and its Affiliates' Representatives not to, issue a press release or other public announcement or otherwise make any public disclosure with respect to this Agreement or the subject matter hereof without the prior written consent of the other party hereto (which consent shall not be unreasonably withheld or delayed), except as may be required by applicable Law (in which case the party hereto required by applicable Law to issue or make the press release, public announcement or other public disclosure shall allow the other party hereto reasonable time to comment on such press release, public announcement or other public disclosure in advance of the issuance or making thereof). Notwithstanding the foregoing, (i) Seller and Purchaser hereby agree that a press release relating to the consummation of the transactions contemplated by this Agreement in a form to be reasonably agreed by Purchaser and Seller may be issued by Seller following the Closing (such press release, the "*Specified Press Release*"), (ii) any party hereto may, without the consent of the other party hereto, make public disclosures of any information with respect to this Agreement or the subject matter hereof which is the same as the information that has already been publicly disclosed by such party, or the other party hereto, in the Specified Press Release or otherwise in compliance with the foregoing provisions of this Section 6.10(a) and (iii) if a party hereto determines that it must make any disclosure referred to in the immediately preceding sentence pursuant to securities laws or regulations or the rules and regulations of any securities exchange or market, then such party may make such disclosure (and for the avoidance of doubt, without compliance with the foregoing provisions of this Section 6.10(a)), *provided*, that it will use commercially reasonable efforts to notify the other party in advance and allow them to comment on such disclosure, in each case, to the extent practicable under the circumstances.

(b) Except as contemplated by the last sentence of Section 6.10(a), neither Purchaser nor Purchaser Representative shall, without Seller's prior written consent, identify Seller, its Affiliates or its or its Affiliates' trustees, directors, officers, employees or agents in any advertising, press releases, sales literature or other promotional materials to be disseminated to any

Person other than to Purchaser, Purchaser Representative, their Affiliates and their Affiliates' Representatives and investors (and potential investors).

(c) Notwithstanding anything herein to the contrary, Seller may, without the consent of Purchaser, disclose (and nothing herein shall be construed to restrict Seller from disclosing) the Purchase Price and the amount and nature of the Purchased Receivables in Seller's annual and other periodic reports and financial statements.

Section 6.11 Taxes.

(a) Unless otherwise required as a result of a change in applicable tax law or a determination, within the meaning of section 1313(a) of the Code, each Tax Purchaser and Seller agree (1) to treat the purchase of the Purchased Receivables pursuant to this Agreement as a sale by Seller to Purchaser and a purchase by Purchaser from Seller for U.S. federal and all applicable state and local income tax purposes and, accordingly, the respective Tax Purchaser as the owner of its respective portion of the Purchased Receivables; (2) any and all Purchased Receivables remitted by Seller to a Tax Purchaser pursuant to Section 6.2(a) or otherwise under this Agreement as received by Seller as agent for Tax Purchaser and collected by Seller on behalf of Tax Purchaser.

(b) Each Tax Purchaser and Purchaser Representative agrees (i) to notify Seller and the Escrow Agent promptly in writing if (A) such Tax Purchaser or Purchaser Representative becomes ineligible to use or deliver any Applicable Withholding Certificate or other tax form previously delivered pursuant to this Agreement, or (B) any Applicable Withholding Certificate or other tax form previously delivered pursuant to this Agreement ceases to be accurate or complete, and (ii) to the extent such Tax Purchaser or Purchaser Representative is legally eligible to do so, to provide to Seller and the Escrow Agent any additional tax forms or information relating to any Applicable Withholding Certificate (A) upon reasonable request by Seller or the Escrow Agent and (B) subject to clause (i)(A) of this Section 6.11(a), promptly upon any Applicable Withholding Certificate or other tax form previously delivered pursuant to this Agreement becoming obsolete.

(c) Each Tax Purchaser and Purchaser Representative agrees to notify Seller and the Escrow Agent promptly if the statements in Section 5.10 (if made as of any date after the Closing Date) cease, or because of any change of Law or any act or omission planned, suffered or performed by such Tax Purchaser or Purchaser Representative, would in the future cease, to be true.

(d) Licensee, Seller, the Escrow Agent or any other applicable withholding agent shall be entitled to deduct (or cause to be deducted) from any amount of Purchased Receivables or other amounts payable hereunder (but for this sentence) to such Tax Purchaser or Purchaser Representative any income or other tax that Licensee, Seller, the Escrow Agent or such other withholding agent determines is required to be withheld with respect to such amount; provided, for the avoidance of doubt, that no amounts payable to any Tax Purchaser under this Agreement shall be reduced by any deduction or withholding of taxes attributable to any item of income other than Purchased Receivables or other amounts payable to Purchaser under this Agreement (including past royalty payments to Seller). If Licensee, Seller, the Escrow Agent or any other applicable withholding agent is required to withhold or deduct any such tax, the

applicable withholding agent shall remit (or cause to be remitted) any amount withheld or deducted pursuant to this Section 6.11(d) to the relevant taxing authority in accordance with applicable tax Law. Any amount withheld and deducted by Licensee, Seller, the Escrow Agent or any other withholding agent pursuant to the first sentence of this Section 6.11(d) shall be treated, for purposes of determining the Total Net Amount as of any date, as paid to the relevant Tax Purchaser only to the extent that such amount is deducted or withheld in respect of Connection Taxes. Seller shall use commercially reasonable efforts to give or cause to be given to each Tax Purchaser such assistance and such information concerning the reasons for deduction or withholding of any taxes as may be reasonably necessary to enable such Tax Purchaser to claim exemption therefrom (or a rate reduction), or credit therefor, and, in each case, to furnish such Tax Purchaser with proper evidence of the taxes withheld and remitted to the relevant taxing authority.

Section 6.12 Further Actions. From and after the Closing, each of Purchaser, Purchaser Representative and Seller shall, at the expense of the requesting party, execute and deliver such additional documents, certificates and instruments, and perform such additional acts, as may be reasonably requested and necessary or appropriate to carry out all of the provisions of this Agreement and to give full effect to and consummate the transactions contemplated by this Agreement.

Section 6.13 Prosecution and Maintenance of Listed Patents.

(a) As between Purchaser and Seller, Seller shall have the sole right, but not the obligation, to Prosecute patent applications and issue patents within the Listed Patents, in Seller's sole discretion.

(b) Subject to Section 6.13(c), Seller shall use commercially reasonable efforts to continue to Prosecute, or to cause GSK and Penn to Prosecute, any patent applications, patent term extensions (including supplementary protection certificates), or issued patents, with respect to the Listed Patents.

(c) Purchaser acknowledges that Penn controls Prosecution of the Listed Patents, with Seller having certain rights to review. Purchaser acknowledges and agrees that (a) the rights and obligations under this Section 6.13 are subject to the rights of the ReGenX Licensors set forth in the GSK Agreement and Penn Agreement with respect to the Listed Patents, and (b) Seller's obligations under this Agreement only apply to the extent of Seller's rights with respect to participation in Prosecuting the Listed Patents under the GSK Agreement and the Penn Agreement.

Section 6.14 Enforcement of Listed Patents.

(a) *Notice and Consultation*. In the event that Purchaser or a Responsible Employee of Seller becomes aware of any potential Competitive Infringement of any Listed Patents by a third party, then promptly (and in any event within [****] Business Days) following Purchaser or such Responsible Employee of Seller, respectively, becoming aware of such Competitive Infringement, Purchaser or Seller, respectively, shall inform the other party hereto of such Competitive Infringement. Seller shall provide to Purchaser a copy of any written notice of any Competitive Infringement delivered or received by Seller (other than any such notice provided

by Purchaser) as soon as practicable (and in any event within [****] Business Days) following such delivery or receipt by Seller. Promptly following a Responsible Employee of Seller becoming aware of any Competitive Infringement (whether as a result of being notified by the Licensee pursuant to Section 7.2.1 of the License Agreement, notified by Purchaser pursuant to this Section 6.14(a), or otherwise), Seller and Purchaser shall consult with each other with a view to determining the appropriate course of action to take with respect to such Competitive Infringement (in each case, subject to the terms and conditions of the License Agreement).

(b) *Enforcement*. Subject to Section 6.14(e), if Seller has the right pursuant to Section 7.2.2 of the License Agreement and applicable Law to institute suit or other legal proceedings to enforce any of the Listed Patents in respect of any Competitive Infringement, then promptly (and in any event within [****] Business Days) following a Responsible Employee of Seller becoming aware of such right of Seller, Seller shall provide notice of such right to Purchaser. In the event that Seller declines to exercise such right, Seller shall promptly give notice of such declination to Purchaser, and the Required Royalty Party shall have [****] Business Days to require that Seller proceed, in consultation with the Required Royalty Party, to institute such a suit or other legal proceeding and to use commercially reasonable efforts to enforce the Listed Patents in respect of such Competitive Infringement, and to exercise such rights and remedies relating to such Competitive Infringement as shall be available to Seller under applicable Law, but, in each case, subject to the terms and conditions of the License Agreement and this Agreement. In connection with any such enforcement of the Listed Patents, Seller may employ any counsel, so long as such counsel is acceptable to the Required Royalty Party (such acceptance not to be unreasonably withheld or delayed).

(c) *Allocation of Proceeds of Enforcement*. Subject to Section 6.14(e), to the extent in respect of a Competitive Infringement, the Proceeds of any enforcement of any of the Listed Patents (i) by Seller pursuant to this Section 6.14 and Section 7.2 of the License Agreement or (ii) by Seller together with the Licensee pursuant to Section 7.2 of the License Agreement, in each case of the immediately foregoing clauses (i) and (ii), after deduction (and reimbursement to Seller and Purchaser) of all costs and expenses (including attorneys' fees and expenses) incurred by Seller and Purchaser in connection with such enforcement pursuant to the first two sentences of Section 6.14(d) below, shall be allocated between, and paid to, Seller and Purchaser in proportion to their respective interests in the Receivables (taking into account any variation in such interests over different time periods, if applicable) (such payment, after giving effect to such deduction and reimbursement, is referred to in the immediately succeeding sentence as the "net payment"). To the extent not on account of Purchased Receivables, the Proceeds of any enforcement of any of the Listed Patents after deduction (and reimbursement to Seller and Purchaser) of all costs and expenses (including attorneys' fees and expenses) incurred by Seller and Purchaser in connection with such enforcement pursuant to the first two sentences of Section 6.14(d) below, shall be paid to Seller. Notwithstanding anything to the contrary herein, and for the avoidance of doubt, it is understood and agreed that Purchaser shall not be entitled to receive any such net payment if the Threshold Date or the Call Closing Date has occurred.

(d) *Allocation of Costs of Enforcement*. All costs and expenses (including attorneys' fees and expenses) incurred by Seller in connection with any enforcement of any of the Listed Patents in respect of a Competitive Infringement shall be borne by Seller and Purchaser in proportion to their respective interests in the Receivables (taking into account any variation in such

interests over different time periods, if applicable). Purchaser shall fund any retainers or advances required by the counsel employed by Seller for such enforcement (such amounts to be credited or deducted from the actual amounts owed by Seller and Purchaser under the immediately preceding sentence). Nothing contained herein shall limit Purchaser from retaining, at its sole cost, separate outside counsel who shall be permitted, where reasonably practicable, to consult with the lead counsel selected pursuant to Section 6.14(b) for such enforcement.

(e) *ReGenX Licensors*. Purchaser acknowledges and agrees that (a) the rights and obligations under this Section 6.14 are subject to the rights of the ReGenX Licensors under the GSK Agreement and Penn Agreement (including any consent or approval rights or rights to control or participate in any enforcement actions); and (b) Seller's obligations under this Agreement only apply to the extent that Seller has any rights with respect to enforcing the Listed Patents under the GSK Agreement and the Penn Agreement. Furthermore, Licensee acknowledges the following:

(i) All monies recovered upon the final judgment or settlement of any action with respect to Competitive Infringement will also need to be allocated to the ReGenX Licensors (a) to reimburse the costs and expenses (including reasonable attorneys' fees and costs) of such licensors, (b) to take into account the royalties payable to such licensors; and (c) to take into account the relative extent of such licensors' financial participation in such action, if applicable.

(ii) The ReGenX Licensors retain the continuing right to intervene at their own expense and join Seller in any claim or suit for infringement of the Listed Patents.

(iii) In any infringement prosecuted by the ReGenX Licensors, all financial recoveries will be entirely retained by such licensors.

(iv) The written consent of the ReGenX Licensors will be required (a) for any decision that would have a materially adverse effect on the validity, scope of patent claims, or enforceability of the Listed Patents and (b) for any settlement or compromise of any infringement suit that would impose any obligations or restrictions on either of the ReGenX Licensors, or grants any rights to the Listed Patents other than rights that Seller has the right to grant under the License Agreement.

Section 6.15 Defense of Third Party Infringement Claims. In the event that Purchaser or a Responsible Employee of Seller becomes aware of any claim or suit by any third party for Competitive Infringement of a patent or other intellectual property of such third party as further described in Section 7.3 of the License Agreement ("*Third Party Infringement Claim*"), then promptly (and in any event within [****] Business Days) following Purchaser or such Responsible Employee of Seller, respectively, becoming aware of such Third Party Infringement Claim, Purchaser or Seller, respectively, shall inform the other party hereto of such Third Party Infringement Claim. Seller shall provide to Purchaser a copy of any written notice of any Third Party Infringement Claim delivered or received by Seller (other than any such notice provided by Purchaser) as soon as practicable (and in any event within [****] Business Days) following such

delivery or receipt by Seller. Promptly following a Responsible Employee of Seller becoming aware of any Third Party Infringement Claim (whether as a result of being notified by the Licensee pursuant to Section 7.3 of the License Agreement, notified by Purchaser pursuant to this Section 6.15(a), or otherwise), Seller and Purchaser shall consult with each other with a view to determining the appropriate course of action to take with respect to such Third Party Infringement Claim (in each case, subject to the terms and conditions of the License Agreement).

Section 6.16 Acknowledgment and Agreement by Purchaser; Limitation of Seller's Duties and Obligations.

(a) Notwithstanding any provision of this Agreement (including other provisions of this Article VI) to the contrary, nothing contained in this Agreement shall obligate Seller to (i) take any action, or omit to take any action, that (A) would conflict with, violate or cause a violation of, contravene or cause a default under, the License Agreement, the GSK Agreement or any applicable Law or any Judgment binding upon, or any guidelines or policies of, Seller, (B) would otherwise, in the judgment of Seller (exercised reasonably), adversely affect in any material respect Seller, including by means of exposing Seller to material liability (whether in relation to the transactions contemplated by the License Agreement, the GSK Agreement or otherwise), or (C) would, or would involve any disclosure that would, result in the loss or waiver of any attorney-client privilege available to Seller; *provided*, that Seller shall use its commercially reasonable efforts to implement arrangements that would permit such action, omission or disclosure while preserving such privilege; or (ii) assign or otherwise transfer any Seller IP Assets to Purchaser or any other Person.

(b) Notwithstanding any provision of this Agreement (including other provisions of this Article VI) to the contrary, nothing contained in this Agreement shall be construed to prevent, limit or restrict Seller from exercising, pursuing and enforcing its rights in respect of any Excluded Asset in a commercially reasonable manner (including by means of assertion and enforcement of, and the taking of other actions with respect to, the Seller IP Assets); *provided*, *however*, that (i) all costs and expenses (including attorneys' fees and expenses) incurred by Seller in connection therewith shall be borne solely by Seller, (ii) Seller shall not, in connection therewith, terminate the License Agreement without the prior written consent of the Required Royalty Party (such consent not to be unreasonably withheld or delayed) and (iii) in connection therewith Seller shall not, without the prior written consent of the Required Royalty Party (such consent not to be unreasonably withheld or delayed), execute or agree to execute any proposed Modification of the License Agreement or the GSK Agreement if such Modification would reasonably be expected to adversely affect in any material respect the value of the Purchased Receivables (it being understood and agreed that any proposed Modification to the provisions of the License Agreement governing the amount or calculation of the Purchased Receivables (including the Royalty Deductions) or the procedures for payment of the Purchased Receivables, or the GSK Agreement governing the amount or calculation of the payments related to Zolgensma pursuant to Section 3.3 or the procedures for payment of such payments, shall be deemed, for purposes of this Section 6.16(b), to have such an effect).

Section 6.17 Seller's Commercially Reasonable Efforts and Judgment. It is understood and agreed that, in determining whether Seller's efforts or judgments are "commercially reasonable" with respect to any covenant that specifically references such term in this Article VI,

Seller shall be deemed to be acting or making a judgment in a commercially reasonable manner if Seller would reasonably be expected to act in the same manner if Seller had the sole right, title and interest in and to the Receivables and the Proceeds.

Section 6.18 Commercialization. If Purchaser shall so request (by written notice to Seller), Seller and Purchaser shall consult with each other from time to time regarding the status of the Licensee's, its Affiliates' and its sub-licensees' compliance with the development, commercialization, marketing and promoting obligations under Section 4.1 of the License Agreement.

Section 6.19 Licensee Instruction Letter. Prior to the termination of this Agreement pursuant to Section 8.14(a), Seller shall not, without Purchaser's prior written consent, deliver any further directions to Licensee regarding the payment of the Receivables of the type referred to in paragraph no. 3 of the Licensee Instruction Letter.

Section 6.20 Purchaser Consent Rights. It is understood and agreed that, in determining whether Purchaser would be reasonable in withholding its consent to a proposed action pursuant to Article VI, Purchaser shall be deemed to be acting reasonably in withholding its consent if (a) Purchaser would reasonably be expected to suffer adverse economic consequences as a result of the action to which its consent is sought and (b) Purchaser shall not be provided with reasonable compensation in respect thereof.

Section 6.21 Penn Agreement. To the extent the GSK Agreement is terminated in accordance with Article 6 thereof, the obligations in Section 6.1, Section 6.3 and Section 6.16 applicable to the GSK Agreement shall thereafter apply *mutatis mutandis* to the Penn Agreement, effective as of the date of such termination.

ARTICLE VII

INDEMNIFICATION

Section 7.1 Obligation of Parties to Indemnify.

(a) *Indemnification by Seller*. Subject to the limitations set forth in this Article VII, from and after the Closing, Seller shall indemnify Purchaser against any and all Losses incurred by Purchaser or its directors, officers, employees or agents (each, a "*Purchaser Indemnified Party*"), to the extent arising or resulting from any of the following:

(i) any breach of any representation or warranty made by Seller in this Agreement; it being understood that, for purposes of this Section 7.1(a)(i), and without limiting the applicability of Section 7.4(a), in determining the amount of any Loss arising therefrom, any qualifications in the representations or warranties of Seller in this Agreement with respect to material adverse effect, materiality, material or similar standards shall be disregarded and will not have any effect (but, for the avoidance of doubt, such qualifications shall not be disregarded for purposes of determining whether a breach of any representation or warranty has occurred, and therefore, whether any indemnification is owed by Seller under this Section 7.1(a)(i);

(ii) any breach of any covenant or agreement of Seller contained in any of the Transaction Documents;

(iii) (A) the matters described in Sections 2(c)(i), (c)(ii) and (d)(iii) of the Schedule of Exceptions and (B) solely to the extent Purchaser is joined or made a party to an action, suit or proceeding relating thereto, the matters described in Sections 2(c)(iii), (d)(i), (d)(ii) and (d)(iv) of the Schedule of Exceptions, in the case of this clause (B) to the extent Purchaser (solely in its capacity as such) incurs direct Losses (excluding Lost Profits and any diminution in value of the Purchased Receivables (including the timing, amount or duration thereof)) as a result of such action, suit or proceeding;

(iv) the Excluded Obligations and Liabilities.

(b) *Indemnification by Purchaser and Purchaser Representative*. Subject to the limitations set forth in this Article VII, from and after the Closing, each of Purchaser and Purchaser Representative shall indemnify Seller against any and all Losses incurred by Seller or its trustees, directors, officers, employees or agents (each, a "*Seller Indemnified Party*"), to the extent arising or resulting from any of the following:

(i) any breach of any representation or warranty made by Purchaser and Purchaser Representative in this Agreement; it being understood that, for purposes of this Section 7.1(b)(i), and without limiting the applicability of Section 7.4(b), in determining the amount of any Loss arising therefrom, any qualifications in the representations or warranties of Purchaser or Purchaser Representative in this Agreement with respect to material adverse effect, materiality, material or similar standards shall be disregarded and will not have any effect (but, for the avoidance of doubt, such qualifications shall not be disregarded for purposes of determining whether a breach of any representation or warranty has occurred, and therefore, whether any indemnification is owed by Purchaser or Purchaser Representative under this Section 7.1(b)(i);

(ii) any breach of any covenant or agreement of Purchaser or Purchaser Representative contained in any of the Transaction Documents; and

(iii) the Assumed Obligations and Liabilities.

Section 7.2 Procedures Relating to Indemnification for Third Party Claims.

(a) *Notice of Third Party Claim*. In order for a party (an "*Indemnified Party*") to be entitled to any indemnification under this Article VII in respect of Losses arising out of or involving a claim or demand made by any Person other than Purchaser or Seller against a Purchaser Indemnified Party or a Seller Indemnified Party, as applicable (a "*Third Party Claim*"), the Indemnified Party must notify the party from whom indemnification is sought under this Article VII (the "*Indemnifying Party*") promptly in writing (including in such notice a brief description of the Third Party Claim, including damages sought or estimated, to the extent actually known or reasonably capable of estimation by the Indemnified Party); *provided*, *however*, that the failure to promptly provide such notice shall not affect the indemnification provided under this Article VII except to the extent that the Indemnifying Party has been actually prejudiced as a result of such failure. Thereafter, the Indemnified Party shall deliver to the Indemnifying Party, promptly after

the Indemnified Party's receipt thereof, copies of all documents (including court papers) received by the Indemnified Party relating to such Third Party Claim.

(b) *Defense of Third Party Claims*. The Indemnifying Party shall be entitled to participate in the defense of any Third Party Claim and, if it so chooses, to assume the defense thereof, at its own expense, with counsel selected by the Indemnifying Party; *provided*, that such counsel is not reasonably objected to by the Indemnified Party. If the Indemnifying Party elects to assume the defense of any Third Party Claim, the Indemnifying Party shall not be liable to the Indemnified Party for legal expenses subsequently incurred by the Indemnified Party in connection with the defense thereof, except that, if the Indemnifying Party and the Indemnified Party have conflicting interests or different defenses available with respect to such Third Party Claim (such that, based on the advice of counsel, representation by the same counsel would be inappropriate), the Indemnified Party may hire its own separate counsel (*provided*, that such counsel is not reasonably objected to by the Indemnifying Party) with respect to such Third Party Claim and the related action or suit, and the reasonable fees and expenses of such counsel shall be considered Losses for purposes of this Agreement. If the Indemnifying Party elects to assume the defense of any Third Party Claim, the Indemnifying Party shall permit the Indemnified Party to participate in, but not control, the defense of such Third Party Claim through counsel chosen by the Indemnified Party, *provided*, that such counsel is not reasonably objected to by the Indemnifying Party and, except in the circumstances described in the immediately preceding sentence, the fees and expenses of such counsel shall be borne by the Indemnified Party. The Indemnifying Party shall be liable for the reasonable fees and expenses of counsel employed by the Indemnified Party in the defense of a Third Party Claim (which shall all be considered Losses for purposes of this Agreement) for any period during which the Indemnifying Party has not assumed the defense thereof (other than during the period prior to the time the Indemnified Party shall have notified the Indemnifying Party of such Third Party Claim).

(c) *Cooperation*. The parties hereto shall cooperate in the defense or prosecution of any Third Party Claim, with such cooperation to include (i) the retention of and the provision to the Indemnifying Party of records and information that are reasonably relevant to such Third Party Claim and (ii) the making available of employees on a mutually convenient basis for providing additional information and explanation of any material provided hereunder. If the Indemnifying Party shall have assumed the defense of a Third Party Claim, the Indemnified Party shall agree to any settlement, compromise or discharge of such Third Party Claim that the Indemnifying Party may recommend and that by its terms obligates the Indemnifying Party to pay the full amount of the liability (if any) in connection with such Third Party Claim and which does not involve any non-monetary penalties and which releases the Indemnified Party completely and unconditionally in connection with such Third Party Claim. Regardless of whether the Indemnifying Party shall have assumed the defense of a Third Party Claim, the Indemnified Party shall not be entitled to be indemnified or held harmless pursuant to this Article VII if the Indemnified Party shall settle such Third Party Claim without the prior written consent of the Indemnifying Party (such consent not to be unreasonably withheld or delayed).

Section 7.3 Procedures Relating to Indemnification for Other Claims. In order for an Indemnified Party to be entitled to any indemnification under this Article VII in respect of Losses that do not arise out of or involve a Third Party Claim, the Indemnified Party must notify the Indemnifying Party promptly in writing (including in such notice a brief description of the claim

for indemnification and the Loss, including damages sought or estimated, to the extent actually known or reasonably capable of estimation by the Indemnified Party); *provided*, *however*, that the failure to promptly provide such notice shall not affect the indemnification provided under this Article VII except to the extent that the Indemnifying Party has been actually prejudiced as a result of such failure.

Section 7.4 Limitations on Indemnification.

(a) *Seller*. Notwithstanding anything in this Agreement to the contrary, Seller shall not have any liability:

(i) under any clause of Section 7.1(a) with respect to any individual item (or any series of related items) if the Loss related thereto is less than [****];

(ii) under clause (i) of Section 7.1(a) unless the aggregate liability for all Losses suffered by the Purchaser Indemnified Parties thereunder exceeds [****], and then only to the extent of such excess; or

(iii) under clause (i) of Section 7.1(a), on any day on which such indemnity claim under clause (i) of Section 7.1(a) is paid by Seller, in excess of the Cap Amount for such day. "*Cap Amount*" means, for any day on which an indemnity claim under clause (i) of Section 7.1(a) is paid by Seller, the excess of [****].

(b) *Purchaser*. Notwithstanding anything in this Agreement to the contrary, Purchaser shall not have any liability:

(i) under any clause of Section 7.1(b) with respect to any individual item (or any series of related items) if the Loss related thereto is less than [****];

(ii) under clause (i) of Section 7.1(b) unless the aggregate liability for all Losses suffered by the Seller Indemnified Parties thereunder exceeds [****], and then only to the extent of such excess; or

(iii) under clause (i) of Section 7.1(b) in excess of [****].

Section 7.5 Survival of Representations and Warranties. The representations and warranties contained in this Agreement shall survive the Closing solely for purposes of Section 7.1 and shall terminate on the Applicable Survival Date. No party hereto shall have any liability or obligation of any nature with respect to any representation or warranty after the termination thereof, unless the other party hereto shall have delivered a notice to such party pursuant to Section 7.2(a) or Section 7.3, claiming such a liability or obligation under Section 7.1, prior to the Applicable Survival Date. "*Applicable Survival Date*" means (a) in the case of the representations and warranties contained in Sections 4.1, 4.2, 4.3, 4.9(a), 4.9(b), 4.9(c), 4.10(a), 4.10(b), 4.10(c), 4.13(a), 4.13(b), 4.13(c), 4.15, 5.1, 5.2 and 5.3, [****], (b) in the case of the representations and warranties contained in Sections 4.9(d), 4.9(e), 4.9(k), 4.9(n), 4.10(d), 4.10(e), 4.13(d) and 4.13(e), [****] and (c) in all other cases, [****].

Section 7.6 No Implied Representations and Warranties. Purchaser acknowledges and agrees that, (x) other than the representations and warranties of Seller specifically contained in Article IV, there are no representations or warranties of Seller or any other Person either expressed or implied (for the benefit of Purchaser) with respect to the Royalty Payments, the Receivables, the Seller IP Assets, the License Agreement, the GSK Agreement, the Penn Agreement, any sub-license granted under the License Agreement or the transactions contemplated by the Transaction Documents, the License Agreement, the GSK Agreement, the Penn Agreement, or any sub-license granted under the License Agreement and (y) that it does not rely on, and shall have no remedies in respect of, any representation or warranty not specifically set forth in Article IV. Without limiting the foregoing, Purchaser acknowledges and agrees that Purchaser, together with its Affiliates and its and its Affiliates' Representatives, have made their own investigation of the Royalty Payments, the Receivables, the Seller IP Assets, the License Agreement, the GSK Agreement, the Penn Agreement, any sub-license granted under the License Agreement and the transactions contemplated by the Transaction Documents, the License Agreement, the GSK Agreement, the Penn Agreement and any sub-license granted under the License Agreement and are not relying on, and shall have no remedies in respect of, (a) any implied warranties or (b) any representation or warranty whatsoever as to the future amount or potential amount of the Royalty Payments and the Receivables, as to the validity or value of the Seller IP Assets, or as to the creditworthiness of the Licensee (or any of its Affiliates), any sub-licensee party to a sub-license granted under the License Agreement (or any of its Affiliates), except as otherwise expressly set forth in this Agreement.

Section 7.7 Exclusive Remedy. Other than for breaches of any covenants or agreements set forth in Section 6.9 or Article VIII, the parties hereto acknowledge and agree that, from and after the Closing, this Article VII (including Section 7.4 and Section 7.5) shall provide such parties' sole and exclusive remedy with respect to any matter or claim arising out of, relating to or in connection with any of the Transaction Documents or any of the transactions contemplated thereby, except that any such claim or matter based upon fraud, deliberate or willful breach of covenant or willful misconduct shall not be subject to or limited by this Article VII.

Section 7.8 Limitations on Damages. Notwithstanding anything to the contrary in this Agreement or any of the other Transaction Documents, in no event shall either party hereto be liable (including under Section 7.1) for any (i) special, indirect, incidental, exemplary, punitive, multiple or consequential damages or (ii) loss of use, business interruption, loss of any contract or other business opportunity or good will, in each case of clauses (i) and (ii), of the other party hereto (other than any such damages or losses occasioned by any breach of the covenants or agreements set forth in Section 6.9), whether or not caused by or resulting from the actions of such party or the breach of its covenants, agreements, representations or warranties under any of the Transaction Documents (except as aforesaid) and whether in contract, tort or breach of statutory duty or otherwise, even if such party has been advised of the possibility of such damages; *provided*, *however*, that the foregoing shall not limit in any way Seller's liability in respect of Lost Profits.

ARTICLE VIII

MISCELLANEOUS

Section 8.1 Headings. The captions to the Articles, Sections and subsections hereof are not a part of this Agreement but are for convenience only and shall not be deemed to limit or otherwise affect the construction of the provisions of this Agreement.

Section 8.2 Notices. All notices and other communications under this Agreement to a party hereto shall be in writing and shall be sent by email with PDF attachment, internationally recognized overnight delivery service or personal delivery to the following address of such party, or to such other address as shall be designated from time to time by such party in accordance with this Section 8.2:

If to:	Address:	With copies to (which shall not constitute service of process):
Seller	REGENXBIO Inc. 9600 Blackwell Road Suite 210 Rockville, MD 20850 *Attention*: Patrick Christmas Email: pchristmas@regenxbio.com	Covington & Burling LLP The New York Times Building 620 Eighth Avenue New York, NY 10018 *Attention*: Peter A. Schwartz Email: pschwartz@cov.com
Purchaser	c/o HCR Collateral Management LLC 300 Atlantic Street Suite 600 Stamford, CT 06901 *Attention*: Clarke B. Futch Anthony Rapsomanikis Email: clarke.futch@hcroyalty.com anthony.rapsomanikis@hcroyalty.com	c/o HCR Collateral Management LLC 300 Atlantic Street Suite 600 Stamford, CT 06901 *Attention*: Chief Legal Officer Email: royalty-legal@hcroyalty.com Morgan, Lewis & Bockius LLP 1701 Market Street Philadelphia, PA 19103 *Attention*: Andrew Mariniello Email: andrew.mariniello@morganlewis.com

All notices and communications under this Agreement shall be effective upon receipt by the addressee. Notwithstanding anything to the contrary in this Section 8.2, all notices and communications under Sections 7.2(a) and 7.3 and all service of legal process shall be sent by internationally recognized overnight delivery service or personal delivery.

Section 8.3 No Personal Liability. It is expressly understood and agreed by Seller, Purchaser and Purchaser Representative that:

(a) each of the representations, warranties, covenants and agreements in the Transaction Documents made on the part of Seller is made by Seller and is not intended to be nor is a personal representation, warranty, covenant or agreement of any other Person, including those Persons named in the definition of "Knowledge of Seller" and any other Representative of Seller or Seller's Affiliates (the "*Non-Warranting Parties*");

(b) other than Seller, no Person, including the Non-Warranting Parties, shall have any liability whatsoever for breach of any representation, warranty, covenant or agreement made in the Transaction Documents on the part of Seller or in respect of any claim or matter arising out of, relating to or in connection with the Transaction Documents or the transactions contemplated thereby; and

(c) the provisions of this Section 8.3 are intended to benefit each and every one of the Non-Warranting Parties and shall be enforceable by each and every one of them to the fullest extent permitted by Law.

Section 8.4 Expenses. Other than the fees, costs and expenses of the Escrow Agent, all fees, costs and expenses (including any legal, accounting, financial advisory and banking fees) incurred in connection with the preparation, negotiation, execution and delivery of the Transaction Documents and to consummate the transactions contemplated thereby shall be paid by the party hereto incurring such fees, costs and expenses. The fees, costs and expenses of the Escrow Agent shall be borne in the manner specified in the Escrow Agreement.

Section 8.5 Assignment

(a) *By Purchaser*. Neither this Agreement nor any of Purchaser's rights, interests or obligations hereunder (including Purchaser's rights in respect of the Purchased Receivables) may be assigned, novated, delegated or transferred, in whole or in part, by Purchaser without the prior written consent of Seller (such consent not to be unreasonably withheld, conditioned or delayed), and any such purported assignment, novation, delegation or transfer without such consent shall be void ab initio and of no effect; *provided*, *however*, that Purchaser may assign this Agreement in accordance with the provisions of Schedule 8.5.

(b) *By Seller*. Neither this Agreement nor any of Seller's rights, interests or obligations hereunder may be assigned, novated, delegated or otherwise transferred, in whole or in part, by operation of Law, merger, change of control, or otherwise, by Seller without the prior written consent of Purchaser, and any such purported assignment, novation, delegation or transfer without such consent shall be void ab initio and of no effect; *provided*, *however*, that following the Closing, Seller may, upon prior written notice to Purchaser, but without the prior written consent of Purchaser, assign this Agreement and Seller's rights, interests and obligations hereunder to any Affiliate of Seller or to any successor in interest (whether by merger, acquisition, asset purchase or otherwise) to all or substantially all of the business to which the License Agreement relates if such assignee (i) succeeds, or in connection with such transaction shall succeed, to all of Seller's right, title and interest in and to the License Agreement; (ii) agrees in a writing to be bound by all

the provisions of this Agreement as if such assignee were the "Seller" under this Agreement and (iii) [****].

(c) *Successors and Assigns*. Subject to the provisions of Section 8.5 and Schedule 8.5, this Agreement shall be binding upon, inure to the benefit of and be enforceable by, the parties hereto and their respective permitted successors and assigns. For the avoidance of doubt, the provisions of Section 6.11 will inure to the benefit of any assignee of Purchaser.

Section 8.6 Amendment and Waiver.

(a) This Agreement may be amended, modified or supplemented only in a writing signed by both parties hereto. Any provision of this Agreement may be waived only in a writing, which writing must be signed by the party hereto granting such waiver.

(b) No failure or delay on the part of either party hereto in exercising any right, power or remedy hereunder shall operate as a waiver thereof, nor shall any single or partial exercise of any such right, power or remedy preclude any other or further exercise thereof or the exercise of any other right, power or remedy. No course of dealing between the parties hereto shall be effective to amend, modify, supplement or waive any provision of this Agreement.

Section 8.7 Entire Agreement. This Agreement, including the Exhibits and Schedules attached to this Agreement, sets forth the entire agreement and understanding between the parties hereto as to the subject matter hereof. All express or implied agreements, promises, assurances, arrangements, representations, warranties and understandings as to the subject matter hereof, whether oral or written, heretofore made are superseded by this Agreement.

Section 8.8 Independent Contractors. The parties hereto recognize and agree that each is operating as an independent contractor and not as an agent, partner or fiduciary of the other. Each party agrees not to refer to the other as a "partner" or the relationship as a "partnership" or "joint venture" or other kind of legal entity or legal form.

Section 8.9 No Third Party Beneficiaries. Except to the extent otherwise contemplated by Section 8.3, this Agreement is for the sole benefit of Seller and Purchaser and their respective permitted successors and assigns, and nothing herein expressed or implied shall give or be construed to give to any Person, other than the parties hereto and such successors and assigns, any legal or equitable rights hereunder. For the avoidance of doubt, indemnification under Article VII in respect of Losses incurred by a Purchaser Indemnified Party or a Seller Indemnified Party may only be enforced by Purchaser or Seller, respectively, and not by any other Person.

Section 8.10 Governing Law. This Agreement shall be governed by and construed and interpreted in accordance with the Laws of State of New York without regard to the conflicts of Laws principles thereof to the extent that such principles would require or permit the application of the Laws of a jurisdiction other than the State of New York.

Section 8.11 Jurisdiction; Venue; Service of Process; Waiver of Jury Trial. Each party hereto irrevocably submits to the exclusive jurisdiction of (a) the courts of the State of New York located in New York County, New York and (b) the United States District Court for the Southern District of New York for the purposes of any suit, action or other proceeding arising out of, relating

to or in connection with this Agreement or any transaction contemplated hereby. Each party hereto agrees to commence any action, suit or other proceeding arising out of, relating to or in connection with this Agreement or any transaction contemplated hereby in the United States District Court for the Southern District of New York or if such suit, action or other proceeding may not be brought in such court for jurisdictional reasons, in the courts of the State of New York located in New York County, New York. Each party hereto irrevocably and unconditionally waives any objection to the laying of venue of any action, suit or other proceeding arising out of, relating to or in connection with this Agreement or any transaction contemplated hereby in (i) the courts of the State of New York located in New York County, New York or (ii) the United States District Court for the Southern District of New York, and hereby further irrevocably and unconditionally waives, and shall not assert by way of motion, defense, or otherwise, in any such suit, action or proceeding, any claim that it is not subject personally to the jurisdiction of the above-named courts, that its property is exempt or immune from attachment or execution, that the suit, action or proceeding is brought in an inconvenient forum, that the venue of the suit, action or proceeding is improper, or that this Agreement and the transactions contemplated hereby and thereby may not be enforced in or by any of the above-named courts. **Each party hereto irrevocably and unconditionally waives any right to trial by jury with respect to any proceeding arising out of, relating to or in connection with this Agreement or any transaction contemplated hereby.**

Section 8.12 Severability. If any term or provision of this Agreement is held to be invalid, illegal or unenforceable by a court, arbitrator or Governmental Entity of competent jurisdiction, such invalidity, illegality or unenforceability shall not affect any other term or provision of this Agreement, which shall remain in full force and effect, and the parties hereto shall replace such term or provision with a new term or provision permitted by applicable Law and having an economic effect as close as possible to the invalid, illegal or unenforceable term or provision. The holding of a term or provision to be invalid, illegal or unenforceable in a jurisdiction shall not have any effect on the application of such term or provision in any other jurisdiction.

Section 8.13 Counterparts. This Agreement may be executed in any number of counterparts and by the parties hereto in separate counterparts, each of which when so executed shall be deemed to be an original and all of which taken together shall constitute one and the same agreement. Copies of executed counterparts transmitted by email with PDF attachment shall be considered original executed counterparts.

Section 8.14 Termination of Agreement.

(a) Subject to Section 8.14(b), this Agreement shall continue in full force and effect until the earliest of (i) the Call Closing Date, (ii) the Threshold Date and (iii) the date on which Purchaser has received the last payment of Purchased Receivables pursuant to the License Agreement (such earliest date, the "*Termination Date*"). Immediately upon the Termination Date, this Agreement shall terminate, save for any rights, obligations or claims of either party hereto which have accrued prior to the Termination Date (along with any corresponding limitations of liability in respect thereof).

(b) The following provisions shall survive any termination of this Agreement pursuant to this Section 8.14: Article I (Definitions; Interpretation), Section 6.9 (Confidentiality),

Section 6.10 (Public Announcements; Use of Names), Section 6.16 (Acknowledgment and Agreement by Purchaser; Limitation of Seller's Duties and Obligations), Article VII (Indemnification) (but only if a claim under Article VII is pending on the date of termination of this Agreement) and Article VIII (Miscellaneous); *provided*, *however*, that Article VII shall survive only until the final resolution of such claim and the satisfaction of all obligations hereunder related to such claim. The termination of this Agreement for any reason shall not release either party from any obligation or liability which, at the time of such expiration or termination, has already accrued to the other party.

(c) Immediately upon the Termination Date, each Party shall execute and deliver to the other Party all documents as such Party shall reasonably request to evidence the termination of this Agreement.

Section 8.15 Purchaser Representative. Seller and Purchaser acknowledge that HCR Agent has been appointed by the entities set forth on Schedule 1.1 as the representative, agent, proxy and attorney in fact (coupled with an interest) of Purchaser for all respects under this Agreement ("*Purchaser Representative*"). The Purchasers shall be bound by and responsible for any actions taken by Purchaser Representative hereunder in proportion to their respective interests hereunder. Unless notified in writing by HCR Agent or any successor Person acting as Purchaser Representative that HCR Agent or such Person has resigned as Purchaser Representative and a new Purchaser Representative has been appointed in accordance with this Section 8.15, Seller shall be entitled (a) to coordinate all communications under this Agreement with HCR Agent or such Person (in each case, in its capacity as Purchaser Representative), (b) to act upon the directions, instructions and notices of HCR Agent or such Person (in each case, in its capacity as Purchaser Representative) and (c) to make any payments required to be made hereunder to Purchaser to HCR Agent or such Person (in each case, in its capacity as Purchaser Representative), and the receipt of any such payment by HCR Agent shall relieve Seller of any further obligation to Purchaser with respect thereto. Prior to any such resignation of HCR Agent or any successor Person as Purchaser Representative becoming effective, a majority in interest of the Purchasers shall select and appoint a new Purchaser Representative from among the Purchasers and their Affiliates or that is otherwise acceptable to Seller in its reasonable discretion by prior written notice to Seller, and in each case such Person shall thereafter be considered the Purchaser Representative for all purposes hereunder.

[*Signature Pages Follow*]

IN WITNESS WHEREOF, the parties hereto have caused this Agreement to be executed by their respective representatives thereunto duly authorized as of the date first above written.

SELLER:

REGENXBIO INC.

/s/ Kenneth T. Mills
Name: Kenneth T. Mills
Title: President and Chief Executive Officer

PURCHASER:

HEALTHCARE ROYALTY PARTNERS IV, L.P.
By: HealthCare Royalty GP IV LLC, its general partner

By: /s/ Clarke B. Futch
Name: Clarke B. Futch
Title: Managing Partner

HCRP OVERFLOW FUND, L.P.
By: HCRP Overflow Fund GP, LLC, its general partner

By: /s/ Clarke B. Futch
Name: Clarke B. Futch
Title: Managing Partner

HCR STAFFORD FUND, L.P.
By: HCR Stafford Fund GP, LLC, its general partner

By: /s/ Clarke B. Futch
Name: Clarke B. Futch
Title: Managing Partner

HCR POTOMAC FUND, L.P.
By: HCR Potomac Fund GP, LLC, its general partner

By: /s/ Clarke B. Futch
Name: Clarke B. Futch
Title: Managing Partner

HCR CANARY FUND, L.P.
By: HCR Canary Fund GP, LLC, its general partner

By: /s/ Clarke B. Futch
Name: Clarke B. Futch
Title: Managing Partner

PURCHASER REPRESENTATIVE:

HCR COLLATERAL MANAGEMENT, LLC

By: /s/ Clarke B. Futch
Name: Clarke B. Futch
Title: Managing Partner

PURCHASER ENTITIES

HealthCare Royalty Partners IV, L.P.
HCRP Overflow Fund, L.P.
HCR Stafford Fund, L.P.
HCR Potomac Fund, L.P.
HCR Canary Fund, L.P.

EXHIBIT 21.1

Subsidiaries of REGENXBIO Inc.

Name of Subsidiary	Jurisdiction of Incorporation or Organization
REGENXBIO EU Limited	Ireland

EXHIBIT 23.1

CONSENT OF INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM

We hereby consent to the incorporation by reference in the Registration Statements on Form S-3 (No. 333-226691) and Form S-8 (No. 333-229910, 333-223466, 333-216508, 333-209899, 333-206984, 333-236664) of REGENXBIO Inc. of our report dated March 1, 2021 relating to the financial statements and the effectiveness of internal control over financial reporting, which appears in this Form 10-K.

/s/ PricewaterhouseCoopers LLP
Arlington, Virginia
March 1, 2021

EXHIBIT 31.1

CERTIFICATION

I, Kenneth T. Mills, certify that:

1. I have reviewed this Annual Report on Form 10-K of REGENXBIO Inc.;

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:

   a. designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

   b. designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

   c. evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

   d. disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting.

5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

   a. all significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

   b. any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: March 1, 2021

/s/ Kenneth T. Mills

Kenneth T. Mills
President and Chief Executive Officer
(Principal Executive Officer)

EXHIBIT 31.2

CERTIFICATION

I, Vittal Vasista, certify that:

1. I have reviewed this Annual Report on Form 10-K of REGENXBIO Inc.;

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:

   a. designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

   b. designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

   c. evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

   d. disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting.

5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

   a. all significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

   b. any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: March 1, 2021

/s/ Vittal Vasista

Vittal Vasista
Chief Financial Officer
(Principal Financial and Accounting Officer)

EXHIBIT 32.1

CERTIFICATION

In connection with the Annual Report of REGENXBIO Inc. (the "Registrant") on Form 10-K for the year ended December 31, 2020 as filed with the Securities and Exchange Commission on the date hereof (the "Report"), the undersigned, Kenneth T. Mills, President, Chief Executive Officer and Director of the Registrant, and Vittal Vasista, Chief Financial Officer of the Registrant, certify, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that to their respective knowledge:

(1) The Report fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934; and

(2) The information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Registrant.

Date: March 1, 2021

/s/ Kenneth T. Mills

Kenneth T. Mills
President and Chief Executive Officer
(Principal Executive Officer)

Date: March 1, 2021

/s/ Vittal Vasista

Vittal Vasista
Chief Financial Officer
(Principal Financial and Accounting Officer)

This certification is made solely for the purposes of 18 U.S.C. Section 1350, subject to the knowledge standard contained therein, and not for any other purpose. A signed original of this written statement required by Section 906 has been provided to the Registrant and will be retained by the Registrant and furnished to the United States Securities and Exchange Commission or its staff upon request.

This certification accompanies the Form 10-K to which it relates, is not deemed filed with the Securities and Exchange Commission and is not to be incorporated by reference into any filing of the Registrant under the Securities Act of 1933 or the Securities Exchange Act of 1934 (whether made before or after the date of the Form 10-K), irrespective of any general incorporation language contained in such filing.