UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
WASHINGTON, DC 20549

FORM 10-K

(Mark One)

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

For the fiscal year ended December 31, 2025

Or

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

For the transition period from _____ to _____

Commission file number **001-15771**

ABEONA THERAPEUTICS INC.

(Exact name of registrant as specified in its charter)

Delaware	**83-0221517**
(State or Other Jurisdiction of incorporation or Organization)	(I.R.S. Employer Identification No.)

6555 Carnegie Avenue, 4th Floor
Cleveland, OH 44103
(Address of principal executive offices, zip code)

(646) 813-4701
(Registrant's telephone number, including area code)

Securities registered pursuant to Section 12(b) of the Securities Exchange Act of 1934:

Title of each class	Trading Symbol(s)	Name of each exchange on which registered
Common Stock, $0.01 par value	ABEO	Nasdaq Capital Market

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

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

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

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

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

Large accelerated filer ☐
Non-accelerated filer ☒
Emerging growth company ☐

Accelerated filer ☐
Smaller reporting 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. Yes ☐ No ☒

If securities are registered pursuant to Section 12(b) of the Act, indicate by check mark whether the financial statements of the registrant included in the filing reflect the correction of an error to previously issued financial statements. Yes ☐ No ☒

Indicate by check mark whether any of those error corrections are restatements that required a recovery analysis of incentive-based compensation received by any of the registrant's executive officers during the relevant recovery period pursuant to § 240.10D-1(b). Yes ☐ No ☒

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

The aggregate market value of the voting and non-voting common equity held by non-affiliates computed by reference to the average bid and asked price of such common equity, as of June 30, 2025, was approximately $273,252,724.

The number of shares outstanding of the registrant's common stock as of March 11, 2026 was 57,049,023.

ABEONA THERAPEUTICS INC.
Annual Report on Form 10-K
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FORWARD-LOOKING STATEMENTS

This Form 10-K (including information incorporated by reference) contains statements that express management's opinions, expectations, beliefs, plans, objectives, assumptions or projections regarding future events or future results and therefore are, or may be deemed to be, "forward-looking statements" within the meaning of Section 27A of the Securities Act of 1933, as amended, and Section 21E of the Securities Exchange Act of 1934, as amended. Words such as "expects," "anticipates," "intends," "plans," "believes," "could," "would," "seeks," "estimates," and variations of such words and similar expressions, and the negatives thereof, are intended to identify such forward-looking statements. Such "forward-looking statements" speak only as of the date made and are not guarantees of future performance and involve certain risks, uncertainties, estimates, and assumptions by management that are difficult to predict. Various factors, some of which are beyond the Company's control, could cause actual results to differ materially from those expressed in, or implied by, such forward-looking statements. In addition, we disclaim any obligation to update any forward-looking statements to reflect events or circumstances after the date of this report, except as may otherwise be required by the federal securities laws.

Forward-looking statements necessarily involve risks and uncertainties, and our actual results could differ materially from those anticipated in forward-looking statements due to a number of factors. These statements include statements about: our ability to successfully commercialize ZEVASKYN® and generate future revenue; our plans to continue development of AAV-based gene therapies designed to treat ophthalmic diseases; our pipeline of product candidates, including the achievement of or expected timing, progress and results of clinical development, clinical trials and potential regulatory approvals; our dependence upon our third-party customers and vendors and their compliance with applicable regulations; our estimates regarding expenses, capital requirements, and needs for additional financing; our intellectual property position and our ability to obtain, maintain and enforce intellectual property protection and exclusivity for our proprietary assets; our estimates regarding the size of the potential markets for ZEVASKYN® and our product candidates, the strength of our commercialization strategies and our ability to serve and supply those markets; and future economic conditions or performance.

Important factors that could affect performance and cause results to differ materially from management's expectations are described in the sections entitled "Risk Factors" and "Management's Discussion and Analysis of Financial Condition and Results of Operations" in this Form 10-K. These factors include: our ability to maintain existing and obtain additional regulatory approvals of ZEVASKYN® and any future product candidates; our ability to successfully commercialize and market ZEVASKYN® and any future product candidates, if approved, and the timing of any commercialization and marketing efforts; our ability to manufacture sufficient batches of ZEVASKYN® to meet demand; our ability to activate additional qualified treatment centers to administer ZEVASKYN® on patients; our ability to access our existing at-the-market sale agreement; our ability to access additional financial resources and/or our financial flexibility to reduce operating expenses if required; our ability to obtain additional equity funding from current or new stockholders; the potential impact of unpredicted changes in the structure and/or administration of the United States government or its agencies; our ability to out-license technology and/or other assets, deferring and/or eliminating planned expenditures, restructuring operations and/or reducing headcount, and sales of assets; the dilutive effect that raising additional funds by selling additional equity securities would have on the relative equity ownership of our existing investors, including under our existing at-the-market sale agreement; the outcome of any interactions with the FDA or other regulatory agencies relating to any of our products or product candidates; our ability to continue to secure and maintain regulatory designations for our product candidates; our ability to develop manufacturing capabilities compliant with current good manufacturing practices for our product candidates; our ability to manufacture cell and gene therapy products and produce an adequate product supply to support clinical trials and potentially future commercialization; the rate and degree of market acceptance of our product candidates for any indication once approved; our ability to meet our obligations contained in license agreements to which we are party; and macroeconomic uncertainty resulting from changes to U.S. trade policy, including current or future tariffs or other trade restrictions.

This Form 10-K includes our trademarks, trade names and service marks, such as "ZEVASKYN®" and "AIM™," which are protected under applicable intellectual property laws and are the property of Abeona Therapeutics Inc. or its subsidiaries. Solely for convenience, trademarks, trade names and service marks referred to in this report appear without the ® and ™ symbols, but such references are not intended to indicate, in any way, that we will not assert, to the fullest extent under applicable law, our rights or the right of the applicable licensor to these trademarks, trade names and service marks. We do not intend our use or display of other parties' trademarks, trade names or service marks to imply, and such use or display should not be construed to imply, a relationship with, or endorsement or sponsorship of us by, these other parties.

PART I

ITEM 1. BUSINESS

Business

Abeona Therapeutics Inc., a Delaware corporation (together with our subsidiaries, "we," "our," "Abeona" or the "Company"), is a commercial-stage biopharmaceutical company developing cell and gene therapies for life-threatening diseases. On April 28, 2025, the U.S. Food and Drug Administration ("FDA") approved ZEVASKYN® (prademagene zamikeracel) gene-modified cellular sheets, also known as ZEVASKYN®, as the first and only autologous cell-based gene therapy for the treatment of wounds in adult and pediatric patients with recessive dystrophic epidermolysis bullosa ("RDEB"), a serious and debilitating genetic skin disease. There is no cure for RDEB, and ZEVASKYN® is the only FDA-approved product to treat RDEB wounds with a single surgical application. ZEVASKYN® was granted Orphan Drug and Rare Pediatric Disease designations by the FDA

ZEVASKYN® is manufactured at our current Good Manufacturing Practices ("cGMP") manufacturing facility in Cleveland, Ohio. Treatments are available through ZEVASKYN® qualified treatment centers, a network of centers that are selected based on their expertise in cell and gene therapy and trained to administer ZEVASKYN®. As of March 2026, we have activated 4 qualified treatment centers and are in discussions with additional centers as we continue to expand the ZEVASKYN® qualified treatment network.

The Company's development portfolio also features adeno-associated virus ("AAV")-based gene therapies designed to treat ophthalmic diseases with high unmet need using novel AIM™ capsids. Abeona's novel AAV capsids are being evaluated to improve tropism profiles for a variety of devastating diseases.

We partner with leading academic researchers, patient advocacy organizations, caregivers and other biotechnology companies to develop and deliver therapies that address the underlying cause of a broad spectrum of rare genetic diseases for which no effective treatment options exist today.

Our Mission and Strategy

Our strategy consists of:

Commercializing ZEVASKYN® and Advancing and Commercializing our Cell and Gene Therapy Programs.

Through our cell and gene therapy expertise in research and development, we believe we are positioned to introduce efficacious and safe therapeutics to transform the standard of care in devastating diseases and establish our leadership position in the field. We are commercializing ZEVASKYN® by ourselves and may develop future strategic partnerships for ZEVASKYN® and we intend to commercialize our other assets either by ourselves or through strategic partnerships, subject to FDA approval.

Developing Novel In-Vivo Gene Therapies Using AIM™ Capsid Technology.

We are researching and developing AAV-based gene therapies using novel AAV capsids both derived from the licensed AIM™ Capsid Technology Platform and invented by the Company. We plan to continue to develop chimeric AAV capsids capable of improved tissue targeting for various indications and that can potentially evade immunity to wild-type AAV vectors.

Leveraging our Leadership Position in Commercial-Scale Cell and Gene Therapy Manufacturing.

We established cGMP, commercial and clinical-scale manufacturing capabilities for engineered cell and gene therapies in our state-of-the-art Cleveland, Ohio facility. We believe that our manufacturing platform provides us with distinct advantages, including flexibility, scale, reliability, and the potential for reduced development risk, reduced cost, and faster times to market. We have focused on establishing internal Chemistry Manufacturing and Controls ("CMC") capabilities that drive value for our organization through process development, assay development and manufacturing. We have also deployed robust quality systems governing all aspects of product lifecycle from preclinical through commercial stage.

Establishing Additional Cell and Gene Therapy Franchises and Adjacencies through In-Licensing and Strategic Partnerships.

We seek to be the partner of choice in cell and gene therapy treatments and have closely collaborated with leading academic institutions, key opinion leaders, patient foundations, and industry partners to accelerate research and development, understand the needs of patients and their families, and generate novel intellectual property.

Maintaining and Growing our IP Portfolio.

We seek patent rights for various aspects of our programs, including vector engineering and construct design, our production process, and all features of our clinical products, including compositions of matter and methods of manufacture, administration, and delivery. We expect to continue to expand our intellectual property portfolio by aggressively seeking patent rights for promising aspects of our product engine and product candidates.

ZEVASKYN® for the Treatment of RDEB

Disease Overview

RDEB belongs to a broad group of genetic skin disorders known as epidermolysis bullosa. Patients with RDEB have a defect in the COL7A1 gene, resulting in the inability to produce Type VII collagen, which plays a vital role in skin functioning by anchoring the skin's dermal and epidermal layers to one another.

As a result of the genetic defect, RDEB patients have fragile skin, which can easily damage to produce open and blistering wounds, disfiguring scars throughout the body, fused fingers and toes, limits in range of motion at joints (e.g., arms and legs), corneal abrasions, and an abnormal narrowing of the esophagus. Long-term RDEB patients can suffer from anemia, infections and are at high risk of developing aggressive squamous cell carcinomas, infections, and premature death. The most severe patients are approximately 20 times more likely to die by 30 years of age than the general population.

Similar to other rare diseases, the incidence and prevalence of RDEB are not well defined. Incidence of 0.2 to 3.05 per million births and prevalence of 0.14 to 1.35 per million people have been observed across different geographies, primarily estimated by limited population analyses of clinical databases or registries (Eichstadt et al.; Clinical, Cosmetic and Investigational Dermatology, 2019). Using genetic modeling of COL7A1 variants, Stanford University estimated the incidence of RDEB to be approximately 63 per million births, and prevalence could be up to 3,850 patients in the U.S., whose wounds may benefit from COL7A1-mediated treatments such as ZEVASKYN®. Based on claims analysis, we estimate that approximately 750 moderate to severe RDEB patients in the U.S. would be ZEVASKYN® eligible patients (Clearview Claims Analysis, 2024).

RDEB patients have active disease, with the majority of their wounds typically greater than 20 cm^2 in size (Stanford University; Solis, D., et al., 2017). In 2020, a survey of RDEB patients reported that approximately 60% have active wounds covering greater than 30% of their bodies (Bruckner et al.; Orphanet Journal of Rare Diseases, 2020). Wounds covering up to approximately 80% of body surface area have been recorded in some EB patients (Hirsch et al.; Nature Research, 2017).

In our VIITAL™ phase 3 and phase 1/2a clinical trials, ZEVASKYN® was applied as a one-time surgical procedure onto RDEB wounds and has shown up to 12 years of durable wound healing and associated pain reduction even in the tough-to-treat large, chronic RDEB wounds. Patients evaluated in the VIITAL™ phase 3 trial had some of the worst wounds. These wounds were large (> 20cm^2) and, on average, had remained open for 6.2 years, and in some cases up to 21 years, prior to ZEVASKYN® treatment. Most RDEB patients have large and chronic wounds that carry the highest burden, including the need for frequent lengthy dressing changes, pain, pruritus (itch), risk of infection, and developing skin cancer.

Current Management of RDEB

RDEB wound management currently consists of lengthy and labor-intensive supportive care to limit contamination and infection, and reduction in mechanical forces that produce new blisters. Care usually includes treatment of new blisters by lancing and draining. Wounds are then dressed with non-adherent material, covered with padding for stability and protection, and secured with an elastic wrap for integrity. In a cost analysis conducted by Debra of America, based on 3,274 patient health insurance claims from private insurance, the annual cost of care for dystrophic epidermolysis bullosa (DEB) was found to be 465% greater than the annual cost to the healthcare system from all people and a substantial share of this burden stems from ongoing wound-care needs. For many patients, these wound-care expenses represent a major, persistent financial strain on both families and the healthcare system, reflecting the chronic and resource-intensive nature of RDEB management.

RDEB patients also have periodic surgeries to relieve disease related issues such as narrowing of their esophagus, fusing of fingers, and corneal abrasions.

In 2023, Vyjuvek® and Filsuvez® were approved by the FDA for treatment of wounds associated with DEB and wounds associated with Junctional (JEB) and DEB, respectively.

RDEB patients continue to seek durable treatments for addressing their wounds in the current treatment landscape.

Our Program History

ZEVASKYN® is a commercial product comprised of autologous epidermal gene-modified sheets in which a functioning COL7A1 gene is inserted into a patient's own skin cells (keratinocytes) using a retrovirus vector. The gene-modified keratinocytes are then grown into credit card-sized sheets and surgically applied to the patient to restore Type VII collagen expression and skin function.

Results from a completed Phase 1/2a study that enrolled seven patients and treated 38 large and chronic RDEB wounds at Stanford University showed that ZEVASKYN® was well-tolerated and resulted in significant and durable wound healing (Siprashvili, Z., et al., 2016), with up to eight years of follow-up after a single surgical application (So. Y, Nazaraoff, et al., Orphanet Journal Rare Disease 2022). To date, there have been no reported serious adverse events.

In November 2022, we announced positive topline data from our VIITAL™ study. The pivotal phase 3 VIITAL™ study evaluated the efficacy, safety, and tolerability of ZEVASKYN® in 43 large chronic wound pairs in 11 subjects with RDEB. The large chronic wounds randomized and treated in VIITAL™ measured greater than 20 cm^2 of surface area and had remained open for a minimum of six months and a maximum of 21 years (mean 6.2 years). The co-primary endpoints of the study were assessed at the six-month timepoint for: (1) the proportion of RDEB wound sites with greater than or equal to 50% healing from baseline, comparing randomized treated with matched untreated (control) wound sites, as determined by direct investigator assessment; and (2) patient-reported pain reduction associated with wound dressing change assessed by the mean differences in scores of the Wong-Baker FACES® Pain Rating Scale between randomized treated and matched untreated (control) wounds.

The VIITAL™ study met both co-primary efficacy endpoints demonstrating statistically significant, clinically meaningful improvements in wound healing and pain reduction in large chronic RDEB wounds. ZEVASKYN® was shown to be well-tolerated with no serious treatment-related adverse events observed, consistent with past clinical experience. There were no deaths or no instances of positive replication-competent retrovirus, no systemic immunologic responses were reported during the study, as well as no squamous cell carcinoma at treatment sites after application of ZEVASKYN®. Two subjects reported at least one serious adverse event unrelated to ZEVASKYN®. Four subjects reported related treatment emergent adverse events, including procedural pain, muscle spasms and pruritis. Infections unrelated to ZEVASKYN® were observed in eight patients.

On April 28, 2025, the FDA approved ZEVASKYN® as the first and only autologous cell-based gene therapy for the treatment of wounds in adult and pediatric patients with RDEB. ZEVASKYN® has been granted Regenerative Medicine Advanced Therapy ("RMAT"), Breakthrough Therapy, Orphan Drug and RPD designations by the FDA as well as Orphan Drug designation by the EMA.

Among the potential benefits of Orphan Drug designation are a potential seven years of market exclusivity following FDA approval, potentially preventing FDA approval of another product deemed to be the same as the approved product for the same indication, waiver of application fees, and tax credits for qualified clinical testing expenses conducted after orphan designation is received. A sponsor who receives an approval for a BLA with RPD designation may qualify for a Priority Review Voucher ("PRV"), subject to final determination by the FDA. A PRV may be used to receive an expedited review of a subsequent marketing application for a different product or sold to another company. We received a PRV upon ZEVASKYN®'s approval, and on May 9, 2025, we entered into a definitive asset purchase agreement that transferred the PRV to a third party. The PRV sale was completed in June 2025 following early termination of the applicable waiting period for U.S. antitrust review of the transaction. We received gross proceeds of $155.0 million from the sale of the PRV.

We have prepared our current cGMP facility in Cleveland, Ohio for manufacturing commercial grade ZEVASKYN® drug product to support our commercial launch of ZEVASKYN®. ZEVASKYN® study drug product for all our VIITAL™ study participants was manufactured at our Cleveland facility.

Commercial Operations

Our commercialization strategy centers on establishing and expanding a network of qualified treatment centers with the clinical expertise and infrastructure required to administer our therapy. As of March 2026, we had activated four qualified treatment centers. These centers were selected based on their expertise in areas such as cell and gene therapy and have undergone specialized training to administer ZEVASKYN®.

Treatment involves obtaining a biopsy from the patient and shipping the biopsied cells to our manufacturing facility, where the patient specific product is manufactured as multilayer cellular sheets containing gene-corrected keratinocytes. Following testing, the product is then shipped back to the qualified treatment center where the patient receives treatment.

We treated our first ZEVASKYN® patient in the fourth quarter of 2025.

As part of commercial launch efforts, we continue to engage with multiple stakeholders across the healthcare system, including leading EB hospital institutions, private and public health insurers, as well as the patient and physician community. To date, we have activated four qualified treatment centers that now can identify and treat patients with ZEVASKYN®. These qualified treatment centers are geographically dispersed across the U.S. and include Ann & Robert H. Lurie Children's Hospital of Chicago, Lucile Packard Children's Hospital Stanford, Children's Hospital Colorado, and The University of Texas Medical Branch (UTMB) in Galveston, Texas. We have secured broad insurance coverage for ZEVASKYN® from multiple national and regional commercial insurers as well as from the CMS (Centers for Medicare and Medicaid Services). ZEVASKYN® has coverage from all Medicaid programs across 50 US states and Puerto Rico. Effective January 1, 2026, CMS also has issued a permanent J-code for ZEVASKYN® that we expect will simplify claims and reimbursement processing between qualified treatment centers and all payer types.

Developing Next-Generation Cell and Gene Therapy

ABO-503 for the treatment of X-linked Retinoschisis ("XLRS")

Disease Overview and Program Overview

XLRS is a rare, monogenic retinal disease that results in the irreversible loss of photoreceptor cells and severe visual impairment. XLRS is caused by mutations in the RS1 protein, which is normally secreted by retinal photoreceptors and bipolar neurons and functions to mediate cell-cell adhesion. XLRS is characterized by abnormal splitting of the layers of the retina, resulting in poor visual acuity, which can progress to legal blindness. The incidence of XLRS is estimated to be between 1 in 5,000 and 1 in 20,000 in males, with an estimated prevalence of 35,000 in the United States and Europe combined. There are currently no disease modifying therapies approved for XLRS, but because the genetics of the disease are well understood, early intervention via gene therapy has significant potential to reverse or stabilize disease progression at early stages and prevent vision loss.

ABO-503, composed of a functional human RS1 packaged in the novel AIM™ capsid AAV204, has shown preclinical efficacy following delivery to the retina in a mouse model of XLRS. Preclinical studies have demonstrated robust RS1 expression in the retina, improved cone photoreceptor density and overall photoreceptor cell survival, as well as a restoration of outer retina architecture. Results of these studies were presented at the American Society of Gene and Cell Therapy ("ASGCT") Annual Meeting in May 2023. A pre-IND meeting for ABO-503 was conducted with the FDA in April 2023 and provided Abeona with comprehensive feedback to support a future IND submission. Due to focus on ZEVASKYN® commercialization efforts, animal efficacy and toxicology studies and cGMP manufacturing of clinical grade material has been postponed to 2026.

ABO-504 for the Treatment of Stargardt Disease

Disease Overview and Program Overview

Autosomal recessive Stargardt disease, the most common form of juvenile macular degeneration with estimated incidence of 1 in 8,000 to 10,000 people, causes vision loss in children and young adults. The most common form of Stargardt disease is caused by mutations in the ABCA4 gene, which prevent removal of toxic compounds from photoreceptor cells that results in photoreceptor cell death and progressive vision loss. There are currently no FDA approved treatments available, and to date, development of investigational gene modifying therapies has remained challenging in part due to the large size of the ABCA4 gene, which exceeds the encapsidation capacity of a single AAV capsid.

Abeona's internal research and development team developed ABO-504, which is designed to efficiently reconstitute the full-length ABCA4 gene by implementing a dual AAV vector strategy using the Cre-LoxP recombinase system. Abeona previously reported preclinical data demonstrating the ability of the dual AAV vector system to produce full length ABCA4 protein in cell culture. Recent proof-of-concept studies, presented at the 2023 ASGCT Annual Meeting, have extended these findings by showing expression of ABCA4 mRNA and full-length ABCA4 protein in the retina of subretinally dosed abca4-/- knockout mice, at levels similar to endogenous ABCA4 in wild-type animals. A pre-IND meeting for ABO-504 was conducted with the FDA in June 2023 and provided Abeona with comprehensive feedback to support a future IND submission.

ABO-505 for the Treatment of Autosomal Dominant Optic Atrophy ("ADOA")

Disease Overview and Program Overview

ADOA, a form of hereditary vision loss associated with retinal ganglion cell ("RGC") death, is predominantly caused by mutations in the Opa1 gene. Opa1, a dynamin-related GTPase, acts to stabilize the inner mitochondrial membrane and acts in mitochondrial fusion and inner membrane remodeling. Mutant phenotypes present with a progressive loss of RGCs that result in optic nerve degeneration and legal blindness with a loss of visual acuity, optic disc pallor, and color vision deficits. ADOA affects approximately 1 in 30,000 people worldwide. Currently, there is no approved treatment for people living with ADOA.

ABO-505 is designed to express a functional copy of human Opa1 in the retina following para-retinal injection. ABO-505 aims to take advantage of the robust optic nerve and RGC transduction ability of AAV204 to deliver its genetic payload to the cells most affected by ADOA. Preclinical studies have confirmed expression of Opa1 in both cell culture and the retinas of dosed wild-type and disease model animals. Initial efficacy results suggest an improvement in retinal signaling to the brain and improved visual acuity in treated mutant mice. These studies were presented at the ASGCT Annual Meeting in May 2023.

Gene Therapy Treatments anchored in AIM™ Vector Platform

In 2016, we licensed a library of novel AAV capsids from UNC. The AIM™ vector system is a platform of AAV capsids capable of widespread central nervous system gene transfer and can be used to confer high transduction efficiency for various therapeutic indications. In partnership with academic institutions, our own scientific research teams have identified capsids within the AIM™ capsid library showing strong potential to successfully target and reach the central nervous system (including the retina) as well, lung, muscle, liver, and other tissues. Based on continuing research by Abeona and our research partners, we have observed improvements in gene delivery to specific tissues compared to currently available AAV technology. We believe AIM™ vectors also have the potential for redosing subjects who previously received certain AAV gene therapy or subjects who have pre-existing antibodies to naturally occurring AAV serotypes.

In July 2024, we entered into a non-exclusive agreement with Beacon Therapeutics ("Beacon") under which Beacon will evaluate Abeona's patented AAV204 capsid for the development and commercialization of potential gene therapies for select ophthalmology indications. Following a 12-month evaluation period, Beacon exercised its option to take a worldwide, non-exclusive license to use AAV204 in connection with up to five gene or disease targets. Beacon will also have the right to use AAV204 for up to four additional nominated gene or disease targets subject to certain conditions. We received an upfront payment upon Beacon's exercise of its option to license AAV204, with additional payments upon the achievement of certain development, regulatory, and sales milestones, along with tiered royalties on worldwide net sales for licensed products incorporating AAV204.

Strategic Licensing Agreements

We have out-licensed certain clinical and research programs, including for the treatment of Sanfilippo syndrome type A (MPS IIIA) to Ultragenyx Pharmaceutical Inc. ("Ultragenyx")) and Rett syndrome to Taysha Gene Therapies, Inc. ("Taysha"). Under the terms of our agreement with Ultragenyx, we are eligible to receive payments based on the achievement of certain sales milestones and royalties on net sales. Under our agreements with Taysha, we are eligible to receive payments based on certain clinical, regulatory, and sales milestones and royalties on net sales. On February 25, 2026, the Company, UNC and Taysha jointly terminated both the license agreement between Abeona and UNC and the corresponding sublicense agreement between Abeona and Taysha relating to Taysha's development program for TSHA-118 for CLN1 disease.

Leveraging Leadership Position in Commercial-Scale Cell and Gene-Therapy Manufacturing

We have established a cGMP manufacturing facility, the Elisa Linton Center located in Cleveland, Ohio at 6555 Carnegie Avenue, which enables us to enhance supply chain control, establish tighter quality control testing, increase supply capacity, reduce production costs and gain manufacturing for ZEVASKYN®. Our facility is led by a team of highly skilled production, process/assay development, and quality control scientists with expertise in cell and gene therapy, particularly in cell culture, upstream manufacturing, downstream purification, assay development and wet lab techniques.

We have advanced our in-house manufacturing capabilities for ZEVASKYN®. The product is manufactured as multilayer cellular sheets containing gene-corrected keratinocytes that is fastened to a petrolatum gauze backing with surgical titanium ligating clips. Engineered keratinocyte sheets expressing functional Type VII collagen are applied over wound areas, providing immediate wound coverage and allowing wound healing. A key component to the ZEVASKYN® drug product manufacturing process is the retroviral vector, which delivers the functional copy of the Collagen VII Alpha 1 cDNA to the patient's own cells. We manufacture the LZRSE-Col7A1 gamma retroviral vector at our Cleveland facility.

Our AAV vector manufacturing process uses the triple plasmid transient transfection method. We insert ("transfect") many copies of three DNA plasmids encoding the specific therapeutic gene sequence, or transgene, the capsid coding sequence, and helper sequences into AAV-293 cells using a serum-free, suspension-based bioreactor vector production technology. During an incubation period following transfection, each cell produces AAV vectors through biosynthesis using the cells' natural machinery. At the end of the incubation period, the newly generated AAV vectors are harvested, filtered, and purified in a multi-step process.

We have established and maintained strong and collaborative relationships with third-party companies specializing in the testing of cell and gene therapy material to complement our process and assay development needs.

We have made significant investments in developing optimized manufacturing processes and believe that our processes and methods developed to date provide a comprehensive manufacturing process for ZEVASKYN® and AAV-based vector therapies, including:

- sufficient scale to support commercial manufacturing requirements for ZEVASKYN®;
- processes related to biopsy, cell collection, storage and transportation as part of manufacturing for ZEVASKYN®;
- processes related to product release testing for ZEVASKYN®;
- processes related to the manufacture and release testing of retroviral vector;
- establishing transportation and packaging processes and materials for finished ZEVASKYN® product;
- proprietary AAV vector manufacturing processes and techniques that produce a highly purified product candidate;
- AAV serum-free suspension technology that is readily scalable;
- multiple assays to accurately characterize our process and the AAV vectors we produce; and
- a series of purification processes, which may be adapted and customized for multiple different AAV capsids, with a goal of higher concentrations of active vectors, and that are essentially free of empty capsids.

We believe that these investments will enable us to develop best-in-class, next-generation cell and gene therapy products.

Maintain Strong Intellectual Property Protection

We strive to protect our commercially important proprietary technology, inventions, and know-how, including by seeking, maintaining, and defending patent rights, both for inventions developed internally and for inventions licensed from third parties. We also rely on trade secrets and know-how relating to our proprietary technology platforms, continuing technological innovation, and in-licensing opportunities to develop, strengthen and maintain our position in the field of cell and gene therapy. We may also rely on the additional protections afforded by data exclusivity (currently 12 years for biologics), other market exclusivities such as orphan drug exclusivity, and patent term extensions, where applicable.

Our success may depend in part on our ability to obtain and maintain patents and other protections for commercially important technology, inventions, and know-how related to our business; defend and enforce our patents; preserve the confidentiality of our trade secrets; and operate without infringing the valid enforceable patents and other intellectual property rights of third parties. Our ability to stop third parties from making, having made, using, selling, offering to sell, or importing our products may depend on the extent to which we have rights under valid and enforceable licenses, patents, or trade secrets that cover these activities. In some cases, these rights may need to be enforced by third-party licensors. With respect to both licensed and company-owned intellectual property, we may not be granted patents with respect to any of our pending patent applications or with respect to any patent applications filed by us in the future, nor can we be sure that any of our existing patents or any patents that may be granted to us in the future will be commercially useful in protecting our commercial products and methods of manufacturing the same.

We are actively seeking U.S. and international patent protection, together with our licensors, for a variety of technologies, including AAV capsids, AAV-based biological products, methods of designing novel AAV constructs, compositions and methods for treating diseases of interest, including RDEB, and methods for manufacturing, packaging, and transporting our product candidates. We also intend to seek patent protection or rely upon trade secret rights to protect other technologies that may be used to discover and validate targets and that may be used to identify and develop novel biological products. We seek protection, in part, through confidentiality and proprietary information agreements. We are a party to various license agreements that give us rights to use specific technologies in our research and development, and future commercialization.

Licensed Technologies and Intellectual Property

1. ***Recessive Dystrophic Epidermolysis Bullosa***

   To support our EB franchise, we licensed a patent family from Stanford University covering ZEVASKYN® and its use in the treatment of RDEB. Patents covering our investigational ZEVASKYN® product have been granted in the United States (U.S. Patent Nos. 12,110,504; 12,173,314; and 12,385,010), by the European Patent Office (EP3400287B1), by the Japan Patent Office (JP7159048, JP7555380), and in other geographical regions, and are expected to expire in early 2037. Patent applications remain pending in the United States which, if granted, would be expected to expire in 2037. A patent covering the packaging and transport system for ZEVASKYN® has been granted in the United States (U.S. Patent No. 12,144,340) and is expected to expire in mid-2040.

   We may also rely on the additional protection afforded by data exclusivity (currently 12 years for biologics like ZEVASKYN®), other market exclusivity such as orphan drug exclusivity (currently seven years), and patent term extensions, where applicable.

2. AIM™ Capsids

We have an exclusive license to an international patent family from The University of North Carolina at Chapel Hill ("UNC") covering novel AAV capsids ("AIM™ capsids") that may potentially be used to deliver a wide variety of therapeutic transgenes to human cells to treat genetic diseases. National stage applications directed to the AIM™ capsids have been filed in the United States, Europe, and other geographical regions. The first U.S. patent in this patent family, U.S. Patent No. 10,532,110 (the "'110 Patent"), was issued to UNC on January 14, 2020. The '110 Patent is entitled to 352 days of patent term adjustment and will not expire before November 6, 2036. The second U.S. patent in this patent family, U.S. Patent No. 10,561,743 (the "'743 Patent"), was issued to UNC on February 18, 2020. The '743 Patent will not expire before November 20, 2035. A third U.S. patent in this patent family, U.S. Patent No. 11,491,242 (the "'242 Patent") issued on November 8, 2022. The '242 Patent is entitled to 429 days of patent term adjustment and will not expire before January 22, 2037. Patents have also been granted in Australia (AU2015349759 and AU2022201540), Israel (IL252072), New Zealand (NZ731673), and Russia (RU2727015). We have exclusive rights to these patents under our license with UNC.

We also own a second patent family directed to certain AAV capsids and have filed national stage applications in the United States, Europe and other geographical regions. U.S. Patent No. 12,454,701 (the "'701 Patent"), was issued on October 28, 2025. The '701 patent is entitled to 1179 days of patent term adjustment and will not expire before February 25, 2043. A patent has also been granted in Japan (JP7590968).

3. Rett Syndrome

We have licensed rights to one patent family from UNC and two patent families from The University Court of the University of Edinburgh ("U. Edinburgh") and The University Court of the University of Glasgow ("U. Glasgow") relating to gene therapy for the treatment of Rett Syndrome. The patent family licensed from UNC at Chapel Hill is directed to viral genomes designed to regulate expression of the MeCP2 gene, which is mutated in patients with Rett Syndrome. This patent family has pending applications in the United States, Europe and other geographical regions. Patents issuing from these applications would have a 20-year expiration date of no earlier than 2039. U.S. Patent No. 12,311,034 was issued to UNC on May 27, 2025 in this family. The patent families licensed from U. Edinburgh and U. Glasgow are directed to expression cassettes for MeCP2 polypeptides and to synthetic MeCP2 polypeptides. The patent family directed to MeCP2 expression cassettes has pending applications in the United States, Europe and other geographical regions. The patent family directed to synthetic MeCP2 polypeptides has pending applications in the United States and other geographical regions. Patents issuing from applications in the Edinburgh patent families would have a 20-year expiration date of no earlier than 2038. U.S. Patent No. 11,969,479 was issued to U. Edinburgh and U. Glasgow in in this patent family on April 20, 2024. In October 2020, we entered into an agreement exclusively sublicensing these UNC and University of Edinburgh patent rights to Taysha Gene Therapies, Inc.

4. ***Multipartite AAV Delivery of Large Transgenes***

   We own three patent families directed to multipartite delivery of large transgenes using AAV vectors. For two of these patent families we have filed national stage applications in the United States, Europe and other geographical regions. Patents issuing from these applications are not expected to expire before 2041 for the first patent family, or before 2044 for the second patent family. A European patent application in the first patent family (EP4182467) is allowed and will be validated in European states in 2026. We have also filed a U.S. provisional application in the third patent family. Patents issuing from the provisional application are not expected to expire before 2046.

5. ***New AAV Capsids and Ophthalmic Disease Treatment via Para-retinal AAV Administration***

   We own a patent family directed to (i) novel AAV capsid proteins and (ii) treating ophthalmic diseases via para-retinal administration of AAV vectors and have filed national stage applications in the United States, Europe, and other geographical regions. Patents issuing from these applications are not expected to expire before 2042.

6. ***Treatment of Dominant Optic Atrophy and X-linked Retinoschisis***

   We own a patent family directed to compositions and methods for treating dominant optic atrophy and X-linked retinoschisis and have filed national stage applications in the United States, Europe, and other geographical regions. Patents issuing from these applications are not expected to expire before 2043.

We expect to explore in due course strategies to support patent term extensions for all of our patent portfolios.

U.S. Biologic Products Development Process

In the United States, the FDA regulates biologic products including gene therapy products under the Federal Food, Drug, and Cosmetic Act ("FDCA"), the Public Health Service Act ("PHSA"), and regulations implementing these laws. The FDCA, PHSA and their corresponding regulations govern, among other things, the testing, manufacturing, safety, efficacy, labeling, packaging, storage, record keeping, distribution, advertising, and promotion of biologic products. Applications to the FDA are required before conducting human clinical testing of biologic products. FDA approval also must be obtained before marketing of biologic products. Gene therapy studies may also need to comply with the National Institutes of Health ("NIH") Guidelines for Research Involving Recombinant or Synthetic Nucleic Acid Molecules ("NIH Guidelines"), which includes additional requirements, such as the review and approval of the study by an Institutional Biosafety Committee.

Within the FDA, the Center for Biologics Evaluation and Research ("CBER") regulates gene therapy products. Within CBER, the review of gene therapy and related products is consolidated in the Office of Tissues and Advanced Therapies ("OTAT") and the FDA has established the Cellular, Tissue and Gene Therapies Advisory Committee ("CTGTAC"), a panel of medical and scientific experts and consumer representatives, to advise CBER on its reviews. The FDA has issued a growing body of guidance documents on CMC, clinical investigations and other areas of gene therapy development, all of which are intended to facilitate the industry's development of gene therapy products.

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

- completion of preclinical laboratory tests and in vivo studies in accordance with the FDA's current Good Laboratory Practice ("GLP") regulations and applicable requirements for the humane use of laboratory animals or other applicable regulations;
- submission to the FDA of an application for an IND, which allows human clinical trials to begin unless the FDA objects within 30 days;
- approval by an independent institutional review board ("IRB"), reviewing each clinical site before each clinical trial may be initiated;

- performance of adequate and well-controlled human clinical trials according to the FDA's Good Clinical Practice ("GCP") regulations, and any additional requirements for the protection of human research subjects and their health information, to establish the safety and efficacy of the proposed biologic product candidate for its intended use;
- development of manufacturing processes to ensure the product candidate's identity, strength, quality, purity, and potency;
- preparation and submission to the FDA of a BLA for marketing approval that includes substantial evidence of safety, purity and potency from results of nonclinical testing and clinical trials;
- satisfactory completion of an FDA pre-approval inspection of the manufacturing facility or facilities where the biologic product candidate is produced to assess compliance with cGMP and to assure that the facilities, methods and controls are adequate to preserve the biologic product candidate's identity, safety, strength, quality, potency and purity;
- potential FDA audit of the nonclinical and clinical trial sites that generated the data in support of the BLA; and
- payment of user fees and the FDA review and approval, or licensure, of the BLA. BLA application fees for products designated as orphan drugs by the FDA are waived.

Before testing any biologic product candidate on humans, including a gene therapy product candidate, the product candidate must undergo preclinical testing. Preclinical tests, also referred to as nonclinical studies, include laboratory evaluations of product chemistry, toxicity, and formulation, as well as in vivo studies to assess the potential safety and activity of the product candidate. The conduct of the preclinical tests must comply with federal regulations and requirements including GLPs.

If a gene therapy trial is conducted at, or sponsored by, institutions receiving NIH funding for recombinant DNA research, the study must also comply with the NIH Guidelines. Compliance with the NIH Guidelines is mandatory for investigators at institutions receiving NIH funds for research involving recombinant DNA. However, many companies and other institutions, not otherwise subject to the NIH Guidelines, voluntarily follow them.

The clinical trial 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 trial on a clinical hold. In such a case, the IND sponsor and the FDA must resolve any outstanding concerns before the clinical trial can begin. The FDA also may impose clinical holds on a biologic product candidate at any time before or during clinical trials due to safety concerns or non-compliance. If the FDA imposes a clinical hold, trials may not commence or recommence without FDA authorization and then only under terms authorized by the FDA.

Human clinical trials under an IND

Clinical trials involve the administration of the biologic product candidate to healthy volunteers or patients under the supervision of qualified investigators, which generally are physicians not employed by, or under the control of, the trial sponsor. Investigators must also provide certain information to the clinical trial sponsors to allow the sponsors to make certain financial disclosures to the FDA. Clinical trials are conducted under protocols detailing, among other things, the objectives of the clinical trial, dosing procedures, subject selection and exclusion criteria and the parameters to be used to monitor subject safety, including stopping rules that assure a clinical trial 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 trials must be conducted and monitored in accordance with the FDA's regulations comprising the GCP requirements, including the requirement that all research subjects provide informed consent.

Further, each clinical trial must be reviewed and approved by an IRB at or servicing each institution at which the clinical trial will be conducted. An IRB is charged with protecting the welfare and rights of trial participants and considers items such as whether the risks to individuals participating in the clinical trials are minimized and are reasonable in relation to anticipated benefits. The IRB also approves communications to study subjects before a study commences at that site and the form and content of the informed consent that must be signed by each clinical trial subject, or his or her legal representative, and must monitor the clinical trial until completed. Clinical trials involving recombinant DNA also must be reviewed by an institutional biosafety committee ("IBC"), a local institutional committee that reviews and oversees basic and clinical research that utilizes recombinant DNA at that institution. The IBC assesses the safety of the research and identifies any potential risk to public health or the environment.

Information about certain clinical trials, including a description of the study and study results, must be submitted within specific timeframes to NIH for public dissemination on their clinicaltrials.gov website. Sponsors or distributors of investigational products for the diagnosis, monitoring, or treatment of one or more serious diseases or conditions must also have a publicly available policy on evaluating and responding to requests for expanded access requests.

Investigational biologics and therapeutic substances imported into the United States are also subject to regulation by the FDA. Further, the export of investigational products outside of the United States is subject to regulatory requirements of the receiving country as well as U.S. export requirements under the FDCA.

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

- Phase 1: The biologic product candidate initially is introduced into healthy human subjects and tested for safety, dosage tolerance, absorption, metabolism, distribution, excretion and, if possible, to gain an early understanding of its effectiveness. In the case of some product candidates for severe or life-threatening diseases, especially when the product candidate may be too inherently toxic to ethically administer to healthy volunteers, the initial human testing is often conducted in patients.

- Phase 2: The biologic product candidate is evaluated in a limited patient population to identify possible adverse effects and safety risks, to preliminarily evaluate the efficacy of the product candidate for specific targeted diseases and to determine dosage tolerance, optimal dosage and dosing schedule.

- Phase 3: The biologic product candidate is administered to an expanded patient population at geographically dispersed clinical trial sites in adequate and well-controlled clinical trials to generate sufficient data to statistically confirm the efficacy and safety of the product for approval. These clinical trials are intended to establish the overall risk/benefit ratio of the product candidate and provide an adequate basis for product labeling. Typically, two phase 3 trials are required by the FDA for product approval. Under some limited circumstances, however, the FDA may approve a BLA based upon a single phase 3 clinical study plus confirmatory evidence or a single large multicenter trial without confirmatory evidence.

Additional kinds of data may also help to support a BLA, such as patient experience data. Real world evidence may also support a BLA, and, for appropriate indications sought through supplemental BLAs, data summaries may provide marketing application support. For genetically targeted products and variant protein targeted products intended to address an unmet medical need in one or more patient subgroups with a serious or life threatening rare disease or condition, the FDA may allow a sponsor to rely upon data and information previously developed by the sponsor or for which the sponsor has a right of reference, that was submitted previously to support an approved application for a product that incorporates or utilizes the same or similar genetically targeted technology or a product that is the same or utilizes the same variant protein targeted drug as the product that is the subject of the application.

Post-approval clinical trials, sometimes referred to as phase IV clinical trials, may be conducted or may be required by FDA after initial approval. These clinical trials are used to gain additional experience from the treatment of patients in the intended therapeutic indication, particularly for long-term safety follow-up.

During all phases of clinical development, regulatory agencies require extensive monitoring and auditing of all clinical activities, clinical data, and clinical trial investigators. Annual progress reports detailing the results of the clinical trials must be submitted to the FDA.

Written IND safety reports must be promptly submitted to the FDA, IRBs, IBCs, and the investigators for serious and unexpected adverse events; any findings from other trials, in vivo laboratory tests or in vitro testing that suggest a significant risk for human subjects; any clinically important increase in the rate of a serious suspected adverse reaction over that listed in the protocol or investigator brochure, or other safety information. The sponsor must submit an IND safety report within 15 calendar days after the sponsor determines that the information qualifies for reporting. The sponsor also must notify the FDA of any unexpected fatal or life-threatening suspected adverse reaction within seven calendar days after the sponsor’s initial receipt of the information.

The FDA, the sponsor or its data safety monitoring board, may suspend a clinical trial 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 trial at its institution if the clinical trial is not being conducted in accordance with the IRB's requirements or if the biologic product candidate has been associated with unexpected serious harm to patients. The FDA or an IRB may also impose conditions on the conduct of a clinical trial.

Additional regulation for gene therapy clinical trials

In addition to the regulations discussed above, there are a number of additional standards that apply to clinical trials involving the use of gene therapy. The FDA has issued various guidance documents regarding gene therapies, which outline additional factors that the FDA will consider at each of the above stages of development and relate to, among other things: the proper preclinical assessment of gene therapies; the CMC information that should be included in an IND application; the proper design of tests to measure product efficacy in support of an IND or BLA application; and long term patient and clinical study subject follow up and reporting requirements. The FDA has also issued draft guidance specific to the development of gene therapy products for neurodegenerative diseases as such products may face special challenges related to CMCs and clinical and preclinical development, due to the nature of the products and potential patient population (e.g., children), the heterogeneity of neurodegenerative disorders, the route of administration, the volume of the product that can be administered, the delivery device, and the study population size.

Compliance with cGMP requirements

Manufacturers of biologics must comply with applicable cGMP regulations for both clinical and commercial supply. Manufacturers and others involved in the manufacture and distribution of such products at the commercial stage also must register their establishments with the FDA and certain state agencies and list the manufactured products. Recently, the information that must be submitted to FDA regarding manufactured products was expanded through the Coronavirus Aid, Relief, and Economic Security, or CARES Act to include the volume of drugs produced during the prior year. Both domestic and foreign manufacturing establishments must register and provide additional information to the FDA upon their initial participation in the manufacturing process. Establishments may be subject to periodic, unannounced inspections by government authorities to ensure compliance with cGMP requirements and other laws. Discovery of problems may result in a government entity placing restrictions on a product, manufacturer, or holder of an approved BLA, and may extend to requiring withdrawal of the product from the market. The FDA will not approve an application unless it determines that the manufacturing processes and facilities comply with cGMP requirements and are adequate to ensure consistent production of the product within required specification.

Concurrent with clinical trials, companies usually complete additional preclinical studies and must also develop additional information about the physical characteristics of the biologic product candidate as well as finalize a process for manufacturing the product candidate in commercial quantities in accordance with cGMP requirements. To help reduce the risk of the introduction of adventitious agents or of causing other adverse events with the use of biologic products, the PHSA 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 requirements, the sponsor must develop methods for testing the identity, strength, quality, potency and purity of the final biologic product. Additionally, appropriate packaging must be selected and tested, and stability studies must be conducted to demonstrate that the biologic product candidate does not undergo unacceptable deterioration over its shelf life.

U.S. review and approval processes

The results of the preclinical tests and clinical trials, together with detailed information relating to the product's CMC and proposed labeling, among other things, are submitted to the FDA as part of a BLA requesting approval to market the product for one or more indications.

For gene therapies, selecting patients with applicable genetic defects is a necessary condition for effective treatment. For the therapies we are currently developing, we believe that diagnoses based on symptoms, in conjunction with existing genetic tests developed and administered by laboratories certified under the Clinical Laboratory Improvement Amendments, are sufficient to select appropriate patients and will be permitted by the FDA. For future therapies, however, it may be necessary to use FDA-cleared or FDA-approved diagnostic tests to select patients or to assure the safe and effective use of therapies in appropriate patients. The FDA refers to such tests as in vitro companion diagnostic devices and the combination of the in vitro companion diagnostic device and the therapeutic would be considered to be a combination product.

The use of the two products together must be shown to be safe and effective for the proposed intended use and the labeling of the two products must reflect their combined use. In some cases, the device component may require a separate premarket submission; for example, when the device component is intended for use with multiple drug products. Sponsors of clinical studies using investigational devices are required to comply with FDA's investigational device exemption regulations. Once approved or cleared, the sponsor of the device component submission (or the combination product submission, if both components are covered by one premarket submission) would need to comply with FDA's post-market device requirements, including establishment registration, device listing, device labeling, unique device identifier, quality system regulation, medical device reporting, and reporting of corrections and removals requirements.

The FDA has 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 diagnostic device at the same time that the FDA approves the therapeutic product. The type of premarket submission required for a companion diagnostic device will depend on the FDA classification of the device. A premarket approval, or PMA, application is required for high-risk devices classified as Class III; a 510(k) premarket notification is required for moderate risk devices classified as Class II; and a *de novo* request may be used for novel devices not previously classified by the FDA that are low or moderate risk.

The FDA may, however, approve a therapeutic product without the concurrent approval or clearance of a diagnostic device when the therapeutic product is intended to treat serious and life-threatening conditions for which no alternative exists and the FDA determines that the benefits from the use of the drug/biologic outweigh the risks from the lack of an approved/cleared companion diagnostic. The FDA would also consider whether additional protections, such as risk evaluation and mitigation strategies, or REMS, or post-approval requirements, are necessary. At this point, it is unclear how the FDA will apply this policy to our gene therapy candidates. Should the FDA deem genetic tests used for selecting appropriate 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 for a BLA. 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 biologic product candidate for the claimed indications in all relevant pediatric subpopulations and to support dosing and administration for each pediatric subpopulation for which the product candidate is safe and effective. The FDA may grant deferrals for submission of data or full or partial waivers. Unless otherwise required by regulation, PREA does not apply to any biologic product candidate for an indication for which orphan designation has been granted.

Under the PDUFA, each BLA must be accompanied by a substantial user fee that must be paid at the time of the first submission of the application, even if the application is being submitted on a rolling basis. The FDA adjusts the PDUFA user fees on an annual basis. Fee waivers or reductions are available in certain circumstances, including a waiver of the application fee for the first application filed by a small business. Additionally, no user fees are assessed on BLAs for product candidates designated as orphan drugs, unless the product candidate also includes a non-orphan indication.

The FDA reviews a BLA within 60 days of submission to determine if it is substantially complete before the agency accepts it for filing. The FDA may refuse to accept for filing any BLA that it deems incomplete or not properly reviewable at the time of submission and may request additional information. In that 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 candidate is safe and potent, or effective, for its intended use, has an acceptable purity profile and whether the product candidate is being manufactured in accordance with cGMP to assure and preserve the product candidate's identity, safety, strength, quality, potency, and purity. The FDA may refer applications for novel biologic products or biologic products that present difficult questions of safety or efficacy to an advisory committee, typically 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 product approval process, the FDA also will determine whether a REMS is necessary to ensure the safe use of the product candidate. A REMS could include medication guides, physician communication plans and elements to assure safe use, such as restricted distribution methods, patient registries, and other risk minimization tools. 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 candidate is manufactured. The FDA will not approve the product candidate unless it determines that the manufacturing processes and facilities comply with cGMP requirements and are adequate to assure consistent production of the product candidate within required specifications. Additionally, before approving a BLA, the FDA typically will inspect one or more clinical sites to assure that the clinical trials were conducted in compliance with IND trial requirements and GCP requirements.

On the basis of the BLA and accompanying information, including the results of the inspection of the manufacturing facilities, the FDA may issue an approval letter or a complete response letter. An approval letter authorizes commercial marketing of the biologic product with specific prescribing information for specific indications. A CRL generally outlines the deficiencies in the submission and may require substantial additional testing or information for the FDA to reconsider the application. If a CRL is issued, the applicant may either: resubmit the marketing application, addressing all of the deficiencies identified in the letter; withdraw the application; or request an opportunity for a hearing. If those deficiencies have been addressed to the FDA's satisfaction in a resubmission of the BLA, the FDA will issue an approval letter.

If a product candidate receives regulatory approval, the approval may be significantly limited to specific diseases, patient populations, and dosages or the indications for use may otherwise be limited. Further, the FDA may require that certain contraindications, warnings, or precautions be included in the product labeling. The FDA also may not approve label statements that are necessary for successful commercialization and marketing. 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 trials, sometimes referred to as phase IV clinical trials, designed to further assess a biologic product's safety and effectiveness, and testing and surveillance programs to monitor the safety of approved products that have been commercialized.

The FDA has agreed to specified performance goals in the review of BLAs under the PDUFA. One such goal is to review 90% of standard BLAs in 10 months after the FDA accepts the BLA for filing, and 90% of priority BLAs in six months, whereupon a review decision is to be made. The FDA does not always meet its PDUFA goal dates for standard and priority BLAs and its review goals are subject to change from time to time. The review process and the PDUFA goal date may also be extended if new information is submitted to the application.

Orphan drug designation

Under the Orphan Drug Act, the FDA may designate a biologic product as an "orphan drug" if it is intended to treat a rare disease or condition (generally meaning that it affects fewer than 200,000 individuals in the United States, or more in cases in which there is no reasonable expectation that the cost of developing and making a biologic product available in the United States for treatment of the disease or condition will be recovered from sales of the product). Additionally, sponsors must present a plausible hypothesis for clinical superiority to obtain orphan drug designation if there is a product already approved by the FDA that is considered by the FDA to be the same as the already approved product and is intended for the same indication. This hypothesis must be demonstrated to obtain orphan exclusivity. 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. If granted, prior to product approval, orphan drug designation entitles a party to financial incentives such as opportunities for grant funding towards clinical study costs, tax advantages, and certain user-fee waivers. The tax advantages, however, were limited in the 2017 Tax Cuts and Jobs Act. Orphan product designation does not shorten the duration of the regulatory review and approval process.

If a product with orphan status receives the first FDA approval for the disease or condition for which it has such designation, the product is entitled to orphan product exclusivity, meaning that the FDA may not approve any other applications to market the same drug or biologic 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 or if the party holding the exclusivity fails to assure the availability of sufficient quantities of the drug to meet the needs of patients with the disease or condition for which the drug was designated. Orphan product sameness decisions are an evolving space. FDA has issued a final guidance document on how the agency will determine the "sameness" of gene therapy products. Pursuant to the guidance, "sameness" will depend on the product's transgene expression, viral vectors groups and variants, and other product features that may have a therapeutic effect. Generally, minor differences between gene therapy products will not result in a finding that two products are different. Any FDA sameness determinations could impact our ability to receive approval for our product candidates and to obtain or retain orphan drug exclusivity. Competitors additionally may receive approval of different products for the same 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 medicinal product status in the European Union has similar, but not identical, benefits.

Expedited development and review programs

The FDA is authorized to expedite the review of BLAs in several ways. Under the Fast Track program, the sponsor of a biologic product candidate may request the FDA to designate the product for a specific indication as a Fast Track product concurrent with or after the filing of the IND. Biologic products are eligible for Fast Track designation if they are intended to treat a serious or life-threatening condition and demonstrate the potential to address unmet medical needs for the condition. Fast Track designation applies to the combination of the product candidate and the specific indication for which it is being studied. In addition to other benefits, such as the ability to have greater interactions with the FDA, the FDA may initiate review of sections of a Fast Track BLA before the application is complete, a process known as rolling review. This "rolling review" is available if the applicant provides and the FDA approves a schedule for the remaining information.

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.

- *Breakthrough therapy designation:* To qualify for the breakthrough therapy program, product candidates must be intended to treat a serious or life-threatening disease or condition, and preliminary clinical evidence must indicate that such product candidates may demonstrate substantial improvement on one or more clinically significant endpoints over existing therapies. The FDA will seek to ensure the sponsor of a breakthrough therapy product candidate receives the following: intensive guidance on an efficient drug development program; intensive involvement of senior managers and experienced staff on a proactive, collaborative, and cross-disciplinary review; and rolling review.

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

- *Accelerated approval:* Drug or biologic 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. Accelerated approval means that a product candidate may be approved on the basis of adequate and well-controlled clinical trials establishing that the product candidate has an effect on a surrogate endpoint that is reasonably likely to predict a clinical benefit, or on the basis of an effect on a clinical endpoint other than survival or irreversible morbidity or mortality or other clinical benefit, taking into account the severity, rarity and prevalence of the condition and the availability or lack of alternative treatments. As a condition of approval, the FDA may require that a sponsor of a drug or biologic product candidate receiving accelerated approval perform adequate and well-controlled post-marketing clinical trials. In addition, the FDA currently requires as a condition for accelerated approval pre-approval of promotional materials. Failure to conduct required post-approval studies, or confirm a clinical benefit during post-marketing studies, will allow the FDA to withdraw the drug or biologic from the market on an expedited basis.

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

Finally, with passage of the 21st Century Cures Act (the "Cures Act") in December 2016, Congress authorized the FDA to accelerate review and approval of products designated as regenerative advanced therapies. A product is eligible for this designation if it is a regenerative medicine therapy (which may include a cell or gene therapy) that is intended to treat, modify, reverse, or cure a serious or life-threatening disease or condition and preliminary clinical evidence indicates that the drug has the potential to address unmet medical needs for such disease or condition. The benefits of a regenerative advanced therapy designation include early interactions with the FDA to expedite development and review, benefits available to breakthrough therapies, potential eligibility for priority review and accelerated approval based on surrogate or intermediate endpoints.

Post-approval requirements

Rigorous and extensive FDA regulation of biologic products continues after approval, particularly with respect to cGMP requirements. Manufacturers 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 biologic products include reporting of cGMP deviations that may affect the identity, potency, purity and overall safety of a distributed product, record-keeping requirements, reporting of adverse events, reporting updated safety and efficacy information, and complying with electronic record and signature requirements.

To help reduce the increased risk of the introduction of adventitious agents, the PHSA emphasizes the importance of manufacturing controls for products whose attributes cannot be precisely defined. The PHSA also provides authority to the FDA to immediately suspend licenses in situations where there exists a danger to public health, to prepare or procure products in the event of shortages and critical public health needs, and to authorize the creation and enforcement of regulations to prevent the introduction or spread of communicable diseases in the United States and between states. After a BLA is approved, the product also may be subject to official lot release. If the product is subject to official lot 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 tests performed on the lot. The FDA also may perform certain confirmatory tests on lots of some products before releasing the lots for distribution. In addition, the FDA conducts laboratory research related to the regulatory standards on the safety, purity, potency, and effectiveness of biologic products.

There also are continuing annual program user fee requirements for approved products, excluding orphan products. In addition, manufacturers and other entities involved in the manufacture and distribution of approved therapeutics are subject to periodic announced and unannounced inspections by the FDA and these state agencies for compliance with cGMP and other requirements, which impose certain procedural and documentation requirements upon the company and third-party manufacturers.

A sponsor also must comply with the FDA's marketing, advertising, and promotion 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"), industry-sponsored scientific and educational activities and promotional activities involving the Internet. A company can make only those claims relating to a product that are approved by the FDA. Physicians, in their independent professional medical judgment, may prescribe legally available products for unapproved indications that are not described in the product's labeling and that differ from those tested and approved by the FDA. Biopharmaceutical companies, however, are required to promote their products only for the approved indications and in accordance with the provisions of the approved label. The FDA and other agencies actively enforce the laws and regulations prohibiting the promotion of off-label uses, and a company that is found to have improperly promoted off-label uses may be subject to significant liability, including, but not limited to, criminal and civil penalties under the FDCA and False Claims Act, exclusion from participation in federal healthcare programs, mandatory compliance programs under corporate integrity agreements, suspension and debarment from government contracts, and refusal of orders under existing government contracts.

In addition, the distribution of prescription biopharmaceutical samples is subject to the Prescription Drug Marketing Act ("PDMA"), which regulates the distribution of samples at the federal level. Both the PDMA and state laws limit the distribution of prescription biopharmaceutical products. Certain reporting related to samples is also required. Free trial or starter prescriptions provided through pharmacies are also subject to regulations under the Medicaid Drug Rebate Program and potential liability under anti-kickback and false claims laws.

Moreover, the enacted Drug Quality and Security Act ("DQSA"), imposed obligations on sponsors of biopharmaceutical products related to product tracking and tracing. Among the requirements of this legislation, sponsors are required to provide certain information regarding the products to individuals and entities to which product ownership is transferred, are required to label products with a product identifier, and are required to keep certain records regarding the product. The transfer of information to subsequent product owners by sponsors is also required to be done electronically. Sponsors must also verify that purchasers of the sponsors' products are appropriately licensed. Further, under this legislation manufacturers have product investigation, quarantine, disposition, and notification responsibilities related to counterfeit, diverted, stolen, and intentionally adulterated products that would result in serious adverse health consequences or death to humans, as well as products that are the subject of fraudulent transactions or which are otherwise unfit for distribution such that they would be reasonably likely to result in serious health consequences or death. Similar requirements additionally are and will be imposed through this legislation on other companies within the biopharmaceutical product supply chain, such as distributors and dispensers, as well as certain sponsor licensees and affiliates.

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. Further, should new safety information arise, additional testing or FDA notification may be required. 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, such as adding new indications and additional labeling claims, are also subject to further FDA review and approval.

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 actions and adverse publicity. These actions could include refusal to approve pending applications or supplemental applications, withdrawal of an approval, clinical hold, suspension or termination of clinical trial by an IRB, warning or untitled letters, product recalls, adverse publicity, product seizures, total or partial suspension of production or distribution, injunctions, fines or other monetary penalties, refusals of government contracts, mandated corrective advertising or communications to healthcare professionals or patients, exclusion from participation in federal and state healthcare programs, debarment, restitution, disgorgement of profits or other civil or criminal penalties.

U.S. patent term restoration and marketing exclusivity

Depending upon the timing, duration, and specifics of FDA approval of product candidates, some of a sponsor's U.S. patents may be eligible for limited patent term extension under the Drug Price Competition and Patent Term Restoration Act of 1984. The Hatch-Waxman Amendments permit a patent restoration term of up to five years to account for patent term lost during 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 generally is 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. This period may also be reduced by any time that the applicant did not act with due diligence. Only one patent applicable to an approved biologic product is eligible for the extension and the application for the extension must be submitted prior to the expiration of the patent. The United States Patent and Trademark Office ("USPTO"), in consultation with the FDA, reviews and approves the application for any patent term extension or restoration.

Pediatric exclusivity

Pediatric exclusivity is a type of non-patent marketing exclusivity in the United States that, if granted, provides for the attachment of an additional six months of marketing protection to the term of any existing regulatory exclusivity, including the non-patent and orphan exclusivity. This six-month exclusivity may be granted if a BLA sponsor submits pediatric data that fairly responds to a written request from the FDA for such data. The data does not need to show the product to be effective in the pediatric population studied; rather, if the clinical trial is deemed to fairly respond to the FDA's request, the additional protection is granted. If reports of requested pediatric studies are submitted to, and accepted by, the FDA within the statutory time limits, whatever statutory or regulatory periods of exclusivity or patent protection that cover the product are extended by six months. This is not a patent term extension, but it effectively extends the regulatory period during which the FDA cannot accept or approve a biosimilar application.

Biosimilars and exclusivity

The Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act ("PPACA"), created an abbreviated approval pathway for biologic products shown to be similar to, or interchangeable with, an FDA-licensed reference biologic product, referred to as biosimilars. For the FDA to approve a biosimilar product, it must find that the biosimilar product is highly similar to the reference product notwithstanding minor differences in clinically inactive components, and that there are no clinically meaningful differences between the reference product and proposed biosimilar product. 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.

A reference biologic is granted 12 years of exclusivity from the time of first licensure of the reference product. An application for a biosimilar product may not be submitted to the FDA until four years following approval of the reference product, and it may not be approved until 12 years thereafter. These exclusivity provisions only apply to biosimilars—companies that rely on their own data and file a full BLA may be approved earlier than 12 years. Moreover, certain changes and supplements to an approved BLA, and subsequent applications filed by the same sponsor, manufacturer, licensor, predecessor in interest, or other related entity do not qualify for the twelve-year exclusivity period. The PHSA also includes provisions to protect reference products that have patent protection. The biosimilar product sponsor and reference product sponsor may exchange certain patent and product information for the purpose of determining whether there should be a legal patent challenge. Based on the outcome of negotiations surrounding the exchanged information, the reference product sponsor may bring a patent infringement suit and injunction proceedings against the biosimilar product sponsor. The biosimilar applicant may also be able to bring an action for declaratory judgment concerning the patent.

In an effort to increase competition in the biologic product marketplace, Congress, the executive branch, and the FDA have taken certain legislative and regulatory steps. For example, in 2020 the FDA finalized a guidance to facilitate product importation. Moreover, the 2020 Further Consolidated Appropriations Act included provisions requiring that sponsors of approved biologic products, including those subject to REMS, provide samples of the approved products to persons developing biosimilar products within specified timeframes, in sufficient quantities, and on commercially reasonable market-based terms. Failure to do so can subject the approved product sponsor to civil actions, penalties, and responsibility for attorney's fees and costs of the civil action. This same bill also includes provisions with respect to shared and separate REMS programs for reference and generic drug products.

Rare Pediatric Disease Priority Review Voucher Program

Under the Rare Pediatric Disease Priority Review Voucher Program, the FDA can award priority review vouchers to sponsors of rare pediatric disease products where the product is intended to treat serious or life-threatening diseases that primarily affect individuals up to age 18. To qualify, the product must contain no active ingredient (including any ester or salt of the active ingredient) that has been previously approved by the FDA. The application must also meet other qualifying criteria, including eligibility for FDA priority review. If the necessary qualifying criteria are met, upon a sponsor's request and product approval, the FDA may award a priority review voucher. This voucher may be transferred and may be redeemed to receive priority review of a subsequent marketing application for a different product. Use of a priority review voucher is subject to an FDA user fee. As these vouchers are transferable, sponsors may sell these vouchers for substantial sums of money. Vouchers may, however, be revoked by the FDA under certain circumstances and sponsors of approved rare pediatric disease products must submit certain reports to the FDA. To take advantage of the benefits of this program, the product must be designated by the FDA for a rare pediatric disease no later than September 30, 2029.

The Rare Pediatric Disease Priority Review Voucher program has been subject to periodic statutory sunset provisions and extensions. Under the current statutory sunset provisions, the FDA may only award a rare pediatric disease priority review voucher if the NDA for the product is approved before September 30, 2029. After September 30, 2029, the FDA may not award any rare pediatric disease priority review vouchers, unless Congress extends the program further.

Government regulation outside of the United States

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

Whether or not a sponsor obtains FDA approval for a product, a sponsor must obtain the requisite approvals from regulatory authorities in foreign countries prior to the commencement of clinical trials or marketing of the product in those countries. Certain countries outside of the United States have a similar process that requires the submission of a clinical trial application, much like the IND, prior to the commencement of human clinical trials. Save where the Clinical Trial Regulation applies (see below) in relation to cross-border trials, in the European Union, for example, a request for a Clinical Trial Authorization ("CTA") must be submitted to the competent regulatory authorities and the competent Ethics Committees in the European Union Member States in which the clinical trial takes place, much like the FDA and the IRB, respectively. Once the CTA request is approved in accordance with the European Union and the European Union Member State's requirements, clinical trial development may proceed.

The requirements and processes governing the conduct of clinical trials, product licensing, pricing and reimbursement vary from country to country. In all cases, the clinical trials are conducted in accordance with GCPs and the applicable regulatory requirements and the ethical principles that have their origin in the Declaration of Helsinki.

Failure to comply with applicable foreign regulatory requirements may result in, among other things, fines, suspension, variation or withdrawal of regulatory approvals, product recalls, seizure of products, operating restrictions, and criminal prosecution.

European Union regulation and exclusivity

To obtain regulatory approval of an investigational biologic product under European Union regulatory systems, applicants must submit a marketing authorization application ("MAA"). The grant of marketing authorization in the European Union 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) 726/2004 of the European Parliament and of the Council laying down Union procedures for the authorization and supervision of medicinal products for human and veterinary use and establishing a European Medicines Agency. Regulation 1394/2007/EC lays down specific rules concerning the authorization, supervision and pharmacovigilance of gene therapy medicinal products, somatic cell therapy medicinal products and tissue engineered products. Manufacturers of advanced therapy medicinal products must demonstrate the quality, safety and efficacy of their products to the EMA which provides an opinion regarding the application for marketing authorization. The European Commission grants or refuses marketing authorization in light of the opinion delivered by EMA.

Innovative medicinal products are authorized in the European Union based on a full marketing authorization application (as opposed to an application for marketing authorization that relies 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 make an MMA relying on data contained in the marketing authorization dossier submitted for the innovative medicinal product to support their application and such generics or biosimilars cannot be placed on the market until 10 years after the first EU marketing of the reference product. The overall 10-year period will be extended to a maximum of 11 years if, during the first eight years of those 10 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. Even if a compound is considered to be a new chemical entity and the innovator is able to gain the period of data exclusivity, another company, nevertheless, could also market another competing medicinal product for the same therapeutic indication if such company obtained marketing authorization based on an MAA with a complete independent data package of pharmaceutical tests, preclinical tests and clinical trials.

Products receiving orphan designation in the European Union can receive 10 years of market exclusivity. During this 10-year period, the competent authorities of the European Union Member States and European Commission may not accept applications or grant marketing authorization for other similar medicinal product for the same orphan indication. There are, however, three exceptions to this principle. 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.

An orphan product can also obtain an additional two years of market exclusivity in the European Union for the conduct of pediatric trials. The 10-year market exclusivity 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 and no longer justifies the maintenance of market exclusivity or if the manufacturer cannot produce sufficient quantities to supply the orphan population.

The criteria for designating an "orphan medicinal product" in the European Union are similar, in principle, to those in the United States. Orphan medicinal products are eligible for financial incentives such as reduction of fees or fee waivers. The application for orphan medicinal product designation must be submitted before the application for marketing authorization. Orphan medicinal product designation does not convey any advantage in, or shorten the duration of, the regulatory review and approval process.

In April 2014, the EU adopted a new Clinical Trials Regulation (EU) No 536/2014 (the "Clinical Trials Regulation"), which replaced the current Clinical Trials Directive 2001/20/EC (the "Clinical Trials Directive") on January 31, 2022. The Clinical Trial Regulation has overhauled the previous system of approvals for clinical trials in the EU whereby all clinical trial approvals were granted purely on a national basis. Specifically, the legislation, which is directly applicable in all member states, aims at simplifying and streamlining the approval of clinical trials in the EU, whereby there is a streamlined application procedure via a single-entry point and strictly defined deadlines for the assessment of clinical trial applications. However, the Clinical Trial Regulation does increase public disclosure requirements in relation to clinical trial information.

In the European Union there are also broadly equivalent regimes for the other issues addressed in relation to U.S. regulation including cGMP requirements, accelerated access (generally through so-called Conditional Marketing Authorizations), pediatric requirements and incentives and patent term restoration (supplementary protection certificates).

Other 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, and entities 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, purchasers, and formulary managers on the other. Although a number of statutory exemptions and regulatory safe harbors exist to protect certain common activities from falling under the Anti-Kickback Statute, these are narrow, and practices may not fall under the applicable safe harbors and exemptions. For example, the United States Department of Health and Human Services recently promulgated a regulation that is effective in two phases. First, the regulation excludes from the definition of "remuneration" limited categories of (a) PBM rebates or other reductions in price to a plan sponsor under Medicare Part D or a Medicaid Managed Care Organization plan reflected in point-of sale reductions in price and (b) PBM service fees. Second, effective January 1, 2023, the regulation expressly provides that rebates to plan sponsors under Medicare Part D either directly to the plan sponsor under Medicare Part D, or indirectly through a pharmacy benefit manager will not be protected under the anti-kickback discount safe harbor. The PPACA amended 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 in order to commit a violation;

- the federal false claims and civil monetary penalties laws, including the civil False Claims Act (the "FCA"), which prohibit, among other things, individuals, or entities from knowingly presenting, or causing to be presented, claims for payment from Medicare, Medicaid or other third-party payors that are false or fraudulent, or making a false statement to avoid, decrease, or conceal an obligation to pay money to the federal government. Certain marketing practices, including off-label promotion, also may implicate the FCA. FCA claims may be pursued by whistleblowers through qui tam actions, even if the government declines to intervene and civil liability may be predicated on reckless disregard for the truth. The PPACA also codified case law that a claim including items or services resulting from a violation of the federal Anti-Kickback Statute constitutes a false or fraudulent claim for purposes of the FCA. Separately, the criminal federal False Claims Act imposes criminal fines or imprisonment against individuals or entities who make or present a claim to the government knowing such claim to be false, fictitious, or fraudulent;

- the federal Physician Payments Sunshine Act, which requires certain manufacturers of drugs, devices, biologics and medical supplies for which payment is available under Medicare, Medicaid, or the Children's Health Insurance Program, with specific exceptions, to report annually to the Centers for Medicare & Medicaid Services ("CMS"), information related to payments and other transfers of value made to or at the request of covered recipients, such as, but not limited to, physicians, physician assistants, nurse practitioners, clinical nurse specialists, certified registered nurse anesthetists and teaching hospitals, as well as ownership and investment interests held by physicians and their immediate family. Payments made to physicians and certain research institutions for clinical trials are included within the ambit of this law. Reported information is made publicly available in searchable formats by CMS;

- additional federal false statements and fraud and abuse statutes prohibit knowingly and willfully executing, or attempting to execute, a scheme to defraud or to obtain, by means of false or fraudulent pretenses, representations or promises, any of the money or property owned by, or under the custody or control of, a healthcare benefit program, regardless of whether the payor is public or private, in connection with the delivery or payment for health care benefits, knowingly and willfully embezzling or stealing from a health care benefit program, willfully obstructing a criminal investigation of a health care offense and knowingly and willfully falsifying, concealing, or covering up by any trick or device a material fact or making any materially false statements in connection with the delivery of, or payment for, healthcare benefits, items, or services relating to healthcare matters. PPACA amended the intent requirement of certain of these criminal statutes under the Health Insurance Portability and Accountability Act of 1996 ("HIPAA") so that a person or entity no longer needs to have actual knowledge of the statute, or the specific intent to violate it, to have committed a violation; 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 European Union and state laws governing the privacy and security of health information in certain circumstances, many of which differ from each other in significant ways, may be stricter than those applicable in the US and may not have the same effect, thus complicating compliance efforts.

Violation 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, debarment from government contracting or refusal of orders under existing contracts, corporate integrity agreements or consent decrees, disgorgement, contractual damages, reputational harm, diminished profits and future earnings, and imprisonment. Furthermore, efforts to ensure that business activities and business arrangements comply with applicable healthcare laws and regulations can be costly.

Data Privacy and Security

- HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act of 2009, "HITECH Act"), and similar state laws impose obligations on certain entities with respect to safeguarding the privacy, security and transmission of protected health information. HIPAA's security and certain privacy standards are directly applicable to persons or organizations of covered entities, other than members of the covered entity's workforce, that create, receive, maintain or transmit protected health information on behalf of a covered entity for a function or activity regulated by HIPAA. The HITECH Act strengthened the civil and criminal penalties that may be imposed against covered entities, business associates and individuals, and gave state attorneys general new authority to file civil actions for damages or injunctions in federal courts to enforce the federal HIPAA laws and seek attorneys' fees and costs associated with pursuing federal civil actions. In addition, other federal and state laws, such as the California Consumer Privacy Act, may regulate the privacy and security of information that we maintain, many of which may differ from each other in significant ways and may not be preempted by HIPAA; and

- the General European Data Protection Regulation ("GDPR"), which became applicable May 25, 2018, harmonizes data privacy laws across Europe. The GDPR sets forth rules relating to the protection with regard to the processing and transfer of personal data as well as an individual's right to the protection of personal data, including medical information and clinical trial related data. In addition, there are rules relating to the export of personal data outside the European Union and in particular there are certain challenges in relation to export to the United States.

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, sales of any product candidates for which regulatory approval for commercial sale is obtained will depend in part on the availability of coverage and adequate reimbursement from third-party payors. Third-party payors include government authorities and health programs in the United States such as Medicare and Medicaid, managed care providers, private health insurers and other organizations. These third-party payors are increasingly reducing reimbursements for medical products and services. 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 and/or application procedure. 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. Additionally, the containment of healthcare costs has become a priority of federal and state governments, and the prices of drugs have been a focus in this effort. The U.S. government, state legislatures and foreign governments have shown significant interest in implementing cost-containment programs, including price controls, required disclosures of pricing and sensitive cost data, requirement for payment of manufacturer rebates and negotiation of supplemental rebates, restrictions on reimbursement and requirements for substitution of generic products. Coverage policies and third-party reimbursement rates may change at any time. Even if favorable coverage and reimbursement status is attained for one or more products for which we receive regulatory approval, less favorable coverage policies and reimbursement rates may be implemented in the future.

In the EU, pricing and reimbursement schemes vary widely from country to country. Some countries provide that products may be marketed only after a reimbursement price has been agreed. Some countries may require the completion of additional studies as part of health technology assessment that compare the cost-effectiveness of a particular product candidate to currently available therapies. EU member states may approve a specific price for a product, or it may instead adopt a system of direct or indirect controls on the profitability of the company placing the product on the market. Other member states allow companies to fix their own prices for products but monitor and control company profits. The downward pressure on health care costs has become intense. As a result, increasingly high barriers are being erected to the entry of new products. In addition, in some countries, cross-border imports from low-priced markets exert competitive pressure that may reduce pricing within a country. Any country that has price controls or reimbursement limitations may not allow favorable reimbursement and pricing arrangements.

Health Reform

The United States and some foreign jurisdictions are considering or have enacted a number of reform proposals to change the healthcare system. There is significant interest in promoting changes in healthcare systems with the stated goals of containing healthcare costs, improving quality, or expanding access. In the United States, the pharmaceutical industry has been a particular focus of these efforts. For example, healthcare reform measures under the Affordable Care Act included increased Medicaid rebates, expanded the 340B drug discount program, and changes requiring manufacturer discounts currently set at 70 percent on Part D utilization in the Part D coverage gap or "donut hole" and multiple provisions that could affect the profitability of our drug products. There is continuing development of value-based pricing and reimbursement models. Moreover, on November 27, 2020, CMS issued an interim final rule implementing a Most Favored Nation payment model under which reimbursement for certain Medicare Part B drugs and biologicals will be based on a price that reflects the lowest per capita Gross Domestic Product-adjusted (GDP-adjusted) price of any non-U.S. member country of the Organization for Economic Co-operation and Development (OECD) with a GDP per capita that is at least sixty percent of the U.S. GDP per capita. Current and future healthcare reform measures may significantly affect our sale of any products, and we continue to face major uncertainty due to the status of major legislative initiatives surrounding healthcare reform.

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 Conservation and Recovery Act and the Toxic Substances Control Act, affect our business. These and other laws govern the use, handling and disposal of various biologic and chemical substances used in, and wastes generated by, operations. If our operations result in contamination of the environment or expose individuals to hazardous substances, we could be liable for damages and governmental fines. Equivalent laws have been adopted in other countries that impose similar obligations.

U.S. Foreign Corrupt Practices Act

The U.S. Foreign Corrupt Practices Act ("FCPA"), prohibits U.S. corporations and individuals from engaging in certain activities to obtain or retain business abroad or to influence a person working in an official capacity. It is illegal to pay, offer to pay or authorize the payment of anything of value to any foreign government official, government staff member, political party, or political candidate in an attempt to obtain or retain business or to otherwise influence a person working in an official capacity. The scope of the FCPA includes interactions with certain healthcare professionals in many countries. Equivalent laws have been adopted in other foreign countries that impose similar obligations.

Competition

Companies that are currently engaged in gene therapy or companies not yet focused on developing cell and gene therapies could at any time decide to develop therapies relevant to our business. Many of our competitors, either alone or with their strategic partners, may have substantially greater financial, technical, and human resources than we do and may have significantly greater experience in the discovery and development of product candidates, obtaining FDA and other regulatory approvals of product candidates and commercializing those product candidates. Accordingly, our competitors may be more successful than us in obtaining approval for product candidates and achieving widespread market acceptance. Our competitors' product candidates may be more effective, or more effectively marketed and sold, than any product candidate 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 product candidates.

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 may compete with us in recruiting and retaining qualified scientific and management personnel and establishing clinical trial sites and subject 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 facing intense and increasing competition as new product candidates enter the market and advanced technologies become available. We expect any product candidates that we develop and commercialize to compete on the basis of, among other things, efficacy, safety, convenience of administration and delivery, price, and the availability of reimbursement from government and other third-party payors.

Our commercial opportunity could be reduced or eliminated if our competitors develop and commercialize products that are safer, more effective, have fewer or less severe side effects, are more convenient or are less expensive than any products that we may develop. Our competitors also may obtain FDA or other regulatory approval for their product candidates more rapidly than we may obtain approval for ours, which could result in our competitors establishing a strong market position before we are able to enter the market.

Corporate Information

Our principal executive office as well as our manufacturing and laboratory facilities are located at 6555 Carnegie Ave, 4th Floor, Cleveland, OH 44103. Our telephone number is (646) 813-4701.

We were incorporated in Wyoming in 1974 as Chemex Corporation, and in 1983 we changed our name to Chemex Pharmaceuticals, Inc. We changed our state of incorporation from Wyoming to Delaware on June 30, 1989. In 1996 we merged with Access Pharmaceuticals, Inc., a private Texas corporation, and changed our name to Access Pharmaceuticals, Inc. On October 24, 2014, we changed our name to PlasmaTech Biopharmaceuticals, Inc. On May 15, 2015, we acquired Abeona Therapeutics LLC and on June 19, 2015, we changed our name to Abeona Therapeutics Inc.

Suppliers

Some of the materials we use are specialized. We obtain materials from several suppliers based in different countries around the world. If materials are unavailable from one supplier, we generally have alternate suppliers available.

Human Capital Resources

As a commercial-stage biopharmaceutical company developing cell and gene therapies for life-threatening diseases, we seek to attract, hire, develop and retain qualified and highly skilled personnel with experience in areas such as research and development and manufacturing operations. We compete for such personnel with numerous pharmaceutical and chemical companies, specialized biotechnology firms and universities. We strive to support our employees' well-being through a transparent, inclusive, and collaborative culture and by providing them with the training, support, and resources to help them succeed professionally.

As of December 31, 2025, we had 226 full-time employees. We have never experienced employment-related work stoppages and believe that we maintain good relations with our personnel. In addition, to complement our internal expertise, we have contracts with scientific consultants, contract research organizations and university research laboratories that specialize in various aspects of drug development including clinical development, regulatory affairs, toxicology, process scale-up and preclinical testing.

Web Availability

We make available free of charge through our website, www.abeonatherapeutics.com, including our annual reports on Form 10-K and other reports that we file with the Securities and Exchange Commission ("SEC") as well as certain of our corporate governance policies, including the charters for the audit, compensation and nominating and corporate governance committees of the Board of Directors (the "Board") and our code of ethics, corporate governance guidelines and whistleblower policy. We will also provide to any person without charge, upon request, a copy of any of the foregoing materials. Any such request must be made in writing to us at: Abeona Therapeutics Inc. c/o Investor Relations, 6555 Carnegie Ave, 4th Floor, Cleveland, OH 44103. The SEC's website, www.sec.gov, contains reports, proxy statements, and other information that we file electronically with the SEC. The content on any website referred to in this Form 10-K is not incorporated by reference in this Form 10-K.

ITEM 1A. RISK FACTORS

Our business, financial condition, financial results, and future growth prospects are subject to a number of risks and uncertainties, including those set forth below. The occurrence of any of the following risks could have a material adverse effect on our business, financial condition, financial results, and future growth prospects. These disclosures reflect our beliefs and opinions as to factors that could materially and adversely affect us and our securities in the future. References to past events are provided by way of example only and are not intended to be a complete listing or a representation as to whether such factors have occurred in the past or their likelihood of occurring in the future.

RISK FACTOR SUMMARY

Our business is subject to numerous risks and uncertainties, including those described in Item 1A "Risk Factors." These risks include, but are not limited to the following:

- We may not be able to successfully manufacture or commercialize ZEVASKYN® and the revenue that we generate from its sales, if any, may be limited.
- Our financial performance depends on the commercial success of ZEVASKYN® and we have limited experience as a commercial-stage company.
- We may encounter challenges with engaging or coordinating with qualified treatment centers needed for the ongoing commercialization of ZEVASKYN®.
- Our cell and gene therapy product candidates are based on proprietary methodologies, which makes it difficult to predict the time and cost of product candidate development and regulatory approval. Additionally, regulatory requirements governing cell and gene therapy products have evolved and may continue to change in the future.
- We may encounter substantial delays in our clinical studies, or we may fail to demonstrate safety and efficacy to the satisfaction of applicable regulatory authorities. Additionally, we may find it difficult to enroll patients in our clinical studies, which could delay or prevent clinical studies of our product candidates.
- We have received and may apply for additional designations such as breakthrough therapy designation, RMAT designation, fast track designation, and rare pediatric disease designation from the FDA intended to facilitate or encourage product candidate development. We may not receive any such designations or be able to maintain them. Moreover, any such designations may not lead to faster development or regulatory review or approval and it does not increase the likelihood that our product candidates will receive marketing approval.
- While certain of our product candidates have received orphan drug designation from the FDA, there is no guarantee that we will be able to maintain this designation, receive this designation for any of our other product candidates, or receive or maintain any corresponding benefits, including periods of exclusivity.
- Even if we obtain regulatory approval for a product candidate, our products will remain subject to regulatory scrutiny.
- We could experience production problems in our manufacturing facility that result in delays in our development or commercialization programs. We may also experience delays in manufacturing if any of our vendors, contract laboratories or suppliers are found to be out of compliance with cGMP.
- If we fail to comply with applicable regulations, the relevant regulatory authority may require remedial measures that may be costly or time-consuming to implement and that may include the suspension of a clinical trial or commercial sales or the closure of a manufacturing facility.
- We expect to rely on third parties, and these third parties may not perform satisfactorily. Additionally, 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.
- Our drug candidates are subject to the risks of failure inherent in the development of pharmaceutical products based on new technologies, and our failure to develop safe and commercially viable drugs would severely limit our ability to become profitable or to achieve significant revenues.
- We may be unable to successfully develop, market, or commercialize our products or our product candidates without establishing new relationships and maintaining current relationships and our ability to successfully commercialize, and market our product candidates could be limited if a number of these existing relationships are terminated.
- We may incur substantial product liability expenses due to the use or misuse of our products for which we may be unable to obtain insurance coverage.
- Our ability to successfully develop and commercialize our drug candidates will substantially depend upon the availability of reimbursement funds for the costs of the resulting drugs and related treatments.

- The market may not accept any pharmaceutical products that we develop, and adverse public perception of gene therapy products may negatively affect demand for, or regulatory approval of, our product candidates.
- We may be subject to federal, state, and foreign healthcare laws and regulations, including fraud and abuse laws, false claims laws, health information privacy and security laws and data privacy laws. If we are unable to comply, or have not fully complied, with such laws, we could face substantial penalties.
- Trends toward managed health care and downward price pressures on medical products and services may limit our ability to profitably sell any drugs that we may develop.
- Our rights to develop and commercialize our product candidates are subject to, in part, the terms and conditions of licenses granted to us by others.
- If we are unable to obtain and maintain patent protection for our product candidates 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.
- Our intellectual property licenses with third parties may be subject to disagreements over contract interpretation.
- We may not be successful in obtaining necessary rights to our product candidates through acquisitions and in-licenses.
- Issued patents covering 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, and intellectual property litigation could cause us to spend substantial resources.
- Third parties may initiate legal proceedings alleging that we are infringing their intellectual property rights, the outcome of which would be uncertain and could harm our business.
- 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.
- If we do not obtain patent term extension and data exclusivity for our product candidates, our business may be harmed.
- We have experienced a history of losses; we expect to incur future losses and we may be unable to obtain necessary additional capital to fund operations in the future. We do not have significant operating revenue and may never achieve profitability.
- We expect to continue to need to raise additional capital to operate our business, and our failure to obtain funding when needed or on terms that are favorable to us may force us to delay, reduce or eliminate our development programs or aspects thereof.
- Failure to achieve and maintain effective internal controls could have a material adverse effect on our business.
- The market price of our common stock may be volatile and adversely affected by several factors.
- Raising additional funds by issuing securities or through licensing or lending arrangements or through our at-the-market sale agreement may cause dilution to our existing stockholders, restrict our operations or require us to relinquish proprietary rights.
- Breaches of data security or unauthorized disclosures of personal information could affect our business or make us subject to liability.

Risks Related to the Commercialization of ZEVASKYN® and our Ability to Generate Revenue

We are in the early stages of commercializing ZEVASKYN® and our limited operating history as a commercial-stage company makes it difficult to predict the long-term success of our business.

We received FDA approval for ZEVASKYN® in 2025, and we are currently in our first full year of commercial sales. Thus, we have limited historical experience operating as a commercial-stage company and limited data on which to base our expectations regarding future revenues, gross margins, operating expenses, and cash flows. Transitioning from a clinical-stage company to a commercial-stage organization requires us to develop, refine, and scale capabilities across sales, marketing, patient services, manufacturing, distribution, compliance, and financial reporting. These activities require significant management attention and financial resources and may present challenges that we have not previously encountered. If we are unable to effectively manage this transition, execute our commercial strategy, or appropriately align our cost structure with revenues, our business, financial condition, cash flow, results of operations, and growth prospects could be adversely affected.

Our financial performance depends on the commercial success of ZEVASKYN® and we have limited experience as a commercial-stage company. As such, we may not be able to successfully commercialize ZEVASKYN® and the revenue that we generate from its sales, if any, may be limited.

Our ability to generate significant revenue from product sales depends on ZEVASKYN®'s successful commercialization. Successful commercialization requires success in many areas, including, but not limited to:

- finding patients who have been diagnosed with RDEB and wish to begin receiving treatment;
- establishing and maintaining relationships with qualified treatment centers who will be treating the patients who receive ZEVASKYN®;
- managing our manufacturing capabilities and supply chain operations in the coordination and delivery of ZEVASKYN® to patients at with qualified treatment centers;
- managing pricing, contracting and reimbursement processes;
- potential post-marketing commitments imposed by regulatory authorities, such as patient registries;
- strength of sales, marketing and distribution support;
- managing working capital and cash flows associated with product commercialization; and
- attracting and retaining employees with relevant commercial, sales, and marketing expertise.

If the patient demand is not as significant as we estimate, or the reasonably predicted population for treatment is narrowed by competition, physician choice, or treatment guidelines, or for any other reason, we may not generate significant revenue from the sale of ZEVASKYN®.

The commercial success of ZEVASKYN® will depend upon the extent of market acceptance by physicians, patients, payors, and other stakeholders.

The degree of market acceptance of ZEVASKYN® depends on several factors, many of which are outside our control, including:

- the perceived clinical efficacy, safety profile and overall benefit-risk profile of ZEVASKYN® compared to alternative therapies;
- relative convenience and ease of administration, including patients' willingness and ability to travel to qualified treatment centers within our network;
- given the complexity of manufacturing ZEVASKYN®, the perception or possibility that issues may continue to arise in the supply of product, which could delay treatment;
- our ability to address any competing products and technological and market developments;
- our ability to educate physicians and other healthcare providers regarding the appropriate use of ZEVASKYN®;
- patient access and affordability;
- inclusion of ZEVASKYN® in clinical guidelines or treatment pathways;
- the effectiveness of our sales and marketing efforts; and
- availability of coverage and reimbursement from government and other third-party payers.

If ZEVASKYN® does not achieve broad market acceptance, we may not generate sufficient revenues to achieve or sustain profitability.

Our revenues currently depend on sales of ZEVASKYN®, which increases our exposure to risks associated with a single product.

Because ZEVASKYN® is our only approved product, our revenues depend highly on its commercial success. Any adverse development affecting ZEVASKYN®, including safety concerns, regulatory actions, supply disruptions, competitive pressures, unfavorable clinical data, or changes in reimbursement, could materially and adversely affect our business, financial condition, cash flow, and results of operations. We do not expect to have additional commercial products in the near term, and we may not be able to successfully develop or acquire additional products.

We may encounter challenges with engaging or coordinating with qualified treatment centers needed for the ongoing commercialization of ZEVASKYN®.

Our commercial strategy is to engage epidermolysis bullosa centers of excellence as qualified treatment centers for the collection of patient biopsy and administration of the drug product once manufactured. To ensure that the qualified treatment centers are prepared to collect biopsies and to ship them to our product in accordance with our specifications and regulatory requirements, we train and conduct quality assessments of each center as part of engagement. These qualified treatment centers are the first and last points on our complex supply chain to reach patients in the commercial setting. We may encounter challenges or delays in engaging and interacting with our qualified treatment centers, and such challenges could impact a qualified treatment centers' willingness and ability to administer ZEVASKYN®.

Furthermore, we may fail to manage the logistics of collecting and shipping patient material to the manufacturing site and shipping the drug product back to the patient. Logistical and shipment delays and problems caused by us, our third-party vendors, or other factors not in our control, such as weather, could prevent or delay the manufacture of or delivery of ZEVASKYN® to patients. If our qualified treatment centers fail to perform satisfactorily, we may suffer reputational, operational, or business harm. Additionally, delays with treatment at the qualified treatment centers due to, for instance, the patient's schedule or health condition or such center's capacity, or due to the need for multiple biopsies, could result in a patient becoming medically ineligible for our treatment or selecting an alternative treatment, the drug product becoming unusable, or loss of medical coverage, which would have a material adverse effect on commercial sales. These delays may also affect our relationship with our qualified treatment center network. Any failure in our engagement or interaction with our qualified treatment centers due to delays in treatment or complications related to manufacturing, among other things, may limit patient access to our therapies and, accordingly, have a material adverse effect on our commercial forecasts and business.

Moreover, we are required to maintain a complex chain of identity and chain of custody with respect to patient material as it moves through the manufacturing process, from the qualified treatment center to the manufacturing facility, and back to the patient. Failure to maintain chain of identity and chain of custody could result in adverse patient outcomes, loss of product, or regulatory action.

The manufacturing, testing and delivery of ZEVASKYN® present significant challenges for us, and we may not be able to produce ZEVASKYN® at the quality, quantities, or timing needed to support commercialization.

The manufacturing of ZEVASKYN® is complex and requires significant expertise. Even with the relevant experience and expertise, manufacturing cell therapy products often leads to difficulties in production, particularly in scaling out and validating initial production, managing the transition from clinical manufacturing to commercial manufacturing, and ensuring that the product meets required specifications. These problems include difficulties with production costs and yields, quality control, quality assurance testing, operator error, scarcity of qualified manufacturing and quality control testing personnel, shortages of any production raw materials as well as compliance with strictly enforced federal, state and foreign regulations.

We are susceptible to production interruptions that may impede our ability to manufacture cell and gene therapy products and produce an adequate product supply to support commercialization of ZEVASKYN®. Several factors could cause production interruptions, including equipment malfunctions, facility contamination, raw material shortages or contamination, natural disasters, public health emergencies, disruption in utility services, human error, or disruptions in the operations of our suppliers. ZEVASKYN® and product candidates are biologic drugs requiring processing steps that are more complex than those required for most chemical pharmaceuticals. We characterize our processes and products, and perform testing to ensure the safety, quality and efficacy of each product produced. While we take significant measures to fully understand and characterize each product, the steps we take may not be sufficient to ensure that a given lot will perform in the intended manner.

For example, we manufactured a full batch of ZEVASKYN® following patient biopsy collection in August 2025 that, despite being a bonafide drug product, could not be released because a rapid sterility assay, mandated by the FDA as a release assay during the final stage of the BLA review, initially yielded a false positive result for sterility. Although we resumed biopsy collection in November 2025 upon completion of assay optimization and the necessary regulatory submission for its implementation, this false positive caused a manufacturing rejection, which caused a delay in our launch of ZEVASKYN®. Additional or similar issues associated with manufacturing and testing can have an adverse impact on our business, financial condition, cash flow, and results of operations.

There are several risks specific to the manufacturing process for ZEVASKYN® that require close attention. As an autologous product there are challenges associated with viability of biopsies as an incoming material. Due to variables such as the fragility of RDEB skin and site of the biopsy, initiation of autologous keratinocyte growth and expansion can be challenging or may be extended beyond the scheduled timing. Another concern during manufacturing is the slowing of cell proliferation, resulting in extended manufacturing time. If pre-release criteria are not met, the production process must be stopped, and a new biopsy must be obtained. If release criteria are out of range, epidermal sheets must be discarded and the manufacturing process must be repeated.

We rely on third-party suppliers for our manufacturing of ZEVASKYN®, and supply interruptions could disrupt commercialization.

Our reliance on third-party suppliers for the manufacturing of ZEVASKYN® exposes us to risks, including manufacturing delays or disruptions, quality control failures, regulatory compliance issues, capacity constraints, and financial instability of suppliers. Any interruption in the manufacture or supply of ZEVASKYN® could impair our ability to meet demand and adversely affect our commercial efforts.

We currently do not have a backup manufacturer to supply manufacturing material for ZEVASKYN®. An alternative manufacturer would need to be qualified through regulatory filings, which could result in production delays. Regulatory authorities also may require additional clinical trials if a new supplier is relied upon for commercial production. Accordingly, identifying and contracting with alternative manufacturer or supplier would significantly affect our ability to meet demand for ZEVASKYN®.

Post-marketing requirements and ongoing regulatory obligations could restrict or delay commercialization.

Following FDA approval, we remain subject to ongoing regulatory obligations, including post-marketing requirements, pharmacovigilance reporting, quality system regulation compliance, and potential FDA inspections. If we fail to comply with these requirements, the FDA may impose sanctions, including warning letters or other enforcement actions, fines, product recalls or withdrawals, restrictions on marketing, or suspension or withdrawal of approval. In addition, previously unknown adverse events may be identified after broader commercial use, which could result in changes to the labeling of ZEVASKYN®, restrictions on its use, or withdrawal from the market.

Our commercialization efforts may expose us to increased risk of product liability and other litigation.

The commercialization of ZEVASKYN® exposes us to the risk of product liability claims and other litigation, including claims related to adverse events, off-label promotion, false advertising, pricing, or reimbursement practices. Even if we are successful in defending ourselves against such claims, litigation could be costly, time-consuming, and damaging to our reputation. If we are unable to obtain or maintain adequate insurance coverage on acceptable terms, our financial condition could be adversely affected.

Our commercial success depends in part on our ability to protect and enforce our intellectual property rights relating to ZEVASKYN®.

Our ability to maintain market exclusivity for ZEVASKYN® depends on our intellectual property portfolio and regulatory exclusivities. If our patents are challenged, invalidated, circumvented or expire earlier than expected, or if we are unable to enforce our intellectual property rights effectively, competitors may develop and commercialize competing products more rapidly than anticipated, which could significantly harm our commercial prospects.

Our products and product candidates may face competition sooner than anticipated.

The 12-year exclusivity granted to ZEVASKYN® may not adequately protect us from biosimilar or other product competition. There may also be changes in regulatory exclusivity policies. For example, there have been efforts to decrease the biologic period of exclusivity to a shorter timeframe. Future proposed budgets, international trade agreements and other arrangements or proposals may affect periods of exclusivity. If another company pursues approval of a product that is biosimilar to ZEVASKYN® or any other biologic product for which we receive FDA approval, we may need to pursue costly and time-consuming patent infringement actions, which may include certain statutorily specified regulatory steps before an infringement action may be brought. Biosimilar applicants may also be able to bring an action for declaratory judgment concerning our patents, requiring that we spend time and money defending the action.

Risks related to manufacturing

We could experience production problems in our manufacturing facilities that result in delays in our development or commercialization programs or otherwise adversely affect our business.

We are susceptible to production interruptions that may impede our ability to manufacture cell and gene therapy products and produce an adequate product supply to support commercialization or clinical trials. Several factors could cause production interruptions, including equipment malfunctions, facility contamination, raw material shortages or contamination, natural disasters, public health emergencies, disruption in utility services, human error, or disruptions in the operations of our suppliers. Our product candidates are biologic drugs requiring processing steps that are more complex than those required for most chemical pharmaceuticals. We characterize our processes and products, and perform testing to ensure the safety, quality and efficacy of each product produced. While we take significant measures to fully understand and characterize each product, the steps we take may not be sufficient to ensure that a given lot will perform in the intended manner.

We employ multiple steps to control our manufacturing process to ensure that the products or product candidate is made strictly and consistently in compliance with the process. Problems with the manufacturing process, including even minor deviations from the normal process, could result in product defects or manufacturing failures that result in lot failures, product recalls, product liability claims, or insufficient inventory. We may encounter problems achieving adequate quantities and quality of clinical grade materials that meet FDA, EU or other applicable standards or specifications with consistent and acceptable production yields and costs. In addition, the FDA, 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, EMA or other foreign regulatory authorities may require that we not distribute a lot until the agency authorizes its release. Slight deviations in the manufacturing process, including those affecting quality attributes and stability, may result in unacceptable changes in the product that could result in lot failures or product recalls for approved and marketed products.

Lot failures or product recalls could cause us to delay sales, product launches, or clinical trials, which could be costly to us and otherwise harm our business, financial condition, cash flow, 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 facilities could make us a less attractive collaborator for potential partners, including qualified treatment centers for ZEVASKYN®, larger pharmaceutical companies, and academic research institutions, which could limit our access to additional attractive development programs. Problems in our manufacturing process including in internal and external facilities providing supply necessary for manufacturing or challenges with procuring supplies, such as due to global trade policies, also could restrict our ability to meet customer or clinical trial supply demand, and as well as market demand for ZEVASKYN® or any future product candidates for which we may receive marketing approval.

If we or any of our vendors, contract laboratories or suppliers are found to be out of compliance with cGMP or other regulations, we may experience delays or disruptions in manufacturing while we implement corrective actions or work with these third parties to remedy the violation or while we work to identify suitable replacement vendors, contract laboratories or suppliers.

To maintain regulatory approval for commercial manufacturing, we will need to continue to ensure that all our processes, methods and equipment are compliant with cGMP and perform extensive audits of vendors, contract laboratories and suppliers. The cGMP requirements govern quality control of the manufacturing process and documentation policies and procedures. Complying with cGMP requires us to spend 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 will be subject to possible regulatory action and may not be permitted to sell ZEVASKYN®.

We may rely on third parties to conduct aspects of our product manufacturing, and these third parties may not perform satisfactorily. We also may rely on third parties to produce certain materials for our product candidates and, therefore, we cannot control every aspect of their activities.

We and our third-party suppliers, laboratories, and manufacturers may be unable to comply with our specifications, cGMP requirements and with other FDA, state, and foreign regulatory requirements.

Inadequate control of production processes can lead to the introduction of adventitious agents or other contaminants, or to inadvertent changes in the properties or stability of a product candidate that may not be detectable in final product testing. If we or our contract manufacturers cannot successfully manufacture material that conforms to our specifications and the strict regulatory requirements of the FDA or other regulatory authorities, we or our contract manufacturers will not be able to secure or maintain regulatory approval for such manufacturing facilities. Any such deviations may also require remedial measures that may be costly and/or time-consuming for us or a third party to implement and may include the temporary or permanent suspension of a clinical trial or commercial sales or the temporary or permanent closure of a facility. Any such remedial measures imposed upon or by us or third parties with whom we contract could materially harm our business. Any delays in obtaining products or product candidates that comply with the applicable regulatory requirements may result in delays to our sales of ZEVASKYN® as well as clinical trials, product approvals, and commercialization for our other product candidates. It may also require that we conduct additional studies.

If any inspection or audit by regulatory authorities 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.

Regulatory authorities may inspect or audit the manufacturing facilities for our products and product candidates at any time. Any such remedial measures imposed upon us could materially harm our business, financial condition, cash flow, results of operations and prospects. If we fail to comply with applicable cGMP regulations, FDA and foreign regulatory authorities could 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, cash flow, results of operations and prospects to be materially harmed. Additionally, if supply from our facility is interrupted, there could be a significant disruption in commercial supply of any of our product candidates for which we obtain marketing approval, and in clinical supply for our product candidates.

If we, our collaborators, or any third-party manufacturers we engage fail to comply with environmental, health and safety laws and regulations, we could become subject to fines or penalties or incur costs that could harm our business.

We, our collaborators, and any third-party manufacturers we engage 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 materials. Our operations also produce hazardous waste products. We generally contract with third parties for the disposal of these materials and wastes. We cannot eliminate the risk of contamination or injury from these materials. In the event of contamination or injury resulting from our use of hazardous materials, we could be held liable for any resulting damages, and any liability could exceed our resources. We also could incur significant costs associated with civil or criminal fines and penalties.

Although we maintain general liability insurance and workers' compensation insurance for certain costs and expenses that we may incur due to injuries to our employees resulting from the use of hazardous materials or other work-related injuries, this insurance may not provide adequate coverage against potential liabilities. We do not maintain insurance for environmental liability or toxic tort claims that may be asserted against us in connection with our storage or disposal of biologic and hazardous materials.

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 our 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 harm our business, financial condition, cash flow, results of operations and prospects.

We may be unable to successfully develop, market, or commercialize our products or our product candidates without establishing new relationships and maintaining current relationships and our ability to successfully commercialize, and market our product candidates could be limited if a number of these existing relationships are terminated.

Our strategy for the research, development and commercialization of our potential pharmaceutical products may require us to enter into various arrangements with corporate and academic collaborators, licensors, licensees and others, in addition to our existing relationships with other parties. Specifically, we may seek to joint venture, sublicense or enter into other marketing arrangements with parties that have an established marketing capability, or we may choose to pursue the commercialization of such products on our own. We may, however, be unable to establish such additional collaborative arrangements, license agreements, or marketing agreements as we may deem necessary to develop, commercialize and market our potential pharmaceutical products on acceptable terms. Furthermore, since we maintain and establish arrangements or relationships with third parties, our business may depend upon the successful performance by these third parties of their responsibilities under those arrangements and relationships. If we are unwilling or unable to perform our obligations under any license or collaboration arrangement, a third party may have the right to terminate such arrangement with us.

We are subject to extensive governmental regulation, which increases our cost of doing business and may affect our ability to commercialize any new products that we may develop.

The FDA and comparable agencies in foreign countries impose substantial requirements upon the introduction of pharmaceutical products through lengthy and detailed laboratory, preclinical and clinical testing procedures and other costly and time-consuming procedures to establish safety and efficacy. All of our drugs and drug candidates require receipt and maintenance of governmental approvals for commercialization. Preclinical and clinical trials and manufacturing of our drug candidates will be subject to the rigorous testing and approval processes of the FDA and corresponding foreign regulatory authorities. Satisfaction of these requirements typically takes a significant number of years and can vary substantially based upon the type, complexity, and novelty of the product.

Due to the time-consuming and uncertain nature of the drug candidate development process and the governmental approval process described above, we cannot be certain when we, independently or with our collaborative partners, might submit a BLA for FDA or other regulatory review. Further, our ability to commence and/or complete development projects will be subject to our ability to raise enough funds to pay for the development costs of these projects. Government regulation also affects the manufacturing and marketing of pharmaceutical products. Government regulations may delay marketing of our potential drugs for a considerable or indefinite period of time, impose costly procedural requirements upon our activities and furnish a competitive advantage to larger companies or companies more experienced in regulatory affairs. Delays in obtaining governmental regulatory approval could adversely affect our marketing as well as our ability to generate significant revenues from commercial sales.

Our drug candidates may not receive FDA or other regulatory approvals on a timely basis or at all. Moreover, if regulatory approval of a drug candidate is granted, such approval may impose limitations on the indicated use for which such drug may be marketed. Even if we obtain initial regulatory approvals for our drug candidates, our drugs and our manufacturing facilities would be subject to continual review and periodic inspection, and later discovery of previously unknown problems with a drug, manufacturer or facility may result in restrictions on the marketing or manufacture of such drug, including withdrawal of the drug from the market. The FDA and other regulatory authorities stringently apply regulatory standards and failure to comply with regulatory standards can, among other things, result in fines, denial or withdrawal of regulatory approvals, product recalls or seizures, operating restrictions, and criminal prosecution.

We may incur substantial product liability expenses due to the use or misuse of our products for which we may be unable to obtain insurance coverage.

Our business exposes us to potential liability risks that are inherent in the testing, manufacturing, and marketing of pharmaceutical products. These risks expand with commercialization and we may face substantial liability for damages in the event of adverse side effects, including injury or death, or product defects identified with any of our products that are marketed to the public or product candidates that are used in clinical tests. Product liability actions can also have regulatory consequences, including the withdrawal of clinical trial participants and potential termination of clinical trial sites or entire clinical programs, and the initiation of investigations, and enforcement actions by regulators, product recalls, withdrawals, revocation of approvals, labeling, marketing, or promotional restrictions.

Product liability insurance for the biotechnology industry is generally expensive, if available at all, and as a result, we may be unable to obtain insurance coverage at acceptable costs or in a sufficient amount in the future, if at all. We may be unable to satisfy any claims for which we may be held liable as a result of the use or misuse of products which we developed, manufactured, or sold and any such product liability claim could adversely affect our business, operating results, or financial condition.

Intense competition may limit our ability to successfully develop and market commercial products.

The biotechnology and pharmaceutical industries are intensely competitive and subject to rapid and significant technological change. Our competitors in the U.S. and elsewhere are numerous and include, among others, major multinational pharmaceutical and chemical companies, specialized biotechnology firms and universities and other research institutions. Many of our competitors have and employ greater financial and other resources, including larger research and development, marketing, and manufacturing organizations. As a result, our competitors may successfully develop technologies and drugs that are more effective or less costly than any that we have or are developing, which could render our technology and future products obsolete and noncompetitive.

In addition, some of our competitors have greater experience than we do in conducting preclinical and clinical trials and obtaining FDA and other regulatory approvals. Accordingly, our competitors may succeed in obtaining FDA or other regulatory approvals for drug candidates more rapidly than we can. Companies that complete clinical trials, obtain required regulatory agency approvals, and commence commercial sale of their drugs before their competitors may achieve a significant competitive advantage. Drugs resulting from our research and development efforts or from our joint efforts with collaborative partners therefore may not be commercially competitive with our competitors' existing products or products under development.

Healthcare reform measures could hinder or prevent our product candidates' commercial success.

Any government-adopted reform measures could adversely affect the pricing of healthcare products and services in the U.S. or internationally and the amount of reimbursement available from governmental agencies or other third-party payors. The continuing efforts of the U.S. and foreign governments, insurance companies, managed care organizations and other payors of health care services to contain or reduce health care costs may adversely affect our ability to set prices for our products which we believe are fair, restrict coverage and reimbursement, or require payment of increased rebates and our ability to generate revenues and achieve and maintain profitability.

New laws, regulations and judicial decisions, or new interpretations of existing laws, regulations, and decisions, which relate to healthcare availability, methods of delivery or payment for products and services, or sales, marketing, or pricing, may limit our potential revenue, and we may need to revise our research and development programs. The pricing and reimbursement environment may change in the future and become more challenging due to several reasons including new healthcare legislation or regulation and fiscal challenges faced by government health administration authorities. Specifically, in both the U.S. and some foreign jurisdictions, there have been a number of legislative and regulatory proposals to change the health care system in ways that could affect our ability to sell our products profitably.

We also cannot predict the likelihood, nature or extent of government regulation that may arise from future legislation or administrative or executive action, either in the United States or abroad.

We may be subject, directly or indirectly, to federal, state, and foreign healthcare laws and regulations, including fraud and abuse laws, false claims laws and health information privacy and security laws. If we are unable to comply, or have not fully complied, with such laws, we could face substantial penalties.

If we obtain FDA approval for any of our product candidates and begin commercializing those products in the United States, our operations will be directly, or indirectly through our prescribers, customers, and purchasers, subject to various federal and state laws and regulations, including, without limitation, the federal Anti-Kickback Statute, the federal civil and criminal false claims act, the civil monetary penalties statute, HIPAA, and the Physician Payments Sunshine Act and regulations. These laws are further described in the U.S. Biologic Products Development Process section of this annual report. These laws will impact, among other things, our proposed sales, marketing, and educational programs. In addition, we may be subject to data privacy laws by both the federal government and the states in which we conduct our business. Failure to comply with these laws could 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, debarment from government contracting or refusal of orders under existing contracts, corporate integrity agreements or consent decrees, disgorgement, contractual damages, reputational harm, diminished profits and future earnings, and imprisonment. Furthermore, efforts to ensure that business activities and business arrangements comply with applicable healthcare laws and regulations can be costly. Comparable laws and regulations apply internationally.

We are subject to extensive laws and regulations related to data privacy, and our failure to comply with these laws and regulations could harm our business.

Numerous foreign, federal, and state laws and regulations govern collection, dissemination, use and confidentiality of personally identifiable health information, including state privacy and confidentiality laws (including state laws requiring disclosure of breaches), HIPAA and the European Union's General Data Protection Regulation ("GDPR"). These laws and regulations are increasing in complexity and number and may change frequently and sometimes conflict.

HIPAA establishes a set of national privacy and security standards for the protection of individually identifiable health information, including protected health information ("PHI"), by health plans, certain healthcare clearinghouses and healthcare providers that submit certain covered transactions electronically, or covered entities, and their "business associates," which are persons or entities that perform certain services for, or on behalf of, a covered entity that involve creating, receiving, maintaining or transmitting PHI. While we are not currently a covered entity or business associate under HIPAA, we may receive identifiable information from these entities. Failure to protect this information properly could subject us to HIPAA's criminal penalties, which may include fines up to $250,000 per violation and/or imprisonment.

GDPR imposes numerous requirements on entities that process personal data in the context of an establishment in the European Economic Area ("EEA") or that process the personal data of data subjects who are located in the EEA. These requirements include, for example, establishing a basis for processing, providing notice to data subjects, developing procedures to vindicate expanded data subject rights, implementing appropriate technical and organizational measures to safeguard personal data, and complying with restrictions on the cross-border transfer of personal data from the EEA to countries that the European Union does not consider to have in place adequate data protection legislation, such as the United States. GDPR additionally establishes heightened obligations for entities that process "special categories" of personal data, such as health data. Nearly all clinical trials involve the processing of these "special categories" of personal data, and thus processing of personal data collected during the course of clinical trials is subject to heightened protections under GDPR.

Moreover, California adopted the California Consumer Privacy Act of 2018 ("CCPA"), which went into effect in January 2020. The CCPA has been characterized as the first "GDPR-like" privacy statute to be enacted in the United States because it mirrors a number of the key provisions of the GDPR. The CCPA establishes a new privacy framework for covered businesses in the State of California, by creating an expanded definition of personal information, establishing new data privacy rights for consumers imposing special rules on the collection of consumer data from minors, and creating a new and potentially severe statutory damages framework for violations of the CCPA and for businesses that fail to implement reasonable security procedures and practices to prevent data breaches.

The legislative and regulatory landscape for privacy and data security continues to evolve, and there has been an increasing focus on privacy and data security issues which may affect our business. Failure to comply with current and future laws and regulations could result in government enforcement actions (including the imposition of significant penalties), criminal and/or civil liability for us and our officers and directors, private litigation and/or adverse publicity that negatively affects our business.

Trends toward managed health care, health technology assessment, and downward price pressures on medical products and services may limit our ability to profitably sell any drugs that we may develop.

Lower prices for pharmaceutical products or reduced profitability may result from:

- third-party-payors' increasing challenges to the prices charged for medical products and services, including by limiting coverage and reimbursement and requiring payment of increased manufacturer rebates;
- the trend toward managed health care in the U.S. and the concurrent growth of Health Maintenance Organizations ("HMOs") and similar organizations that can control or significantly influence the purchase of healthcare services and products; and
- state, federal, and foreign legislative proposals to control drug prices, reform healthcare or reduce government insurance programs.

The cost containment measures that healthcare providers are instituting, including practice protocols and guidelines and clinical pathways, and the effect of any healthcare reform, could limit our ability to profitably sell any drugs that we may successfully develop. Moreover, any future legislation or regulation, if any, relating to the healthcare industry or third-party coverage and reimbursement, may cause our business to suffer.

Changes in and uncertainty surrounding U.S. trade policy could have a material adverse impact on our business, financial condition, cash flow, and results of operations.

The ongoing trade tensions between the U.S. and other jurisdictions have resulted in multiple rounds of tariffs and anticipated tariffs affecting a wide range of products and jurisdictions and has indicated an intention to continue developing new trade policies, including with respect to the pharmaceutical industry. In response, certain foreign governments have announced or implemented retaliatory tariffs and other protectionist measures. These developments have created a dynamic and unpredictable trade landscape, which may adversely affect our business, results of operations, financial conditions and prospects.

Current or future tariffs or other trade restrictions may result in increased research and development expenses, including with respect to increased costs associated with raw materials, laboratory equipment, and research materials and components. In addition, such tariffs may increase our supply chain complexity and could also potentially disrupt our existing supply chain. Unlike consumer goods, pharmaceuticals face unique regulatory constraints that make rapid supply chain adjustments particularly difficult and costly. Tariffs and trade restrictions affecting the import of materials necessary for manufacturing or clinical trials could result in manufacturing delays for ZEVASKYN® or hinder our ability to establish cost-effective production capabilities, as well as in delays to our development timelines for our pre-clinical product candidates, negatively affecting our growth prospects. Increased development costs and extended development timelines could place us at a competitive disadvantage compared to companies operating in regions with more favorable trade relationships and could reduce investor confidence, negatively impacting our ability to secure additional financing on favorable terms or at all. Tariffs and trade restrictions.

If we are unable to obtain necessary raw materials or product components in sufficient quantity and in a timely manner due to disruptions in the global supply chain caused by macroeconomic events and conditions, the development, testing and clinical trials of our product candidates may be delayed or infeasible, and regulatory approval or commercial launch of any resulting product may be delayed or not obtained, which could significantly harm our business.

The complexity of announced or future tariffs may also increase the risk that we or our customers or suppliers may be subject to civil or criminal enforcement actions in the United States or foreign jurisdictions related to compliance with trade regulations. Foreign governments may also adopt non-tariff measures, such as procurement preferences or informal disincentives to engage with, purchase from or invest in U.S. entities, which may limit our ability to compete internationally and attract non-U.S. investment, employees, customers and suppliers. Foreign governments may also take other retaliatory actions against U.S. entities, such as decreased intellectual property protection, increased enforcement actions, or delays in regulatory approvals, which may result in heightened international legal and operational risks. In addition, the United States and other governments have imposed and may continue to impose additional sanctions, such as trade restrictions or trade barriers, which could restrict us from doing business directly or indirectly in or with certain countries or parties and may impose additional costs and complexity to our business.

Risks related to the discovery and development of our product candidates

Our cell and gene therapy product candidates are based on proprietary methodologies, which makes it difficult to predict the time and cost of product candidate development and subsequently obtaining regulatory approval. Only a few gene therapy products have been approved in the U.S. and the EU.

We have concentrated our therapeutic product research and development efforts on our cell and gene therapy platform, and our future success depends in part on the successful development of this therapeutic approach. There can be no assurance that any development problems we experience in the future related to our cell and gene therapy platform will not cause significant delays or unanticipated costs, or that such development problems can be solved. We may also experience delays in developing a sustainable, reproducible and commercial-scale manufacturing process or transferring that process to commercial partners, which may prevent us from completing our clinical studies or commercializing our products on a timely or profitable basis, if at all.

In addition, the clinical study requirements of the FDA, the EMA, and other regulatory agencies and the criteria these regulators use to determine the safety and efficacy of a product candidate vary substantially according to the type, complexity, novelty and intended use and market of the potential products. The regulatory approval process for novel product candidates can be more expensive and take longer than for other, better known or more extensively studied pharmaceutical or other product candidates. Given that only a few gene therapy products have been approved in the Western world, it is not possible to predict how long it will take or how much it will cost to obtain regulatory approvals for our product candidates in the United States, the EU or other jurisdictions. Approvals by the EMA and the European Commission may not be indicative of what the FDA may require for approval.

Our ability to successfully develop and commercialize our product candidates will substantially depend upon the availability of reimbursement funds for the costs of the resulting drugs and related treatments.

Market acceptance and sales of our product candidates may depend on coverage and reimbursement policies and health care reform measures. Decisions about formulary coverage as well as levels at which government authorities and third-party payors, such as private health insurers and health maintenance organizations, reimburse patients for the price they pay for our products as well as levels at which these payors pay directly for our products, where applicable, could affect whether we are able to commercialize these products. We cannot be sure that reimbursement will be available for any of these products. Also, we cannot be sure that coverage or reimbursement amounts will not reduce the demand for, or the price of, our products. We have not commenced efforts to have our product candidates reimbursed by the government or third-party payors. If coverage and reimbursement are not available or are available only at limited levels, we may not be able to commercialize our product candidates. In recent years, officials have made numerous proposals to change the health care system in the U.S. These proposals include measures that would limit or prohibit payments for certain medical treatments or subject the pricing of drugs to government control. In addition, in many foreign countries, particularly the countries of the European Union, the pricing of prescription drugs is subject to government control. If our products are or become subject to government regulation that limits or prohibits payment for our products, or that subjects the price of our products to governmental control, we may not be able to generate revenue, attain profitability or commercialize our products.

As a result of legislative proposals and the trend towards managed health care in the U.S., third-party payors are increasingly attempting to contain health care costs by limiting both coverage and the level of reimbursement of new drugs. They may also impose strict prior authorization requirements and/or refuse to provide any coverage of uses of approved products for medical indications other than those for which the FDA has granted market approvals. As a result, significant uncertainty exists as to whether and how much third-party payors will reimburse patients for their use of newly approved drugs, which in turn will put pressure on the pricing of drugs.

Our drug candidates are subject to the risks of failure inherent in the development of pharmaceutical products based on new technologies, and our failure to develop safe and commercially viable drugs would severely limit our ability to become profitable or to achieve significant revenues.

We may be unable to successfully commercialize our product candidates if some or all of our product candidates are found to be unsafe or ineffective or otherwise fail to meet applicable regulatory standards or receive necessary regulatory clearances. Additionally, our product candidates may be deemed too difficult to develop into commercially viable drugs. We may encounter difficulty in manufacturing or marketing our product candidates on a large scale, and proprietary rights of third parties may preclude us from marketing our drug candidates. Moreover, competitors may be able to market superior or equivalent drugs successfully. Failure to successfully commercialize our product candidates would have a material adverse effect on our business.

Adverse public perception of gene therapy products may negatively affect demand for, or regulatory approval of, our product candidates.

Our product candidates involve altering genes, and the clinical and commercial success of our product candidates will depend in part on public acceptance of the use of gene altering therapies for the treatment of genetic diseases. Public attitude may be influenced by claims that gene therapy is unsafe, unethical, or immoral, and, as a result, our product candidates may not gain the acceptance of the public or the medical community. Negative public reaction to gene therapy in general could result in greater government regulation and stricter labeling requirements of gene therapy products, including any of our product candidates, and could cause a decrease in the demand for any products we may develop. Adverse public opinion also may adversely affect our ability to enroll patients in clinical trials.

The market may not accept any pharmaceutical products that we develop, thereby materially impairing our ability to generate revenue from such products.

The product candidates that we are attempting to develop may compete with drugs manufactured and marketed by other pharmaceutical companies. The degree of market acceptance of any drugs developed by us will depend on a number of factors, including the establishment and demonstration of the clinical efficacy and safety of our drug candidates, the potential advantage of our drug candidates over existing therapies and the reimbursement policies of government and third-party payors. Physicians, patients, or the medical community in general may not accept or use any drugs that we may develop independently or with our collaborative partners and if they do not, our business could suffer.

Regulatory requirements governing cell and gene therapy products have evolved and may continue to change in the future.

Regulatory requirements in the United States and in other jurisdictions governing gene therapy products have changed frequently and will continue to change in the future as scientific knowledge is acquired. The FDA and EMA have each expressed interest in further regulating gene therapy. For example, the FDA has established the Office of Tissues and Advanced Therapies within CBER to consolidate the review of gene therapy and related products, and the Cellular, Tissue and Gene Therapies Advisory Committee to advise CBER on its review. Over the last few years, the FDA, through CBER, has provided significant guidance regarding the development of gene therapies. Additionally, the EMA advocates a risk-based approach to the development of a gene therapy product. Agencies at both the federal and state level in the United States, as well as the U.S. congressional committees and other governments or governing agencies, have also expressed interest in further regulating the biotechnology industry. Such action may delay or prevent commercialization of some, or all, of our product candidates. These regulatory review agencies, committees and advisory groups and the new requirements and guidelines they promulgate may lengthen the regulatory review process, require us to perform additional or larger studies, increase our development costs, lead to changes in regulatory positions and interpretations, delay or prevent approval and commercialization of these treatment candidates or lead to significant post-approval studies, 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 requirements and guidelines. If we fail to do so, we may be required to delay or discontinue development of our product candidates. Delay or failure to obtain, or unexpected costs in obtaining, the regulatory approval necessary to bring a potential product to market could decrease our ability to generate sufficient product revenue to maintain our business.

We may encounter substantial delays in our clinical studies, such as clinical holds, 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 studies to demonstrate the safety, purity and potency, and efficacy, of the product candidates in humans. Clinical testing is expensive, time-consuming, and uncertain as to outcome. This is especially true for rare or complicated diseases. We cannot guarantee that any clinical studies will be conducted as planned or completed on schedule, if at all. A failure of one or more clinical studies can occur at any stage of testing.

The results of preclinical studies, preliminary study results, and early clinical trials of our product candidates may not be predictive of the results of later-stage clinical trials or the ultimately completed trial. Product candidates in later stages of clinical trials may fail to show the desired safety and efficacy traits despite having progressed through preclinical studies and initial clinical trials. Preclinical and early clinical studies may also reveal unfavorable product candidate characteristics, including safety concerns.

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

- regulators or IRBs may not authorize us or our investigators to commence or continue a clinical trial, conduct a clinical trial at a prospective trial site, or amend trial protocols, or regulators or IRBs may require that we modify or amend our clinical trial protocols;
- we may experience delays in reaching, or fail to reach, agreement on acceptable clinical trial contracts or clinical trial protocols with prospective trial sites and our contract research organizations (“CROs’);
- regulators may require us to perform additional or unanticipated clinical trials to obtain approval or we may be subject to additional post-marketing testing, surveillance, or REMS requirements to maintain regulatory approval;
- flaws in a clinical trial may not become apparent until the trial is well advanced;
- clinical trials of our product candidates may produce negative or inconclusive results, or our studies may fail to reach the necessary level of statistical significance, and we may decide, or regulators may require us, to conduct additional clinical trials or abandon product development programs;
- clinical trials of our product candidates may require us to provide follow-up patient visits for safety for a minimum of five years even if we were to terminate and/or abandon a product development program;

- our third-party contractors may fail to comply with regulatory requirements or the clinical trial protocol, or fail to meet their contractual obligations to us in a timely manner, or at all, or we may be required to engage in additional clinical trial site monitoring;
- we, the regulators, or IRBs may require the suspension or termination of clinical research for various reasons, including noncompliance with regulatory requirements or a finding that the participants are being exposed to unacceptable health risks, undesirable side effects, or other unexpected characteristics (alone or in combination with other products) of the product candidate, or due to findings of undesirable effects caused by a chemically or mechanistically similar therapeutic or therapeutic candidate;
- changes in marketing approval and regulatory review policies or changes in or the enactment of additional statutes or regulations;
- the cost of clinical trials of and marketing applications for our product candidates may be greater than we anticipate;
- the supply or quality of our product candidates or other materials necessary to conduct clinical trials may be insufficient or inadequate;
- we may decide, or regulators may require us, to conduct or gather, as applicable, additional clinical trials, analyses, reports, data, or preclinical trials, or we may abandon product development programs;
- we may fail to reach an agreement with regulators or IRBs regarding the scope, design, or implementation of our clinical trials. For instance, the FDA or comparable foreign regulatory authorities may require changes to our study design that make further study impractical or not financially prudent;
- we may have delays in adding new investigators or clinical trial sites, or we may experience a withdrawal of clinical trial sites;
- there may be regulatory questions or disagreements regarding interpretations of data and results, or new information may emerge regarding our product candidates;
- we may make changes to our product candidates or their manufacturing process that necessitate additional studies or that result in our product candidates not performing as expected;
- the FDA or comparable foreign regulatory authorities may disagree with our study design, including endpoints, or our interpretation of data from preclinical studies and clinical trials or find that a product candidate's benefits do not outweigh its safety risks;
- the FDA or comparable foreign regulatory authorities may not accept data from studies with clinical trial sites in foreign countries;
- the FDA or comparable regulatory authorities may disagree with our intended indications;
- the FDA or comparable foreign regulatory authorities may fail to approve or subsequently find fault with the manufacturing processes or our contract manufacturer's manufacturing facility for clinical and future commercial supplies;
- the data collected from clinical trials of our product candidates may not be sufficient to the satisfaction of the FDA or comparable foreign regulatory authorities to support the submission of a marketing application, or other comparable submission in foreign jurisdictions or to obtain regulatory approval in the United States or elsewhere;
- if one of our product candidates does not receive marketing approval in one country, it may impact our ability to receive marketing approval in other countries;
- the FDA or comparable regulatory authorities may take longer than we anticipate to make a decision on our product candidates; and
- we may not be able to demonstrate that a product candidate provides an advantage over current standards of care or current or future competitive therapies in development.

Delays in launching clinical trials resulting from FDA or other regulatory actions, such as a clinical hold letter, would delay the commercialization of our product candidates and our ability to generate revenue, which would have an adverse effect on our business.

Significant delays relating to any preclinical or clinical trials 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. This may prevent us from receiving marketing approvals and impair our ability to successfully commercialize our product candidates. If any of the foregoing were to occur, our business, financial condition, cash flow, results of operations, and prospects will be materially harmed.

We may find it difficult to enroll patients in our clinical studies, which could delay or prevent clinical studies of our product candidates.

Identifying and qualifying patients to participate in clinical studies of our product candidates is critical to our success. The timing of our clinical studies depends on the speed at which we can recruit eligible patients to participate in testing our product candidates. We have experienced delays in some of our clinical studies due to the ultra-rare nature of the diseases we aim to treat, and we may experience similar delays in the future. If patients are unwilling to participate in our cell and gene therapy studies because of negative publicity from adverse events in the biotechnology or gene therapy industries or for other reasons, including competitive clinical studies for similar patient populations, the timeline for recruiting patients, conducting studies, and obtaining regulatory approval of potential products may be delayed. These delays could result in increased costs, delays in advancing our product development, delays in testing the effectiveness of our technology or termination of the clinical studies altogether.

We may not be able to identify, recruit or enroll enough patients, or those with required or desired characteristics to achieve diversity in a study, to complete our clinical studies in a timely manner. Patient enrollment is affected by factors including:

- severity of the disease under investigation;
- design of the study protocol;
- size and nature of the patient population;
- eligibility criteria for and design of the study in question;
- perceived risks and benefits of the product candidate under study, including as a result of adverse effects observed in similar or competing therapies;
- proximity and availability of clinical study sites for prospective patients;
- availability of competing therapies and clinical studies;
- efforts to facilitate timely enrollment in clinical studies;
- ability to compensate patients for their time and effort;
- risk that enrolled patients will drop out before completion or not return for post-treatment follow-up;
- inability to obtain or maintain patient informed consents;
- effectiveness of publicity created by clinical trial sites regarding the trial;
- patient referral practices of physicians; and
- ability to monitor patients adequately during and after treatment.

We also plan to seek initial marketing approval in the European Union in addition to the U.S. Our ability to successfully initiate, enroll and complete a clinical study in any foreign country is subject to additional risks unique to conducting business in foreign countries, such as different standards for the conduct of clinical studies; different laws, medical standards, and regulatory requirements; and the ability to establish or manage relationships with treatment centers, contract research organizations and physicians.

If we have difficulty enrolling enough patients to conduct our clinical studies as planned our development costs may increase, the time for completion of clinical trials may increase, we may need to delay, limit or terminate ongoing or planned clinical studies, any of which would have an adverse effect on our business.

Our products or product candidates may cause undesirable side effects or have other properties that could delay or prevent their regulatory approval or commercialization.

Undesirable side effects caused by our products or product candidates, including adverse events associated with our product candidates, could interrupt, delay, or halt clinical trials and could result in the denial of regulatory approval or more limited approvals by the FDA, EMA or other regulatory authorities for any or all targeted indications, or the inclusion of unfavorable information in our product labeling, such as limitations on the indicated uses or populations for which the products may be marketed or distributed, a label with significant safety warnings, including boxed warnings, contraindications, and precautions, a label without statements necessary or desirable for successful commercialization, or may result in requirements for costly post-marketing testing and surveillance, or other requirements, including REMS, to monitor the safety or efficacy of the products. These could in turn prevent us from commercializing our products or product candidates and generating revenues from their sale.

In addition, if we or others identify undesirable side effects caused by our product candidates after receipt of marketing approval, the regulatory authorities may require the addition of restrictive labeling statements. Regulatory authorities may withdraw their approval of the product. We also may be required to change the way the product is administered or conduct additional clinical trials. Any of these events could prevent us from achieving or maintaining market acceptance of the affected products or product candidate or could substantially increase the costs and expenses of commercializing the products or product candidate, which in turn could delay or prevent us from generating significant revenues from its sale or adversely affect our reputation.

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

We cannot commercialize a product until the appropriate regulatory authorities have reviewed and approved the product candidate. Even if our product candidates demonstrate safety and efficacy in clinical studies, the regulatory agencies 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 advisory group or 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 changes in regulatory agency policy during the period of product development, clinical studies, and the review process. Regulatory agencies also may approve a treatment candidate for fewer or more limited indications, populations, or uses than requested or may grant approval subject to the performance of post-marketing studies, surveillance, or other requirements. In addition, regulatory agencies may not approve the labeling claims that are necessary or desirable for the successful commercialization of our treatment candidates, or may require significant safety warnings, including black box warnings, contraindications, and precautions. For example, the development of our product candidates for pediatric use is an important part of our current business strategy, and if we are unable to obtain regulatory approval for the desired age ranges, our business may suffer.

We have received and may apply for additional designations intended to facilitate or encourage product candidate development. We may not receive any such designations or be able to maintain them. Moreover, any such designations may not lead to faster development or regulatory review or approval and it does not increase the likelihood that our product candidates will receive marketing approval.

Our product candidates have received regulatory designations including breakthrough therapy designation, RMAT designation, fast track designation, and rare pediatric disease designation from the FDA. In the future and as appropriate, we may seek additional product designations. Receipt of such a designation is within the discretion of the FDA. Even if we believe one of our product candidates meets the criteria for a designation, the FDA may disagree. In any event, the receipt of such a designation for a product candidate may not result in a faster development process, review, or approval compared to product candidates considered for approval under conventional FDA procedures and does not assure ultimate marketing approval by the FDA. In addition, the FDA may later decide that the product candidates no longer meet the designation conditions, in which case any granted designations may be revoked. Finally, specifically with respect to our rare pediatric disease designations, if we are not able to obtain FDA approval of our designated product candidates before the statute sunsets, we would not be eligible to receive priority review vouchers.

There is no guarantee that we will be able to obtain or maintain orphan drug designation for our product candidates or receive or maintain any corresponding benefits, including periods of exclusivity.

While orphan drug designation provides certain advantages, it neither shortens the development time nor the regulatory review time of a product candidate nor gives the product candidate any advantage in the regulatory review or approval process. Generally, if a product candidate with orphan drug designation subsequently receives marketing approval before another product considered by the FDA or comparable foreign regulatory authorities to be the same, for the same orphan indication, the product is entitled to a period of marketing exclusivity, which precludes the FDA or comparable foreign regulatory authorities from approving another marketing application for the same drug or biologic for the same indication for seven years. We may not be able to obtain any future orphan drug designations that we apply for, orphan drug designations do not guarantee that we will be able to successfully develop our product candidates, and there is no guarantee that we will be able to maintain any orphan drug designations that we receive. For instance, orphan drug designation may be revoked if the FDA finds that the request for designation contained an untrue statement of material fact or omitted material information, or if the FDA finds that the product candidate was not eligible for designation at the time of the submission of the request. Moreover, we may ultimately not receive any period of regulatory exclusivity if our product candidates are approved. For instance, we may not receive orphan product regulatory exclusivity if the indication for which we receive FDA approval is broader than the designation. Orphan exclusivity may also be lost for the same reasons that the designation may be lost. Orphan exclusivity may further be lost if we are unable to assure a sufficient quantity of the product to meet the needs of patients with the rare disease or condition.

Even if we obtain orphan exclusivity for any of our current or future product candidates, that exclusivity may not effectively protect the product from competition as different products can be approved for the same condition or products that are the same as ours can be approved for different conditions. Even after an orphan product is approved, the FDA or comparable foreign regulatory authorities can also subsequently approve a product containing the same principal molecular features for the same condition if the FDA concludes that the later product is clinically superior. The FDA may further grant orphan drug designation to multiple sponsors for the same compound or active molecule and for the same indication. If another sponsor receives FDA or comparable foreign regulatory authority approval for such product before we do, we would be prevented from launching our product for the orphan indication for a period of at least seven years unless we can demonstrate clinical superiority. The FDA's thinking around sameness with respect to gene therapies, and thus the circumstances when clinical superiority would need to be shown, is evolving. While the agency has issued guidance on the topic, certain decisions may need to be made on a case by case basis, given the novelty of the technology. Moreover, third-party payors may reimburse for products off-label even if not indicated for the orphan condition.

Even if we obtain regulatory approval for a product candidate, our products will remain subject to regulatory scrutiny.

Even if we obtain regulatory approval in a jurisdiction, regulatory authorities may still impose significant restrictions on the indicated uses or marketing of our product candidates or impose ongoing requirements for potentially costly post-approval studies, post-market surveillance or patient or drug restrictions. Moreover, the FDA and comparable foreign regulatory authorities will continue to closely monitor the safety profile of any product even after approval, including gene therapy specific requirements for long term follow up. Additionally, the holder of an approved BLA is obligated to monitor and report adverse events and any failure of a product to meet the specifications in the BLA. The holder of an approved BLA must also submit new or supplemental applications and obtain FDA approval for certain changes to the approved product, product labeling or manufacturing process. Advertising and promotional materials must comply with FDA rules and are subject to FDA review, in addition to other potentially applicable federal and state laws.

In addition, product manufacturers and their facilities are subject to payment of user fees and continual review and periodic inspections by the FDA and other regulatory authorities for compliance with cGMP and adherence to commitments made in the BLA. If we or a regulatory agency discovers previously unknown problems with a product, such as adverse events of unanticipated severity or frequency, or that the product is less effective than previously thought, or problems with the facility where the product is manufactured, a regulatory agency may impose restrictions relative to that product or the manufacturing facility, including requiring recall or withdrawal of the product from the market or suspension of manufacturing.

If we fail to comply with applicable regulatory requirements following approval of any of our product candidates or during product development, or if we later discovery previously unknown safety, efficacy, or manufacturing issues, the following may result:

- restrictions on manufacturing, distribution, marketing, or labeling of such products, including restrictions on the indication or approved patient population, and required additional warnings, such as black box warnings, contraindications, and precautions;
- requirements to conduct post-marketing studies or clinical trials, or to institute risk mitigation strategies, such as REMS;
- issuance of corrective information;
- the product may become less competitive, we may face reputational harm, or we may face liability for any harm caused to patients or subjects;

- modifications on the way the product is administered;
- modifications on promotional pieces;
- issuance of warning, untitled, or cyber letters asserting that we are in violation of the law, or of safety alerts, Dear Healthcare Provider letters, press releases, or other communications containing warnings or other safety information about the product;
- injunction or imposition civil or criminal penalties or monetary fines, restitution, or disgorgement of profits or revenues;
- suspension or withdrawal of regulatory approval;
- suspension or termination of any ongoing clinical studies;
- refusal to approve a pending marketing application, such as a BLA or supplements to a BLA submitted by us;
- seizure, detention, or recall of product;
- refusal to permit the import or export of our products; or
- refusal to allow us to enter into supply contracts, including government contracts, exclusion from federal healthcare programs, FDA debarment, consent decrees, or corporate integrity agreements.

Any government investigation of alleged violations of law could require us to expend significant time and resources in response and could generate negative publicity. The occurrence of any event or penalty described above may inhibit our ability to commercialize our product candidates and generate revenues.

The FDA's policies may change, and additional government regulations may be enacted, that could prevent, limit or delay regulatory approval of our product candidates, that could limit the marketability of our product candidates, or that could impose additional regulatory obligations on us. For example, a change in administration in the U.S. may result in new, revised, postponed or frozen regulatory requirements and associated compliance obligations. Changes in medical practice and standard of care may also impact the marketability of our product candidates. If we are slow or unable to adapt to changes in existing requirements, standards of care, or the adoption of new requirements or policies, or if we are not able to maintain regulatory compliance, we may lose any marketing approval that we may have obtained and be subject to regulatory enforcement action.

Should any of the above actions take place, they could adversely affect our ability to achieve or sustain profitability. Further, the cost of compliance with post-approval regulations may have a negative effect on our operating results and financial condition.

Disruptions at FDA and other government agencies, such as those that may be caused by funding shortages, could hinder their ability to hire, retain or deploy key leadership and other personnel, or otherwise prevent new or modified products from being developed, approved, or commercialized in a timely manner or at all, which could negatively impact our business.

The ability of FDA to review and approve new products can be affected by a variety of factors, including government budget and funding levels, statutory, regulatory, and policy changes, FDA's ability to hire and retain key personnel and accept the payment of user fees, and other events that may otherwise affect FDA's ability to perform routine functions. Average review times at the agency have fluctuated in recent years as a result. Disruptions at FDA and other agencies may also increase the time necessary to meet with and provide feedback to entities developing drug products, review and/or approve our submissions, conduct inspections, issue regulatory guidance, or otherwise authorize our actions requiring regulatory approval, which would adversely affect our business. In addition, government funding of FDA and other government agencies on which our operations may rely, including those that fund research and development activities, is subject to the political process, which is inherently fluid and unpredictable. For example, the executive branch recently established the Department of Government Efficiency, which implemented a federal government hiring freeze and large-scale layoffs of current federal employees and also announced additional efforts to reduce federal employee headcount and the size of the federal government.

It is unclear how these executive actions or other potential actions by the executive branch will have an impact on the regulatory authorities that oversee our business. These budgetary pressures may reduce FDA's ability to perform its responsibilities. If a significant reorganization or reduction in FDA's workforce occurs, FDA's budget is significantly reduced, or there are other disruptions at FDA and other agencies, more time may be necessary for biological products, or biologics, or modifications to approved biologics to be reviewed and/or approved by necessary government agencies, which could increase our costs and would adversely affect our business. In addition, if the current government shutdown continues, it could significantly impact the ability of FDA to timely review and process our regulatory submissions, which could have a material adverse effect on our business. For example, over the last several years, the United States government has shut down several times and certain regulatory agencies, such as FDA, have had to furlough critical employees and stop critical activities. Additionally, Congress may introduce and ultimately pass healthcare-related legislation that could impact the drug approval process.

Risks related to our reliance on third-parties

We expect to rely on third parties to conduct some or all aspects of our viral vector production, drug product manufacturing, research and preclinical, and clinical testing, and these third parties may not perform satisfactorily.

We do not expect to independently conduct all aspects of our viral vector production, drug product manufacturing and distribution, research and preclinical, and clinical testing. We currently rely, and expect to continue to rely, on third parties with respect to these matters. In some cases, these third parties are academic, research or similar institutions that may not apply the same quality control protocols utilized in certain commercial settings.

Our reliance on these third parties for research and development activities reduces our control over these activities but does not relieve us of our responsibility to ensure compliance with all required regulations and study protocols. For example, for product candidates that we develop and commercialize on our own, we remain responsible for ensuring that each of our IND-enabling studies and clinical studies are conducted in accordance with the study plan and protocols, and that our viral vectors and drug products are manufactured in accordance with GMP as applied in the relevant jurisdictions. We must also ensure that our preclinical trials are conducted in accordance with GLPs, as appropriate. Moreover, the FDA and comparable foreign regulatory authorities require us to comply with GCPs for conducting, recording, and reporting the results of clinical trials to assure that data and reported results are credible and accurate and that the rights, integrity, and confidentiality of trial participants are protected. Regulatory authorities enforce these requirements through periodic inspections. If we or any of our third-party service providers fail to comply with applicable regulatory requirements, we or they may be subject to enforcement or other legal actions, the data generated in our trials or manufacturing development may be deemed unreliable, and the FDA or comparable foreign regulatory authorities may require us to perform additional studies and manufacturing development. If these third parties do not successfully carry out their contractual duties, meet expected deadlines, conduct our studies in accordance with regulatory requirements or our stated study plans and protocols, or manufacture our viral vectors and drug products in accordance with cGMP, or if they need to be replaced or if the quality or accuracy of the data they obtain is compromised due to the failure to adhere to our protocols, regulatory requirements or for other reasons, we will not be able to complete, or may be delayed in completing, the preclinical and clinical studies and manufacturing process validation activities required to support future IND, MAA and BLA submissions and approval of our product candidates.

Any of these third parties may terminate their engagements with us at any time. If we need to enter into alternative arrangements, it could delay our product development activities. Any of these events could lead to clinical study delays or failure to obtain regulatory approval or impact our ability to successfully commercialize future products. Some of these events could be the basis for FDA action, including injunction, recall, seizure or total or partial suspension of production.

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 to manufacture our vectors and our product candidates, and because we collaborate with various organizations and academic institutions on the advancement of our cell and gene therapy platform, 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, collaborative research agreements, consulting agreements or other similar agreements with our collaborators, advisors, employees, 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, such as trade secrets. Despite the contractual provisions employed when working with third parties, the need to share trade secrets and other confidential information increases the risk that such trade secrets become known by our competitors, are inadvertently incorporated into the technology of others, or are disclosed or used in violation of these agreements. Given that our proprietary position is based, in part, on our know-how and trade secrets, a competitor's discovery of our trade secrets or other unauthorized use or disclosure would impair our competitive position and may have a material adverse effect on our business.

In addition, these agreements typically restrict the ability of our collaborators, advisors, employees, and consultants to publish data potentially relating to our trade secrets. Our academic collaborators typically have rights to publish data, provided that we are notified in advance and may delay publication for a specified time in order to secure our intellectual property rights arising from the collaboration. In other cases, publication rights are controlled exclusively by us, although in some cases we may share these rights with other parties. We also conduct joint research and development programs that may require us to share trade secrets under the terms of our research and development partnerships or similar agreements. Despite our efforts to protect our trade secrets, our competitors may discover our trade secrets, either through breach of these agreements, independent development or publication of information including our trade secrets in cases where we do not have proprietary or otherwise protected rights at the time of publication. A competitor's discovery of our trade secrets would impair our competitive position and have an adverse impact on our business.

Risks related to our intellectual property

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

We rely 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 product candidates. These and other licenses may not provide exclusive rights to use such intellectual property and technology in all relevant fields of use and in all territories in which we may wish to develop or commercialize our technology and products in the future. As a result, we may not be able to prevent competitors from developing and commercializing competitive products in territories included in all of our licenses. These licenses may also require us to grant back certain rights to licensors and to pay certain amounts relating to sublicensing patent and other rights under the agreement.

In some circumstances, particularly in-licenses with academic institutions, we may not have the right to control the preparation, filing and prosecution of patent applications, or to maintain the patents, covering in-licensed technologies. Therefore, in those cases we cannot be certain that these patents and applications will be prosecuted, maintained and enforced in a manner consistent with the best interests of our business. 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 adversely affected. In certain circumstances, we have or may license technology from third parties on a non-exclusive basis. In such instances, other licensees may have the right to enforce our licensed patents in their respective fields, without our oversight or control. Those other licensees may choose to enforce our licensed patents in a way that harms our interest, for example, by advocating for claim interpretations or agreeing on invalidity positions that conflict with our positions or our interest. In addition to the foregoing, the risks associated with patent rights that we license from third parties will also apply to patent rights we may own in the future.

Further, in many of our license agreements we are responsible for bringing any actions against any third party for infringing the patents we have licensed. Certain of our license agreements also require us to meet development milestones to maintain the license, including establishing a set timeline for developing and commercializing products and minimum yearly 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 intellectual property rights of the licensor that are not subject to the licensing agreement;
- the sublicensing of patent and other rights under our collaborative development relationships;
- our diligence obligations under the license agreement and what activities satisfy those diligence obligations;
- the inventorship 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 any dispute over in-licensed intellectual property prevents or impairs our ability to maintain our current licensing arrangements on acceptable terms, we may be unable to successfully develop and commercialize the affected product candidates.

If we fail to comply with our obligations under these license 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 develop, manufacture, or market products covered by the license or may face other penalties under the agreements. Termination of these agreements or reduction or elimination of our rights under these agreements may result in our having to negotiate new or reinstated agreements with less favorable terms or cause us to lose our rights under these agreements, including our rights to important intellectual property or technology. It is possible that such termination may occur even if we believe that we have complied with our obligations under a license agreement, if a dispute arises between us and a licensor.

Furthermore, to the extent that the research resulting in certain of our licensed patent rights and technology was funded by the U.S. government, the government may have certain rights, or march-in rights, to such patent rights and technology. When new technologies are developed with U.S. government funding, the U.S. government generally obtains certain rights in any resulting patents, including a non-exclusive, royalty-free license authorizing the U.S. government, or a third party on its behalf, 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 U.S. 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, or a third party on its behalf, of such rights could harm our competitive position, business, financial condition, cash flow, results of operations and prospects.

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

Our success depends, in large part, on our and our licensors' ability to obtain and maintain patent protection in the United States and other countries with respect to our proprietary product candidates and manufacturing technology. We and our licensors have sought, and we intend to seek in the future, to protect our proprietary positions 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 have and may not in the future be able to file, prosecute, maintain, enforce, or license all necessary or desirable patent applications at a reasonable cost or in a timely manner. For example, in some cases, the work of certain academic researchers in the gene therapy field has entered the public domain, which may compromise our ability to obtain patent protection for certain inventions related to or building upon such prior work. Consequently, we will not be able to obtain any such patents to prevent others from using our technology for, and developing and marketing competing products to treat, these indications. 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.

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 and our licensors' patent rights are highly uncertain. Our pending and future patent applications may not result in patents being issued which protect our technology or product candidates or which effectively prevent others from commercializing competitive technologies and product candidates. In particular, during prosecution of any patent application, the issuance of any patents based on the application may depend upon our ability to generate additional preclinical or clinical data that support the patentability of our proposed claims. We may not be able to generate sufficient additional data on a timely basis, or at all. 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 and our licensors' patent protection.

We may not be aware of all third-party intellectual property rights potentially relating to our 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 whether 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. Databases for patents and publications, and methods for searching them, are inherently limited, so it is not practical to review and know the full scope of all issued and pending patent applications. As a result, the issuance, scope, validity, enforceability, and commercial value of our and our licensed patent rights are uncertain.

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 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 or increase our financial or other obligations to our licensors.

The agreements under which we currently license intellectual property or technology from third parties are complex, and certain provisions in such agreements may be susceptible to multiple interpretations. The resolution of any contract interpretation disagreement that may arise could narrow what we believe to be the scope of our rights to the relevant intellectual property or technology or increase what we believe to be our financial or other obligations under the relevant agreement, either of which could harm our business, financial condition, cash flow, results of operations and prospects.

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

We currently have rights to certain intellectual property, through licenses from third parties, to develop our product candidates. Because our programs may require the use of proprietary rights held by third parties, the growth of our business likely will depend, in part, on our ability to acquire, in-license or use these proprietary rights. We may be unable to acquire or in-license any compositions, methods of use, processes, or other intellectual property rights from third parties that we identify as necessary for our 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 assign or license rights to us. We also may be unable to license or acquire third-party intellectual property rights on terms that would allow us to make an appropriate return on our investment.

We sometimes collaborate with non-profit and academic institutions to accelerate our preclinical research or development under written agreements with these institutions. Typically, these institutions 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 an 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 develop 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 be required to expend significant time and resources to redesign our product candidates or the methods for manufacturing them or to develop or license replacement technology, all of which may not be feasible on a technical or commercial basis. If we are unable to do so, we may be unable to develop or commercialize the affected product candidates, which could harm our business significantly.

Issued patents covering 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 we or one of our licensing partners initiate legal proceedings against a third party to enforce a patent covering one of our product candidates, the defendant could counterclaim that the patent covering our product 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 lack of novelty, obviousness, lack of written description or non-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 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 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 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. 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, collaborators, contractors, and other third-parties. 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 harm our business.

Our commercial success depends upon our ability and the ability of our collaborators to develop, manufacture, market and sell our product candidates and use our proprietary technologies without infringing 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 become party to, or threatened with, infringement litigation claims regarding our product candidates and technology, including claims from competitors or from non-practicing entities that have no relevant product revenue and against whom our own patent portfolio may have no deterrent effect. Moreover, we may 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 or derivation proceedings, post grant review and *inter partes* review before the USPTO or foreign patent offices. 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 adversely affect our ability to commercialize our product candidates or any other of our product candidates or technologies covered by the asserted third-party patents.

To successfully challenge the validity of any such U.S. patent in federal court, we would need to overcome a statutory presumption of validity. As this burden is a high one requiring us to prove by clear and convincing evidence 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. Similar challenges exist in other jurisdictions. 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 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 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 rights. A finding of infringement could prevent us from manufacturing and commercializing our product candidates or force us to cease some of our business operations, which could harm our business. In addition, we may be forced to redesign our product candidates, seek new regulatory approvals, and indemnify third parties pursuant to contractual agreements. Claims that we have misappropriated the confidential information or trade secrets of third parties could have a similar negative impact on our business, reputation, 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.

If we fail in prosecuting or 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 prosecuting or defending against such claims, litigation could result in substantial costs and be a distraction to management.

If we do not obtain patent term extension and data exclusivity for our product candidates, our business may be 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 ("PTE") under the Drug Price Competition and Patent Term Restoration Act of 1984 (the "Hatch-Waxman Amendments"). The Hatch-Waxman Amendments permit a PTE of up to five years as compensation for patent term lost during the FDA regulatory review process. PTE 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 per FDA-approved product, and only those claims covering the approved drug, a method for using it or a method for manufacturing it may be extended. Further, certain of our licenses currently or in the future may not provide us with the right to control decisions of the licensor or its other licensees with respect to PTE under the Hatch-Waxman Act. Thus, if one of our important licensed patents is eligible for PTE, and it covers a product of another licensee in addition to our own product candidate, we may not be able to obtain that extension if the other licensee seeks and obtains that extension first. Moreover, 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, or the applicable time-period or the scope of patent protection afforded during any such extension could be less than we request. If we are unable to obtain PTE or the duration of any such extension is less than we request, the period during which we will have the right to exclusively market our product may be shortened and our competitors may obtain approval of competing products following our patent expiration, and our revenue could be materially reduced.

Risks related to our financial condition and capital requirements

We have experienced a history of losses; we expect to incur future losses and we may be unable to obtain necessary additional capital to fund operations in the future.

We have recorded minimal revenue to date and have incurred an accumulated deficit of $742.1 million through December 31, 2025. The net income for the year ended December 31, 2025, was $71.2 million due to the gain on sale of our priority review voucher. Excluding that gain, our net loss for the year ended December 31, 2025 would have been $81.2 million. Our losses have resulted principally from costs incurred in research and development activities related to our efforts to develop clinical drug candidates and from the associated administrative costs.

We require substantial capital to commercialize ZEVASKYN®, for our development programs and operating expenses, to pursue regulatory clearances and to prosecute and defend our intellectual property rights. We expect to continue to incur significant expenses and increasing operating losses for the foreseeable future. We anticipate that our expenses will increase substantially if and as we:

- continue commercialization efforts for ZEVASKYN®;
- seek regulatory and marketing approvals for our product candidates that successfully complete clinical studies;
- continue our research and preclinical and clinical development of our product candidates;
- further develop the manufacturing process for our vectors or our product candidates;
- expand the scope of our current clinical studies for our product candidates;
- change or add additional manufacturers or suppliers;
- seek to identify and validate additional product candidates;
- acquire or in-license other product candidates and technologies;
- make milestone or other payments under any license agreements;
- maintain, protect and expand our intellectual property portfolio;
- establish a sales, marketing and distribution infrastructure in the United States and Europe to commercialize any products for which we may obtain marketing approval;
- attract and retain skilled personnel;
- build additional infrastructure to support our operations as a larger public company and our product development and planned future commercialization efforts, including manufacturing capacity; and
- experience any delays or encounter issues with any of the above.

The net losses we incur may fluctuate significantly from quarter to quarter and year to year, such that a period-to-period comparison of our results of operations may not be a good indication of our future performance. In any particular quarter or quarters, our operating results could be below the expectations of securities analysts or investors, which could cause our stock price to decline.

We do not have significant operating revenue and may never achieve profitability.

To date, we have funded our operations primarily through public offerings of our common stock. Our ability to achieve significant revenue or profitability depends upon our ability to commercialize ZEVASKYN® and complete the development of our drug candidates, and to develop and obtain patent protection and regulatory approvals for our drug candidates. We are not expecting any significant revenues in the short-term from our product candidates. Furthermore, we may not be able to ever successfully identify, develop, commercialize, patent, manufacture, obtain required regulatory approvals or market any products. Moreover, even if we do identify, develop, commercialize, patent, manufacture, or obtain required regulatory approvals to market additional products, we may not generate revenues or royalties from commercial sales of these products for a significant number of years, if at all. Therefore, our operations are subject to all the risks inherent in the establishment of a new business enterprise.

If the estimates we make, or the assumptions on which we rely, in preparing our consolidated financial statements are incorrect, our actual results may vary from those reflected in our projections and accruals.

Our consolidated financial statements have been prepared in accordance with U.S. GAAP. The preparation of these consolidated financial statements requires us to make estimates and judgments that affect the reported amounts of our assets, liabilities, revenues and expenses, and related disclosure of contingent assets and liabilities. We base our estimates on historical experience and on various other assumptions that we believe to be reasonable under the circumstances. However, we cannot assure you that our estimates, or the assumptions underlying them, will be correct. We may be incorrect in our assumptions regarding the applicability of drug pricing programs and rebates that may be applicable to ZEVASKYN® and future product candidates, which may result in our under- or over-estimating our anticipated product revenues, especially as applicable laws and regulations governing pricing evolve over time.

We may need to raise additional capital to operate our business, and our failure to obtain funding when needed or on terms that are favorable to us may force us to delay, reduce or eliminate our development programs or commercialization efforts.

We may need to raise additional capital to fund our future operations and we cannot be certain that funding will be available to us on acceptable terms on a timely basis, or at all. We expect to continue to spend substantial amounts on regulatory approval efforts, product development (including commercialization activities), and conducting potential future preclinical or clinical trials for our product candidates. Our ability to raise capital through the sale of securities may be limited by our number of authorized shares of common stock and various rules of the SEC and the Nasdaq that place limits on the number and dollar amount of securities that we may sell.

If we fail to raise additional funds on acceptable terms or at all, we may be unable to complete planned preclinical and clinical trials, obtain approval of our product candidates from the FDA and other regulatory authorities, or successfully commercialize any of our product candidates. In addition, we could be forced to delay, discontinue, or curtail product development, or forego licensing in attractive business opportunities. Any additional sources of financing will likely involve the issuance of our equity or debt securities, which will have a dilutive effect on our stockholders. Also, the terms of any financing may adversely affect the holdings or the rights of our stockholders and the issuance of additional securities, whether common stock, preferred stock or debt, by us, or the possibility of such issuance, may cause the market price of our shares to decline.

Further, if we are unable to obtain funding on a timely basis, we may be required to significantly curtail, delay, or discontinue one or more of our research or development programs or the commercialization of any product candidates or be unable to expand our operations or otherwise capitalize on our business opportunities, as desired, which could materially affect our business, financial condition, cash flow, and results of operations.

Failure to achieve and maintain effective internal controls could have a material adverse effect on our business.

Effective internal controls are necessary for us to provide reliable financial reports. If we cannot provide reliable financial reports, our operating results could be harmed. All internal control systems, no matter how well designed, have inherent limitations. Therefore, even those systems determined to be effective can provide only reasonable assurance with respect to financial statement preparation and presentation.

Any failure to implement required new or improved controls, or difficulties encountered in their implementation, could harm our operating results, or cause us to fail to meet our reporting obligations. Failure to achieve and maintain an effective internal control environment could cause investors to lose confidence in our reported financial information, which could have a material adverse effect on our stock price. Failure to comply with Section 404 of the Sarbanes-Oxley Act could also potentially subject us to actions or investigations by the SEC or other regulatory authorities.

Our ability to use our net operating loss carryforwards to offset future taxable income and taxes may be subject to certain limitations.

As of December 31, 2025, we had $310.7 million of U.S. federal net operating loss (“NOL”) carryforwards, $11.6 million of state NOL carryforwards, $5.2 million of general business credit carryforwards, and $0.1 million of state credits, which may be utilized against future federal and state income taxes. Of the federal NOLs, $308.1 million do not expire and may be carried forward indefinitely, subject to the limitation that they may offset no more than 80% of taxable income in any tax year. The remaining federal NOLs expire between 2026 and 2037. State NOL carryforwards have expiration periods that vary by jurisdiction based on applicable state tax laws. The federal general business credits begin to expire in 2043, and the state credits expire in 2026.

Generally, a change of more than 50% in the ownership of a company’s stock, by value, over a three-year period constitutes an ownership change for U.S. federal income tax purposes or applicable state tax law. An ownership change may limit our ability to use our NOL carryforwards attributable to the period prior to the change.

During the year ended December 31, 2025, we completed a Section 382 study to evaluate whether historical equity transactions resulted in an ownership change within the meaning of Section 382 of the Internal Revenue Code. Based on this analysis, we determined that there were multiple ownership changes. As a result, certain NOL carryforwards will not be realizable due to the Section 382 limitations. We had previously recorded a full valuation allowance against the deferred tax assets associated with these NOLs. Accordingly, the $96.6 million reduction in gross deferred tax assets resulting from the Section 382 analysis was fully offset by a corresponding reduction in the valuation allowance and did not affect income tax expense or net income for the year ended December 31, 2025.

If we experience any future ownership changes, we could be limited in our ability to use our NOLs and tax credits in future years in which we have taxable income, we will pay more taxes than if we were able to fully utilize our NOLs and tax credits, and we could be required to pay taxes earlier than we would otherwise be required, which could cause such NOLs to expire unused. This could adversely affect our results of operations.

General Risk Factors

The market price of our common stock may be volatile and adversely affected by several factors.

The market price of our common stock can fluctuate significantly in response to various factors and events, including:

- our ability to execute our business plan, including commercialize ZEVASKYN®;
- our ability to integrate operations, technology, products, and services;
- operating results below expectations;
- announcements concerning product development results, including clinical trial results;
- regulatory or legal developments in the U.S. or EU, including decisions from regulatory agencies relating to ZEVASKYN® or our product candidates;
- our failure to successfully manage and sustain the commercial launch of ZEVASKYN®, including failure to manage our supply chain operations in the coordination and delivery of drug product to patients at qualified treatment centers;
- litigation or public concern about the safety of ZEVASKYN® or our product candidates;
- our issuance of additional securities, including debt or equity or a combination thereof, which will be necessary to fund our operating expenses;
- announcements of technological innovations or new products by us or our competitors;
- loss of any strategic relationship;
- industry developments, including, without limitation, changes in healthcare policies or practices or third-party reimbursement policies;
- economic and other external factors; and
- period-to-period fluctuations in our financial results.

In addition, the securities markets have experienced significant price and volume fluctuations from time to time that are unrelated to the operating performance of particular companies. These market fluctuations may also materially and adversely affect the market price of our common stock.

Raising additional funds by issuing securities or through licensing or lending arrangements or through our at-the-market sale agreement would cause dilution to our existing stockholders, restrict our operations, or require us to relinquish proprietary rights.

If we raise additional capital by issuing equity securities, the share ownership of existing stockholders will be diluted. Meanwhile, any future debt financing may involve covenants that limit our ability to incur liens or additional debt, pay dividends, redeem, or repurchase our common stock, make certain investments or engage in certain merger, consolidation, or asset sale transactions. In addition, if we raise additional funds through licensing arrangements or the disposition of any of our assets, it may be necessary to relinquish potentially valuable rights to our product candidates or grant licenses on terms that are not favorable to us.

The terms of any financing may adversely affect the holdings or the rights of our stockholders and the issuance of additional securities, whether equity or debt, or the possibility of such issuance, may cause the market price of our shares to decline. We may sell shares or other securities in other offerings, including under our open market sale agreement, at a price per share that is less than the prices per share paid by other investors, and investors purchasing shares of our common stock, preferred stock or other securities in the future could have rights superior to existing stockholders. The sale of additional equity or convertible securities would dilute all of our stockholders and the terms of these securities may include liquidation or other preferences that adversely affect our existing stockholders.

Actual or potential sales of our common stock by our employees, including our executive officers, pursuant to pre-arranged stock trading plans could cause our stock price to fall or prevent it from increasing for numerous reasons, and actual or potential sales by such persons could be viewed negatively by other investors.

In accordance with the guidelines specified under Rule 10b5-1 of the Securities Exchange Act of 1934, as amended, and our policies regarding stock transactions, a number of our employees, including executive officers and members of our board of directors, have adopted and may continue to adopt stock trading plans pursuant to which they have arranged to sell shares of our common stock from time to time in the future. Generally, sales under such plans by our executive officers and directors require public filings. Actual or potential sales of our common stock by such persons could cause the price of our common stock to fall or prevent it from increasing for numerous reasons.

Significant disruptions of information technology ("IT") systems, breaches of data security, or unauthorized disclosures of personal information (including sensitive personal information) could adversely affect our business and could subject us to liability or reputational damage.

We operate information systems that contain limited amounts of client data. As a routine element of our business, we collect, analyze, and retain data pertaining to the clinical trials we conduct for our products. Unauthorized third parties could attempt to gain entry to such information systems to steal data or disrupt the systems or for financial gain. Like other companies we may experience threats and incursions to our data and systems, including malicious software and viruses, phishing, business email compromise and social engineering attacks or other cyber-attacks. The number and complexity of these threats continue to increase over time.

We have implemented and maintain security systems measures and safeguards, which we believe to be reasonable, to protect our information systems and confidential information, including personal information, and that of our customers, clients and suppliers that is held or processed by us, against unauthorized access or disclosure and to prevent, detect, contain, respond to, and mitigate security-related threats and potential incidents. We undertake ongoing improvements to the security of our systems, connected devices, and information-sharing products in order to minimize potential vulnerabilities, in accordance with industry and regulatory standards. Despite such efforts, our safeguards may fail, or we may be subject to breaches of our security resulting in unauthorized access to our facilities or information systems and the information we are trying to protect. Moreover, our business or operations may be affected in the event our customers, clients and suppliers experience data security incidents, cyber-attacks or extended interruptions of their services or systems.

We are continuously evaluating and, where appropriate, enhancing our IT systems to address our planned growth, including to support our planned manufacturing operations. There are inherent costs and risks associated with implementing the enhancements to our IT systems, including potential delays in access to, or errors in, critical business and financial information, substantial capital expenditures, additional administrative time and operating expenses, retention of sufficiently skilled personnel to implement and operate the enhanced systems, demands on management time, and costs of delays or difficulties in transitioning to the enhanced systems, any of which could harm our business and results of operations. In addition, the implementation of enhancements to our IT systems may not result in productivity improvements at a level that outweighs the costs of implementation, or at all.

While we do not believe cybersecurity incidents have resulted in any material impact on our business, operations or financial results or our ability to service our customers or run our business, past and future incidents resulting in unauthorized access to our facilities or information systems, or those of our suppliers, or accidental loss or disclosure of proprietary or confidential information about us, our clients or our customers could result in, among other things, a total shutdown of our systems that would disrupt our ability to conduct business or pay vendors and employees, violations of applicable privacy and other laws, significant legal and financial exposure, damage to our reputation, and a loss of investor confidence in our security measures. Additional impacts from cybersecurity incidents could include remediation costs to our customers or business partners, such as liability for stolen assets or information, repairs of system damage, and incentives for continued business; increased cybersecurity protection costs, which may include the costs of making organizational changes, deploying additional personnel, resources and security technologies, training employees, and engaging third-party experts and consultants; lost revenue resulting from the unauthorized use of proprietary information or the failure to retain or attract business partners following an incident; increased insurance premiums; and damage to the Company's competitiveness, stock price, and long-term shareholder value. In addition, cybersecurity risks and data security incidents could lead to unfavorable publicity, governmental inquiry and oversight, regulatory actions by federal, state and non-U.S. governmental authorities, litigation by affected parties and possible financial obligations for damages related to the theft or misuse of such information, any of which could have a material adverse effect on our profitability and cash flow.

For information regarding our processes and practices related to information and cybersecurity, please see Item 1C of this report, "Cybersecurity".

ITEM 1B. UNRESOLVED STAFF COMMENTS

Not Applicable.

ITEM 1C. Cybersecurity

Cybersecurity Management and Strategy

In the ordinary course of our business, we collect, use, store, and transmit confidential, financial, sensitive, proprietary, personal, and health-related information. The secure maintenance of this information and our information technology systems is important to our operations and business strategy. To this end, we consider cybersecurity, along with other significant risks that we face, within our overall enterprise risk management framework, and have implemented processes designed to assess, identify, and manage risks from potential unauthorized occurrences on or through our information technology systems that may result in adverse effects on the confidentiality, integrity, and availability of these systems and the data residing therein. These processes are managed and monitored by a dedicated Director of Information Technology and an Information Technology Security and Risk Manager. We have developed a cybersecurity program following the National Institute of Standards and Technology cybersecurity framework that includes mechanisms, controls, technologies, and systems designed to prevent or mitigate data loss, theft, misuse, or other security incidents or vulnerabilities affecting the data and maintain a stable information technology environment. For example, we conduct penetration and vulnerability testing, and data recovery testing on a periodic basis. In addition, we consult with outside advisors and experts, when appropriate, to assist with assessing, identifying, and managing cybersecurity risks, including to anticipate future threats and trends, and their impact on the Company's risk environment.

Third-Party Risk Management

We have processes to evaluate third-party service providers and vendors that have access to sensitive systems and company data, which may include due diligence procedures such as assessments of that service provider's cybersecurity posture or a recommendation of specific mitigation controls. Following an assessment, we determine and prioritize service provider risk based on potential threat impact and likelihood, and such risk determinations drive the level of due diligence and ongoing compliance monitoring required for each service provider.

Education and Awareness

We also provide cybersecurity training to our employees and are formalizing an ongoing information security training program for active employees and relevant consultants to address matters such as phishing, email security, social engineering and training on data privacy.

Governance

Our Director of Information Technology, who reports to our CFO, and the Information Technology Security and Risk Manager are responsible for assessing and managing cybersecurity risks. Our Director of Information Technology has over 25 years of experience managing information technology and cybersecurity. He has a bachelor's degree in electrical engineering from Wright State University as well as a master's degree in business administration from Ashland University. He has certifications from various information technology vendors as well as experience in implementing security frameworks such as International Organization for Standardization ("ISO") 27001 and the National Institute of Standards and Technology. Our Information Technology Security and Risk Manager has a PhD in a scientific field and various information security certifications such as Certified Ethical Hacker and Holistic Information Security Practitioner. She also has decades of experience in managing information technology environments and information security such as security architecture, security operations and governance risk and compliance.

We report on our information security program, including the results of periodic testing, to the Audit Committee of the Board of Directors on a quarterly basis. Our Board's Audit Committee is responsible for overseeing our cybersecurity and information security procedures. The Audit Committee reviews management presentations concerning cybersecurity-related issues, including information security, technology risks, policies, and risk mitigation programs. The Audit Committee reports matters to the Board of Directors as needed. Our CFO, with the support of our Director of Information Technology, Information Technology Security and Risk Manager and third-party consultants, assesses and manages cybersecurity risk, including preventing, mitigating, detecting, and addressing cybersecurity incidents, if any. Our CFO also works closely with other management positions and external legal counsel to ensure that we understand our cybersecurity risk management responsibilities. In case of a cybersecurity incident or breach, our incident response plan defines in detail reporting and escalation processes to management and the Board of Directors.

Current Cybersecurity Risk Posture

We have not identified any cybersecurity incidents or threats that have materially affected us or are reasonably likely to materially affect us. However, like other companies in our industry, we and our third-party vendors have from time-to-time experienced threats to and security incidents relating to information systems. Additional information on cybersecurity risks we face is discussed in Part I, Item 1A, "Risk Factors."

ITEM 2. PROPERTIES

Our corporate headquarters are located in Cleveland, Ohio, where we currently lease approximately 73,100 square feet of manufacturing, laboratory and office space. Those leases expire in December 2030. We leased 10,400 square feet of office space located in New York, New York. That lease expired in September 2025.

We believe that our facilities are sufficient to meet our current needs and that suitable space will be available as and when needed for potential future expansion.

ITEM 3. LEGAL PROCEEDINGS

We are not currently subject to any material pending legal proceedings.

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

Our common stock has traded on the Nasdaq Capital Market ("Nasdaq") under the symbol "ABEO" since June 22, 2015.

The number of record holders of our common stock as of March 11, 2026 was 312.

Dividend Policy

We have never declared or paid any cash dividends on our common stock, and we do not anticipate paying any cash dividends on our common stock in the foreseeable future. The payment of dividends, if any, in the future is within the discretion of our Board of Directors and will depend on our earnings, capital requirements and financial condition and other relevant facts. We currently intend to retain all future earnings, if any, to finance the development and growth of our business.

Recent Sales of Unregistered Securities

None.

Issuer Repurchases of Equity Securities

None.

ITEM 6. [RESERVED]

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

You should read the following discussion and analysis together with our consolidated financial statements and related notes included in this Form 10-K. This discussion and analysis contains forward-looking statements, which involve risks and uncertainties. As a result of many factors, such as those described under "Forward-Looking Statements," "Risk Factors" and elsewhere in this Form 10-K, our actual results may differ materially from those anticipated in these forward-looking statements.

OVERVIEW

We are a commercial-stage biopharmaceutical company developing cell and gene therapies for life-threatening diseases. On April 28, 2025, the FDA approved ZEVASKYN® (prademagene zamikeracel) gene-modified cellular sheets, also known as ZEVASKYN®, as the first and only autologous cell-based gene therapy for the treatment of wounds in adult and pediatric patients with RDEB, a serious and debilitating genetic skin disease. There is no cure for RDEB, and ZEVASKYN® is the only FDA-approved product to treat RDEB wounds with a single application. ZEVASKYN® was granted Orphan Drug and Rare Pediatric Disease designations by the FDA.

ZEVASKYN® is manufactured at our current cGMP manufacturing facility in Cleveland, Ohio, and is made available through ZEVASKYN® qualified treatment centers.

Our development portfolio also features adeno-associated virus ("AAV") based gene therapies designed to treat ophthalmic diseases with high unmet need using novel AIM™ capsids. Abeona's novel, next-generation AAV capsids are being evaluated to improve tropism profiles for a variety of devastating diseases.

Preclinical Pipeline

Our preclinical programs are investigating the use of novel AAV capsids in AAV-based therapies for serious genetic eye diseases, including ABO-504 for Stargardt disease, ABO-503 for X-linked retinoschisis ("XLRS") and ABO-505 for autosomal dominant optic atrophy ("ADOA"). We completed pre-Investigational New Drug Application ("pre-IND") meetings with the FDA regarding the preclinical development plans and regulatory requirements to support first-in-human trials.

Recent Developments

Since we resumed manufacturing operations in mid-January after a planned facility shutdown, a patient treatment has been completed, multiple biopsies have been collected for scheduled ZEVASKYN® treatments in the coming weeks, and additional biopsies are scheduled.

RESULTS OF OPERATIONS

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

	For the year ended December 31,		Change	
($ in thousands)	**2025**	**2024**	**$**	**%**
Revenues:				
Product revenue, net	$ 2,420	$ —	$ 2,420	100%
License and other revenues	3,400	—	3,400	100%
Total revenues	5,820	—	5,820	100%
Costs and expenses:				
Cost of sales	1,532	—	1,532	100%
Royalties	1,893	—	1,893	100%
Research and development	26,812	34,360	(7,548)	(22)%
Selling, general and administrative	65,031	29,851	35,180	118%
Total costs and expenses	95,268	64,211	31,057	48%
Loss from operations	(89,448)	(64,211)	(25,237)	39%
Interest income	5,556	4,246	1,310	31%
Interest expense	(3,740)	(4,208)	468	(11)%
Change in fair value of warrant and derivative liabilities	6,139	(755)	6,894	(913)%
Gain from sale of priority review voucher, net	152,366	—	152,366	100%
Other income, net	410	1,194	(784)	(66)%
Income (loss) before income taxes	71,283	(63,734)	135,017	(212)%
Income tax expense	100	—	100	100%
Net income (loss)	$ 71,183	$ (63,734)	$ 134,917	(212)%

Product revenue, net

On April 28, 2025, the FDA approved ZEVASKYN® as the first and only autologous cell-based gene therapy for the treatment of wounds in adult and pediatric patients with RDEB. Product revenue, net, resulting from the sale of ZEVASKYN®, for the year ended December 31, 2025 was $2.4 million. On December 8, 2025, we announced the first commercial patient treatment with FDA-approved ZEVASKYN® at Lucile Packard Children's Hospital Stanford in Palo Alto, CA. There was no product revenue for the year ended December 31, 2024 as the approval by the FDA for ZEVASKYN® did not occur until 2025.

License and other revenues

License and other revenues for the year ended December 31, 2025 was $3.4 million as compared to nil for the same period of 2024. The revenue in 2025 consists primarily of revenue resulting from achieving a clinical development milestone under a sublicense agreement we entered into with Taysha in October 2020 relating to an investigational AAV-based gene therapy for Rett syndrome. Additionally in 2025, we also recorded $0.4 million resulting from a third party exercising its option to license certain of our AAV capsids. There was no license or other revenue in 2024 as no clinical development milestones were met in 2024.

Cost of sales

Cost of sales during the year ended December 31, 2025 was $1.5 million and primarily includes costs associated with the first commercial patient treatment with FDA-approved ZEVASKYN® in December of 2025 and costs associated with the August 2025 production of a full batch of ZEVASKYN® that could not be released due to technical issues that arose in implementing the rapid sterility lot release assay that was mandated by the FDA during BLA review. There was no cost of sales in the same period of 2024, as ZEVASKYN® was approved by the FDA in April 2025.

Royalties

Total royalty expenses were $1.9 million for the year ended December 31, 2025, as compared to nil for the same period of 2024. The increase in was primarily due to royalties owed to our licensors resulting from the milestone due from Taysha related to Rett syndrome.

Research and development

Research and development expenses include, but are not limited to, payroll and personnel expenses, preclinical lab supplies, preclinical and development costs, clinical trial costs, preclinical manufacturing and manufacturing facility costs, costs associated with regulatory approvals, preclinical depreciation on lab supplies and manufacturing facilities, and preclinical consultant-related expenses.

Total research and development spending for the year ended December 31, 2025 was $26.8 million, as compared to $34.4 million for the same period of 2024, a decrease of $7.6 million. The reduction in expenses was primarily due to costs capitalized into inventory and engineering runs and other production costs that are no longer considered research and development due to FDA approval of ZEVASKYN® in April of 2025.

We expect our research and development activities to continue as we work towards advancing our product candidates towards potential regulatory approval, reflecting costs associated with the following:

- employee and consultant-related expenses;
- preclinical and developmental costs;
- clinical trial costs;
- the cost of acquiring and manufacturing clinical trial materials; and
- costs associated with regulatory approvals.

Selling, general and administrative

Selling, general and administrative expenses primarily consist of payroll and personnel costs, office facility costs, public reporting company related costs, professional fees (e.g., legal expenses), selling and other costs for commercial launch and other general operating expenses not otherwise included in research and development expenses. We expect our selling, general, and administrative costs to continue to increase as we expand our commercialization of ZEVASKYN® and advance other product candidates toward potential regulatory approval.

Total selling, general and administrative expenses were $65.0 million for the year ended December 31, 2025, as compared to $29.9 million for the same period of 2024, an increase of $35.1 million. The increase in expenses was primarily due to increases in commercial costs of $2.3 million, related to our continued commercialization efforts, increases in salaries and stock-based compensation of $18.6 million due to new hires, and $4.8 million of costs related to engineering runs with the remainder due to other commercial costs upon FDA approval in April of 2025.

Interest income

Interest income was $5.6 million for the year ended December 31, 2025, as compared to $4.2 million in the same period of 2024. The increase resulted from higher earnings on short-term investments driven by increased average short-term investment balances.

Interest expense

Interest expense was $3.7 million for the year ended December 31, 2025, as compared to $4.2 million in the same period of 2024. Interest expense was due to the credit facility we entered into in January 2024 and decreased as a result of the July 2025 amendment to the credit facility reducing the interest rate for the senior secured term loan thereunder from 13.5% to 11.75%.

Change in fair value of warrant and derivative liabilities

We issued stock purchase warrants that are required to be classified as a liability and valued at fair market value at each reporting period. In addition, the conversion feature in our loan agreement is required to be classified as a liability and valued at fair market value at each reporting period.

The change in fair value of warrant liabilities resulted in a gain of $6.1 million for the year ended December 31, 2025. The gain in the fair value of warrant liabilities was primarily due to the decrease in our stock price as of December 31, 2025 compared to December 31, 2024 and to the shorter expected term period over period.

The change in fair value of warrant and derivative liabilities was a loss of $0.8 million for the year ended December 31, 2024. The loss on the fair value of warrant and derivative liabilities was primarily due to the increase in our stock price year over the year offset by a reduced term of each of the warrants and derivative liabilities. At September 30, 2024, the conversion feature in our loan agreement no longer met the criteria of a derivative liability, and the derivative liability was reclassified to equity.

Gain from sale of priority review voucher, net

In May 2025, we sold our PRV awarded to us following the FDA approval of ZEVASKYN®. We received gross proceeds of $155.0 million during the year ended December 31, 2025 and recognized a gain from the PRV sale of $152.4 million, net of transaction costs of $2.6 million, as it did not have a carrying value at the time of sale.

Other income, net

Other income, net was $0.4 million for the year ended December 31, 2025, as compared to $1.2 million in the same period of 2024. The change was primarily a result of the refundable job creation tax credit of $0.5 million received in 2024 that was not received in 2025.

Income tax expense

We recorded a current income tax expense of $0.1 million for the year ended December 31, 2025. We did not record an income tax expense for the year ended December 31, 2024 as we generated sufficient tax losses, after consideration of discrete items. The current income tax expense for the year ended December 31, 2025 was primarily driven by pre-tax income from the gain on sale of the PRV.

LIQUIDITY AND CAPITAL RESOURCES

Cash Flows for the Years Ended December 31, 2025 and 2024

($ in thousands)	For the year ended December 31, 2025	2024
Total cash, cash equivalents and restricted cash (used in) provided by:		
Operating activities	$ (76,326)	$ (56,015)
Investing activities	105,028	(39,240)
Financing activities	26,040	104,139
Net increase in cash, cash equivalents and restricted cash	$ 54,742	$ 8,884

Operating activities

Net cash used in operating activities was $76.3 million for the year ended December 31, 2025, primarily comprised of our net income of $71.2 million, offset by decreases in operating assets and liabilities of $5.4 million, the $152.4 million gain on sale of priority review voucher for which the cash proceeds are recorded in investing activities, and net non-cash charges of $10.2 million. Non-cash charges consisted primarily of $6.1 million of gain as a result of the change in fair value of warrant and derivative liabilities, $10.8 million of stock-based compensation and $2.5 million of depreciation and amortization.

Net cash used in operating activities was $56.0 million for the year ended December 31, 2024, primarily comprised of our net loss of $63.7 million and decreases in operating assets and liabilities of $4.4 million, partially offset by net non-cash charges of $12.1 million. Non-cash charges consisted primarily of $0.8 million of the change in fair value of warrant and derivative liabilities, $6.6 million of stock-based compensation, $1.5 million of non-cash interest expense and $2.0 million of depreciation and amortization.

Investing activities

Net cash provided by investing activities was $105.0 million for the year ended December 31, 2025, primarily comprised of net proceeds from sale of priority review voucher of $152.4 million, proceeds from maturities of short-term investments of $167.3 million, offset by purchases of short-term investments of $206.6 million and capital expenditures of $8.0 million.

Net cash used in investing activities was $39.2 million for the year ended December 31, 2024, primarily comprised of purchases of short-term investments of $157.0 million and capital expenditures of $2.4 million, partially offset by proceeds from maturities of short-term investments of $120.2 million.

Financing activities

Net cash provided by financing activities was $26.0 million for the year ended December 31, 2025, primarily comprised of proceeds of $17.3 million from open market sales of common stock pursuant to the ATM Agreement (as defined below) and proceeds of $8.8 million from the exercise of stock purchase warrants.

Net cash provided by financing activities was $104.1 million for the year ended December 31, 2024, primarily comprised of proceeds of $70.2 million in net proceeds from our May 2024 underwritten offering, $15.5 million from open market sales of common stock pursuant to the ATM Agreement (as defined below) and net proceeds of $19.0 million from our credit facility entered into in January 2024.

We have historically funded our operations primarily through our sale of equity securities, our most recent gain on sale of our PRV, and strategic collaboration arrangements.

Our principal source of liquidity is cash, cash equivalents, restricted cash and short-term investments, collectively referred to as our cash resources. As of December 31, 2025, our cash resources were $191.4 million. We believe that our current cash and cash equivalents, restricted cash and short-term investments are sufficient to fund operations through at least the next 12 months from the date of this annual report on Form 10-K. We may need to secure additional funding to carry out all of our planned research and development and potential commercialization activities. If we are unable to obtain additional financing or generate license or product revenue, the lack of liquidity and sufficient capital resources could have a material adverse effect on our future prospects.

We have an open market sale agreement with Jefferies LLC (as amended, the "ATM Agreement") pursuant to which we may sell from time to time, through Jefferies LLC, shares of our common stock for an aggregate sales price of up to $75.0 million. Any sales of shares pursuant to this agreement are made under our effective "shelf" registration statement on Form S-3 that is on file with and has been declared effective by the SEC. We sold 3,510,889 shares of our common stock under the ATM Agreement and received $17.3 million of net proceeds during the year ended December 31, 2025. We sold 2,825,954 shares of our common stock under the ATM Agreement and received $15.5 million of net proceeds during the year ended December 31, 2024. Under the ATM Agreement and as of December 31, 2025, we have remaining shares of our common stock for an aggregate sales price of up to $51.5 million.

Since our inception and excluding the gain on sale of our priority review voucher, we have incurred negative cash flows from operations and have expended, and expect to continue to expend, substantial funds to complete our planned product development and commercialization efforts. Excluding the gain on sale of our priority review voucher, we have not been profitable since inception and to date have received limited revenues from the sale of products or licenses. As a result, we have incurred significant operating losses and negative cash flows from operations since our inception and anticipate such losses and negative cash flows will continue until ZEVASKYN® can provide sufficient revenue for us to be profitable and generate positive cash flow.

We may incur losses for the next several years as we continue to invest in commercialization, product research and development, preclinical studies, clinical trials, and regulatory compliance and cannot provide assurance that we will ever be able to generate sufficient product sales or royalty revenue to achieve profitability on a sustained basis, or at all.

If we raise additional funds by selling additional equity securities, the relative equity ownership of our existing investors will be diluted, and the new investors could obtain terms more favorable than previous investors. If we raise additional funds through collaborations, strategic alliances, or licensing arrangements with third parties, we may have to relinquish valuable rights to our technologies, future revenue streams, research programs, or product candidates or grant licenses on terms that may not be favorable to us. If we are unable to raise additional funds through equity or debt financing when needed, we may be required to delay, limit, or terminate our product development programs or any future commercialization efforts or grant rights to develop and market product candidates to third parties that we would otherwise prefer to develop and market ourselves.

Our future capital requirements and adequacy of available funds depend on many factors, including:

- the successful commercialization of ZEVASKYN®;
- the successful development, regulatory approval and commercialization of our cell and gene therapy and other product candidates;
- the ability to establish and maintain collaborative arrangements with corporate partners for the research, development, and commercialization of products;
- continued scientific progress in our research and development programs;
- the magnitude, scope and results of preclinical testing and clinical trials;
- the costs involved in filing, prosecuting, and enforcing patent claims;
- the costs involved in conducting clinical trials;
- competing technological developments;
- the cost of manufacturing and scale-up;
- the ability to establish and maintain effective commercialization arrangements and activities; and
- the successful outcome of our regulatory filings.

Due to uncertainties and certain of the risks described above, our ability to successfully commercialize our product candidates, our ability to obtain applicable regulatory approval to market our product candidates, our ability to obtain necessary additional capital to fund operations in the future, our ability to successfully manufacture our products and our product candidates in clinical quantities or for commercial purposes, government regulation to which we are subject, the uncertainty associated with preclinical and clinical testing, intense competition that we face, the potential necessity of licensing technology from third parties and protection of our intellectual property, it is not possible to reliably predict future spending or time to completion by project or product category or the period in which material net cash inflows from significant projects are expected to commence. If we are unable to timely complete a particular project, our research and development efforts could be delayed or reduced, our business could suffer depending on the significance of the project and we might need to raise additional capital to fund operations, as discussed in the risks above.

We plan to continue our policy of investing any available funds in suitable certificates of deposit, money market funds, government securities and investment-grade, interest-bearing securities. We do not invest in derivative financial instruments.

Contractual Obligations

We enter into agreements in the normal course of business with clinical research organizations for clinical trials and clinical manufacturing organizations for supply manufacturing and with vendors for preclinical research studies and other services and products for operating purposes. These contractual obligations are cancelable at any time by us, generally upon prior written notice to the vendor.

Operating lease amounts represent future minimum lease payments under our non-cancelable operating lease agreements. The total future payments for our operating lease obligations that had commenced as of December 31, 2025 were $6.2 million, of which $1.0 million is due in the next twelve months and the remaining payments are due over the terms of the respective leases. The minimum lease payments above do not include any related common area maintenance charges or real estate taxes.

In addition, we are also party to other license agreements that include contingent payments. However, contingent payments related to these license agreements are not disclosed as the satisfaction of these contingent payments is uncertain as of December 31, 2025 and, if satisfied, the timing of payment for these amounts was not reasonably estimable as of December 31, 2025. Commitments related to the license agreements include contingent payments that will become payable if and when certain development, regulatory and commercial milestones are achieved. During the next 12 months, certain contingent payments could become due upon sales of ZEVASKYN® or any other developmental milestones for sub-licensed products related to such license agreements.

Critical Accounting Estimates

The preparation of financial statements in accordance with accounting principles generally accepted in the United States of America requires management to make estimates and assumptions that affect the reported amounts and related disclosures in the financial statements. Management considers an accounting estimate to be critical if:

- it requires assumptions to be made that were uncertain at the time the estimate was made, and
- changes in the estimate or different estimates that could have been selected could have a material impact in our results of operations or financial condition.

While we base our estimates and judgments on our experience and on various other factors that we believe to be reasonable under the circumstances, actual results could differ from those estimates and the differences could be material.

While our significant accounting policies are described in greater detail in Note 2 to our consolidated financial statements appearing elsewhere in this Annual Report, we believe that the following accounting policies are the most critical to the judgements and estimates used in the preparation of our consolidated financial statements.

Revenue Recognition

Product Revenue

After FDA approval of ZEVASKYN® in April 2025, we began commercial marketing and made our first product sale in Q4 2025. ASC 606, *Revenue from Contracts with Customers*, ("ASC 606") requires us to make estimates of variable consideration, including in our contracts, to be included in the transaction price. Revenue from product sales is recognized at the point in time that the customer obtains control of the product, which is typically upon the completion of a final quality inspection of the product at the qualified treatment centers. There is no obligation for the qualified treatment centers to use ZEVASKYN®, and we have no contractual right to receive payment until the final quality inspection of the product at the qualified treatment centers, and transfer of control is completed.

Revenue from product sales is reduced at the time of recognition for payor rebates, co-payment assistance and prompt pay discounts, which are attributed to various commercial arrangements and government programs. Our contracts can include the right to receive an outcomes-based rebate and a subsequent treatment discount of ZEVASKYN® under certain conditions. We have determined that the rebate and discount create a material right and we allocate the transaction consideration to ZEVASKYN® and the material right on a relative standalone selling price basis. Transaction consideration allocated to the material right is deferred and recognized when either (a) the subsequent purchase of ZEVASKYN® occurs, or (b) the time period during which a subsequent purchase of ZEVASKYN® is made, expires.

As of December 31, 2025, our sales contained no material estimates as the applicable government rebate was known at the time of revenue recognition and no other material rights were present.

License and other revenues

We enter into license agreements that are within the scope of ASC 606, under which it may exclusively license rights to research, develop, manufacture and commercialize its product candidates to third parties. The terms of these arrangements typically include payments of one or more of the following: non-refundable, upfront license fees; reimbursement of certain costs; customer option exercise fees; development, regulatory and commercial milestone payments; and royalties on net sales of licensed products.

If the license to our intellectual property is determined to be distinct from the other performance obligations identified in the arrangement, we recognize revenue from non-refundable, upfront fees allocated to the license when the license is transferred to the customer and the customer is able to use and benefit from the license. In assessing whether a performance obligation is distinct from the other performance obligations, we consider factors such as the research, development, manufacturing and commercialization capabilities of the collaboration partner and the availability of the associated expertise in the general marketplace. In addition, we consider whether the collaboration partner can benefit from a performance obligation for its intended purpose without the receipt of the remaining performance obligation, whether the value of the performance obligation is dependent on the unsatisfied performance obligation, whether there are other vendors that could provide the remaining performance obligation, and whether it is separately identifiable from the remaining performance obligation. For licenses that are combined with other performance obligations, we utilize judgment to assess the nature of the combined performance obligation to determine whether the combined performance obligation is satisfied over time or at a point in time and, if over time, the appropriate method of measuring progress for purposes of recognizing revenue. We evaluate the measure of progress each reporting period and, if necessary, adjust the measure of performance and related revenue recognition. The measure of progress, and thereby periods over which revenue should be recognized, are subject to estimates by management and may change over the course of the research and development and licensing agreement. Such a change could have a material impact on the amount of revenue we record in future periods.

Milestone Payments

At the inception of each arrangement that includes research or development milestone payments, we evaluate whether the milestones are considered probable of being achieved and estimate the amount to be included in the transaction price using the most likely amount method. If it is probable that a significant cumulative revenue reversal would not occur, the associated milestone value is included in the transaction price. An output method is generally used to measure progress toward complete satisfaction of a milestone. Milestone payments that are not within our control or the licensee, such as regulatory approvals, are not considered probable of being achieved until those approvals are received. We evaluate factors such as the scientific, clinical, regulatory, commercial, and other risks that must be overcome to achieve the particular milestone in making this assessment. There is considerable judgment involved in determining whether it is probable that a significant cumulative revenue reversal would not occur. At the end of each subsequent reporting period, we re-evaluate the probability of achievement of all milestones subject to constraint and, if necessary, adjust our estimate of the overall transaction price. Any such adjustments are recorded on a cumulative catch-up basis, which would affect revenue and earnings in the period of adjustment.

Collaborative Arrangements

We analyze our collaboration arrangements to assess whether such arrangements 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 and therefore within the scope of ASC 808, *Collaborative Arrangements* (“ASC 808”). This assessment is performed throughout the life of the arrangement based on changes in the responsibilities of all parties in the arrangement. For collaboration arrangements within the scope of ASC 808 that contain multiple elements, we first determine which elements of the collaboration are deemed to be within the scope of ASC 808 and which elements of the collaboration are more reflective of a vendor-customer relationship and therefore within the scope of ASC 606. For elements of collaboration arrangements that are accounted for pursuant to ASC 808, an appropriate recognition method is determined and applied consistently, generally by analogy to ASC 606. Amounts that are owed to collaboration partners are recognized as an offset to collaboration revenue as such amounts are incurred by the collaboration partner. For those elements of the arrangement that are accounted for pursuant to ASC 606, the Company applies the five-step model described above under ASC 606.

Accrued Expenses

As part of the process of preparing our consolidated financial statements, we are required to estimate our accrued expenses. This process involves reviewing open contracts and purchase orders, communicating with our personnel to identify services that have been performed on our behalf and estimating the level of service performed and the associated costs incurred for the services when we have not yet been invoiced or otherwise notified of the actual costs. The majority of our service providers invoice us in arrears for services performed on a pre-determined schedule or when contractual milestones are met; however, some require advance payments. We make estimates of our accrued expenses as of each balance sheet date in our consolidated financial statements based on facts and circumstances known to us at that time. If the actual timing of the performance of services or the level of effort varies from our estimate, we adjust the accrual or amount of prepaid expense accordingly. To date, we have not made any material adjustments to our prior estimates of accrued expenses.

Share-Based Compensation Expense

We have applied the fair value recognition provisions of Financial Accounting Standards Board Accounting Standards Codification, or ASC, Topic 718, *Compensation—Stock Compensation* ("ASC 718"), to account for stock-based compensation. We recognize compensation costs related to stock-based awards granted based on the estimated fair value of the awards on the date of grant.

ASC 718 requires all stock-based payments, including grants of stock options and restricted stock, to be recognized in the consolidated statements of operations and comprehensive income based on their grant-date fair values. Compensation expense for stock options, restricted stock awards and restricted stock units is recognized on a straight-line basis based on the grant-date fair value over the associated service period of the award, which is generally the vesting term.

Determining the amount of stock-based compensation to be recorded requires us to develop estimates of the fair value of stock-based awards as of their measurement date. We recognize stock-based compensation expense over the requisite service period, which is the vesting period of the award. Calculating the fair value of stock-based awards requires that we make assumptions. We estimate the fair value of its stock options using the Black-Scholes option pricing model, which requires the input of subjective assumptions, including: (i) the expected stock price volatility; (ii) the expected term of the award; (iii) the risk-free interest rate; and (iv) expected dividends.

We estimate the expected term of stock options using the "simplified" method as prescribed by SEC Staff Accounting Bulletin No. 107, *Share-Based Payments*, whereby the expected term equals the arithmetic mean of the vesting term and the original contractual term of the option. The risk-free interest rates are based on US Treasury securities with a maturity date commensurate with the expected term of the associated award. The Company has never paid and does not expect to pay dividends in the foreseeable future. The Company accounts for forfeitures as they occur. Stock-based compensation expense recognized in the financial statements is based on awards for which service conditions are expected to be satisfied.

Stock option-based compensation expense recognized for the years ended December 31, 2025 and 2024 was $0.3 million and $1.1 million, respectively. Restricted stock-based compensation expense recognized for the years ended December 31, 2025 and 2024 was $10.5 million and $5.6 million, respectively.

Warrants

We determine the accounting and value of any issued warrants in accordance with ASC 480*, Distinguishing Liabilities from Equity* and ASC 815, *Derivatives and Hedging*. We measure the value of any liability classified warrants on their issuance date based on their fair value using the Black-Scholes pricing model. Inputs used in the model include assumptions for expected volatility, risk-free interest rate, dividend yield and estimated expected term. Certain inputs used in this Black-Scholes pricing model may fluctuate in future periods based upon factors that are outside of our control, including a potential change in control. A significant change in one or more of these inputs used in the calculation of the fair value may cause a significant change to the fair value of our warrant liabilities, which could also result in material non-cash gains or losses being reported in the Company's statement of operations and comprehensive income (loss). In addition, the inputs we utilized to value our warrant liabilities are highly subjective. The assumptions used in calculating the fair value of our warrant liabilities represent our best estimates, but these estimates involve inherent uncertainties and the application of management judgment. As a result, if factors change and we use different assumptions, the fair value of the warrant liabilities may be materially different in the future.

The change in fair value of warrant liability recognized for the years ended December 31, 2025 and 2024 resulted in a gain of $6.1 million and a loss of $0.8 million, respectively.

ITEM 7A. QUANTITATIVE AND QUALITATIVE DISCLOSURES ABOUT MARKET RISK

Not applicable.

ITEM 8. FINANCIAL STATEMENTS AND SUPPLEMENTARY DATA

Financial statements required by this Item are incorporated in this Annual Report on Form 10-K starting on page F-1 hereto. Reference is made to Item 15 of this Form 10-K.

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

None.

ITEM 9A. CONTROLS AND PROCEDURES

Evaluation of Disclosure Controls and Procedures

Our management, with the participation of our principal executive officer and principal financial officer, has evaluated the effectiveness of our disclosure controls and procedures (as defined in Rules 13a-15(e) and 15d-15(e) under the Securities Exchange Act of 1934, as amended (the "Exchange Act")), as of the end of the period covered by this Annual Report on Form 10-K. Based on such evaluation, our principal executive officer and principal financial officer have concluded that as of such date, our disclosure controls and procedures were effective.

Management's Annual 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, 2025, 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). Our management's assessment of the effectiveness of our internal control over financial reporting included testing and evaluating the design and operating effectiveness of our internal controls. In our management's opinion, we have maintained effective internal control over financial reporting as of December 31, 2025, based on criteria established in the COSO 2013 framework.

Because we are a non-accelerated filer and smaller reporting company, Deloitte & Touche LLP, our independent registered public accounting firm, is not required to attest to or issue a report on the effectiveness of our internal control over financial reporting.

Inherent Limitations of Internal Controls

Our management, including our principal executive officer and principal financial officer, does not expect that our disclosure controls and procedures or our internal controls will prevent all errors and all fraud. A control system, no matter how well conceived and operated, can provide only reasonable, not absolute, assurance that the objectives of the control system are met. 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, within the Company have been detected. These inherent limitations include the realities that judgments in decision-making can be faulty, and that breakdowns can occur because of a simple error or mistake. Additionally, controls can be circumvented by the individual acts of some persons, by collusion of two or more people, or by management override of the control. The design of any system of controls also is based in part upon certain assumptions about the likelihood of future events, and there can be no assurance that any design will succeed in achieving its stated goals under all potential future conditions. Over time, controls may become inadequate because of changes in conditions, or the degree of compliance with the policies or procedures may deteriorate. 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. Because of the inherent limitations in a cost-effective control system, misstatements due to error or fraud may occur and not be detected.

Changes in Internal Control over Financial Reporting

There have been no changes in our internal control over financial reporting, as such term is defined in Rules 13a-15(f) and 15d-15(f) promulgated under the Exchange Act, during the fourth quarter of 2025 that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

ITEM 9B. OTHER INFORMATION

During the fiscal quarter ended December 31, 2025, none of our directors or officers (as defined in Rule 16a-1 under the Exchange Act) adopted, modified or terminated a "Rule 10b5-1 trading arrangement" or "non-Rule 105b-1 trading arrangement" (as those terms are defined in Item 408 of Regulation S-K).

ITEM 9C. DISCLOSURE REGARDING FOREIGN JURISDICTIONS THAT PREVENT INSPECTIONS

Not applicable.

PART III

ITEM 10. DIRECTORS, EXECUTIVE OFFICERS AND CORPORATE GOVERNANCE

Information appearing in our Notice of Annual Meeting of Stockholders and Proxy Statement for the 2026 Annual Meeting of Stockholders (the "2026 Proxy Statement"), including information appearing under "Proxy Statement Summary," "Corporate Governance Matters," and "Audit Committee Matters" is incorporated herein by reference. We will file the 2026 Proxy Statement with the SEC pursuant to Regulation 14A within 120 days after the end of the fiscal year.

We have adopted a Code of Business Conduct and Ethics (the "Code") that applies to all of our employees (including executive officers) and directors. The Code is available on our website at www.abeonatherapeutics.com under the heading "Investors & Media—Corporate Governance—Governance—Governance Documents." We intend to satisfy the disclosure requirement regarding any amendment to, or waiver from a provision of the Code applicable to any executive officer or director, by posting such information on our website.

ITEM 11. EXECUTIVE COMPENSATION

Information contained in the 2026 Proxy Statement, including information appearing under "Corporate Governance Matters," "Compensation of Directors," and "Executive Compensation" in the 2026 Proxy Statement, is incorporated herein by reference.

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

Information contained in the 2026 Proxy Statement, including information appearing under "Security Ownership of Certain Beneficial Owners and Management" in the 2026 Proxy Statement, is incorporated herein by reference.

ITEM 13. CERTAIN RELATIONSHIPS AND RELATED TRANSACTIONS AND DIRECTOR INDEPENDENCE

Information contained in the 2026 Proxy Statement, including information appearing under "Corporate Governance Matters" and "Compensation of Directors" in the 2026 Proxy Statement, is incorporated herein by reference.

ITEM 14. PRINCIPAL ACCOUNTING FEES AND SERVICES

Information contained in the 2026 Proxy Statement, including information appearing under "Independent Registered Public Accounting Firm Fees and Services" in the 2026 Proxy Statement, is incorporated herein by reference.

PART IV

ITEM 15. EXHIBITS, FINANCIAL STATEMENT SCHEDULES

a. Financial Statements.

The following financial statements are submitted as part of this report:

	Page
Report of Independent Registered Public Accounting Firm (PCAOB 034)	F-1
Consolidated Balance Sheets at December 31, 2025 and 2024	F-3
Consolidated Statements of Operations and Comprehensive Income (Loss) for 2025 and 2024	F-4
Consolidated Statements of Stockholders' Equity for 2025 and 2024	F-5
Consolidated Statements of Cash Flows for 2025 and 2024	F-6
Notes to Consolidated Financial Statements	F-7

b. Exhibits

Exhibit Index

Exhibits:	Description of Document
3.1	Restated Certificate of Incorporation of Abeona Therapeutics Inc. (incorporated by reference to Exhibit 3.1 of our Form 10-Q for the quarter ended March 31, 2019)
3.2	Certificate of Amendment to Restated Certificate of Incorporation of Abeona Therapeutics Inc. (incorporated by reference to Exhibit 3.1 of our Form 8-K filed on June 30, 2022)
3.3	Amended and Restated Bylaws of Abeona Therapeutics Inc.
3.4	Form of Certificate of Designation of Preferences, Rights and Limitations of Series A Convertible Redeemable Preferred Stock (incorporated by reference to Exhibit 3.1 of our Form 8-K filed on May 2, 2022)
3.5	Form of Certificate of Designation of Preferences, Rights and Limitations of Series B Convertible Redeemable Preferred Stock (incorporated by reference to Exhibit 3.2 of our Form 8-K filed on May 2, 2022)
4.1	Description of Capital Stock of Abeona Therapeutics Inc. (incorporated by reference to Exhibit 4.4 of our Form 10-K for the year ended December 31, 2019)
4.2	Form of Pre-Funded Warrant (incorporated by reference to Exhibit 4.1 of our Form 8-K filed on May 3, 2024)
4.3	Warrant to Purchase Common Stock, by and between Abeona Therapeutics, Inc. and Avenue Venture Opportunities Fund, L.P., dated as of January 8, 2024 (incorporated by reference to Exhibit 4.1 of our Form 8-K filed on January 8, 2024)
4.4	Warrant to Purchase Common Stock, by and between Abeona Therapeutics, Inc. and Avenue Venture Opportunities Fund II, L.P., dated as of January 8, 2024 (incorporated by reference to Exhibit 4.2 of our Form 8-K filed on January 8, 2024)

4.5	Warrant to Purchase Common Stock, by and between Abeona Therapeutics Inc. and Avenue Venture Opportunities Fund, L.P., dated as of July 18, 2025 (incorporated by reference to Exhibit 4.1 of our Form 8-K filed on July 18, 2025)
4.6	Warrant to Purchase Common Stock, by and between Abeona Therapeutics Inc. and Avenue Venture Opportunities Fund II, L.P., dated as of July 18, 2025 (incorporated by reference to Exhibit 4.2 of our Form 8-K filed on July 18, 2025)
10.1*	401(k) Plan (incorporated by reference to Exhibit 10.20 of our Form 10-K for the year ended December 31, 1999)
10.2*	2005 Equity Incentive Plan (incorporated by reference to Exhibit 1 of our Proxy Statement filed on April 18, 2005)
10.3*	2015 Equity Incentive Plan, as amended (incorporated by reference to Exhibit 99.1 to our Form S-8 filed on August 30, 2022)
10.4*	Second Amended and Restated 2023 Equity Incentive Plan (incorporated by reference to Appendix A of our Definitive Proxy Statement on Schedule 14A filed with the Securities and Exchange Commission on November 12, 2024)
10.5	2023 Employment Inducement Equity Incentive Plan (incorporated by reference to Exhibit 99.1 to our Form S-8 filed on October 10, 2023)
10.6	Director Designation Agreement dated November 15, 2007, between the Company and SCO Capital Partners LLC (incorporated by reference to Exhibit 10.26 of our Form S-1 filed on March 11, 2008)
10.7	Agreement and Plan of Merger, dated May 5, 2015, by and among the Company, PlasmaTech Merger Sub Inc., Abeona Therapeutics LLC and Paul A. Hawkins, in his capacity as Member Representative (incorporated by reference to Exhibit 10.1 to our Form 10-Q for the quarter ended June 30, 2015)
10.8	Form of Indemnification Agreement, between the Company and directors and officers of the Company (incorporated by reference to Exhibit 10.1 to our Form 8-K filed on October 16, 2020)
10.9*	Letter Agreement, dated October 6, 2021, between the Company and Vishwas Seshadri (incorporated by reference to Exhibit 10.6 of our Form 10-K for the year ended December 31, 2021)
10.10*	Letter Agreement, dated September 16, 2021, between the Company and Brendan O'Malley (incorporated by reference to Exhibit 10.11 of our Form 10-K for the year ended December 31, 2021)
10.11*	Letter Agreement, dated February 28, 2022, between the Company and Joseph Vazzano (incorporated by reference to Exhibit 10.1 of our Form 10-Q for the quarter ended March 31, 2022)
10.12	Open Market Sale Agreement, dated August 17, 2018, by and between the Company and Jefferies LLC (incorporated by reference to Exhibit 1.1 of Form 8-K filed on August 20, 2018)
10.13	Amendment No. 1 to Open Market Sale Agreement, dated November 19, 2021, amending the Open Market Agreement, by and between the Company and Jefferies LLC, dated August 17, 2018 (incorporated by reference to Exhibit 1.2 of Form 8-K filed on November 19, 2021)
10.14	Form of Securities Purchase Agreement between Abeona Therapeutics Inc. and the investors thereto, dated April 29, 2022 (incorporated by reference to Exhibit 10.1 of our Form 8-K filed on May 2, 2022)

10.15	Form of Registration Rights Agreement by and among Abeona Therapeutics Inc. and the investors named therein, dated April 29, 2022 (incorporated by reference to Exhibit 10.2 of our Form 8-K filed on May 2, 2022)
10.16+	License Agreement by and between Abeona Therapeutics Inc. and Ultragenyx Pharmaceutical Inc., dated May 16, 2022 (incorporated by reference to Exhibit 10.3 of our Form 10-Q for the quarter ended June 30, 2022)
10.17	Retention Bonus Letter, dated June 15, 2023, to Vishwas Seshadri, Ph.D. (incorporated by reference to Exhibit 10.3 of our Form 10-Q for the quarter ended June 30, 2023)
10.18	Retention Bonus Letter, dated June 15, 2023, to Joseph Vazzano. (incorporated by reference to Exhibit 10.4 of our Form 10-Q for the quarter ended June 30, 2023)
10.19	Retention Bonus Letter, dated June 15, 2023, to Brendan O'Malley, Ph.D. (incorporated by reference to Exhibit 10.5 of our Form 10-Q for the quarter ended June 30, 2023)
10.20	Securities Purchase Agreement, dated July 3, 2023 (incorporated by reference to Exhibit 10.1 of our Form 8-K filed on July 3, 2023)
10.21	Loan and Security Agreement, by and among Abeona Therapeutics, Inc., MacroChem Corporation, Abeona Therapeutics LLC, Avenue Venture Opportunities Fund, L.P., as Agent, and Avenue Venture Opportunities Fund II, L.P., dated as of January 8, 2024 (incorporated by reference to Exhibit 10.1 of our Form 8-K filed on January 8, 2024)
10.22	Supplement to the Loan and Security Agreement, by and among Abeona Therapeutics, Inc., MacroChem Corporation, Abeona Therapeutics LLC, Avenue Venture Opportunities Fund, L.P., as Agent, and Avenue Venture Opportunities Fund II, L.P., dated as of January 8, 2024 (incorporated by reference to Exhibit 10.2 of our Form 8-K filed on January 8, 2024)
10.23	Priority Review Voucher Asset Purchase Agreement dated May 9, 2025 (incorporated by reference to Exhibit 10.1 of our Form 10-Q for the quarter ended June 30, 2025)
10.24	First Amendment to Loan and Security Agreement and Supplement, by and among Abeona Therapeutics Inc., MacroChem Corporation, Abeona Therapeutics LLC, Avenue Venture Opportunities Fund, L.P., as Agent, and Avenue Venture Opportunities Fund II, L.P., dated as of July 18, 2025 (incorporated by reference to Exhibit 10.1 of our Form 8-K filed on July 18, 2025)
19	Policy on Insider Trading and Confidentiality
21	Subsidiaries of the registrant
23.1	Consent of Deloitte & Touche LLP
31.1	Principal Executive Officer Certification Pursuant to Rule 13a-14(a) of the Securities Exchange Act of 1934
31.2	Principal Financial Officer Certification Pursuant to Rule 13a-14(a) of the Securities Exchange Act of 1934
32	Certification Pursuant to 18 U.S.C. Section 1350, as Adopted Pursuant to Section 906 of the Sarbanes-Oxley Act of 2002
97	Policy Relating to Recovery of Erroneously Awarded Compensation
101.INS	Inline XBRL Instance Document
101.SCH	Inline XBRL Taxonomy Extension Schema
101.CAL	Inline XBRL Taxonomy Extension Calculation Linkbase Document
101.DEF	Inline XBRL Taxonomy Extension Definition Linkbase Document
101.LAB	Inline XBRL Taxonomy Extension Label Linkbase Document
101.PRE	Inline XBRL Taxonomy Extension Presentation Linkbase Document
104	Cover Page Interactive Data File (formatted as inline XBRL and contained in Exhibit 101)

* Management contract or compensatory plan or arrangement.

+ Portions of this exhibit have been omitted pursuant to Item 601(b)(10)(iv) of Regulation S-K.

ITEM 16. FORM 10-K SUMMARY

None.

SIGNATURES

Pursuant to the requirements of Section 13 or 15(d) of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned, thereunto duly authorized.

ABEONA THERAPEUTICS INC.

Date: March 17, 2026

By: */s/ Vishwas Seshadri*
Vishwas Seshadri
President and Chief Executive Officer
(Principal 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.

Date: March 17, 2026

/s/ Vishwas Seshadri
Vishwas Seshadri
President, Chief Executive Officer and Director
(Principal Executive Officer)

Date: March 17, 2026

/s/ Joseph Vazzano
Joseph Vazzano
Chief Financial Officer
(Principal Financial and Accounting Officer)

Date: March 17, 2026

/s/ Leila Alland
Leila Alland, Director

Date: March 17, 2026

/s/ Mark J. Alvino
Mark J. Alvino, Director

Date: March 17, 2026

/s/ Michael Amoroso
Michael Amoroso, Director
Chairman of the Board

Date: March 17, 2026

/s/ Faith L. Charles
Faith L. Charles, Director

Date: March 17, 2026

/s/ Eric Crombez, MD
Eric Crombez, MD, Director

Date: March 17, 2026

/s/ Christine Silverstein
Christine Silverstein, Director

Date: March 17, 2026

/s/ Donald A. Wuchterl
Donald A. Wuchterl, Director

Date: March 17, 2026

/s/ Bernhardt G. Zeiher, MD, FCCP, FACP
Bernhardt G. Zeiher, MD, FCCP, FACP, Director

REPORT OF INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM

To the shareholders and the Board of Directors of Abeona Therapeutics Inc.

Opinion on the Financial Statements

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

Basis for Opinion

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

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

Our audits included performing procedures to assess the risks of material misstatement of the financial statements, whether due to error or fraud, and performing procedures that respond to those risks. Such procedures included examining, on a test basis, evidence regarding the amounts and disclosures in the financial statements. Our audits also included evaluating the accounting principles used and significant estimates made by management, as well as evaluating the overall presentation of the financial statements. We believe that our audits provide a reasonable basis for our opinion.

Critical Audit Matter

The critical audit matter communicated below is a matter arising from the current-period audit of the financial statements that was communicated or required to be communicated to the audit committee and that (1) relates to accounts or disclosures that are material to the financial statements and (2) involved our especially challenging, subjective, or complex judgments. The communication of critical audit matters does not alter in any way our opinion on the financial statements, taken as a whole, and we are not, by communicating the critical audit matter below, providing a separate opinion on the critical audit matter or on the accounts or disclosures to which it relates.

Revenue- Product revenue, net— Refer to Notes 2 and 3 to the financial statements

Critical Audit Matter Description

As more fully described in Notes 2 and 3 to the financial statements, product revenue is generated from sales of ZEVASKYN®, which received regulatory approval and reached its commercialization stage upon the treatment of the first patient in 2025. The Company's contracts can include the right to receive both an outcome-based rebate and a subsequent treatment discount.

Revenue from product sales is recognized at the point in time that the customer obtains control of the product. The Company has determined that the rebate and discount create a material right and allocates the transaction consideration to the product and material right on a relative standalone selling price basis. Consideration allocated to the material right is deferred and recognized when the subsequent purchase occurs or the option expires.

We identified the Company's initial application of Revenue from Contracts with Customers ("ASC 606") to its product revenue as a critical audit matter, given the complexity involved with the identification of material rights and in the estimation of the standalone selling price of the material right. Auditing these conclusions involved especially subjective judgment and audit effort.

How the Critical Audit Matter Was Addressed in the Audit

Our audit procedures related to the application of ASC 606 to the Company's product revenue included the following, among others:

- We evaluated the Company's significant account policies related to revenue recognition for reasonableness.
- For a selection of revenue agreements, we obtained and read the underlying agreement between the Company and its customers.
- With the assistance of professionals in our firm having expertise in the accounting treatment for revenue arrangements, we evaluated the Company's assessment of the accounting treatment for such arrangements, including the identification of material rights and the methodology used to estimate the standalone selling price of the material right. We evaluated the Company's determination of the allocation of the transaction price to the product and the material right using a relative standalone selling price methodology.

/s/ Deloitte & Touche LLP

Morristown, New Jersey
March 16, 2026

We have served as the Company's auditor since 2023.

ABEONA THERAPEUTICS INC. AND SUBSIDIARIES
Consolidated Balance Sheets
($ in thousands, except share and per share amounts)

	December 31, 2025	December 31, 2024
ASSETS		
Current assets:		
Cash and cash equivalents	$ 78,437	$ 23,357
Short-term investments	112,967	74,363
Restricted cash	—	338
Accounts receivable, net	6,147	—
Inventory	5,493	—
Other receivables	568	1,652
Prepaid expenses and other current assets	1,294	1,143
Total current assets	204,906	100,853
Property and equipment, net	9,921	4,430
Operating lease right-of-use assets	3,962	3,552
Other assets	781	96
Total assets	$ 219,570	$ 108,931
LIABILITIES AND STOCKHOLDERS' EQUITY		
Current liabilities:		
Accounts payable	$ 7,889	$ 3,441
Accrued expenses	8,467	6,333
Current portion of long-term debt	12,222	5,926
Current portion of operating lease liability	864	823
Accrued taxes	126	—
Other current liabilities	2	64
Total current liabilities	29,570	16,587
Long-term operating lease liabilities	4,069	3,262
Long-term debt	7,813	13,037
Warrant liabilities	18,902	32,014
Total liabilities	60,354	64,900
Commitments and contingencies		
Stockholders' equity:		
Preferred stock - $0.01 par value; authorized 2,000,000 shares; No shares issued and outstanding as of December 31, 2025 and 2024, respectively	—	—
Common stock - $0.01 par value; authorized 200,000,000 shares; 55,043,413 and 45,644,091 shares issued and outstanding as of December 31, 2025 and 2024, respectively	550	457
Additional paid-in capital	900,603	856,824
Accumulated deficit	(742,075)	(813,258)
Accumulated other comprehensive loss	138	8
Total stockholders' equity	159,216	44,031
Total liabilities and stockholders' equity	$ 219,570	$ 108,931

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

ABEONA THERAPEUTICS INC. AND SUBSIDIARIES
Consolidated Statements of Operations and Comprehensive Income (Loss)
($ in thousands, except share and per share amounts)

	For the years ended December 31,	
	2025	**2024**
Revenues:		
Product revenue, net	$ 2,420	$ —
License and other revenues	3,400	—
Total revenues	5,820	—
Costs and expenses:		
Cost of sales	1,532	—
Royalties	1,893	—
Research and development	26,812	34,360
Selling, general and administrative	65,031	29,851
Total costs and expenses	95,268	64,211
Loss from operations	(89,448)	(64,211)
Interest income	5,556	4,246
Interest expense	(3,740)	(4,208)
Change in fair value of warrant and derivative liabilities	6,139	(755)
Gain from sale of priority review voucher, net	152,366	—
Other income, net	410	1,194
Income (loss) before income taxes	71,283	(63,734)
Income tax (benefit) expense	100	—
Net income (loss)	$ 71,183	$ (63,734)
Basic income (loss) per common share	$ 1.34	$ (1.55)
Dilutive income (loss) per common share	$ 1.01	$ (1.55)
Weighted average number of common shares outstanding:		
Basic	52,952,917	41,048,206
Dilutive	66,135,821	41,048,206
Other comprehensive income (loss):		
Change in unrealized gains related to available-for-sale debt securities	130	74
Comprehensive income (loss)	$ 71,313	$ (63,660)

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

ABEONA THERAPEUTICS INC. AND SUBSIDIARIES
Consolidated Statements of Stockholders' Equity
($ in thousands, except share amounts)

	Common Stock		Additional Paid-in Capital	Accumulated Deficit	Accumulated Other Comprehensive Income (Loss)	Total Stockholders' Equity
	Shares	Amount				
Balance at December 31, 2023	26,523,878	$ 265	$ 764,151	$ (749,524)	$ (66)	$ 14,826
Stock-based compensation expense	—	—	6,628	—	—	6,628
Issuance of common stock in connection with restricted share awards, net of cancellations and shares settled for tax withholding settlement	1,780,713	19	(545)	—	—	(526)
Issuance of common stock, net of offering costs under open market sale agreement (ATM)	2,825,954	28	15,447	—	—	15,475
Issuance of common stock in connection with public offering, net of offering costs	12,285,056	123	70,030	—	—	70,153
Issuance of common stock upon exercise of pre-funded warrants, net of shares settled	2,228,490	22	(22)	—	—	—
Reclassification of derivative liability	—	—	1,135	—	—	1,135
Net loss	—	—	—	(63,734)	—	(63,734)
Other comprehensive income	—	—	—	—	74	74
Balance at December 31, 2024	45,644,091	$ 457	$ 856,824	$ (813,258)	$ 8	$ 44,031
Stock-based compensation expense	—	—	10,779	—	—	10,779
Issuance of common stock in connection with restricted share awards, net of cancellations and shares settled for tax withholding settlement	2,320,696	23	(60)	—	—	(37)
Issuance of common stock, net of offering costs under open market sale agreement (ATM)	3,510,889	35	17,265	—	—	17,300
Issuance of common stock upon exercise of warrants	3,567,737	35	8,742	—	—	8,777
Reclassification of warrant liability	—	—	7,053	—	—	7,053
Net income	—	—	—	71,183	—	71,183
Other comprehensive income	—	—	—	—	130	130
Balance at December 31, 2025	55,043,413	$ 550	$ 900,603	$ (742,075)	$ 138	$ 159,216

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

ABEONA THERAPEUTICS INC. AND SUBSIDIARIES
Consolidated Statements of Cash Flows
($ in thousands)

	For the year ended December 31,	
	2025	**2024**
Cash flows from operating activities:		
Net income (loss)	$ 71,183	$ (63,734)
Adjustments to reconcile net income (loss) to cash used in operating activities:		
Depreciation and amortization	2,549	2,004
Stock-based compensation expense	10,779	6,628
Change in fair value of warrant and derivative liabilities	(6,139)	755
Accretion and interest on short-term investments	889	276
Amortization of right-of-use lease assets	1,014	903
Non-cash interest	1,152	1,538
Gain on disposal of property and equipment	—	(2)
Gain from sale of priority review voucher	(152,366)	—
Change in operating assets and liabilities:		
Accounts receivable	(6,147)	—
Inventory	(5,493)	—
Other receivables	1,084	792
Prepaid expenses and other current assets	(151)	(564)
Other assets	(685)	181
Accounts payable and accrued expenses	6,540	1,507
Accrued taxes	126	—
Lease liabilities	(576)	(1,315)
Change in payable to licensor	—	(5,000)
Other current liabilities	(85)	16
Net cash used in operating activities	(76,326)	(56,015)
Cash flows from investing activities:		
Proceeds from sale of priority review voucher, net of transaction costs of $2.6 million	152,366	—
Capital expenditures	(7,975)	(2,446)
Proceeds from disposal of property and equipment	—	18
Purchases of short-term investments	(206,634)	(157,010)
Proceeds from maturities of short-term investments	167,271	120,198
Net cash provided by (used in) investing activities	105,028	(39,240)
Cash flows from financing activities:		
Proceeds from ATM sales of common stock, net of issuance costs	17,300	15,475
Payments related to net settlement of restricted share awards	(37)	(526)
Proceeds from underwritten sales of common stock, net of issuance costs	—	70,153
Proceeds from exercise of warrants	8,777	—
Proceeds from issuance of long-term debt	—	20,000
Payment of debt issuance costs	—	(963)
Net cash provided by financing activities	26,040	104,139
Net increase in cash, cash equivalents and restricted cash	54,742	8,884
Cash, cash equivalents and restricted cash at beginning of period	23,695	14,811
Cash, cash equivalents and restricted cash at end of period	$ 78,437	$ 23,695
Supplemental cash flow information:		
Cash and cash equivalents	$ 78,437	$ 23,357
Restricted cash	—	338
Total cash, cash equivalents and restricted cash	$ 78,437	$ 23,695
Supplemental non-cash flow information:		
Right-of-use asset obtained in exchange for new operating lease liabilities	$ 1,424	$ —
Derivative and warrant additions associated with loan and security agreement	$ 80	$ 1,042
Reclassification of derivative and warrant liability to equity	$ 7,053	$ 1,135
Changes in accrued property and equipment	$ (406)	$ 471
Cash paid for interest	$ 2,589	$ 2,670
Cash paid for taxes	$ —	$ 7

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

NOTE 1 – NATURE OF OPERATIONS

Background

Abeona Therapeutics Inc. (together with the Company's subsidiaries, "Abeona" or the "Company"), a Delaware corporation, is a commercial-stage biopharmaceutical company developing cell and gene therapies for life-threatening diseases. On April 28, 2025, the U.S. Food and Drug Administration ("FDA") approved ZEVASKYN® (prademagene zamikeracel) gene-modified cellular sheets, also known as ZEVASKYN®, as the first and only autologous cell-based gene therapy for the treatment of wounds in adult and pediatric patients with recessive dystrophic epidermolysis bullosa ("RDEB"), a serious and debilitating genetic skin disease. The Company's development portfolio also features adeno-associated virus ("AAV")-based gene therapies designed to treat ophthalmic diseases with high unmet need using novel AIM™ capsids.

Liquidity

In accordance with Accounting Standards Codification ("ASC") 205-40, *Going Concern*, the Company has evaluated whether there are conditions and events, considered in the aggregate, that raise substantial doubt about the Company's ability to continue as a going concern within one year after the date the accompanying consolidated financial statements were issued.

As a biopharmaceutical organization, the Company has devoted substantially all of its resources since inception to research and development activities for ZEVASKYN® and other product candidates, business planning, raising capital, establishing its intellectual property portfolio, acquiring or discovering product candidates, and providing selling, general and administrative support for these operations.

As a result, the Company has incurred significant operating losses and negative cash flows from operations since its inception, other than the year ended December 31, 2025 with the gain on sale of its Priority Review Voucher ("PRV"). The Company anticipates such losses and negative cash flows will continue until ZEVASKYN® can provide sufficient revenue for the Company to be profitable and generate positive cash flows. Through December 31, 2025, the Company has relied primarily on its sale of equity securities, its proceeds from the sale of its PRV, and strategic collaboration arrangements to finance its operations. The Company expects that its capital resources will be sufficient to fund its operating expenses and capital expenditure requirements for at least the next 12 months from the issuance date of these consolidated financial statements. The Company may need to raise additional capital to fully implement its business plans through the issuance of equity, borrowings, or strategic alliances with partner companies. However, if such financing is not available at adequate levels, the Company would need to reevaluate its operating plans.

NOTE 2 – SUMMARY OF SIGNIFICANT ACCOUNTING POLICIES

A summary of the significant accounting policies applied in the preparation of the accompanying consolidated financial statements follows:

Principles of Consolidation

The consolidated financial statements include the financial statements of Abeona Therapeutics Inc. and the Company's wholly-owned subsidiaries. All intercompany balances and transactions have been eliminated in consolidation.

Use of Estimates

The preparation of consolidated financial statements in conformity with accounting principles generally accepted in the United States of America ("U.S. GAAP") requires management to make estimates and assumptions that affect the reported amount of assets and disclosure of contingent assets and liabilities at the date of the consolidated financial statements and the reported amounts of revenue and expenses during the reported period. The Company's significant estimates include, but are not limited to, variable consideration associated with revenue recognition and the determination of the standalone selling price of material rights, fair value of warrant and derivative liabilities, the incremental borrowing rate related to the Company's operating leases, stock-based compensation, accrued expenses, impairment of long-lived assets and income taxes. Due to the uncertainty inherent in such estimates, actual results could differ from these estimates and assumptions.

Cash and Cash Equivalents

The Company considers all highly liquid investments with a maturity of three months or less when purchased to be cash equivalents. The Company maintains deposits primarily in financial institutions, which may at times exceed amounts covered by insurance provided by the U.S. Federal Deposit Insurance Corporation ("FDIC"). The Company has not experienced any losses related to amounts in excess of FDIC limits.

Restricted Cash

Restricted cash served as collateral for leased office space that expired in September 2025.

Short-term Investments

Short-term investments consist of investments in U.S. treasury securities, U.S. federal agency securities and certificates of deposit. The Company determines the appropriate classification of the securities at the time they are acquired and evaluates the appropriateness of such classifications at each balance sheet date. The Company classifies its short-term investments as available-for-sale pursuant to Accounting Standards Codification ("ASC") 320, *Investments – Debt and Equity Securities*. Investments classified as current have maturities of less than one year. The Company reviews its short-term investments for other-than-temporary impairment whenever the fair value of a marketable security is less than the amortized cost and evidence indicates that a short-term investment's carrying amount is not recoverable within a reasonable period of time.

Accounts Receivable

Accounts receivable represents amounts arising from product sales and licensing revenue and is recorded net of allowances for prompt payment discounts, returns, and credit losses. The Company estimates an allowance for credit losses by considering factors such as the aging of its accounts receivable, the history of write offs for uncollectible accounts, and the credit quality of its significant customers, the current economic environment/macroeconomic trends, supportable forecasts, and other relevant factors. The Company reviews the credit quality of its accounts receivables by monitoring the aging of its accounts receivable, the history of write offs for uncollectible accounts, and the credit quality of its significant customers, the current economic environment/macroeconomic trends, supportable forecasts, and other relevant factors. The Company has no historical write-offs of its accounts receivable and the Company has determined that an allowance for credit losses is not required as of December 31, 2025.

Accounts receivable, net comprises the following categories (in thousands):

	For the year ended December 31,	
	2025	**2024**
Product sales	$ 3,147	$ —
License revenues	3,000	—
Total accounts receivable, net	$ 6,147	$ —

Other Receivables

Other receivables include employee retention credits ("ERC"), sublease rent receivables and other miscellaneous receivables that are expected to be collected within the next twelve months. As of December 31, 2025 and 2024, the Company had ERC receivables of $0.5 million and $1.6 million, respectively, which was recorded in other receivables and as a component of other income, net in the consolidated statements of operations and comprehensive income (loss).

Concentration of Credit Risk and Off-Balance Sheet Risk

Financial instruments that subject the Company to credit risk primarily consist of cash and cash equivalents, short-term investments, accounts receivable, net and other receivables. The Company maintains its cash and cash equivalent balances with high-quality financial institutions and, consequently, the Company believes that such funds are subject to minimal credit risk. The Company is exposed to credit risk in the event of default by the financial institutions to the extent amounts recorded on the consolidated balance sheets are in excess of insured limits. The Company has not experienced any credit losses in such accounts and does not believe it is exposed to any significant credit risk on these funds. The Company's investment securities, which primarily consist of U.S. federal agency securities, U.S. treasury securities and certificates of deposit, potentially subject the Company to concentrations of credit risk. The Company has no financial instruments with off-balance sheet risk of loss.

Inventory and Costs of Sales

The Company capitalizes inventory costs associated with products when future economic benefit is expected to be realized. These costs consist of raw materials, manufacturing-related costs, personnel costs, facility costs, and other indirect overhead costs. Prior to receiving FDA approval for ZEVASKYN® in April 2025, the Company expensed costs related to inventory for clinical and pre-commercial purposes directly to research and development expense. Following the FDA's approval of ZEVASKYN®, the Company began capitalizing inventory related to commercialized products held for sale, in-process of production for sale, and raw materials to be used in the manufacturing of inventory.

The Company values its inventory at the lower-of-cost and net realizable value, on a first-in, first-out basis. The Company adjusts the net realizable value of any excess, obsolete or unsalable inventory in the period in which they are identified. Such impairment charges, should they occur, are recorded within cost of sales.

Cost of sales includes inventory and period costs related to overhead and manufacturing costs of ZEVASKYN® during the twelve months ended December 31, 2025, including costs associated with the manufacturing of non-conforming products. Prior to receiving FDA approval in April 2025, costs associated with the manufacturing of ZEVASKYN® were expensed as research and development costs.

Property and Equipment

Property and equipment are recorded at cost. Depreciation is provided using the straight-line method over estimated useful lives ranging from three to five years. Leasehold improvements are amortized over the shorter of the asset's useful life or the life of the lease term ranging from five to ten years. Expenditures for major renewals and betterments that extend the useful lives are capitalized. Expenditures for normal maintenance and repairs are expensed as incurred. The cost of assets sold or abandoned, and the related accumulated depreciation are eliminated from the accounts and any gains or losses are recognized in the accompanying consolidated statements of operations of the respective period.

Leases

The Company accounts for leases in accordance with ASC 842, *Leases*. Right-of-use lease assets represent the Company's right to use an underlying asset for the lease term and lease liabilities represent the Company's obligation to make lease payments arising from the lease. The measurement of lease liabilities is based on the present value of future lease payments over the lease term. As the Company's leases do not provide an implicit rate, the Company uses its incremental borrowing rate based on the information available at the lease commencement date in determining the present value of future lease payments. The right-of-use asset is based on the measurement of the lease liability and includes any lease payments made prior to or on lease commencement and excludes lease incentives and initial direct costs incurred, as applicable. Rent expense for the Company's operating leases is recognized on a straight-line basis over the lease term. The Company does not have any leases classified as finance leases.

The Company's leases do not have significant rent escalation, holidays, concessions, material residual value guarantees, material restrictive covenants or contingent rent provisions. The Company's leases include both lease (e.g., fixed payments including rent, taxes, and insurance costs) and non-lease components (e.g., common-area or other maintenance costs), which are accounted for as a single lease component as the Company has elected the practical expedient to group lease and non-lease components for all leases.

Most leases include one or more options to renew. The exercise of lease renewal options is typically at the Company's sole discretion; therefore, the majority of renewals to extend the lease terms are not included in the Company's right-of-use assets and lease liabilities as they are not reasonably certain of exercise. The Company regularly evaluates the renewal options and when they are reasonably certain of exercise, the Company includes the renewal period in its lease term.

Impairment of Long-Lived Assets

Long-lived assets consist of property and equipment, licensed technology, and right-of-use assets. The Company tests its long-lived assets for impairment when events and circumstances indicate that the carrying value of an asset or group of assets may not be fully recoverable. If indicators are present or changes in circumstance suggest that impairment may exist, the Company assesses the recoverability of the affected long-lived assets or group of assets by determining whether the carrying value of such assets or group of assets can be recovered through undiscounted future operating cash flows. If the carrying amount is not recoverable, the Company measures the amount of any impairment by comparing the carrying value of the asset or group of assets to its fair value.

Credit Losses

The Company reviews its available-for-sale investments for credit losses on a collective basis by major security type and in line with the Company's investment policy. As of December 31, 2025, the Company's available-for-sale investments were in securities that are issued by the U.S. treasury, U.S. federal agencies and certificates of deposits, are highly rated, and have a history of zero credit losses. The Company reviews the credit quality of its accounts receivables by monitoring the aging of its accounts receivable, the history of write offs for uncollectible accounts, and the credit quality of its significant customers, the current economic environment/macroeconomic trends, supportable forecasts, and other relevant factors. The Company's accounts receivables are with customers that do not have a history of uncollectibility nor a history of significantly aged accounts receivables. As of December 31, 2025, the Company did not recognize a credit loss allowance for its investments or accounts receivable.

Segments

The Company determines and presents operating segments based on the information that is internally provided to the Company's chief operating decision maker ("CODM"), its Chief Executive Officer, in accordance with ASC 280, *Segment Reporting*. The Company has determined that it operates in a single business segment, which is a commercial-stage biopharmaceutical company developing cell and gene therapies for life-threatening diseases. Refer to Note 17– Segment Information for further information related to the Company's segment.

Revenue Recognition

The Company accounts for contracts with customers in accordance with ASC 606, *Revenue from Contracts with Customers* ("ASC 606"). ASC 606 applies to all contracts with customers, except for contracts that are within the scope of other standards. Under ASC 606, an entity recognizes revenue when its customer obtains control of promised goods or services, in an amount that reflects the consideration that the entity expects to receive in exchange for those goods or services. To determine revenue recognition for arrangements that an entity determines are within the scope of ASC 606, the entity performs the following five steps: (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 a performance obligation. The Company only applies the five-step model to contracts when it is probable that the entity will collect the consideration it is entitled to in exchange for the goods or services it transfers to the customer.

At contract inception, once the contract is determined to be within the scope of ASC 606, the Company assesses the goods or services promised within each contract, determines those that are performance obligations and assesses whether each promised good or service is distinct. The Company then recognizes as revenue the amount of the transaction price that is allocated to the respective performance obligation when (or as) the performance obligation is satisfied.

As part of the accounting for these arrangements, the Company applies significant judgment to determine: (a) the number of performance obligations based on the determination under step (ii) above; (b) the transaction price under step (iii) above; and (c) the stand-alone selling price for each performance obligation identified in the contract for the allocation of transaction price in step (iv) above.

With respect to the transaction price, to the extent the transaction price includes variable consideration, the Company estimates the amount of variable consideration that should be included in the transaction price using the expected value method or most likely amount method, depending upon the nature of the underlying variable consideration. As it pertains to license agreement, the Company primarily applies the most likely amount method, except for sales-based royalties, to estimating variable consideration. The Company determines the standalone selling price for performance obligations in its contracts with customers using an adjusted market approach, until such time sales transaction volume is at sufficient level to establish standalone selling price using observable inputs.

Product Revenue

The Company generates revenue from sales in the United States of its commercially approved ZEVASKYN®. The Company's customers for ZEVASKYN® are qualified treatment centers. Revenue from product sales is a single performance obligation recognized at the point in time when the customer obtains control of the product, which is typically upon the completion of a final quality inspection of the product at the qualified treatment center. There is no obligation for the qualified treatment centers to use ZEVASKYN®, and the Company has no contractual right to receive payment until the final quality inspection of the product at the qualified treatment centers and transfer of control is completed.

The Company is a party to various commercial arrangements and government programs, which include payor rebates, co-payment assistance and prompt pay discounts, which impact the transaction price and represent forms of variable consideration. Revenue from product sales is reduced at the time of recognition for these forms of variable consideration. The Company's contracts can include the right to receive an outcomes-based rebate and a subsequent treatment discount of ZEVASKYN® under certain conditions. The Company has determined that the rebate and discount create a material right and allocates transaction consideration to ZEVASKYN® and the material right on a relative standalone selling price basis. The standalone selling price for ZEVASKYN® is the wholesale acquisition cost. The standalone selling price for the material right is determined by quantifying the discount a customer would receive upon exercise of the option adjusting for the likelihood the option will be exercised. Transaction consideration allocated to the material right is deferred and recognized when either (a) the subsequent purchase of ZEVASKYN® occurs, or (b) the time period during which a subsequent purchase of ZEVASKYN® could be made, expires. There was no deferral of revenue for the years ended December 31, 2025 or 2024.

License and other revenues

The Company enters into license agreements that are within the scope of ASC 606, under which it may exclusively license rights to research, develop, manufacture and commercialize its product candidates to third parties. The terms of these arrangements typically include payment to the Company of one or more of the following: non-refundable, upfront license fees; reimbursement of certain costs; customer option exercise fees; development, regulatory and commercial milestone payments; and royalties on net sales of licensed products.

If the license to the Company's intellectual property is determined to be distinct from the other performance obligations identified in the arrangement, the Company recognizes revenue from non-refundable, upfront fees allocated to the license when the license is transferred to the customer and the customer is able to use and benefit from the license. In assessing whether a performance obligation is distinct from the other performance obligations, the Company considers factors such as the research, development, manufacturing and commercialization capabilities of the collaboration partner and the availability of the associated expertise in the general marketplace. In addition, the Company considers whether the collaboration partner can benefit from a performance obligation for its intended purpose without the receipt of the remaining performance obligation, whether the value of the performance obligation is dependent on the unsatisfied performance obligation, whether there are other vendors that could provide the remaining performance obligation, and whether it is separately identifiable from the remaining performance obligation. For licenses that are combined with other performance obligation, the Company utilizes judgment to assess the nature of the combined performance obligation to determine whether the combined performance obligation is satisfied over time or at a point in time and, if over time, the appropriate method of measuring progress for purposes of recognizing revenue. The Company evaluates the measure of progress each reporting period and, if necessary, adjusts the measure of performance and related revenue recognition. The measure of progress, and thereby periods over which revenue should be recognized, are subject to estimates by management and may change over the course of the research and development and licensing agreement. Such a change could have a material impact on the amount of revenue the Company records in future periods.

Milestone Payments

At the inception of each arrangement that includes research or development milestone payments, the Company evaluates whether the milestones are considered probable of being achieved and estimates the amount to be included in the transaction price using the most likely amount method. If it is probable that a significant cumulative revenue reversal would not occur, the associated milestone value is included in the transaction price. Milestone payments that are not within the control of the Company or the licensee, such as regulatory approvals, are not considered probable of being achieved until those approvals are received. The Company evaluates factors such as the scientific, clinical, regulatory, commercial, and other risks that must be overcome to achieve the particular milestone in making this assessment. There is considerable judgment involved in determining whether it is probable that a significant cumulative revenue reversal would not occur. At the end of each subsequent reporting period, the Company re-evaluates the probability of achievement of all milestones subject to constraint and, if necessary, adjusts its estimate of the overall transaction price. Any such adjustments are recorded on a cumulative catch-up basis, which would affect revenue and earnings in the period of adjustment.

Collaborative Arrangements

The Company analyzes its collaboration arrangements to assess whether such arrangements 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 and therefore within the scope of ASC 808, *Collaborative Arrangements* ("ASC 808"). This assessment is performed throughout the life of the arrangement based on changes in the responsibilities of all parties in the arrangement. For collaboration arrangements within the scope of ASC 808 that contain multiple elements, the Company first determines which elements of the collaboration are deemed to be within the scope of ASC 808 and which elements of the collaboration are more reflective of a vendor-customer relationship and therefore within the scope of ASC 606. For elements of collaboration arrangements that are accounted for pursuant to ASC 808, an appropriate recognition method is determined and applied consistently, generally by analogy to ASC 606. Amounts that are owed to collaboration partners are recognized as an offset to collaboration revenue as such amounts are incurred by the collaboration partner. For those elements of the arrangement that are accounted for pursuant to ASC 606, the Company applies the five-step model described above under ASC 606.

Royalties

The Company has license agreements with various third parties. Under these agreements, the Company is obligated to pay royalty payments based on a percentage of net sales or sublicence revenues. Royalties are included in either accounts payable or accrued expenses in the consolidated balance sheets. See Note 13 – License/Supplier Agreements for details of the Company's license agreements and resulting royalties recognized.

Research and Development Expenses

Research and development costs are expensed as incurred. Research and development expenses include, but are not limited to, payroll and personnel expense, lab supplies, preclinical and development cost, clinical trial expense, manufacturing related to clinical phase products, regulatory, and consulting. The cost of materials and equipment or facilities that are acquired for research and development activities and that have alternative future uses are capitalized when acquired.

Selling, General and Administrative Expenses

Selling, general and administrative expenses primarily consist of personnel, contract personnel, personnel-related expenses to support the Company's administrative and operating activities, facility costs, professional expenses (i.e., legal, audit, advisory expenses) and commercial readiness and launch costs.

Income Taxes

Income taxes are accounted for under the asset and liability method. Deferred tax assets and liabilities are recognized for the future tax consequences attributable to differences between the consolidated financial statement carrying amounts of existing assets and liabilities and their respective tax bases and operating loss and tax credit carryforwards. Deferred tax assets and liabilities are measured using enacted tax rates expected to apply to taxable income in the years in which those temporary differences are expected to be recovered or settled. The effect on deferred tax assets and liabilities of a change in tax rates is recognized in income in the period that includes the enactment date. A valuation allowance is provided for deferred tax assets to the extent their realization is in doubt.

The Company accounts for uncertain income tax positions in accordance with ASC 740, *Income Taxes*. Interest costs and penalties related to income taxes are classified as interest expense and selling, general and administrative costs, respectively, in the consolidated financial statements. For the years ended December 31, 2025 and 2024, the Company did not recognize any uncertain tax positions, interest or penalty expense related to income taxes. The Company files U.S. federal and state income tax returns as necessary. The federal return generally has a three-year statute of limitations, and most states have a four-year statute of limitations; however, the taxing authorities are allowed to review the tax year in which the net operating loss was generated when the loss is utilized on a tax return. The Company currently does not have any open income tax audits.

Net Income (Loss) Per Share

Basic net income (loss) per share is computed by dividing net income (loss) attributable to common shareholders by the weighted-average number of shares of common stock outstanding during the period. The weighted average number of shares of common stock includes the weighted average effect of outstanding pre-funded warrants for the purchase of shares of common stock for which the remaining unfunded exercise price is $0.0001 or less per share. Diluted net income (loss) per share is computed based on the weighted average number of shares of common stock plus the effect of dilutive potential common shares outstanding during the period using the treasury stock method and if-converted method. Dilutive potential securities result from outstanding restricted stock, stock options, stock purchase warrants and conversion features in the Company's Loan Agreement (as defined in Note 10 – Debt). When the Company has a net loss during the period, the Company does not include the potential impact of dilutive securities in diluted net loss per share, as the impact of these items is anti-dilutive.

A reconciliation of the numerators and the denominators of the basic and diluted net income (loss) per share computations are as follows (in thousands, except share and per share amounts):

	For the year ended December 31,	
	2025	2024
Numerator:		
Net income (loss) used for basic net income (loss) per share	$ 71,183	$ (63,734)
Effect of dilutive securities:		
Fair value adjustments for warrant liabilities	(4,106)	—
Numerator for dilutive net income (loss) per share - net income (loss) available for common shareholders' after the effect of dilutive securities	$ 67,077	$ (63,734)
Denominator:		
Weighted average number of common shares outstanding - basic	52,952,917	41,048,206
Effect of dilutive shares:		
Shares of common stock issuable upon exercise of stock options	176,170	—
Shares of common stock underlying restricted stock	4,581,249	—
Shares of common stock issuable upon exercise of warrants	7,811,234	—
Shares of common stock issuable upon exercise of conversion feature of loan agreement	614,251	—
Dilutive potential common shares	13,182,904	—
Denominator for dilutive net income (loss) per share - adjusted weighted average shares used in computing net income (loss) per share - dilutive	66,135,821	41,048,206
Earnings per share:		
Basic income (loss) per common share	$ 1.34	$ (1.55)
Dilutive income (loss) per common share	$ 1.01	$ (1.55)

The following table sets forth the potential securities that could potentially dilute basic income (loss) per share in the future that were not included in the computation of diluted net income (loss) per share because to do so would have been anti-dilutive for the periods presented:

	For the year ended December 31,	
	2025	2024
Shares of common stock issuable upon exercise of stock options	—	176,587
Shares of common stock underlying restricted stock	—	3,320,811
Shares of common stock issuable upon exercise of conversion feature of loan agreement	—	614,251
Shares of common stock issuable upon exercise of warrants	1,804,474	9,987,560
Total	1,804,474	14,099,209

Stock-Based Compensation

The Company accounts for stock-based compensation expense in accordance with ASC 718, *Stock Based Compensation*. The Company measures the cost of the employee/director/consultant services received in exchange for an award of equity instruments based on the grant date fair value for the employees and directors and vesting date fair value for consultants of the award. The Company uses the Black-Scholes option pricing model to determine the fair value of options on the grant date which includes assumptions for expected volatility, risk-free interest rate, dividend yield and estimated expected term. The Company uses the closing price of its common stock as quoted on the Nasdaq to determine the fair value of restricted stock. The Company accounts for forfeitures as they occur, which may result in the reversal of compensation costs in subsequent periods as the forfeitures arise. The Company estimates the expected term using the "simplified" method, as outlined in SEC Staff Accounting Bulletin No. 107, "Share-Based Payment."

Derivative Liability

The Company accounts for the fair value of the conversion right embedded within the Loan and Security Agreement in accordance with the guidance in ASC 815, which requires the Company to bifurcate and separately account for the conversion feature as an embedded derivative contained in the Company's Loan and Security Agreement. Accordingly, the Company accounts for the conversion feature as a derivative liability in the consolidated balance sheet. Derivatives are measured at their fair value on the balance sheet. In determining the appropriate fair value, the Company uses a Monte Carlo simulation model, which incorporated assumptions and estimates to value the derivatives. The derivative liability is remeasured at each reporting period with the change in fair value recorded to change in fair value of warrant and derivative liabilities in the consolidated statement of operations and comprehensive income (loss) until the derivative is exercised, expired, reclassified, or otherwise settled. At September 30, 2024, the conversion feature no longer met the criteria of a derivative liability, and the derivative liability was reclassified to equity. There are no outstanding derivative liabilities as of December 31, 2025 or 2024.

Warrants

On May 7, 2024, the Company issued pre-funded warrants to purchase 6,142,656 shares of common stock, with an exercise price of $4.0699 per share (the "2024 Pre-Funded Warrants"). The 2024 Pre-Funded Warrants are classified as equity in accordance with ASC 815, *Derivatives and Hedging*, given the prefunded warrants are indexed to the Company's own shares of common stock and meet the requirements to be classified in equity. The 2024 Pre-Funded Warrants were recorded at their relative fair value at issuance in the stockholders' equity section of the consolidated balance sheet and the 2024 Pre-Funded Warrants are considered outstanding shares in the basic earnings per share calculation given their nominal exercise price. On June 24, 2024, December 2, 2024, and October 29, 2025, 700,000, 1,228,511, and 1,719,944, respectively, of the 2024 Pre-Funded Warrants were exercised, leaving 2,494,181 of 2024 Pre-Funded Warrants outstanding as of December 31, 2025.

On January 8, 2024, the Company issued warrants to purchase up to $2,400,000 worth of shares of the Company's common stock. On January 8, 2024, the January Warrants did not include an explicit share limit and the number of shares issuable under the warrant agreements were variable based on the exercise price and therefore the warrants were liability classified based on a Black-Scholes valuation in accordance with ASC 815 and were recorded at the closing date fair value of $0.2 million which was based on a Black-Scholes option pricing model. The warrants are revalued on each subsequent balance sheet date until such instruments are exercised or expire, with any changes in the fair value between reporting periods recorded in the consolidated statements of operations and comprehensive income (loss). On September 30, 2024, per the terms of the 2024 Loan Agreement Warrants, the exercise price and the number of shares became set at $4.07 per share and 589,681 shares, respectively, all of which are outstanding as of December 31, 2025.

In July 2025, as part of the Loan Agreement Amendment, see Note 10, the Company issued 16,474 common stock warrants, all of which are outstanding as of December 31, 2025. The July 2025 Avenue Warrants (as defined in Note 11 – Equity) expire on July 18, 2030, and have an exercise price per share equal to $6.07. The common stock warrants issued in connection with the Loan Agreement Amendment issuance were determined to be liability classified under ASC 815 as the common stock warrants were not considered indexed to the Company's stock.

On July 6, 2023, the Company issued pre-funded warrants to purchase 2,919,140 shares of common stock, with an exercise price of $4.0299 per share ("2023 Pre-Funded Warrants". The 2023 Pre-Funded Warrants are classified as equity in accordance with ASC 815, *Derivatives and Hedging*, given the prefunded warrants are indexed to the Company's own shares of common stock and meet the requirements to be classified in equity. The 2023 Pre-Funded Warrants were recorded at their relative fair value at issuance in the stockholders' equity section of the consolidated balance sheet and the 2023 Pre-Funded Warrants are considered outstanding shares in the basic earnings per share calculation given their nominal exercise price. On May 9, 2024, 300,000 of the 2023 Pre-Funded Warrants were exercised, leaving 2,619,140 2023 Pre-Funded Warrants outstanding as of December 31, 2025.

On November 3, 2022, the Company issued warrants to purchase 7,609,879 shares of common stock, with an exercise price of $4.75 per share, subject to customary adjustments thereunder. On August 25, 2025 and December 30, 2025, 1,086,956 and 760,870, respectively, of November 3, 2022 warrants were exercised, leaving 5,762,053 of the November 3, 2022 warrants outstanding as of December 31, 2025. On December 17, 2021, the Company issued warrants to purchase 1,788,000 shares of common stock, with an exercise price of $9.75 per share, subject to customary adjustments thereunder. The warrants issued in 2022 and 2021 were determined to be freestanding instruments as they are legally detachable and separately exercisable from each other and from the common stock issued. The common stock warrants are accounted for as liabilities in the consolidated balance sheets at their estimated fair value because they are not indexed to the Company's own stock. The warrants are revalued on each subsequent balance sheet date until such instruments are exercised or expire, with any changes in the fair value between reporting periods recorded in the consolidated statements of operations and comprehensive income (loss).

Recently Adopted Accounting Pronouncements

In December 2023, the FASB issued ASU 2023-09, Income Taxes (Topic 740): *Improvements to Income Tax Disclosures*. ASU 2023-09 is intended to enhance the transparency and decision usefulness of income tax information through improvements to income tax disclosures by requiring additional information related to the effective tax rate reconciliations, income taxes paid, and income tax expense and pretax income by jurisdiction. The Company adopted ASU 2023-09 effective January 1, 2025 on a prospective basis. Accordingly, the enhanced income tax disclosures are presented beginning in fiscal year 2025, and prior period disclosures have not been recast. The adoption of this guidance did not have an impact on the Company's consolidated results of operations, financial position, or cash flows, as the amendments relate solely to disclosure requirements. See Note 15 – Income Taxes for the related enhanced disclosures.

Recently Issued Accounting Pronouncements

In November 2024, the FASB issued ASU No. 2024-03, Income Statement – Reporting Comprehensive Income - Expense Disaggregation Disclosures (Subtopic 220-40): *Disaggregation of Income Statement Expenses*. The amendments in ASU 2024-03 address investor requests for more detailed expense information and require additional disaggregated disclosures in the notes to financial statements for certain categories of expenses that are included on the face of the income statement. This guidance is effective for fiscal years beginning after December 15, 2026, and interim periods within fiscal years beginning after December 15, 2027, with early adoption permitted. The Company is currently evaluating this guidance to determine the impact it may have on its consolidated financial statements.

In September 2025, the FASB issued ASU 2025-07, *Derivatives and Hedging (Topic 815) and Revenue from Contracts with Customers (Topic 606): Derivatives Scope Refinements and Scope Clarification for Share-Based Noncash Consideration from a Customer in a Revenue Contract*. The guidance in ASU 2025-07 refines the scope of derivative accounting under ASC 815 by expanding an existing scope exception to exclude certain non-exchange traded contracts with underlyings based on the operations or activities of one of the contract parties from derivative classification. The ASU also provides guidance under Topic 606 on the accounting for share-based noncash consideration received from a customer in a revenue contract, including measurement and timing considerations. ASU 2025-07 is effective for annual and interim periods beginning after December 15, 2026, with early adoption permitted. The Company is currently evaluating the impact of adopting ASU 2025-07.

In December 2025, the FASB issued ASU 2025-11, *Interim Reporting (Topic 270): Narrow-Scope Improvements.* This standard clarifies current interim reporting requirements on Topic 270 and introduces a disclosure principle requiring entities to disclose events since the end of the last annual reporting period that have a material impact on the entity. This standard will be effective for fiscal years beginning after December 15, 2027, with the option to apply it retrospectively. Early adoption is allowed. Currently, the Company is assessing the potential impact of this guidance on its consolidated financial statement disclosures.

NOTE 3 – REVENUE

Revenue comprises the following categories (in thousands):

	For the year ended December 31,	
	2025	2024
Product revenue, net	$ 2,420	$ —
License and other revenues	3,400	—
Total revenues	$ 5,820	$ —

Product revenue, net

The Company generates product revenue from sales of ZEVASKYN® in the United States. The Company ships and sells ZEVASKYN® directly to qualified treatment centers based on approved agreements. For these sales, the Company recognizes ZEVASKYN® revenue equal to the allocated transaction consideration at the point in time that the completion of a final quality inspection of the product is completed at the qualified treatment centers.

Revenue from product sales is reduced at the time of recognition for payor rebates, co-payment assistance and prompt pay discounts, which are attributed to various commercial arrangements and government programs. Product revenue was reduced by $0.7 million of government rebates based on contracted rebate rates for the year ended December 31, 2025. There were no co-payment assistance or prompt pay discounts for the year ended December 31, 2025.

The Company's contracts can include the right to receive an outcomes-based rebate and a subsequent treatment discount of ZEVASKYN® under certain conditions. The Company has determined that the rebate and discount create a material right and allocates transaction consideration to ZEVASKYN® and the material right on a relative standalone selling price basis. The standalone selling price for ZEVASKYN® is the wholesale acquisition cost. The standalone selling price for the material right is determined by quantifying the discount a customer would receive upon exercise of the option adjusting for the likelihood the option will be exercised. Transaction consideration allocated to the material right is deferred and recognized when either (a) the subsequent purchase of ZEVASKYN® occurs, or (b) the time period during which a subsequent purchase of ZEVASKYN® could be made, expires. There was no deferral of revenue or contract assets and liabilities for the years ended December 31, 2025 or 2024.

License and other revenues

The Company enters into license agreements that are within the scope of ASC 606, under which it may exclusively license rights to research, develop, manufacture and commercialize its product candidates to third parties. The terms of these arrangements typically include payment to the Company of one or more of the following: non-refundable, upfront license fees; reimbursement of certain costs; customer option exercise fees; development, regulatory and commercial milestone payments; and royalties on net sales of licensed products. See Note 13 – License/Supplier Agreements for detailed information on the Company's licenses agreements and revenues from these agreements.

Concentration of credit risk

Potential credit risk exposure for both ZEVASKYN® and licensed revenue has been evaluated for the Company's accounts receivable in accordance with ASC 326, *Financial Instruments – Credit Losses*. The loss percentage is calculated through the use of current and historical economic and financial information. As of December 31, 2025, there were no estimated losses applied to the accounts receivables balance.

The Company's total percentage of revenue and accounts receivable balances were comprised of the following concentrations from its largest customers, based on whose revenue or accounts receivable concentration is greater than 10% of total revenue or total accounts receivable in the periods disclosed below.

For the year ended and as of December 31, 2025	% of Revenue	% of Accounts Receivable
Customer 1	48.1%	51.2%
Customer 2	45.8%	48.8%

There was no revenue or accounts receivable as of December 31, 2024.

NOTE 4 – SHORT-TERM INVESTMENTS

The following table provides a summary of the short-term investments (in thousands):

	December 31, 2025			
	Amortized Cost	Gross Unrealized Gain	Gross Unrealized Loss	Fair Value
Available-for-sale, short-term investments:				
U.S. treasury securities	$ 25,057	31	—	$ 25,088
U.S. federal agency securities	17,772	2	—	17,774
Certificates of deposit	70,000	105	—	70,105
Total available-for-sale, short-term investments	$ 112,829	138	—	$ 112,967

	December 31, 2024			
	Amortized Cost	Gross Unrealized Gain	Gross Unrealized Loss	Fair Value
Available-for-sale, short-term investments:				
U.S. treasury securities	$ 23,990	—	(22)	$ 23,968
U.S. federal agency securities	40,365	10	—	40,375
Certificates of deposit	10,000	20	—	10,020
Total available-for-sale, short-term investments	$ 74,355	30	(22)	$ 74,363

As of December 31, 2025, the available-for-sale securities classified as short-term investments mature in one year or less. The Company carries its available-for-sale securities at fair value in the consolidated balance sheets. Unrealized losses on available-for-sale securities as of December 31, 2025, were not significant and were primarily due to changes in interest rates, including market credit spreads, and not due to increased credit risks associated with specific securities. None of the short-term investments have been in a continuous unrealized loss position for more than 12 months. Accordingly, no other-than-temporary impairment was recorded for the year ended December 31, 2025.

There were no significant realized gains or losses recognized on the sale or maturity of available-for-sale investments during the years ended December 31, 2025 or 2024.

NOTE 5 – INVENTORY

Inventory consists of the following (in thousands):

	As of December 31,	
	2025	2024
Raw materials	$ 5,493	$ —
Work-in-progress	—	—
Finished goods	—	—
Total inventory	$ 5,493	$ —

For the year ended December 31, 2025 and 2024, there were not inventory write-downs.

NOTE 6 – PROPERTY AND EQUIPMENT

Property and equipment are stated at cost and depreciated or amortized using the straight-line method based on useful lives as follows (in thousands):

		As of December 31,	
	Useful lives (years)	2025	2024
Laboratory equipment	5	$ 10,061	$ 8,868
Furniture, software and office equipment	3 to 5	1,962	1,113
Leasehold improvements	Shorter of remaining lease term or useful life	15,116	8,805
Construction-in-progress		—	624
Subtotal		27,139	19,410
Less: accumulated depreciation		(17,218)	(14,980)
Total property and equipment, net		$ 9,921	$ 4,430

In 2024, construction-in-progress related to leasehold improvements for the Company's new office space as well as for conversion of existing office space into additional manufacturing space to increase ZEVASKYN® manufacturing capacity, all of which was completed in 2025.

Depreciation and amortization on property and equipment was $2.5 million and $2.0 million for the years ended December 31, 2025 and 2024, respectively. The Company incurred a gain on disposal of nil and $2,000 during the years ended December 31, 2025 and 2024, respectively, which is reflected in other income, net in the consolidated statements of operations and comprehensive income (loss).

The Company capitalized into inventory $0.2 million relating to depreciation associated with manufacturing equipment and production facilities for the year ended December 31, 2025. The capitalized costs associated are added to inventory and are expensed through cost of sales in the consolidated statement of operations and comprehensive income (loss) upon the commercial sales of ZEVASKYN®.

NOTE 7 – FAIR VALUE MEASUREMENTS

The Company calculates the fair value of the Company's assets and liabilities that qualify as financial instruments and includes additional information in the notes to the consolidated financial statements when the fair value is different than the carrying value of these financial instruments. The estimated fair value of other receivables, prepaid expenses and other current assets, other assets, accounts payable, accrued taxes and accrued expenses approximate their carrying amounts due to the relatively short maturity of these instruments. The estimated fair value of the Loan Agreement (as Defined in Note 10 – Debt) as of December 31, 2025 and December 31, 2024, was $21.2 million and $24.7 million, respectively. Both observable and unobservable inputs were used to determine the fair value of long-term debt, which was classified within the Level 3 category.

U.S. GAAP defines fair value as the exchange price that would be received for an asset or paid to transfer a liability (an exit price) in the principal or most advantageous market for the asset or liability in an orderly transaction between market participants at the measurement date. This guidance establishes a three-level fair value hierarchy that prioritizes the inputs used to measure fair value. The hierarchy requires entities to maximize the use of observable inputs and minimize the use of unobservable inputs. The three levels of inputs used to measure fair value are as follows:

- Level 1 - Quoted prices in active markets for identical assets or liabilities.
- Level 2 - Observable inputs other than quoted prices included in Level 1, such as quoted prices for similar assets and liabilities in active markets; quoted prices for identical or similar assets and liabilities in markets that are not active; or other inputs that are observable or can be corroborated by observable market data.
- Level 3 - Unobservable inputs that are supported by little or no market activity and that are significant to the fair value of the assets and liabilities. This includes certain pricing models, discounted cash flow methodologies and similar valuation techniques that use significant unobservable inputs.

The Company has segregated all financial assets and liabilities that are measured at fair value on a recurring basis (at least annually) into the most appropriate level within the fair value hierarchy based on the inputs used to determine the fair value at the measurement date in the table below.

The following table provides a summary of financial assets and liabilities measured at fair value on a recurring and non-recurring basis (in thousands):

Description	Fair Value at December 31, 2025	Level 1	Level 2	Level 3
Recurring Assets				
Cash equivalents				
Money market funds	$ 73,854	$ 73,854	$ —	$ —
Money market deposit account	182	182	—	—
Short-term investments				
U.S. treasury securities	25,088	25,088	—	—
U.S. federal agency securities	17,774	—	17,774	—
Certificates of deposit	70,105	—	70,105	—
Total assets measured at fair value	$ 187,003	$ 99,124	$ 87,879	$ —
Liabilities				
Warrant liabilities	$ 18,902	$ —	$ —	$ 18,902
Total liabilities measured at fair value	$ 18,902	$ —	$ —	$ 18,902

Description	Fair Value at December 31, 2024	Level 1	Level 2	Level 3
Recurring Assets				
Cash equivalents				
Money market funds	$ 17,627	$ 17,627	$ —	$ —
Money market deposit account	5,109	5,109	—	—
Short-term investments				
U.S. treasury securities	23,968	23,968	—	—
U.S. federal agency securities	40,375	—	40,375	—
Certificates of deposit	10,020	—	10,020	—
Total assets measured at fair value	$ 97,099	$ 46,704	$ 50,395	$ —
Liabilities				
Warrant liabilities	$ 32,014	$ —	$ —	$ 32,014
Total liabilities measured at fair value	$ 32,014	$ —	$ —	$ 32,014

Warrant Liabilities

As of December 31, 2025 and 2024, the Company had the following outstanding warrants that are classified as warrant liabilities:

	As of December 31,	
	2025	2024
Warrants issued as part of the 2021 public offering, expiration date December 2026, exercise price of $9.75 per share	1,788,000	1,788,000
Warrants issued as part of the 2022 Private Placement Offering, expiration date November 2027, exercise price $4.75 per share	5,762,503	7,609,879
Warrants issued as part of the 2024 Loan Agreement, expiration date January 2029, exercise price $4.07 per share	589,681	589,681
Warrants issued as part of the 2024 Loan Agreement Amendment, expiration date July 2030, exercise price $6.07 per share	16,474	—

The common stock warrants related to the 2021 Public Offering and the 2022 Private Placement are not indexed to the Company's own stock and therefore have been classified as liabilities at their estimated fair value. The common stock warrants issued in connection with the Loan Agreement issuance were determined to be liability classified under ASC 815, *Derivatives and Hedg*ing ("ASC 815") as the common stock warrants were not considered indexed to the Company's stock. Changes in the estimated fair value of the warrant liabilities are recorded as changes in fair value of warrant liabilities in the consolidated statement of operations and comprehensive income (loss).

In January 2024, as part of the Loan Agreement, see Note 10 – Debt, the Company issued warrants to purchase $2.4 million worth of shares of the Company's stock which have an exercise price of $4.07 per share and the shares issuable were calculated at 589,681 shares. In July 2025, as part of the Loan Agreement Amendment, see Note 10 – Debt, the Company issued 16,474 common stock warrants. The July 2025 Avenue Warrants (as defined in Note 11 – Equity) expire on July 18, 2030, and have an exercise price per share equal to $6.07. The common stock warrants issued in connection with the Loan Agreement and the Loan Agreement Amendment were determined to be liability classified under ASC 815 as the common stock warrants were not considered indexed to the Company's stock.

Changes in the estimated fair value of the warrant liabilities is recorded as changes in fair value of warrant liabilities in the consolidated statement of operations and comprehensive income (loss).

The following table provides a summary of the activity on the warrant liabilities (in thousands):

	As of December 31,	
	2025	2024
Beginning warrant liabilities	$ 32,014	$ 31,352
Issuance of warrants	80	220
Reclassification of warrants to equity as part of warrant exercise	(7,053)	—
(Gain) loss recognized in earnings from change in fair value	(6,139)	442
Ending warrant liabilities	$ 18,902	$ 32,014

The warrant liabilities are valued using significant inputs not observable in the market. Accordingly, the warrant liability is measured at fair value on a recurring basis using unobservable inputs and are classified as Level 3 inputs within the fair value hierarchy. Fair value measurements categorized within Level 3 are sensitive to changes in the assumptions or methodology used to determine fair value and such changes could result in a significant increase or decrease in the fair value. The Company's valuation of the common stock warrants utilized the Black-Scholes option-pricing model, which incorporated assumptions and estimates to value the common stock warrants. The Company assessed these assumptions and estimates at the end of each reporting period.

The following table outlines the key inputs for the Black-Scholes option-pricing model:

	As of December 31,	
	2025	2024
Common share price	$5.27	$5.57
Expected term (years)	0.96 – 4.54	1.96 – 4.02
Risk-free interest rate (%)	3.41% – 3.63%	4.16% – 4.24%
Volatility (%)	78.97% – 100.00%	92.64% – 100.00%
Expected dividend yield (%)	0%	0%

Derivative Liabilities

The Conversion Right embedded within the Loan Agreement (see Note 10 – Debt below) required bifurcation as certain adjustments to the conversion price were not indexed to the Company's own stock and therefore the Conversion Right was recorded as a derivative liability. The derivative liability is remeasured at each reporting period with the change in fair value recorded to changes in fair value of warrants and derivative liabilities in the consolidated statement of operations and comprehensive income (loss) until the derivative is exercised, expired, reclassified, or otherwise settled.

On September 30, 2024, pursuant to the Loan Agreement, the conversion price was fixed at $4.88 and is considered indexed to the Company's own stock. At September 30, 2024, the Conversion Right no longer met the criteria of a derivative liability, and the derivative liability was reclassified to equity.

The following table provides a summary of the activity on the derivative liabilities (in thousands):

	As of December 31,	
	2025	2024
Beginning derivative liabilities	$ —	$ —
Fair value of derivatives issued in connection with Loan Agreement	—	822
Loss recognized in earnings from change in fair value	—	313
Reclassification of derivative liability in connection with the Loan Agreement	—	(1,135)
Ending derivative liabilities	$ —	$ —

NOTE 8 – ACCRUED EXPENSES

The following table provides a summary of the components of accrued expenses (in thousands):

	As of December 31,	
	2025	2024
Accrued employee compensation	$ 5,636	$ 4,392
Accrued contracted services and other	2,104	1,941
Accrued rebates	727	—
Total accrued expenses	$ 8,467	$ 6,333

NOTE 9 – LEASES

The Company leases space under operating leases for administrative, manufacturing and laboratory facilities in Cleveland, Ohio. The Company leased office space in New York, New York, which the Company sublet. The lease for office space in New York, New York terminated in September 2025, which was the end of the lease term. The Company also leases certain office equipment under operating leases, which have a non-cancelable lease term of less than one year and the Company has elected the practical expedient to exclude these short-term leases from the Company's right-of-use assets and lease liabilities.

During 2024, the Company signed a lease for 16,566 square feet of office space at 6700 Euclid Avenue, Cleveland, Ohio. Pursuant to the lease agreement, the lease term commences on January 1, 2025, with an initial term through December 30, 2030. Annual lease payments during the term of the lease are approximately $0.3 million. The total lease payments over the duration of the lease term are approximately $1.5 million. The impact of this lease agreement was to increase the Company's operating right-of-use lease assets and operating lease liabilities by $1.0 million on January 1, 2025.

During 2022 and 2023, the Company entered into two sublease agreements with unrelated third parties to occupy the Company's administrative offices in New York, New York. The sublease agreements terminated in September 2025 at the same time the Company's lease terminated.

The following table provides a summary of the Company's operating lease liabilities (in thousands):

	As of December 31,	
	2025	2024
Current operating lease liability	$ 864	$ 823
Non-current operating lease liability	4,069	3,262
Total operating lease liability	$ 4,933	$ 4,085

Lease costs and rent are reflected in selling, general and administrative expenses and research and development expenses in the consolidated statements of operations and comprehensive income (loss), as determined by the underlying activities.

The following table provides a summary of the components of lease costs and rent (in thousands):

	For the year ended December 31,	
	2025	2024
Operating lease cost	$ 1,401	$ 1,288
Variable lease cost	421	380
Short-term lease cost	46	49
Total operating lease costs	$ 1,868	$ 1,717

Cash paid for amounts included in the measurement of operating lease liabilities was $1.7 million and $1.3 million for the years ended December 31, 2025 and 2024, respectively. Cash received as part of tenet leasehold improvement allowance was $0.7 million for the year ended December 31, 2025. There was no cash received for the year ended December 31, 2024.

Future minimum lease payments and obligations, which do not include short-term leases, related to the Company's operating lease liabilities as of December 31, 2025 were as follows (in thousands):

Future minimum lease payments and obligations	Operating Leases
2026	$ 864
2027	1,295
2028	1,325
2029	1,357
2030	1,387
Total undiscounted operating lease payments	6,228
Less: imputed interest	1,295
Present value of operating lease liabilities	$ 4,933

The weighted-average remaining term of the Company's operating leases was 60 months, and the weighted-average discount rate used to measure the present value of the Company's operating lease liabilities was 8.7% as of December 31, 2025.

The Company received $0.4 million and $0.6 million during the years ended December 31, 2025 and 2024, respectively, of sublease income which is recorded in other income, net on the consolidated statements of operations and comprehensive income (loss). The sublease ended on September 30, 2025, and there are no future cash receipts.

NOTE 10 – DEBT

The following table provides a summary of the Company's debt, net of debt issuance costs and discounts (in thousands):

	As of December 31,	
	2025	**2024**
Loan Agreement Principal	$ 20,000	$ 20,000
Accreted final payment fee	711	354
Unamortized debt issuance costs and discounts	(676)	(1,391)
Total long-term debt	20,035	18,963
Less: current maturities	12,222	5,926
Long-term debt, net of current maturities	$ 7,813	$ 13,037

Loan and Security Agreement

On January 8, 2024 (the "Closing Date"), the Company entered into a Loan and Security Agreement, as supplemented by a Supplement, dated as of January 8, 2024 (collectively, the "Loan Agreement") with Avenue Venture Opportunities Fund, L.P., a Delaware limited partnership, as administrative agent and collateral agent ("Avenue" and the "Agent") and Avenue Venture Opportunities Fund II, L.P., a Delaware limited partnership ("Avenue 2" and, together with Avenue, the "Lenders"). The Loan Agreement provides for senior secured term loans (the "Loans") in an aggregate principal amount up to $50 million, with (i) a committed tranche of $20 million advanced on the Closing Date ("Tranche 1"), (ii) a committed tranche of up to $10 million which may be advanced upon the request of the Company between June 30, 2024 and September 30, 2024, subject to the Company obtaining FDA approval of ZEVASKYN® in RDEB, with the issuance of a Priority Review Voucher ("Tranche 2"), and (iii) a discretionary tranche of up to $20 million which may be advanced between March 31, 2025 and March 31, 2026 (the "Discretionary Tranche") provided at the discretion of the Lenders. The Loans are due and payable on July 1, 2027. As of September 30, 2024, the Tranche 2 was no longer available as the Company did not meet the Tranche 2 criteria.

The loan principal is repayable in equal monthly installments beginning on February 1, 2026. On April 28, 2025, with the FDA approval of ZEVASKYN® and in accordance with the Loan Agreement, the start date of the loan principal monthly installments was extended from May 1, 2025 to February 1, 2026. The Loans bear interest at a rate per annum (subject to increase during an event of default) equal to the greater of (i) the prime rate, as published by the Wall Street Journal from time to time, plus 5.00% and (ii) 13.50%. On July 18, 2025, the Company entered into an amendment (the "Amendment") to the Loan Agreement that reduces the interest rate for senior secured term loan owed under the Loan Agreement from 13.5% to a fixed rate of 11.75% per annum. The stated interest rate and effective interest rate as of December 31, 2025 was 11.75% and 18.42%, respectively. In connection with the Amendment, the Company issued the Lenders warrants to purchase up to an aggregate of 16,474 shares of Company common stock (collectively, the "July 2025 Avenue Warrants"). The July 2025 Avenue Warrants expire on July 18, 2030, and have an exercise price per share equal to $6.07.

The Company may, subject to certain parameters, voluntarily prepay the Loans, in whole, at any time. If prepayment occurs after January 8, 2025 and on or before January 8, 2026, the Company is required to pay a fee equal to 2.00% of the principal amount of the Loans; if prepayment occurs after January 8, 2026, the Company is required to pay a fee equal to 1.00% of the principal amount of the Loans. A final payment fee of 5.00% of the principal amount of the funded Tranche 1 Loans, Tranche 2 Loans and Discretionary Tranche Loans is also due upon maturity on July 1, 2027, or any earlier date of prepayment.

The Company's obligations under the Loan Agreement are secured by a pledge of substantially all of the Company's assets. Pursuant to the Loan Agreement, the Company is subject to a financial covenant requiring the Company to maintain at all times $5 million in unrestricted cash. The Loan Agreement also contains affirmative and negative covenants customary for financings of this type that, among other things, limit the ability of the Company and its subsidiaries to (i) incur additional debt, guarantees or liens; (ii) pay dividends; (iii) enter into certain change of control transactions; (iv) sell, transfer, lease, license, or otherwise dispose of certain assets; (v) make certain investments or loans; and (vi) engage in certain transactions with related persons, in each case, subject to certain exceptions. The Loan Agreement also includes events of default customary for financings of this type, in certain cases subject to customary periods to cure, following which the Agent may accelerate all amounts outstanding under the Loans.

Pursuant to the Supplement to the Loan and Security Agreement, Avenue also has the right to convert up to $3 million of the outstanding principal of the Loans into shares of Company common stock (the "Conversion Right") at a price per share equal to 120% of the exercise price of the Warrants (further discussed below) at any time while the Loans are outstanding, subject to certain terms and conditions, including ownership limitations. The Conversion Right required bifurcation as certain adjustments to the conversion price were not indexed to the Company's own stock and therefore the Conversion Right was recorded as a derivative liability. On January 8, 2024, the Conversion Right was recorded at the closing date fair value of $0.8 million which was based on a Monte Carlo simulation model. The derivative liability is remeasured at each reporting period with the change in fair value recorded to change in fair value of warrants and derivative liabilities in the consolidated statement of operations and comprehensive income (loss) until the derivative is exercised, expired, reclassified, or otherwise settled. On September 30, 2024, pursuant to the Loan Agreement, the conversion price was fixed at $4.88 and is considered indexed to the Company's own stock. On September 30, 2024, the Conversion Right no longer met the criteria of a derivative liability and the derivative liability of $1.1 million was reclassified to equity.

In addition, subject to applicable law and specified provisions set forth in the Supplement to the Loan and Security Agreement and solely to the extent permitted under applicable stock exchange rules without requiring stockholder approval, the Lenders may participate in certain equity financing transactions of the Company in an aggregate amount of up to $1 million on the same terms, conditions and pricing offered by the Company to other investors participating in such financing transactions (such right, the "Participation Right"). The Participation Right automatically terminates upon the earliest of (i) July 1, 2027, (ii) such time that the Lenders have purchased $1.0 million of the Company's equity securities in the aggregate pursuant to the Participation Right, and (iii) the repayment in full of all of the obligations under the Loan Agreement.

On the Closing Date and pursuant to the funding of Tranche 1 of the Loan Agreement, the Company issued to each of Avenue and Avenue 2 (collectively, the "Warrant Holders") warrants to purchase up to $480,000 and $1,920,000 of Company common stock, respectively, which is more fully described in Note 11 – Equity below.

The future payment obligations of the principal are as follows (in thousands):

2026	$	12,222
2027		7,778
Total principal	$	20,000

NOTE 11 – EQUITY

Preferred Stock

The aggregate number of authorized shares of the Company's preferred stock is 2,000,000 shares with a par value of one cent ($0.01). There is no preferred stock outstanding as of December 31, 2025 and 2024.

Common Stock and Warrants

Public Offerings

On December 21, 2021, the Company closed an underwritten public offering of 1,788,000 shares of common stock at a public offering price of $9.75 per share and stock purchase warrants to purchase 1,788,000 shares of common stock at an exercise price of $9.75. The net proceeds to the Company were $16.0 million, after deducting $1.5 million of underwriting discounts and commissions and offering expenses payable by the Company. The net proceeds were allocated to the warrant liability as noted below with the remainder of $7.0 million recorded in common stock and additional paid-in capital. In the event of certain fundamental transactions involving the Company, the holders of the stock purchase warrants may require the Company to make a payment based on a Black-Scholes valuation, using specific inputs that are not considered indexed to the Company's stock in accordance with ASC 815, *Derivatives and Hed*ging ("ASC 815"). Therefore, the Company accounted for the stock purchase warrants as liabilities, which were recorded at the closing date fair value of $9.0 million which was based on a Black-Scholes option pricing model. The remainder of the proceeds were allocated to common stock issued and recorded as a component of equity.

As of December 31, 2025, there were 1,788,000 stock purchase warrants outstanding related to this public offering. These stock purchase warrants expire on December 21, 2026. During such time as each warrant is outstanding, the holder of the warrant is entitled to participate in any dividends or other distribution of assets to holders of shares of common stock. There was no warrant activity during the year ended December 31, 2025 and 2024, other than the change in fair value of the warrants for the stock purchase warrants issued as part of this public offering.

On May 7, 2024, the Company sold 12,285,056 shares of its common stock and, in lieu of common stock, pre-funded warrants to purchase 6,142,656 shares of its common stock (the "2024 Pre-Funded Warrants"), for an aggregate purchase price of $75.0 million gross, or $70.2 million net of related costs. The offering price for each share of common stock was $4.07, and the offering price for the 2024 Pre-Funded Warrants was $4.0699, which represents the per share offering price for the Company's common stock less a $0.0001 per share exercise price for each 2024 Pre-Funded Warrant. The 2024 Pre-Funded Warrants are immediately exercisable at a nominal exercise price of $0.0001 per share and may be exercised at any time until the pre-funded warrants are exercised in full. On June 24, 2024, 700,000 of the 2024 Pre-Funded Warrants were exercised, on December 2, 2024, 1,228,531 of the 2024 Pre-Funded Warrants were exercised, and on October 29, 2025, 1,719,944 of the 2024 Pre-Funded Warrants were exercised, leaving 2,494,181 2024 Pre-Funded Warrants outstanding as of December 31, 2025. The 2024 Pre-Funded Warrants are classified as equity in accordance with ASC 815, given the prefunded warrants are indexed to the Company's own shares of common stock and meet the requirements to be classified in equity. The 2024 Pre-Funded warrants were recorded at their relative fair value at issuance in the stockholders' equity section of the consolidated balance sheet and the 2024 Pre-Funded Warrants are considered outstanding shares in the basic and diluted earnings per share calculation for the year ended December 31, 2025 and 2024 given their nominal exercise price.

Open Market Sale Agreement

On August 17, 2018, the Company entered into an open market sale agreement (as amended, the "ATM Agreement") with Jefferies LLC ("Jefferies") pursuant to which, the Company may sell from time to time, through Jefferies, shares of its common stock for an aggregate sales price of up to $75.0 million. Any sales of shares pursuant to this agreement are made under the Company's effective "shelf" registration statement on Form S-3 that is on file with and has been declared effective by the SEC.

The Company sold 3,510,889 and 2,825,954 shares of its common stock under the ATM Agreement during the years ended December 31, 2025 and 2024, respectively, resulting in net proceeds of $17.3 million and $15.5 million during the years ended December 31, 2025 and 2024, respectively.

Private Placement Offering

On November 3, 2022, the Company sold 7,065,946 shares of its common stock, and in lieu of shares of common stock, pre-funded warrants exercisable for 543,933 shares of common stock and accompanying warrants to purchase 7,609,879 shares of its common stock to a group of new and existing institutional investors in a private placement. The offering price for each share of common stock and accompanying warrant was $4.60, and the offering price for each pre-funded warrant and accompanying warrant was $4.59, which equaled the offering price per share of the common stock and accompanying warrant, less the $0.01 per share exercise price of each pre-funded warrant. Each accompanying warrant represents the right to purchase one share of the Company's common stock at an exercise price of $4.75 per share of common stock. The pre-funded warrants were exercised in December 2022 and converted to 543,933 shares of common stock. Total shares sold and converted during the year ended December 31, 2022 were 7,609,879 for an aggregate purchase price of $35.0 million gross, or $32.6 million net of related costs of $1.5 million which was expensed to selling, general and administrative expenses and $0.9 million which was recorded as a reduction to additional paid-in-capital. The net proceeds were allocated to the warrant liability as noted below with the remainder of $12.9 million and $0.1 million recorded in additional paid-in capital and common stock, respectively.

In the event of certain fundamental transactions involving the Company, the holders of the stock purchase warrants may require the Company to make a payment based on a Black-Scholes valuation, using specific inputs that are not considered indexed to the Company's stock in accordance with ASC 815. Therefore, the Company is accounting for the stock purchase warrants as liabilities. On November 3, 2022, the stock purchase warrants were recorded at the closing date fair value of $22.0 million which was based on a Black-Scholes option pricing model. The remainder of the proceeds were allocated to common stock issued and recorded as a component of equity.

As of December 31, 2025, there were 5,762,053 warrants outstanding related to this private placement offering. The warrants expire on November 3, 2027. During such time as each warrant is outstanding, the holder of the warrant is entitled to participate in any dividends or other distribution of assets to holders of shares of common stock. In August 2025, 1,086,956 warrants were exercised for proceeds of $5.2 million. In December 2025, 760,870 warrants were exercised for proceeds of $3.6 million. Other than noted above, there was no additional warrant activity during the years ended December 31, 2025 and 2024, other than the change in fair value of the warrants.

Direct Placement Offering

On July 6, 2023, the Company sold 3,284,407 shares of its common stock, and in lieu of shares of common stock, pre-funded warrants exercisable for 2,919,140 shares of common stock (the "2023 Pre-Funded Warrants"), to a group of existing institutional investors for an aggregate purchase price of $25.0 million gross, or $23.0 million net of related costs. The offering price for each share of common stock was $4.03, and the offering price for the 2023 Pre-Funded Warrants was $4.0299, which represents the per share offering price for the Company's common stock less a $0.0001 per share exercise price for each such 2023 Pre-Funded Warrant. The 2023 Pre-Funded Warrants are immediately exercisable at a nominal exercise price of $0.0001 per share, may be exercised at any time and do not have an expiration date. On May 9, 2024, 300,000 of the 2023 Pre-Funded Warrants were exercised, leaving 2,619,140 2023 Pre-Funded Warrants outstanding as of December 31, 2025. The 2023 Pre-Funded Warrants are classified as equity in accordance with ASC 815, given the 2023 Pre-Funded Warrants are indexed to the Company's own shares of common stock and meet the requirements to be classified in equity. The 2023 Pre-Funded Warrants were recorded at their relative fair value at issuance in the stockholders' equity section of the consolidated balance sheet and the 2023 Pre-Funded Warrants are considered outstanding shares in the basic and diluted earnings per share calculation for the years ended December 31, 2025 and 2024 given their nominal exercise price.

Common Stock Warrants related to the Loan and Security Agreement

On January 8, 2024, in connection with entering into the Loan and Security Agreement, the Company issued to the Warrant Holders warrants to purchase up to $0.5 million and $1.9 million worth of shares, respectively, of Company common stock (collectively, the "January Warrants"). The January Warrants expire on January 8, 2029 and upon issuance, had an exercise price per share equal to the lesser of (i) $4.75 and (ii) the price per share of the Company's next bona fide round of equity financing before September 30, 2024 in which the Company sells or issues shares of its common stock, excluding certain excluded issuances as defined in the Supplement. In connection with the underwritten common stock offering consummated on May 7, 2024, and pursuant to the term of the January Warrants, the exercise price of the January Warrants was reduced to $4.07 per share for 589,681 shares. In addition, upon a change of control where the per share price of the Company common stock is less than or equal to two times that of the exercise price, the Warrant Holders would be entitled to receive the shares of common stock underlying the January Warrants without payment of the exercise price. On January 8, 2024, the January Warrants did not include an explicit share limit and the number of shares issuable under the warrant agreements were variable based on the exercise price, therefore, the January Warrants were liability classified based on a Black-Scholes valuation in accordance with ASC 815 and were recorded at the closing date fair value of $0.2 million which was based on a Black-Scholes option pricing model. On September 30, 2024, per the terms of the January Warrants, the exercise price and the number of shares issuable became set at $4.07 per share and 589,681 shares, respectively.

The Warrant Holders may exercise the January Warrants at any time, or from time to time up to and including January 8, 2029, by making a cash payment equal to the exercise price multiplied by the quantity of shares. The Warrant Holders may also exercise the January Warrants on a cashless basis by receiving a net number of shares calculated pursuant to the formula set forth in the January Warrants. The January Warrants are subject to anti-dilution adjustments for stock dividends, stock splits, and reverse stock splits.

On July 18, 2025, in connection with entering into the Loan Agreement Amendment, the Company issued the Lenders warrants to purchase up to an aggregate of 16,474 shares of Company common stock (collectively, the "July 2025 Avenue Warrants"). The July 2025 Avenue Warrants expire on July 18, 2030 and have an exercise price per share equal to $6.07. In the event of certain fundamental transactions involving the Company, the holders of the stock purchase warrants may require the Company to make a payment based on a Black-Scholes valuation, using specific inputs that are not considered indexed to the Company's stock in accordance with ASC 815. Therefore, the Company accounted for the stock purchase warrants as liabilities, which were recorded at the closing date fair value of $0.1 million which was based on a Black-Scholes option pricing model.

NOTE 12 – STOCK-BASED COMPENSATION

Prior to May 17, 2023, the Company had previously granted stock options and stock awards under the Abeona Therapeutics Inc. 2015 Equity Incentive Plan (the "2015 Incentive Plan"). As of May 17, 2023, no further grants can be made under the 2015 Incentive Plan. The Company now grants stock options and stock awards under the Abeona Therapeutics Inc. 2023 Equity Incentive Plan (the "2023 Incentive Plan") which was approved by stockholders on May 17, 2023. On April 24, 2024, stockholders approved an amendment to the 2023 Incentive Plan to increase the shares authorized for issuance from 1,700,000 shares to 3,200,000 shares. On December 20, 2024, stockholders approved an additional increase in the shares authorized for issuance under the 2023 Incentive Plan from 3,200,000 shares to 8,400,000 shares. As of December 31, 2025, there were 3,298,589 shares available to be granted under the 2023 Incentive Plan. In addition, in 2023, the Company's board of directors approved various restricted stock awards granted to certain new hires as inducement grants. On October 10, 2023, the Company's board of directors approved the Abeona Therapeutics Inc. 2023 Employment Inducement Equity Incentive Plan (the "Inducement Plan"). As of December 31, 2025, there were 214,284 shares available to be granted under the Inducement Plan.

The following table summarizes stock-based compensation (in thousands):

	For the year ended December 31,	
	2025	**2024**
Research and development	$ 1,355	$ 1,561
Selling, general and administrative	9,424	5,067
Total stock-based compensation expense	$ 10,779	$ 6,628

Stock Options

The Company estimates the fair value of each option award on the date of grant using the Black-Scholes option-pricing model. The Company then recognizes the grant date fair value of each option as compensation expense ratably using the straight-line attribution method over the service period (generally the vesting period). The Black-Scholes model incorporates the following assumptions:

- Expected volatility – the Company estimates the volatility of the share price at the date of grant using a "look-back" period which coincides with the expected term, defined below. The Company believes using a "look-back" period which coincides with the expected term is the most appropriate measure for determining expected volatility.

- Expected term – the Company estimates the expected term using the "simplified" method, as outlined in SEC Staff Accounting Bulletin No. 107, "Share-Based Payment."

- Risk-free interest rate – the Company estimates the risk-free interest rate using the U.S. Treasury yield curve for periods equal to the expected term of the options in effect at the time of grant.

- Dividends – the Company uses an expected dividend yield of zero because the Company has not declared nor paid a cash dividend, nor are there any plans to declare a dividend.

The Company did not grant any stock options in the year ended December 31, 2025 and 2024.

The Company accounts for forfeitures as they occur, which may result in the reversal of compensation costs in subsequent periods as the forfeitures arise.

The following table summarizes stock option activity during the year ended December 31, 2025 and 2024.

	Number of Options	Weighted Average Exercise Price	Weighted Average Remaining Contractual Term (years)	Aggregate Intrinsic Value (in thousands)
Outstanding at December 31, 2023	179,001	$ 38.58	6.83	$ 3
Granted	—	$ —	—	$ —
Cancelled/forfeited	(2,414)	$ 33.84	—	$ —
Exercised	—	$ —	—	$ —
Outstanding at December 31, 2024	176,857	$ 38.64	5.83	$ 6
Granted	—	$ —	—	$ —
Cancelled/forfeited	(568)	$ 14.21	—	$ —
Exercised	—	$ —	—	$ —
Outstanding at December 31, 2025	176,019	$ 38.72	4.85	$ 5
Exercisable	175,489	$ 38.82	4.84	$ 4
Unvested	530	$ 4.48	6.38	$ 1

The aggregate intrinsic value of options is calculated as the difference between the exercise price of the underlying options and the fair value of the Company's common stock for those options that had exercise prices lower than the fair value of the Company's common stock. As of December 31, 2025, the total compensation cost related to non-vested option awards not yet recognized was $2,000 with a weighted average remaining vesting period of 0.4 years.

Further information regarding options outstanding under the 2015 Incentive Plan as of December 31, 2025 is summarized below:

Range of Exercise Prices		Number of Options Outstanding	Weighted-Average Remaining Life In Years	Weighted-Average Exercise Price	Number of Options Exercisable	Weighted-Average Remaining Life in Years	Weighted-Average Exercise Price
$ 4.00	$ 22.75	19,520	6.0	$ 16.58	18,990	6.0	$ 16.92
25.50	47.00	104,279	4.5	33.52	104,279	4.5	33.52
54.50	58.50	52,020	5.2	56.96	52,020	5.2	56.96
164.75	183.50	200	3.1	164.75	200	3.1	164.75
		176,019			175,489		

Restricted Stock:

The following table summarizes restricted stock award activity:

	Number of Awards	Weighted Average Grant Date Fair Value Per Unit
Outstanding at December 31, 2023	2,448,169	$ 4.25
Granted	2,065,054	$ 4.95
Cancelled/forfeited	(183,114)	$ 3.78
Vested	(1,009,298)	$ 4.64
Outstanding at December 31, 2024	3,320,811	$ 4.60
Granted	2,405,231	$ 5.30
Cancelled/forfeited	(82,784)	$ 5.00
Vested	(1,462,277)	$ 4.71
Outstanding at December 31, 2025	4,180,981	$ 4.96

As of December 31, 2025, there was $13.7 million of total unrecognized compensation expense related to unvested restricted stock awards, which is expected to be recognized over a weighted average vesting period of 1.8 years. The total fair value of restricted stock awards that vested was $6.9 million and $4.7 million during the years ended December 31, 2025 and 2024, respectively.

NOTE 13 – LICENSE/SUPPLIER AGREEMENTS

License Agreement Relating to Recessive Dystrophic Epidermolysis Bullosa (RDEB)

In 2016, the Company entered into two licensing agreements between the Company and The Board of Trustees of Leland Stanford Junior University (“Stanford”) to develop EB-101 (LZRSE-Col7A1 Engineered Autologous Epidermal Sheets (LEAES)) and EB-201 (AAV DJ COL7A1) and to license the invention “Gene Therapy for Recessive Dystrophic EB using Genetically Corrected Autologous Keratinocytes.” Under the terms of the licensing agreements, the Company paid an upfront of licensing fees in cash and is subject to annual license maintenance fees. In addition, the Company is subject to the achievement of certain milestones, regulatory approval milestone payments, and royalty payments in the low single digits on annual net sales of the licensed product. As of December 31, 2025, the Company paid the remaining milestone payments of $0.3 million which became due upon FDA approval of ZEVASKYN® on April 28, 2025 and is included in selling, general and administrative costs in the consolidated statement of operations and comprehensive income (loss). Under this arrangement the Company recognized $43,000 of royalties due to Stanford during the year ended December 31, 2025 which is included in accrued expenses in the consolidated balance sheet. There were no royalty payments during the year ended December 31, 2024.

License Agreement Relating to Novel AAV Capsids ("AIM™ capsids")

In 2016, the Company licensed an international patent family from The University of North Carolina at Chapel Hill ("UNC") covering novel AAV capsids ("AIM™ capsids") that may potentially be used to deliver a wide variety of therapeutic transgenes to human cells to treat genetic diseases. Under the terms of the licensing agreements, the Company paid an upfront licensing fee in cash and is subject to on-going patent expenses incurred in relation to the patents licensed under this agreement and annual license maintenance fees. In addition, the Company is subject to the achievement of certain milestones, regulatory approval milestone payments, and royalty payments in the low single digits on annual net sales of the licensed product. As of December 31, 2025, as a result of exercise of the option to license certain of the Company's AAV capsids, the Company paid $0.1 million to UNC as a royalty payment under this agreement.

License Agreement Relating to CLN1 Disease

In 2016, the Company licensed from UNC rights to two patent families directed to treating CLN1 disease (also known as infantile Batten disease). Under the terms of the licensing agreements, the Company paid an upfront of licensing fees in cash and is subject to on-going patent expenses incurred in relation to the patents licensed under this agreement and annual license maintenance fees. In addition, the Company is subject to the achievement of certain milestones, regulatory approval milestone payments, and royalty payments in the low single digits on annual net sales of the licensed product. The Company subsequently sublicensed the license to Taysha Gene Therapies ("Taysha"), see detail of the sublicense agreement below. As part of the agreement with UNC, the Company is obligated to pay to UNC a percentage of any sublicense revenue that the Company receives under the agreement. The Company recognizes any payments under this agreement as royalties in the consolidated statement of operations and comprehensive income (loss). As of December 31, 2025 and 2024, no milestone or royalty payments under this agreement have been made. On February 25, 2026, the Company, UNC, and Taysha jointly terminated both the license agreement between Abeona and UNC and the corresponding sublicense agreement between Abeona and Taysha relating to Taysha's development program for TSHA-118 for CLN1 disease.

License Agreement Relating to Rett Syndrome

In 2019, the Company licensed rights to one patent family from UNC and two patent families from The University Court of the University of Edinburgh ("U. Edinburgh") and The University Court of the University of Glasgow ("U. Glasgow") relating to gene therapy for the treatment of Rett Syndrome. Under the terms of the licensing agreements, the Company paid an upfront of licensing fees in cash and is subject to on-going patent expenses incurred in relation to the patents licensed under this agreement and annual license maintenance fees. In addition, the Company is subject to the achievement of certain milestones, regulatory approval milestone payments, and royalty payments in the low single digits on annual net sales of the licensed product. The Company subsequently sublicensed the license to Taysha, see detail of the sublicense agreement below. As part of the agreement with UNC, the Company is obligated to pay to UNC and U. Edinburgh a percentage of any sublicense revenue that the Company receives under the agreement. The Company recognizes any payments under this agreement as royalties in the consolidated statement of operations and comprehensive income (loss). Under this arrangement the Company recognized $1.8 million of royalties due to UNC and U. Edinburgh during the year ended December 31, 2025 which is included in accounts payable in the consolidated balance sheet. There were no royalty payments during the year ended December 31, 2024. All milestone payments during the year were related to clinical milestones achieved by our sublicensor as per the sublicense agreement noted below. Other than the milestones achieved by our sublicensor and the subsequent royalties due to UNC and U. Edinburgh, there were no milestone payments under this agreement have been made during the year ended December 31, 2025 and 2024.

License Agreement Relating to AAV Capsids

In 2024, the Company entered into a license agreement with a third party for certain of the Company's AAV capsids. This agreement had an option to exercise before the terms of the agreement were activated. In June 2025, the third party exercised its option as per the agreement with a payment of $0.4 million included as license and other revenues in the statement of operations and comprehensive income (loss).

The Company assessed the nature of the promised license to determine whether the license has significant stand-alone functionality and evaluated whether such functionality can be retained without ongoing activities by the Company and determined that the license has significant stand-alone functionality. Furthermore, the Company has no ongoing activities associated with the license to support or maintain the license's utility. Based on this, the Company determined that the pattern of transfer of control of the license to the third party was at a point in time.

The transaction price of the contract includes (i) $0.4 million of fixed consideration, (ii) up to $24.0 million of variable consideration in the form of event-based milestone payments, (iii) up to $45.0 million of variable consideration in the form of sales-based milestone payments, and (iv) low single-digit royalty-based payments based on net sales. The Company is obligated to pay a portion of milestone payments and royalties on net sales received from the third party to UNC. The event-based milestone payments are based on certain development and regulatory events occurring. The Company evaluated whether the milestone conditions have been achieved and if it is probable that a significant cumulative revenue reversal would not occur before recognizing the associated revenue. The Company determined that these milestone payments are not within the Company's control or the licensee's control, such as regulatory approvals, and are not considered probable of being achieved until those approvals are received. Accordingly, the Company has fully constrained the $24.0 million in event-based milestone payments until such time that it is probable that a significant cumulative revenue reversal would not occur. The sales-based milestone payments and other royalty-based payments are based on a level of sales for which the license is deemed to be the predominant item to which the royalties relate. The Company will recognize revenue for these payments at the later of (i) when the related sales occur, or (ii) when the performance obligation to which some or all of the royalty has been allocated has been satisfied or partially satisfied. To date, the Company has not recognized any sales-based or royalty revenue resulting from this licensing arrangement.

Under this arrangement, the Company recognized $0.4 million in revenue during the year ended December 31, 2025, and no revenue for year ended December 31, 2024. As of December 31, 2025 and 2024, the Company does not have any contract assets or contract liabilities as a result of this transaction.

Sublicense and Inventory Purchase Agreements Relating to CLN1 Disease

In August 2020, the Company entered into sublicense and inventory purchase agreements with Taysha relating to a potential gene therapy for CLN1 disease. Under the sublicense agreement, Taysha received worldwide exclusive rights to intellectual property and know-how relating to the research, development, and manufacture of the potential gene therapy, which the Company had referred to as ABO-202 and which Taysha referred to as TSHA-118. Under the inventory purchase agreement, the Company sold to Taysha certain inventory and other items related to ABO-202/TSHA-118. The Company assessed the nature of the promised license to determine whether the license has significant stand-alone functionality and evaluated whether such functionality could be retained without ongoing activities by the Company and determined that the license has significant stand-alone functionality. Furthermore, the Company has no ongoing activities associated with the license to support or maintain the license's utility. Based on this, the Company determined that the pattern of transfer of control of the license to Taysha was at a point in time.

The transaction price of the contract included (i) $7.0 million of fixed consideration, (ii) up to $26.0 million of variable consideration in the form of event-based milestone payments, (iii) up to $30.0 million of variable consideration in the form of sales-based milestone payments, and (iv) high single-digit royalty-based payments based on net sales. The Company was obligated to pay a portion of milestone payments and royalties on net sales received from Taysha to UNC. The event-based milestone payments were based on certain development and regulatory events occurring. At inception, the Company evaluated whether the milestone conditions had been achieved and if it was probable that a significant cumulative revenue reversal would not occur before recognizing the associated revenue and determined that these milestone payments were not within the Company's control or the licensee's control, such as regulatory approvals, and were not considered probable of being achieved until those approvals were received. Accordingly, at inception, the Company fully constrained the $26.0 million of event-based milestone payments until such time that it is probable that significant cumulative revenue reversal would not occur. The sales-based milestone payments and other royalty-based payments were to have been based on a level of sales for which the license was deemed to be the predominant item to which the royalties relate. The Company would have recognized revenue for these payments at the later of (i) when the related sales occurred, or (ii) when the performance obligation to which some or all of the royalty had been allocated had been satisfied or partially satisfied. To date, the Company has not recognized any sales-based or royalty revenue resulting from this licensing arrangement. On February 25, 2026, the Company, UNC, and Taysha jointly terminated both the license agreement between Abeona and UNC and the corresponding sublicense agreement between Abeona and Taysha relating to Taysha's development program for TSHA-118 for CLN1 disease.

Under this arrangement, the Company did not recognize any revenue during the years ended December 31, 2025 and 2024, respectively. The Company has no contract assets or liabilities as of December 31, 2025 and 2024 as a result of this transaction.

Sublicense Agreement Relating to Rett Syndrome

In October 2020, the Company entered into a sublicense agreement with Taysha for a gene therapy for Rett syndrome, including intellectual property related to MECP2 gene constructs and regulation of their expression. The agreement grants Taysha worldwide exclusive rights to intellectual property developed by scientists at UNC, U. Edinburgh and the Company, and the Company's know-how relating to the research, development, and manufacture of the gene therapy for Rett syndrome and MECP2 gene constructs and regulation of their expression.

The Company assessed the nature of the promised license to determine whether the license has significant stand-alone functionality and evaluated whether such functionality can be retained without ongoing activities by the Company and determined that the license has significant stand-alone functionality. Furthermore, the Company has no ongoing activities associated with the license to support or maintain the license's utility. Based on this, the Company determined that the pattern of transfer of control of the license to Taysha was at a point in time.

The transaction price of the contract includes (i) $3.0 million of fixed consideration, (ii) up to $26.5 million of variable consideration in the form of event-based milestone payments, (iii) up to $30.0 million of variable consideration in the form of sales-based milestone payments, and (iv) high single-digit royalty-based payments based on net sales. The Company is obligated to pay a portion of milestone payments and royalties on net sales received from Taysha to UNC and U. Edinburgh. The event-based milestone payments are based on certain development and regulatory events occurring. The Company evaluated whether the milestone conditions have been achieved and if it is probable that a significant cumulative revenue reversal would not occur before recognizing the associated revenue. The Company determined that these milestone payments are not within the Company's control or the licensee's control, such as regulatory approvals, and are not considered probable of being achieved until those approvals are received. Accordingly, the Company fully constrained the $26.5 million in event-based milestone payments until such time that it is probable that a significant cumulative revenue reversal would not occur. The sales-based milestone payments and other royalty-based payments are based on a level of sales for which the license is deemed to be the predominant item to which the royalties relate. The Company will recognize revenue for these payments at the later of (i) when the related sales occur, or (ii) when the performance obligation to which some or all of the royalty has been allocated has been satisfied or partially satisfied. To date, the Company has not recognized any sales-based or royalty revenue resulting from this licensing arrangement.

Under this arrangement, the Company recognized revenue of $3.0 million and nil during the years ended December 31, 2025 and 2024, respectively. The revenue recognized was related to clinical milestones achieved by our sublicensor as per the sublicense agreement noted above. As of December 31, 2025, the $3.0 million is included in accounts receivable in the consolidated balance sheet. As of December 31, 2024, the Company did not have any contract assets or contract liabilities as a result of this transaction.

Ultragenyx License Agreement

On May 16, 2022, the Company and Ultragenyx Pharmaceutical Inc. ("Ultragenyx") entered into an exclusive license agreement (the "License Agreement") for AAV gene therapy, ABO-102, for the treatment of Sanfilippo syndrome type A (MPS IIIA). Under the License Agreement, Ultragenyx assumed responsibility for the ABO-102 program from the Company, with the exclusive right to develop, manufacture, and commercialize ABO-102 worldwide. Also pursuant to the License Agreement, following regulatory approval, the Company is eligible to receive tiered royalties from mid-single-digit up to 10% on net sales and up to $30.0 million in commercial milestone payments. Both forms of consideration comprise the transaction price to which the Company expects to be entitled in exchange for transferring the related intellectual property and certain, contractually-specified, transition services to Ultragenyx. The sales-based royalty and milestone payments are subject to the royalty recognition constraint. As such, these fees are not recognized as revenue until the later of: (a) the occurrence of the subsequent sale, and (b) the performance obligation to which they relate has been satisfied. As of December 31, 2025 and 2024, the Company does not have any contract assets or contract liabilities as a result of this transaction.

NOTE 14 – 401(k) PLAN

The Company has a tax-qualified employee savings and retirement plan (the "401(k) Plan") covering all the Company's employees in the United States. Pursuant to the 401(k) Plan, employees may elect to reduce their current compensation by up to the statutorily prescribed annual limit ($23,500 in 2025 and $23,000 in 2024 for employees who are under age 50 and $31,000 in 2025 and $30,500 in 2024 for employees who are age 50 and older) and to have the amount of such reduction contributed to the 401(k) Plan. The 401(k) Plan is intended to qualify under Section 401 of the Internal Revenue Code so that contributions by employees or by us to the 401(k) Plan, and income earned on 401(k) Plan contributions, are not taxable to employees until withdrawn from the 401(k) Plan, and so that contributions by us, if any, will be deductible by us when made. At the direction of each participant, the Company invests the assets of the 401(k) Plan in any of over 50 investment options. Company contributions under the 401(k) Plan were $1.0 million and $0.5 million for the years ended December 31, 2025 and 2024.

NOTE 15 – INCOME TAXES

Income tax expense for each of the following years consists of the following (in thousands):

	For the year ended December 31,	
	2025	**2024**
Current:		
U.S. federal	$ 100	$ —
State and local	—	—
Total current income tax expense	100	—
Deferred:		
U.S. federal	—	—
State and local	—	—
Total deferred income tax expense	—	—
Total income tax expense	$ 100	$ —

A reconciliation of the income tax expense the amount computed by applying the 21% statutory U.S federal income tax rate to income before income taxes after the adoption of ASU 2023-09 as follows:

	For the year ended December 31, 2025	
In thousands except for percentages	**Amount**	**Percent**
US federal statutory tax rate	$ 14,970	21.0%
State and local income taxes, net of federal income tax effect (a)	—	0.0%
Tax credits		
Research and development ("R&D") credit	(2,882)	(4.0)%
R&D credit expired (under statute or 382 study)	3,327	4.6%
Changes in valuation allowance	(36,054)	(50.6)%
Nontaxable or nondeductible items		
Change in FV of warrant liabilities	(1,289)	(1.8)%
Other	141	0.2%
Other adjustments		
Federal NOL's expired (under statute or 382 limitation)	20,284	28.5%
Share-based awards	984	1.4%
Other	619	0.8%
Total income tax expense	$ 100	0.1%

(a) State taxes in New York made up the majority (greater than 50 percent) of the tax effect in this category.

No federal, state and local income taxes were paid during the period.

Changes to US tax law enacted on July 4, 2025, allow for immediate expensing of domestic research and experimentation costs, accelerated depreciation on eligible capital expenditures, and other tax law changes impacting 2025 with certain changes effective in 2026. These changes are reflected in our results for the year ended December 31, 2025.

As previously disclosed for the year ended December 31, 2024, prior to the adoption of ASU 2023-09, the following is a reconciliation of the difference between the effective income tax rate and federal statutory rate (in thousands):

	For the year ended December 31, 2024
Income taxes at U.S. statutory rate	$ (13,384)
State tax, net of federal benefit	(679)
Research and development credit	(1,535)
Deferred true ups	8,032
Valuation allowance	5,418
Change in fair value of warrant liabilities	159
Expired tax losses and credits	2,116
Permanent differences	(127)
Total tax expense	$ —

Deferred taxes are provided for the temporary differences between the financial reporting bases and the tax bases of the Company's assets and liabilities. The temporary differences that give rise to deferred tax assets and liabilities were as follows (in thousands):

	For the year ended December 31,	
	2025	2024
Deferred tax assets:		
Net operating loss carryforwards	$ 65,956	$ 88,059
General business credit carryforwards	5,228	6,000
State credits	77	2,780
Property and equipment	—	1,002
Stock based compensation	2,542	2,463
Intangible assets	652	661
Accrual to cash conversion	892	—
Accruals	—	107
Capitalized research and development	770	13,264
Operating lease liabilities	318	—
Other	91	70
Deferred tax assets before valuation allowance	76,526	114,406
Valuation allowance	(74,922)	(114,406)
Total deferred tax assets	1,604	—
Deferred tax liabilities:		
Property and equipment	(1,333)	—
Right-of-use asset	(271)	—
Total deferred tax liabilities	(1,604)	—
Net deferred tax asset (liability)	$ —	$ —

Net operating Loss and Other Carryforwards

As of December 31, 2025, the Company had $310.7 million of U.S. federal net operating loss ("NOL") carryforwards, $11.6 million of state NOL carryforwards, $5.2 million of general business credit carryforwards, and $0.1 million of state credits. Of the federal NOLs, $308.1 million do not expire and may be carried forward indefinitely, subject to the limitation that they may offset no more than 80% of taxable income in any tax year. The remaining federal NOLs expire between 2026 and 2037. State NOL carryforwards have expiration periods that vary by jurisdiction based on applicable state tax laws. The federal general business credits begin to expire in 2043, and the state credits expire in 2026.

The utilization of NOLs and tax credits that have expiration dates will depend on the Company's ability to generate sufficient taxable income before those attributes expire.

The Internal Revenue Code of 1986, as amended, includes provisions that may limit the Company's ability to utilize its NOLs carryforwards following certain events, including significant changes in ownership. If such limitations apply and the Company generates taxable income in excess of the annually permitted NOL utilization, the Company could incur federal income tax liabilities even though additional NOLs would remain available for use in future years.

During the year ended December 31, 2025, the Company completed a Section 382 study to evaluate whether historical equity transactions resulted in an ownership change within the meaning of Section 382 of the Internal Revenue Code. Based on this analysis, the Company determined that there were numerous ownership changes. As a result, certain NOL carryforwards will not be realizable due to the Section 382 limitations.

The Company had previously recorded a full valuation allowance against the deferred tax assets associated with these NOLs. Accordingly, the $96.6 million reduction in gross deferred tax assets resulting from the Section 382 analysis was fully offset by a corresponding reduction in the valuation allowance and did not affect income tax expense or net income for the year ended December 31, 2025.

Valuation Allowance

At December 31, 2025 and 2024, the Company maintained a full valuation allowance on its deferred tax assets based on a history of cumulative losses. The Company will not record income tax benefits in the financial statements until it is determined that it is more likely than not that the Company will generate sufficient taxable income to realize the deferred income tax assets. In 2025, the valuation allowance decreased by approximately $39.5 million. In 2024, the valuation allowance increased by approximately $5.4 million.

Unrecognized Tax Benefits

At December 31, 2025 and 2024, the Company had no reserves for unrecognized tax benefits.

The Company and its subsidiaries are subject to taxation in the United States. The Company is subject to U.S. federal and state examinations for 2022 and forward, and 2021 and forward, respectively. However, net operating losses are subject to audit in any tax year in which those losses are utilized, notwithstanding the year of origin.

NOTE 16 – COMMITMENTS AND CONTINGENCIES

Litigation

The Company recognizes a liability for a contingency when it is probable that liability has been incurred and when the amount of loss can be reasonably estimated. When a range of probable loss can be estimated, the Company accrues the most likely amount of such loss, and if such amount is not determinable, then the Company accrues the minimum of the range of probable loss. As of December 31, 2025 and 2024, there was no litigation against the Company.

NOTE 17 – SEGMENT INFORMATION

Operating segments are defined as components of an enterprise about which separate discrete information is available for evaluation by the Chief Operating Decision Maker ("CODM"), or decision-making group, in deciding how to allocate resources in assessing performance. The Company is a commercial-stage biopharmaceutical company developing cell and gene therapies for life-threatening diseases and has one reportable segment. The Company's CODM is the chief executive officer.

The accounting policies of the commercial-stage biopharmaceutical segment are the same as those described in the summary of significant accounting policies. The CODM assesses performance for the commercial-stage biopharmaceutical segment based on net income (loss), which is reported on the consolidated statements of operations and comprehensive income (loss) as consolidated net income (loss). The measure of segment assets is reported on the consolidated balance sheet as total consolidated assets. Expenditures for additions to long-lived assets, which include purchases of property and equipment, are included in total consolidated assets reviewed by the chief operating decision maker and are reported on the consolidated statements of cash flows.

To date, the Company has generated limited product revenue. The Company will continue to incur significant expenses and operating losses until ZEVASKYN® can provide sufficient revenue for the Company to be profitable. As such, the CODM uses cash forecast models in deciding how to invest into the commercial-stage biopharmaceutical segment. Such cash forecast models are reviewed to make decisions about allocating resources and assessing the entity-wide operating results and performance. Net income (loss) is used to monitor budget versus actual results. Monitoring budgeted versus actual results is used to make decisions about allocating resources, assessing the performance of the segment and in establishing management's compensation, along with cash forecast models.

The table below summarizes the significant expense categories regularly provided to the CODM for the years ended December 31, 2025, and 2024:

	For the year ended December 31,	
	2025	2024
Revenues:		
Product revenue, net	$ 2,420	$ —
License and other revenues	3,400	—
Total revenues	5,820	—
Cost of sales	1,532	—
Royalties	1,893	—
Research and development costs:		
Salaries & related costs	10,247	15,345
Non-cash stock-based compensation	1,355	1,561
Other research and development costs (a)	15,210	17,454
Total research and development costs	26,812	34,360
Selling, general and administrative costs:		
Salaries & related costs	24,978	10,729
Non-cash stock-based compensation	9,424	5,067
Commercial costs	7,159	4,818
Other selling, general and administrative costs (b)	23,470	9,237
Total selling, general and administrative costs	65,031	29,851
Other segment items, net (c)	160,631	477
Net income (loss)	$ 71,183	$ (63,734)

(a) Other research and development costs include, but are not limited to preclinical lab supplies, preclinical and development costs, clinical trial costs, preclinical manufacturing and manufacturing facility costs, costs associated with preclinical regulatory approvals, preclinical depreciation on lab supplies and manufacturing facilities, and preclinical consultant-related expenses.

(b) Other selling, general and administrative costs primarily consist of office facility costs, public reporting company related costs, professional fees (e.g., legal expenses), regulatory costs, production costs not attributable to cost of sales and other general operating expenses not otherwise included in research and development expenses.

(c) Other segment items include interest income, interest expense, change in fair value of warrant and derivative liabilities, gain on sale of priority review voucher, other income, net and income tax (benefit) expense.

NOTE 18 – SALE OF NONFINANCIAL ASSETS

On May 9, 2025, the Company entered into a definitive asset purchase agreement that transferred the rights to a PRV awarded to the Company following the FDA approval of ZEVASKYN®. The PRV sale was subject to customary closing conditions and was completed in June 2025 following the expiration of applicable U.S. antitrust requirements. The Company accounted for this transaction under ASC Topic 610-20, *Gains and Losses from the Derecognition of Nonfinancial Assets* ("ASC 610-20"). The Company received the gross proceeds of $155.0 million during the year ended December 31, 2025 and recognized a gain, net of transaction costs of $2.6 million, from sale of priority review voucher of $152.4 million on the Company's consolidated statement of operations and comprehensive income (loss) as it did not have a carrying value at the time of sale.

NOTE 19 – SUBSEQUENT EVENTS

In January of 2026, the compensation committee of the board of directors granted various employees and directors restricted stock awards, under which the holders have the right to receive an aggregate of 2,034,526 shares of the Company's common stock. Total stock compensation estimated for these awards at the time of grant was $10.8 million, with $9.2 million vesting in three equal annual installments and $1.6 million vesting in one annual installment. Pursuant to the terms of the awards, the shares not vested are forfeited upon separation from the Company.

Exhibit 3.3

AMENDED AND RESTATED BYLAWS OF

ABEONA THERAPEUTICS INC.

ARTICLE I.

Offices and Agents

1. Principal Office. The principal office of the Corporation may be located within or without the State of Delaware, as designated by the board of directors. The Corporation may have other offices and places of business at such places within or without the State of Delaware as shall be determined by the directors.

2. Registered Office. The registered office of the Corporation required by the General Corporation Law of Delaware must be maintained in the State of Delaware, and it may be, but need not be, identical with the principal office, if located in the state of Delaware. The address of the registered office of the Corporation may be changed from time to time as provided by the General Corporation Law of Delaware.

3. Registered Agent. The Corporation shall maintain a registered agent in the State of Delaware as required by the General Corporation Law of Delaware. Such registered agent may be changed from time to time as provided by the General Corporation Law of Delaware.

ARTICLE II.

Stockholders Meetings

1. Annual Meetings. Unless otherwise determined by the board of directors, the annual meeting of the stockholders of the Corporation shall be held at such date and time as may be determined by the board of directors . The board of directors may postpone, reschedule, or cancel any annual meeting of stockholders.

2. Special Meetings. Special meetings of the stockholders of the Corporation may be called at any time by the chairman of the board of directors, if any, by the president or by resolution of the board of directors. Special meetings of stockholders shall be held on such date as shall be determined by the board of directors and stated in the Corporation's notice of the meeting.

3. Place of Meeting. The annual meeting of the stockholders of the Corporation may be held at any place, either within or without the State of Delaware, as may be designated by the board of directors. The board of directors may, in its sole discretion, determine that a meeting of stockholders shall not be held at any place, but may instead be held solely by means of remote communication as authorized by Section 211(a)(2) of the General Corporation Law of the State of Delaware (the "DGCL") and Article X herein. In the absence of any such designation or determination, meetings of stockholders shall be held at the Corporation's principal executive office.

4. Notice of Meeting. Except as otherwise provided in these Bylaws or by the laws of the State of Delaware, written or printed notice stating the place, date and hour of the meeting and, in the case of a special meeting, the purpose or purposes for which the meeting is called, shall be delivered either personally or by mail to each stockholder of record entitled to vote at such meeting not less than ten (10) nor more than sixty (60) days before the date of the meeting. If mailed, such notice shall be deemed to be delivered when deposited in the United States mail, postage prepaid, directed to the stockholder at his address as it appears on the records of the Corporation. An affidavit of the secretary, assistant secretary, if any, or transfer agent of the Corporation that notice has been given shall, in the absence of fraud, be prima facie evidence of the facts stated therein.

5. Waiver of Notice. Any stockholder, either before, at, or after any stockholders' meeting, may waive notice of the meeting, and his waiver shall be deemed the equivalent of giving notice. Attendance at a stockholders' meeting, either in person or by proxy, by a person entitled to notice thereof shall constitute a waiver of notice of the meeting unless he attends for the express purpose of objecting, at the beginning of the meeting, to the transaction of any business on the ground that the meeting was not lawfully called or convened.

6. Fixing of Record Date. For the purpose of determining stockholders entitled to notice of or to vote at any meeting of stockholders or any adjournment thereof, to express consent to corporate action in writing without a meeting, or entitled to receive payment of any dividend or other distribution or allotment of any rights, or entitled to exercise any rights in respect of any change, conversion or exchange of stock, or for the purpose of any other lawful action, the board of directors of the Corporation may fix, in advance, a record date, which shall not be more than sixty (60) nor less than ten (10) days before the date of the meeting; not more than ten (10) days after the record date for determining stockholders entitled to express consent is fixed; and not more than sixty (60) days prior to the date of any other action. If no record date is fixed: (i) the record date for determining stockholders entitled to notice of or to vote at a meeting of stockholders shall be the close of business on the day next preceding the day on which notice is given, or, if notice is waived, at the close of business on the day next preceding the day on which the meeting was held; (ii) the record date for determining stockholders entitled to express consent to corporate action in writing without a meeting, when no prior action by the board of directors is necessary, shall be the day on which the first written consent is delivered to the Corporation at its principal place of business or such other place as designated by the boards of directors; (iii) the record date for determining stockholders for any other purpose shall be at the close of business on the day on which the board of directors adopts the resolution relating thereto. A determination of stockholders entitled to notice of or to vote at a meeting of stockholders shall apply to any adjournment of the meeting, provided, however, that the board of directors may fix a new record date for the adjourned meeting.

7. Voting List. The officer or agent who has charge of the stock ledger of the Corporation shall prepare and make, at least ten (10) days before every meeting of stockholders, a complete list of the stockholders entitled to vote at the meeting, or any adjournment thereof, arranged in alphabetical order, showing the address of and the number of shares registered in the name of each stockholder. To the extent required by statute, such list shall be open to the examination of any stockholder for any purpose germane to the meeting, during ordinary business hours, for a period of at least ten (10) days prior to the meeting, (i) on a reasonably accessible electronic network, provided that the information required to gain access to such list is provided with the notice of the meeting, or (ii) during ordinary business hours, at the Corporation's principal place of business.

8. Polls. The date and time of the opening and closing of the polls for each matter upon which the stockholders will vote at a meeting shall be announced at the meeting. No ballot, proxies or votes, nor any revocations thereof or changes thereto, shall be accepted by the inspectors after the closing of the polls unless the Court of Chancery upon application by a stockholder shall determine otherwise.

9. Proxies. Any stockholder entitled to vote at a meeting of the stockholders, or to express consent or dissent to corporate action in writing without meeting may authorize another person or persons to act for him by proxy. No proxy shall be voted or acted upon after three (3) years from the date of its execution unless the proxy expressly provides for a longer period. A duly executed proxy shall be irrevocable if it states that it is irrevocable and if, and only as long as, it is coupled with an interest sufficient in law to support an irrevocable power. A proxy may be irrevocable regardless of whether the interest with which it is coupled is an interest in the stock itself or an interest in the Corporation generally.

Without limiting the manner in which a stockholder may authorize another person or persons to act for him by proxy, the following shall constitute a valid means by which a stockholder may grant such authority.

A stockholder may execute a writing authorizing another person or persons to act for him as proxy. Execution may be accomplished by the stockholder or his authorized officer, director, employee or agent signing such writing or causing his signature to be affixed to such writing by any reasonable means including but not limited to, by electronic signature.

A stockholder may authorize another person or persons to act for him as proxy by transmitting or authorizing the transmission of electronic mail or other means of electronic transmission to the person who will be the holder of the proxy or to a proxy solicitation firm, proxy support service organization or like agent duly authorized by the person who will be the holder of the proxy to receive such transmission, provided that any such electronic mail or other electronic transmission must either set forth or be submitted with information from which it can be determined that the electronic mail or other electronic transmission was authorized by the stockholder. If it is determined that such electronic mail or other electronic transmission is valid, the inspectors or, if there are no inspectors, such other persons making that determination shall specify the information upon which they relied.

Any electronic telecommunication or other reliable reproduction of the writing or transmission created pursuant to this Paragraph 9 may be substituted or used in lieu of the original writing or transmission for any and all purposes for which the original writing or transmission could be used, provided that such copy, electronic telecommunication or other reproduction shall be a complete reproduction of the entire original writing or transmission.

10. Voting Rights. Each outstanding share, regardless of class, shall be entitled to one vote, and each fractional share shall be entitled to a corresponding fractional vote on each matter submitted to a vote at a meeting of stockholders except to the extent that the voting rights of the shares of any class or classes are limited or denied by the Certificate of Incorporation.

Persons holding stock in a fiduciary capacity shall be entitled to vote the shares so held. Persons whose stock is pledged shall be entitled to vote, unless in the transfer by the pledgor on the books of the Corporation he has expressly empowered the pledgee to vote thereon, in which case only the pledgee, or his proxy, may represent such stock and vote thereon.

The Corporation's own capital stock belonging to the Corporation or to another corporation, if a majority of the shares entitled to vote in the election or directors of such other corporation is held, directly or indirectly, by the Corporation, shall neither be entitled to vote nor be counted for quorum purposes. Nothing in this section shall be construed as limiting the right of the Corporation to vote stock, including but not limited to its own stock, held by it in a fiduciary capacity.

Shares which have been called for redemption shall not be deemed to be outstanding shares for the purpose of voting or determining the total number of shares entitled to vote on any matter on and after the date on which written notice of redemption has been sent to holders thereof and a sum sufficient to redeem such shares has been irrevocably deposited or set aside to pay the redemption price to the holders of the shares upon surrender of certificates therefor.

If shares or other securities having voting power stand of record in the names of two (2) or more persons, whether fiduciaries, members of a partnership, joint tenants, tenants in common, tenants by the entirety or otherwise, or if two (2) or more persons have the same fiduciary relationship respecting the same shares, unless the secretary of the Corporation is given written notice to the contrary and is furnished with a copy of the instrument or order appointing them or creating the relationship wherein it is so provided, their acts with respect to voting shall have the following effect: (i) if only one (1) votes, his act binds all; (ii) if more than one (1) votes, the act of the majority so voting binds all; (iii) if more than one (1) votes, but the vote is evenly split on any particular matter each faction may vote the securities in question proportionally, or any person voting the shares, or a beneficiary, if any, may apply to the Court of Chancery or such other court as may have jurisdiction to appoint an additional person to act with the persons so voting the shares, which shall then be voted as determined by a majority of such persons and the person appointed by the Court. If the instrument so filed shows that any such tenancy is held in unequal interests, a majority or even split for the purpose of this subsection shall be a majority or even split in interest.

11. Notice of Stockholder Business.

A. Annual Meetings of Stockholders.

1. Nominations of persons for election to the Board of Directors and the proposal of business to be considered by the stockholders may be made at an annual meeting of stockholders (a) pursuant to the Corporation's notice of meeting, (b) by or at the direction of the Board of Directors or (c) by any stockholder of the Corporation who (i) was a stockholder of record at the time of giving of notice provided for in this Bylaw and at the time of the annual meeting, (ii) is entitled to vote at the meeting and (iii) complies with the notice procedures set forth in this Bylaw.

2. For business other than nominations to be properly brought before an annual meeting by a stockholder, the stockholder must have given timely notice thereof in writing to the Secretary pursuant to this Section 11(A) and such other business must otherwise be a proper matter for stockholder action. To be timely, a stockholder's notice shall be delivered to the Secretary at the principal executive offices of the Corporation not earlier than the close of business on the 150th day and not later than the close of business on the 120th day prior to the first anniversary of the preceding year's annual meeting; provided, however, that in the event that the date of the annual meeting is more than 30 days before or after such anniversary date, notice by the stockholder to be timely must be so delivered by close of business on the 10th day following the day on which public announcement of the date of such meeting is first made by the Corporation. In no event shall the public announcement of an adjournment, recess, continuation, rescheduling, or postponement of an annual meeting, or the announcement thereof, commence a new time period (or extend any time period) for the giving of a stockholder's notice as described above.

3. To be in proper form, a stockholder's notice to the Secretary must set forth the following information:

a. as to each such matter such stockholder proposes to bring before the annual meeting:

i. a brief description of the business intended to be brought before the annual meeting;

ii. the text of any proposal relating to such business, including the complete text of any resolutions proposed for consideration at the annual meeting, and if such business includes a proposal to amend the Certificate of Incorporation or these Bylaws, the text of the proposed amendment;

iv. the reasons for conducting such business at the annual meeting (including the text of any reasons for the proposed business that will be disclosed in any proxy statement or supplement thereto to be filed with the Securities and Exchange Commission (the "SEC"));

v. a complete and accurate description of any material interest in such business of the proposing stockholder and any Stockholder Associated Person (as defined below), individually or in the aggregate, in such business, including any anticipated material benefit to the proposing stockholder and any Stockholder Associated Person therefrom; and

vi. any other information relating to such business that would be required to be disclosed in a proxy statement or other filing required to be made by such Proposing Stockholder in connection with the solicitation of proxies in support of such proposed business pursuant to Section 14(a) under the Exchange Act (or pursuant to any law or statute replacing such section) and the rules and regulations promulgated thereunder; and

b. as to the stockholder of record giving notice and any Stockholder Associated Person (as defined below), if any, on whose behalf the proposal is being made:

i. the name and address of such stockholder or any Stockholder Associated Person (including, if applicable, the name and address that appear on the Corporation's stock ledger);

ii. whether such person(s) is/are providing the notice at the request of a beneficial holder of securities of the Corporation;

iii. (1) the class or series and number of all shares of capital stock of the Corporation that are, directly or indirectly, owned beneficially (within the meaning of Rule 13d-3 under the Securities Exchange Act of 1934, as amended (the "Exchange Act")) or of record by such stockholder or any Stockholder Associated Person (specifying the type of ownership), (2) the name of each nominee holder of shares of all stock of the Corporation owned beneficially but not of record by such stockholder or any Stockholder Associated Person, the number of such shares of stock of the Corporation held by each such nominee holder, and any pledge with respect to any such stock, (3) whether and to the extent to which any derivative instrument, swap, option, warrant, short interest, hedge or profit interest or other transaction or series of transactions has been entered into by or on behalf of such stockholder or any Stockholder Associated Person, with respect to stock of the Corporation, and (4) whether and to the extent to which any other transaction, agreement, arrangement, or understanding (including any short position or any borrowing or lending of shares of stock of the Corporation) has been made by or on behalf of such stockholder or any Stockholder Associated Person, the effect or intent of any of the foregoing being to mitigate loss to, or to manage risk or benefit of stock price changes for, such stockholder or any Stockholder Associated Person, or to increase or decrease the voting power or pecuniary or economic interest of such stockholder or any Stockholder Associated Person, with respect to stock of the Corporation;

iv. a complete and accurate description of all agreements, arrangements or understandings (whether written or oral) between or among such stockholder or any Stockholder Associated Person and any other person or persons or entity (naming each such person or entity) in connection with (1) the proposal of such business, including any material interest of such stockholder or any Stockholder Associated Person, in such business, including any anticipated benefit therefrom to such stockholder or any Stockholder Associated Person, and (2) any understanding (whether written or oral) that such stockholder or any Stockholder Associated Person may have reached with a stockholder of the Corporation (including the name of such stockholder) with respect to how such stockholder will vote such stockholder's shares in the Corporation at any meeting of stockholders or take other action in support of any other business or other actions to be taken, by such stockholder or any Stockholder Associated Person;

v. any substantial direct or indirect interest (including any existing or prospective commercial, business or contractual relationship with the Corporation), by security holdings or otherwise, of stockholder or any Stockholder Associated Person in the Corporation or any affiliate thereof, other than an interest arising from the ownership of Corporation securities where such stockholder or any Stockholder Associated Person receives no extra or special benefit not shared on a *pro rata* basis by all other holders of the same class or series;

vi. any direct or indirect interest of stockholder or any Stockholder Associated Person in any contract or arrangement with the Corporation, any affiliate of the Corporation or any principal competitor of the Corporation (including any employment agreement, collective bargaining agreement or consulting agreement);

vii. a representation that (i) no such stockholder or any Stockholder Associated Person has breached any contract or other agreement, arrangement or understanding with the Corporation except as disclosed to the Corporation pursuant hereto and (ii) that such stockholder or any Stockholder Associated Person has complied, and will comply, with all applicable requirements of state law and the Exchange Act with respect to the matters set forth in this Section 11(A);

viii. a complete and accurate description of any pending or, to such stockholder's knowledge, threatened legal proceeding in which such stockholder or any Stockholder Associated Person is a party or participant involving the Corporation, or to such stockholder's knowledge, any current or former officer, director, affiliate or associate of the Corporation;

ix. a representation that the stockholder giving notice intends to appear in person or by proxy at the annual meeting to bring such business before the meeting and an acknowledgment that, if such stockholder (or a qualified representative of such stockholder) does not appear to present such business at such meeting, the Corporation need not present such business for a vote at such meeting, notwithstanding that proxies in respect of such vote may have been received by the Corporation;

x. a representation from such stockholder as to whether such stockholder or any Stockholder Associated Person intends or is part of a group that intends (A) to deliver a proxy statement and/or form of proxy to a number of holders of the Corporation's voting shares reasonably believed by such stockholder to be sufficient to approve or adopt the business to be proposed, (B) to engage in a solicitation (within the meaning of Exchange Act Rule 14a-1(l)) with respect to business, and if so, the name of each participant (as defined in Item 4 of Schedule 14A under the Exchange Act) in such solicitation; and

xi. any other information relating to such stockholder or any Stockholder Associated Person that would be required to be disclosed in a proxy statement or other filing required to be made in connection with the solicitation of proxies by such person with respect to the proposed business to be brought by such person before the annual meeting pursuant to Section 14 of the Exchange Act, and the rules and regulations promulgated thereunder, and any other information relating to such stockholder or any Stockholder Associated Person that would be required to be set forth in a Schedule 13D filed pursuant to Rule 13d-1(a) of the Exchange Act (regardless of whether such person or entity is actually required to file a Schedule 13D).

4. A stockholder providing notice of business proposed to be brought before an annual meeting shall further update and supplement such notice, if necessary, so that the information provided or required to be provided in such notice pursuant to this Section 11(A) shall be true and correct as of the record date for determining the stockholders entitled to receive notice of the annual meeting and such update and supplement shall be delivered either in person or by United States certified mail, postage prepaid, and received by the secretary at the principal executive offices of the Corporation not later than five (5) business days after the record date for determining the stockholders entitled to receive notice of the annual meeting. The update and supplement shall clearly identify the information that has changed since such stockholder's prior submission, it being understood that no such update may cure any deficiencies or inaccuracies with respect to any such prior submission or extend the time period for the delivery of notice pursuant to this Section 11(A). If a stockholder fails to provide such written update within such period, the information as to which such written update relates may be deemed not to have been provided in accordance with this Section 11(A). In addition, the stockholder shall promptly provide any other information reasonably requested by the Corporation. If requested by the Corporation, any supplemental information required under this paragraph shall be provided within 10 (ten) days after it has been requested by the Corporation.

5. No business shall be conducted at the annual meeting of stockholders except business brought before the annual meeting in accordance with the procedures set forth in this Section 11(A); provided, however, that, once business has been properly brought before the annual meeting in accordance with such procedures, nothing in this Section 11(A) shall be deemed to preclude discussion by any stockholder of any such business. Except as otherwise provided by law, the Certificate of Incorporation, or these Bylaws the chair of an annual meeting shall have the power and duty to determine whether any business was properly brought before the annual meeting in accordance with the foregoing procedures, and, if such proposed business is deemed not to have been properly made, to declare to the meeting that the business was not properly brought before the meeting and such business shall be disregarded and declared to be out of order, notwithstanding that proxies with respect to such vote may have been received by the Corporation. Notwithstanding the foregoing provisions of this Section 11(A), unless otherwise required by law, if the stockholder (or a qualified representative of such stockholder) proposing any business to be conducted at the annual meeting does not appear at the annual meeting of stockholders to propose such business, such proposed business shall not be transacted, and no vote shall be taken with respect to such proposed business, notwithstanding that proxies with respect to such vote may have been received by the Corporation.

6. Notwithstanding the foregoing provisions of this Section 11(A), a stockholder shall also comply with all applicable requirements of the Exchange Act with respect to the matters set forth in this Section 11(A). This Section 11(A) is expressly intended to apply to any business proposed to be brought before a meeting of stockholders other than any proposal made pursuant to Rule 14a-8 under the Exchange Act. Nothing contained in this Section 11(A) shall be deemed to affect any rights of stockholders to request inclusion of proposals in the Corporation's proxy statement pursuant to Rule 14a-8 under the Exchange Act (or any successor provision of law).

B. Special Meetings of Stockholders. Only such business shall be conducted at a special meeting of stockholders as shall have been brought before the meeting pursuant to the Corporation's notice of meeting. In no event shall the public announcement of an adjournment of a special meeting commence a new time period for the giving of a stockholder's notice as described above.

12. Inspectors of Election. Prior to holding any meeting of stockholders, the Corporation shall appoint one or more inspectors to act at the meeting and make a written report thereof. The Corporation may designate one or more persons as alternate inspectors to replace any inspector who fails to act. If no inspector or alternate is able to act at a meeting of stockholders, the person presiding at the meeting shall appoint one or more inspectors to act at the meeting. Each inspector, before entering upon the discharge of his duties, shall take and sign an oath faithfully to execute the duties of inspector with strict impartiality and according to the best of his ability.

The inspectors shall (i) ascertain the number of shares outstanding and the voting power of each; (ii) determine the shares represented at a meeting and the validity of proxies and ballots; (iii) count all votes and ballots: (iv) determine and retain for a reasonable period a record of the disposition of any challenges made to any determination by the inspectors, and (v) certify their determination of the number of shares represented at the meeting and their count of all votes and ballots. The inspectors may appoint or retain other persons or entities to assist the inspectors in the performance of the duties of the inspectors.

In determining the validity and counting of proxies and ballots, the inspectors shall be limited to an examination of the proxies, any envelopes submitted with those proxies, any information provided in accordance with Article II, Paragraph 9 of these Bylaws, and any records of the Corporation, except that the inspectors may consider other reliable information for the limited purpose of reconciling proxies and ballots submitted by or on behalf of banks, brokers, their nominees or similar persons which represent more votes than the holder of a proxy is authorized by the record owner to cast or more votes than the stockholder holds of record. If the inspectors consider other reliable information for the limited purpose permitted herein, the inspectors at the time they make their certification shall specify the precise information considered by them including the person or persons from whom they obtained the information, when the information was obtained, the means by which the information was obtained and the basis for the inspectors' belief that such information is accurate and reliable.

13. Quorum. Except as otherwise provided in the Certificate of Incorporation, the presence, in person or by proxy, of the holders of a majority of the outstanding shares entitled to vote shall constitute a quorum at meetings of the stockholders. In all matters, other than the election of directors, the affirmative vote of a majority of the shares present in person or represented by proxy at the meeting and actually voting on the subject matter shall be the act of the stockholders. Directors shall be elected by the vote of the majority of the votes cast by stockholders with respect to that director's election at any meeting of stockholders for the election of directors. In the event any stockholders withdraw from a duly organized meeting at which a quorum was initially present, the remaining shares represented shall constitute a quorum for the purpose of continuing to do business, and the affirmative vote of the majority of the remaining shares represented at the meeting and entitled to vote on the subject matter shall be the act of the stockholders unless the vote of a greater number or voting by classes is required by the DGCL or the Certificate of Incorporation.

14. Election of Directors. Except as provided in Article III, Section 2, of these Bylaws with respect to the filling of vacancies that occur from time to time on the Board of Directors, a nominee for director shall be elected to the Board of Directors by the vote of the majority of the votes cast by stockholders with respect to that director's election at any meeting of stockholders for the election of directors. For purposes of this Section 14, a majority of votes cast shall mean that the number of shares voted "for" a director's election exceeds fifty percent (50%) of the number of votes cast with respect to that director's election. Votes cast shall include a stockholder's direction to withhold authority in each case and shall exclude abstentions with respect to that director's election. Notwithstanding the foregoing, directors shall be elected by a plurality of the votes cast (and not by majority vote) at any meeting of stockholders where the election of directors is a Contested Election (as defined below). For purposes of these Bylaws, an election of directors shall be considered a "Contested Election" if (i) the number of nominees standing for election at any meeting of stockholders exceeds the number of directors to be elected at such meeting, with the determination that an election is "contested" to be made by the Secretary of the Corporation based on whether one or more notices of nomination, purporting to be in compliance with Article VII, Section C the Certificate of Incorporation, were received by the Secretary of the Corporation (provided that the determination that an election is a "Contested Election" shall not prejudice the ability of the Corporation to challenge whether a notice of nomination has been submitted in accordance with Article VII, Section C the Certificate of Incorporation, as applicable), and (ii) such notice of nomination or notices of nomination have not been withdrawn on or prior to the tenth (10th) calendar day preceding the date the Corporation files with the Securities and Exchange Commission ("SEC") its initial definitive proxy statement relating to such meeting of stockholders such that the number of candidates for election as director no longer exceeds the number of directors to be elected at such meeting (regardless of whether or not such proxy statement is thereafter revised or supplemented). If directors are to be elected by a plurality of the votes cast, stockholders shall not be permitted to vote against a nominee.

Each person who is nominated to stand for election as director, whether such nomination is proposed by the Corporation or a stockholder, shall, as a condition to such nomination, tender an irrevocable and executed letter of resignation in advance of the meeting for the election of directors. If a nominee for director is not elected and the nominee is an incumbent director, the Board's Nominating and Corporate Governance Committee (the "Nominating and Corporate Governance Committee") will make a recommendation to the Board as to whether to accept or reject the tendered resignation, or whether other action should be taken. The Board will act on the tendered resignation, taking into account the Nominating and Corporate Governance Committee's recommendation, and make public disclosure of its decision regarding the tendered resignation and the rationale behind the decision within ninety (90) calendar days from the date of the certification of the election results. The Nominating and Corporate Governance Committee, in making its recommendation, and the Board, in making its decision, may each consider any factors or other information that they consider appropriate and relevant. The director who tenders his or her resignation will not participate in the recommendation of the Nominating and Corporate Governance Committee or the decision of the Board with respect to his or her tender of resignation, but may participate in the recommendation or the decision regarding another director's tender of resignation.

15. Advance Notice for Nomination of Directors.

A. Only persons who are nominated in accordance with the following procedures shall be eligible for election as directors of the Corporation, except as may be otherwise provided by the terms of one or more series of Preferred Stock with respect to the rights of holders of one or more series of Preferred Stock to elect directors. Nominations of persons for election to the Board of Directors at any annual meeting of stockholders, or at any special meeting of stockholders called for the purpose of electing directors as set forth in the Corporation's notice of such special meeting, may be made (i) by or at the direction of the Board of Directors or (ii) by any stockholder of the Corporation (x) who is a stockholder of record entitled to vote in the election of directors on the date of the giving of the notice provided for in this Section 15 and on the record date for the determination of stockholders is entitled to vote at such meeting and (y) who complies with the notice procedures set forth in this Section 15.

B. In addition to any other applicable requirements, for a nomination to be made by a stockholder, such stockholder must have given timely notice thereof in proper written form to the Secretary of the Corporation pursuant to Article VII, Section C of the Corporation's Certificate of Incorporation. To be timely, a stockholder's notice to the Secretary must be received by the Secretary at the principal executive offices of the Corporation (i) in the case of an annual meeting, not later than the close of business on the 150th day prior to the first anniversary of the preceding year's annual meeting nor earlier than the close of business on the deadline specified in the Corporation's Certificate of Incorporation for stockholder nomination; provided, however, that in the event that the annual meeting is more than 30 days before or after such anniversary date (or if there has been no prior annual meeting), notice by the stockholder to be timely must be so received by the close of business on the later of (i) the deadline specified in the Corporation's Certificate of Incorporation, or (ii) the 10th day following the day on which public announcement of the date of the annual meeting was first made by the Corporation; and (ii) in the case of a special meeting of stockholders called for the purpose of electing directors, not later than the close of business on the 10th day following the day on which public announcement of the date of the special meeting is first made by the Corporation. In no event shall the adjournment, recess, continuation, rescheduling, or postponement of an annual meeting or special meeting, or the public announcement thereof, commence a new time period (or extend any time period) for the giving of a stockholder's notice as described in this Section 15.

C. Notwithstanding anything in paragraph (b) to the contrary, in the event that the number of directors to be elected to the Board of Directors at an annual meeting is greater than the number of directors whose terms expire on the date of the annual meeting and there is no public announcement by the Corporation naming all of the nominees for the additional directors to be elected or specifying the size of the increased Board before the close of business on the 90th day prior to the anniversary date of the immediately preceding annual meeting of stockholders, a stockholder's notice required by this Section 15 shall also be considered timely, but only with respect to nominees for the additional directorships created by such increase that are to be filled by election at such annual meeting, if it shall be received by the Secretary at the principal executive offices of the Corporation not later than the close of business on the 10th day following the date on which such public announcement was first made by the Corporation.

D. To be in proper written form, a stockholder's notice to the Secretary must set forth the following information:

1. as to each person whom the stockholder proposes to nominate for election as a director:

a. the name, age, business address and residence address of such person;

b. the principal occupation or employment of such person;

c. (i) the class or series and number of all shares of capital stock of the Corporation, if any, that are, directly or indirectly, owned beneficially or of record by such person (specifying the type of ownership), (ii) the name of each nominee holder of shares of all stock of the Corporation owned beneficially but not of record by such person, the number of such shares of stock of the Corporation held by each such nominee holder and any pledge with respect to any of such shares of stock, (iii) whether and the extent to which any derivative instrument, swap, option, warrant, short interest, hedge or profit interest or other transaction has been entered into by or on behalf of such person, with respect to stock of the Corporation, and (iv) whether and the extent to which any other transaction, agreement, arrangement or understanding (including any short position or any borrowing or lending of shares of stock of the Corporation) has been made by or on behalf of such person, the effect or intent of any of the foregoing being to mitigate the loss to, or to manage the risk or benefit of stock price changes for, such person, or to increase or decrease the voting power or pecuniary or economic interest of such person, with respect to stock of the Corporation;

2. a written questionnaire with respect to the background and qualification of such person, completed and executed by such person in the form required by the Corporation (which form such stockholder shall request in writing from the Secretary of the Corporation prior to submitting notice and which the secretary shall provide within ten days after receiving such request);

3. a written representation and agreement completed by such person in the form required by the Corporation providing that such person (which form such stockholder shall request in writing from the Secretary of the Corporation prior to submitting notice and which the Secretary shall provide within ten (10) days after receiving such request): (a) is not and will not become a party to any agreement, arrangement or understanding with, and any commitment or assurance to, any person or entity as to how a person, if elected as a director, will act or vote on any issue or question that has not been disclosed to the Corporation or that could limit or interfere with such person's ability to comply, if elected as a director of the Corporation, with such person's fiduciary duties under applicable law; (b) is not and will not become a party to any agreement, arrangement or understanding with any person or entity other than the Corporation with respect to any direct or indirect compensation, reimbursement or indemnification in connection with service or action as a director or a director nominee that has not been disclosed to the Corporation; (c) will, if elected as a director, comply with all applicable rules of any securities exchanges upon which the Corporation's securities are listed, the Certificate of Incorporation, these Bylaws, all applicable publicly disclosed corporate governance, ethics, conflict of interest, confidentiality, stock ownership and trading policies and all other guidelines and policies of the Corporation generally applicable to directors (which other guidelines and policies will be provided to such person within five (5) business days after the Secretary receives any written request therefor from such person), and all applicable fiduciary duties under state law; (d) intends to serve a full term as a director, if elected; (e) will provide facts, statements and other information in all communications with the Corporation and its stockholders that are or will be true and correct and that do not and will not omit to state any fact necessary in order to make the statements made, in light of the circumstances under which they are made, not misleading; and (f) will tender his or her resignation as a director if the Board of Directors determines that such person failed to comply with the provisions of this Section 15 in all material respects, provides such person notice of any such determination and, if such non-compliance may be cured, such person fails to cure such non-compliance within ten business days after delivery of such notice to such person;

4. a description of any business or personal interests that could place such person in a potential conflict of interest with the Corporation or any of its subsidiaries;

5. any other information relating to such person that would be required to be disclosed in a proxy statement or other filings required to be made in connection with solicitations of proxies for election of directors pursuant to Section 14 of the Exchange Act and the rules and regulations promulgated thereunder (including such person's written consent to being named in the Corporation's proxy statement and form of proxy as a nominee and to serve as a director if elected);

6. a description of all direct and indirect compensation and other material monetary agreements, arrangements and understandings during the past three years, and any other material relationships, between or among any stockholder giving the notice (including the beneficial owner, if any, on whose behalf the nomination is being made) or Stockholder Associated Person, on the one hand, and each such proposed nominee and his or her respective affiliates and associates, on the other hand, including, without limitation, all information that would be required to be disclosed pursuant to Item 404 under Regulation S-K if such any stockholder giving the notice (including the beneficial owner, if any, on whose behalf the nomination is being made) or Stockholder Associated Person were the "registrant" for purposes of such rule and the such proposed nominee were a director or executive officer of such registrant; and

E. as to the stockholder giving the notice, and any Stockholder Associated Person, if any, on whose behalf the nomination is being made:

1. the name and record address of the stockholder giving the notice and the name and address of any Stockholder Associated Person (including, if applicable, the name and address that appear on the Corporation's stock ledger);

2. (a) the class or series and number of all shares of capital stock of the Corporation that are, directly or indirectly, owned beneficially or of record by such stockholder or any Stockholder Associated Person (specifying the type of ownership), (b) the name of each nominee holder of shares of the Corporation owned beneficially but not of record by such stockholder or any Stockholder Associated Person, the number of shares of stock of the Corporation held by each such nominee holder and any pledge with respect to any of such stock, (c) whether and the extent to which any derivative instrument, swap, option, warrant, short interest, hedge or profit interest or other transaction has been entered into by or on behalf of such stockholder or any Stockholder Associated Person, with respect to stock of the Corporation and (d) whether and the extent to which any other transaction, agreement, arrangement or understanding (including any short position or any borrowing or lending of shares of stock of the Corporation) has been made by or on behalf of such stockholder or any Stockholder Associated Person, the effect or intent of any of the foregoing being to mitigate the loss to, or to manage the risk or benefit of stock price changes for, such stockholder or any Stockholder Associated Person, or to increase or decrease the voting power or pecuniary or economic interest of such stockholder or any Stockholder Associated Person, respect to stock of the Corporation;

3. a complete and accurate description of all agreements, arrangements, or understandings (whether written or oral) between or among such stockholder or any Stockholder Associated Person, and any proposed nominee or any other person or persons or entity (naming each such person or entity) pursuant to which (a) the nomination(s) are being made by such stockholder, and any material interest of such stockholder or any Stockholder Associated Person, in such nomination, including any anticipated benefit therefrom to such stockholder or any Stockholder Associated Person, and (b) any understanding that such stockholder or any Stockholder Associated Person may have reached with a stockholder of the Corporation (including the name of such stockholder) with respect to how such stockholder will vote such stockholder's shares in the Corporation in support of any nomination;

4. any direct or indirect interest of such stockholder or any Stockholder Associated Person in any contract or arrangement with the Corporation, any affiliate of the Corporation or any principal competitor of the Corporation (including any employment agreement or consulting agreement);

5. a representation that (a) no such stockholder or any Stockholder Associated Person has breached any contract or other agreement, arrangement or understanding with the Corporation except as disclosed to the Corporation pursuant hereto and (b) that such stockholder or any Stockholder Associated Person has complied, and will comply, with all applicable requirements of state law and Exchange Act with respect to the matters set forth in this Section 15;

6. a complete and accurate description of any pending or, to such stockholder's knowledge, threatened legal proceeding in which such stockholder or any Stockholder Associated Person is a party or participant involving the Corporation or, to such stockholder's knowledge, any current or former officer, director, affiliate or associate of the Corporation;

7. a representation that the stockholder giving notice intends to appear in person or by proxy at the annual meeting of stockholders or special meeting of stockholders to nominate the persons named in such stockholder's notice and an acknowledgment that, if such stockholder (or a qualified representative of such stockholder) does not appear to nominate the persons named in the stockholder's notice at such meeting, such nomination shall be disregarded and no vote shall be taken with respect to such nomination, notwithstanding that proxies in respect of such vote may have been received by the Corporation;

8. a representation from such stockholder as to whether such stockholder or any Stockholder Associated Person intends or is part of a group that intends (a) to deliver a proxy statement and/or form of proxy to a number of holders of the Corporation's voting shares reasonably believed by such stockholder to be sufficient to elect the person named in the stockholder's notice, (b) to solicit proxies in support of the election of any nominee named in the stockholder's notice in accordance with Rule 14a-19 under the Exchange Act, or (c) to engage in a solicitation (within the meaning of Exchange Act Rule 14a-1(l)) with respect to the nomination, and if so, the name of each participant (as defined in Item 4 of Schedule 14A under the Exchange Act) in such solicitation; and

9. any other information relating to such stockholder or any Stockholder Associated Person that would be required to be disclosed in a proxy statement or other filings required to be made in connection with the solicitation of proxies for the election of directors pursuant to Section 14 of the Exchange Act and the rules and regulations promulgated thereunder.

F. A stockholder providing notice of any nomination proposed to be made at an annual meeting of stockholders or a special meeting of stockholders shall further update and supplement such notice, if necessary, so that the information provided or required to be provided in such notice pursuant to this Section 15 shall be true and correct as of the record date for determining the stockholders entitled to receive notice of the annual meeting of stockholders or special meeting of stockholders, and such update and supplement shall be delivered either in person or by United States certified mail, postage prepaid, and received by the secretary at the principal executive offices of the Corporation not later than five (5) business days after the record date for determining the stockholders entitled to receive notice of such annual meeting of stockholders or special meeting of stockholders. The update and supplement shall clearly identify the information that has changed since such stockholder's prior submission. If a stockholder fails to provide such written update within such period, the information as to which such written update relates may be deemed not to have been provided in accordance with this Section 15. For the avoidance of doubt, the obligation to update and supplement as set forth in this paragraph or any other Section of these Bylaws shall not be deemed to extend or waive any applicable deadlines under these Bylaws, cure deficiencies in any notice of nomination or permit a change in the nominee(s) or nomination(s) proposed to be made at a meeting of the stockholders as identified in the notice of nomination. In addition, the stockholder shall promptly provide any other information reasonably requested by the Corporation. If requested by the Corporation, any supplemental information required under this paragraph shall be provided within ten (10) days after it has been requested by the Corporation. In addition, the Board of Directors may require any proposed nominee to submit to interviews with the Board of Directors or any committee thereof, and such proposed nominee shall make himself or herself available for any such interviews within ten (10) days following any reasonable request therefor from the Board of Directors or any committee thereof.

G. In addition to the provisions of this Section 15, a stockholder shall also comply with all of the applicable requirements of the Exchange Act and the rules and regulations thereunder with respect to the matters set forth herein. Nothing in this Section 15 shall be deemed to affect any rights of the holders of Preferred Stock to elect directors pursuant to the Certificate of Incorporation.

H. General.

1. Only such persons who are nominated in accordance with the procedures set forth in this Bylaw shall be eligible to serve as directors and only such business shall be conducted at a meeting of stockholders as shall have been brought before the meeting in accordance with the procedures set forth in this Bylaw. Except as otherwise provided by law, the Certificate of Incorporation or these Bylaws, the chair of the meeting shall have the power and duty to determine whether a nomination or any business proposed to be brought before the meeting was made or proposed, as the case may be, in accordance with the procedures set forth in this Bylaw and, if any proposed nomination or business is not in compliance with this Bylaw, to declare that such defective proposal or nomination shall be disregarded.

2. For purposes of these Bylaws, "public announcement" shall mean disclosure in a press release reported by the Dow Jones News Service, Associated Press or comparable national news service or in a document publicly filed by the Corporation with the Securities and Exchange Commission pursuant to Section 13, 14 or 15(d) of the Exchange Act.

3. For purposes of these Bylaws, a "Stockholder Associated Person" of any stockholder shall mean (i) any person controlling, directly or indirectly, or acting in concert with, such stockholder or any beneficial owner described in the immediately following clause (ii), (ii) any beneficial owner of shares of stock of the corporation owned of record or beneficially by such stockholder and on whose behalf the proposal or nomination, as the case may be, is being made, or (iii) any person controlling, controlled by or under common control with such person referred to in the preceding clauses (i) and (ii).

4. Notwithstanding the foregoing provisions of this Bylaw, a stockholder shall also comply with all applicable requirements of the Exchange Act and the rules and regulations thereunder with respect to the matters set forth in this Bylaw. Nothing in this Bylaw shall be deemed to affect any rights (i) of stockholders to request inclusion of proposals in the Corporation's proxy statement pursuant to Rule 14a-8 under the Exchange Act or (ii) of the holders of any series of Preferred Stock if and to the extent provided for under law, the Certificate of Incorporation or these Bylaws.

16. Adjournments. If less than a quorum of the outstanding shares entitled to vote is represented at any meeting of the stockholders, a majority of the shares so represented may adjourn the meeting from time to time for a period not to exceed thirty (30) days at any one adjournment, without further notice, provided the time and place thereof are announced at the meeting at which the adjournment is taken. At the adjourned meeting, the Corporation may transact any business which might have been transacted at the original meeting. Any meeting of the stockholders may adjourn from time to time until its business is completed. If the adjournment is for more than thirty (30) days, or if after the adjournment a new record date is fixed for the adjourned meeting, a notice of the adjourned meeting shall be given to each stockholder of record entitled to vote at the meeting.

17. Informal Act by Stockholders. Any action required to be taken at a meeting of stockholders, or any action which may be taken at a meeting of stockholders, may be taken without a meeting, without prior notice and without a vote, if a consent or consents in writing setting forth the action so taken, shall be signed by the holders of outstanding stock having not less than the minimum number of votes that would be necessary to authorize or take such action at a meeting at which all shares entitled to vote thereon were present and voted shall be delivered to the Corporation by said consent or consents delivered at its principal place of business or such other place as designated by the board of directors. Delivery made to the Corporation shall be by hand or by certified or registered mail, return receipt requested.

ARTICLE III.

Board of Directors

1. Number, Qualifications and Term of Office. Except as otherwise provided in the Certificate of Incorporation or the General Corporation Law of Delaware, the business and affairs of the Corporation shall be managed under the direction of a board of directors consisting of from three to fifteen members. Each director shall be a natural person of the age of fifteen years or older, but does not need to be a resident of the state of Delaware or a stockholder of the Corporation. The board of directors, by resolution, may increase or decrease the number of directors from time to time. Except as otherwise provided in these Bylaws or in the Certificate of Incorporation, the board of directors shall be divided into three (3) classes as nearly equal in number as possible. Each director in each class shall be elected at the appropriate annual meeting of stockholders, as determined by the Certificate of Incorporation, and shall hold office for a term of three (3) years and until his successor is elected and qualified or until his earlier resignation or removal. No decrease in the number of directors shall have the effect of shortening the term of any incumbent director.

2. Vacancies and Newly Created Directorships. Vacancies and newly created directorships resulting from any increase in the authorized number of directors elected by all of the stockholders having the right to vote as a single class shall be filled solely by a majority of the directors then in office, although less than a quorum, or by a sole remaining director. Any directors so chosen shall hold office until the next election of the class for which such director shall have been chosen, and until their successors shall be elected and qualified. No decrease in the number of directors constituting the board of directors shall shorten the term of any incumbent director.

If at any time of filling any vacancy or newly created directorship, the directors then in office shall constitute less than a majority of the whole board, the Court of Chancery may, upon application of any stockholder or stockholders holding at least ten percent (10%) of the total number of shares at the time outstanding having the right to vote for such directors, summarily order an election to be held to fill any such vacancies or newly created directorships, or to replace the directors chosen by the directors then in office as aforesaid, which election shall be governed by Section 211 of the General Corporation Law of Delaware.

Any director may resign at any time by giving written notice to the president or to the secretary of the Corporation. Such resignation shall take effect at the future time specified therein; and unless otherwise specified therein, the acceptance of such resignation shall not be necessary to make it effective. Any vacancy occurring on the board of directors created by the resignation of a director, may be filled by the affirmative vote of a majority of directors then in office, including those who have so resigned. The vote thereon shall take effect when such resignation or resignations shall become effective. A director elected to fill a vacancy shall be elected for the unexpired term of his predecessor in office.

3. Removal. Any director or the entire board of directors may be removed in accordance with the provisions of Article VII Subparagraph D of the Certificate of Incorporation.

4. Compensation. Any director may be paid any one or more of the following: his expenses, if any, of attendance at meetings; a fixed sum for attendance at each meeting; or a stated salary as director. No such payment shall preclude any director from serving the Corporation in any other capacity and receiving compensation therefor. A director shall also be entitled to receive options for the acquisition of shares of stock of the corporation.

ARTICLE IV.

Meetings of the Board

1. Place of Meetings. The regular or special meetings of the board of directors or any committee designated by the board may be held at the principal office of the Corporation or at any other place within or without the State of Delaware that a majority of the board of directors or any such committee, as the case may be, may designate from time to time by resolution.

2. Regular Meetings. The board of directors shall meet each year immediately after the annual meeting of the stockholders for the purpose of electing officers and transacting such other business as may come before the meeting. The board of directors or any committee designated by the board may provide, by resolution, for the holding of additional regular meetings without other notice than such resolution.

3. Special Meetings. Special meetings of the board of directors or any committee designated by the board may be called at any time by the chairman of the board, if any, by the president or by a majority of the members of the board of directors or any such committee, as the case may be.

4. Notice of Meetings. Notice of the regular meetings of the board of directors or any committee designated by the board need not be given. Except as otherwise provided by these Bylaws or the laws of the State of Delaware, written notice of each special meeting of the board of directors or any such committee setting forth the time and the place of the meeting shall be given to each director not less than two (2) days prior to the time fixed for the meeting by sending a copy of the notice through the United States mail, charges prepaid, to the address of each director appearing on the books of the Corporation, or not less than twenty-four (24) hours prior to the time fixed for the meeting either personally, personally by telephone or by sending a copy of the notice by electronic mail or other means of electronic transmission to an address of each director appearing on the books of the Corporation. Neither the business to be transacted at, nor the purpose of, any regular or special meeting of the board of directors need be specified in the notice or waiver of notice of such meeting.

5. Waiver of Notice. A director may in writing waive notice of any special meeting of the board of directors or any committee, either before, at, or after the meeting; and his waiver shall be deemed the equivalent of giving notice. Attendance of a director at a meeting shall constitute waiver of notice of that meeting unless he attends for the express purpose of objecting to the transaction of business because the meeting has not been lawfully called or convened.

6. Quorum. At meetings of the board of directors or any committee designated by the board a majority of the number of directors fixed by these Bylaws or a majority of the members of any such committee, as the case may be, shall be necessary to constitute a quorum for the transaction of business. If a quorum is present, the act of the majority of directors in attendance shall be the act of the board of directors or any such committee, as the case may be, unless the act of a greater number is required by these Bylaws, the Certificate of Incorporation or the General Corporation Law of Delaware. One or more directors may participate in meetings of the board of directors as authorized by Subparagraph 11 of this Article IV by conference telephone, while the remaining director or directors are physically present at the meeting.

7. Presumption of Assent. A director who is present at a meeting of the board or committee designated by the board when corporate action is taken is deemed to have assented to the action taken unless: (i) he objects at the beginning of such meeting to the holding of the meeting or the transacting of business at the meeting; (ii) he contemporaneously requests that his dissent from the action taken be entered in the minutes of such meeting; or (iii) he gives written notice of his dissent to the presiding officer of such meeting before its adjournment or to the secretary of the Corporation immediately after adjournment of such meeting. The right of dissent as to a specific action taken in a meeting of a board or committee thereof is not available to a director who votes in favor of such action.

8. Reliance on Books of Account or Reports. Any member of the board of directors or any committee designated by the board of directors shall, in the performance of his duties, be fully protected in relying in good faith upon the records of the Corporation and upon such information, opinions, reports or statements presented to the Corporation by any of its officers, or employees, or committees of the board of directors, or by any other person as to matters the members reasonably believes are within such other persons professional or expert competence and who has been selected with reasonable care by or on behalf of the Corporation, or in relying in good faith upon other records of the Corporation.

9. Committees. The board of directors may, by a resolution passed by a majority of the whole board designate one (1) or more committees, each committee to consist of one (1) or more directors of the corporation. The board may designate one or more directors as alternate members of any committee who may replace any absent or disqualified member at any meeting of the committee. In the absence or disqualification of a member of a committee, the member or members present at any meeting and not disqualified from voting, whether or not he or they constitute a quorum, may unanimously appoint another member of the board of directors to act at the meeting in the place of any such absent or disqualified member.

Any such committee to the extent provided in the resolution of the board of directors shall have and may exercise all of the powers and authority of the board of directors in the management of the business and affairs of the Corporation and may authorize the seal of the Corporation to be affixed to all papers which it may acquire. No such committee shall have the power or authority of the board of directors to: (i) amend the Certificate of Incorporation; (ii) adopt an agreement of merger or consolidation; (iii) recommend to the stockholders the sale, lease or exchange of all or substantially all of the Corporation's property and assets; (iv) recommend to the stockholders a dissolution of the Corporation or a revocation of a dissolution; (v) amend the Bylaws of the Corporation; (vi) or unless expressly provided for by resolution, or in the Certificate of Incorporation, declare a dividend, authorize the issuance of stock or to adopt a certificate of ownership and merger. To the extent authorized by resolution or resolutions providing for the issuance of shares of stock, adopted by the board, a committee may: (i) fix the designations and any of the preferences or rights of such shares relating to dividends, redemption, dissolution, any distribution of assets of the Corporation or the conversion into, or the exchange of such shares for, shares of any other class or classes or any other series of the same or any other class or classes of stock of the Corporation; or (ii) fix the number of shares of any series of stock or authorize the increase or decrease of the shares of any series. If any such delegation of the authority of the board of directors is made as provided herein, all references to the board of directors contained in these Bylaws, the Certificate of Incorporation, the General Corporation Law of Delaware or any other applicable law or regulation relating to the authority so delegated shall be deemed to refer to such committee.

10. Informal Action by Directors. Any action required or permitted to be taken at a meeting of the board of directors or any committee thereof, may be taken without a meeting if all the members of the board or committee, as the case may be, consent thereto in writing, and the writing or writings are filed with the minutes of proceedings of the board or committee. Such consent shall have the same force and effect as a unanimous vote of the directors and may be stated as such in any articles or documents filed with the Secretary of State of Delaware under the General Corporation Law of Delaware.

11. Telephonic Meetings. Members of the board of directors or any committee designated by the board may participate in meeting of such board or committee by means of a conference telephone or similar communications equipment by which all persons participating in the meeting can hear each other at the same time. Participation in such a meeting shall constitute presence in person at the meeting.

ARTICLE V.

Officers and Agents

1. General. The executive officers of the Corporation shall be elected annually by the board of directors at the first meeting of the board held after each annual meeting of the stockholders. If the election of such officers shall not be held at such meeting, such election shall take place as soon thereafter as a meeting may conveniently be held. The officers of the Corporation shall consist of a president, a secretary and a treasurer, or a secretary/treasurer; in addition, one or more vice presidents, a chairman of the board of directors and such other officers, assistant officers, agents and employees that the board of directors may from time to time deem necessary may be elected by the board of directors or be appointed in a manner prescribed by the board.

Two or more offices may be held by the same person. Officers shall hold office until their successors are elected and qualified, unless they are sooner removed from office as provided in these Bylaws. All officers of the Corporation shall be natural persons of the age of eighteen years or older. Officers of the Corporation need not be residents of the State of Delaware or directors or stockholders of the Corporation.

2. General Duties. All officers and agents of the Corporation, as between themselves and the Corporation, shall have such authority and shall perform such duties in the management of the Corporation as may be provided in these Bylaws or as may be determined by resolution of the board of directors not inconsistent with these Bylaws. In all cases where the duties of any officer, agent or employee are not prescribed by the Bylaws or by the board of directors, such officer, agent or employee shall follow the orders and instructions of the president.

Any officer shall have the power to execute and deliver on behalf of and in the name of the Corporation any instrument requiring the signature of an officer of the Corporation, except as otherwise provided in these Bylaws or where the execution and delivery thereof shall be expressly delegated by the board of directors to some other officer or agent of the Corporation. Unless authorized to do so by these Bylaws or by the board of directors, no officer, agent or employee shall have any power or authority to bind the Corporation in any way, to pledge its credit or to render it liable pecuniarily for any purpose or in any amount.

3. Vacancies. When a vacancy occurs in one of the executive offices by reason of death, resignation or otherwise, it shall be filled by a resolution of the board of directors. The officer so selected shall hold office until his successor is chosen and qualified.

4. Salaries. The board of directors shall fix the salaries of the officers of the Corporation. The salaries of other agents and employees of the Corporation may be fixed by the board of directors, or by any committee designated by the board or by an officer to whom that function has been delegated by the board. No officer shall be prevented from receiving such salary by reason of the fact that he is also a director of the Corporation.

5. Removal. Any officer or agent of this Corporation may be removed by the board of directors whenever in its judgment the best interests of the Corporation may be served thereby, but such removal shall be without prejudice to the contract rights, if any, of the person so removed. Election or appointment of an officer or an agent shall not of itself create contract rights.

6. Chairman of the Board. The chairman of the board, if any, shall preside as chairman at meetings of the stockholders and the board of directors. He shall, in addition, have such other duties as the board may prescribe that he perform. At the request of the president, the chairman of the board may, in the case of the president's absence or inability to act, temporarily act in his place. In the case of death of the president or in the case of his absence or inability to act without having designated the chairman of the board to act temporarily in his place, the chairman of the board shall perform the duties of the president, unless the board of directors, by resolution, provides otherwise. If the chairman of the board shall be unable to act in place of the president, any vice president may exercise such powers and perform such duties as provided in section 8 below.

6a. Executive Chairman. The board of directors may from time to time elect or appoint the chairman of the board, if any, to serve as executive chairman. To the extent such position is filled, the executive chairman shall be an executive officer of the Corporation who reports to the board of directors. The executive chairman shall have such powers and duties as the board of directors shall designate from time to time.

6b. Chief Executive Officer. The board of directors shall appoint a chief executive officer, who shall be the Corporation's principal executive officer. All other executive officers, other than the executive chairman, shall report to the chief executive officer. The chief executive officer shall have general and active management and supervision of the business and affairs of the Corporation.

7. President. The president shall be the chief executive officer of the Corporation (unless the board of directors appoints another executive to be the chief executive officer of the Corporation with such duties and responsibilities as the board of directors shall delegate from time to time), and, subject to the control of the board of directors, shall have general supervision of the business and affairs of the Corporation. In the event the position of chairman of the board shall not be occupied or the chairman shall be absent or otherwise unable to act, the president shall preside at meetings of the stockholders and directors and shall discharge the duties of the presiding officer. At each annual meeting of the stockholders the president shall give a report of the business of the Corporation for the preceding fiscal year and shall perform whatever other duties the board of directors may from time to time prescribe. The president may sign, with the secretary or any other proper officer of the Corporation thereunto authorized by the board of directors, certificates for shares of the Corporation, any deeds, mortgages, bonds, contracts, or other instruments which the board of directors has authorized to be executed, except in cases where the signing and execution thereof shall be expressly delegated by the board of directors or by these Bylaws to some other officer or agent of the Corporation, or shall be required by law to be otherwise signed or executed.

8. Vice Presidents. Each vice president shall have such powers and perform such duties as the board of directors may from time to time prescribe or as the president may from time to time delegate to him. At the request of the president, in the case of the president's absence or inability to act, any vice president may temporarily act in his place. In the case of the death of the president, or in the case of his absence or inability to act without having designated a vice president or vice presidents to act temporarily in his place, the board of directors, by resolution, may designate a vice president or vice presidents, to perform the duties of the president. If no such designation shall be made, the chairman of the board of directors, if any, shall exercise such powers and perform such duties, as provided in Section 6 above, but if the Corporation has no chairman of the board of directors, or if the chairman is unable to act in place of the president, all the vice presidents may exercise such powers and perform such duties.

9. Secretary. The secretary shall keep or cause to be kept in books provided for that purpose the minutes of the meetings of the stockholders, executive committee, if any, and any other committees, and of the board of directors; shall see that all notices are duly given in accordance with the provisions of these Bylaws and as required by law; shall be custodian of the records and of the seal of the Corporation and see that the seal is affixed to all documents, the execution of which on behalf of the Corporation under its seal is duly authorized and in accordance with the provisions of these Bylaws; keep a register of the post office address of each stockholder which shall be furnished to the secretary by such stockholder, sign with the president certificates for shares of the Corporation, the issuance of which shall have been authorized by resolution of the board of directors; have a general charge of the stock transfer books of the Corporation; and, in general, shall perform all duties incident to the office of secretary and such other duties as may, from time to time, be assigned to him by the board of directors or by the president. In the absence of the secretary or his inability to act, the assistant secretaries, if any, shall act with the same powers and shall be subject to the same restrictions as are applicable to the secretary.

10. Treasurer. The treasurer shall have custody of corporate funds and securities. He shall keep full and accurate accounts of receipts and disbursements and shall deposit all corporate monies and other valuable effects in the name and to the credit of the Corporation in the depository or depositories of the Corporation selected by the board of directors, and shall render an account of his transactions as treasurer and of the financial condition of the Corporation to the president and/or the board of directors upon request. Such power given to the treasurer to deposit and disburse funds shall not, however, preclude any other officer or employee of the Corporation from also depositing and disbursing funds when authorized to do so by the board of directors. The treasurer shall, if required by the board of directors, give the Corporation a bond in such amount and with such surety or sureties as may be ordered by the board of directors for the faithful performance of duties of his office. The treasurer shall have such other duties as may be from time to time prescribed by the board of directors or the president. In the absence of the treasurer or his inability to act, the assistant treasurers, if any, shall act with the same authority and shall be subject to the same restrictions as are applicable to the treasurer.

11. Delegation of Duties. Whenever an officer is absent, or whenever, for any reason, the board of directors may deem it desirable, the board may delegate the powers and duties of an officer to any other officer or officers or to any director or directors.

12. Bond of Officers. The board of directors may require any officer to give the Corporation a bond in such sum and with such surety or sureties as shall be satisfactory to the board of directors for such terms and conditions as the board of directors may specify, including without limitation for the faithful performance of his duties and for the restoration to the Corporation of all property in his possession or under his control belong to the Corporation.

13. Loans to Director, Officers, Employees. The Corporation may lend money to, guarantee the obligations of and otherwise assist directors, officers and employees of the Corporation, or directors of another corporation of which the Corporation owns a majority of the voting stock to the extent of and in compliance with the General Corporation Laws of Delaware.

ARTICLE VI.

Stock Certificates and the Transfer of Shares

1. Stock Certificates; Uncertificated Shares. The shares of the Corporation shall be represented by certificates, provided that the board of directors of the Corporation may provide by resolution or resolutions that some or all of any or all classes or series of its stock shall be uncertificated shares. Any such resolution shall not apply to shares represented by a certificate until such certificate is surrendered to the corporation. Notwithstanding the adoption of such a resolution by the board of directors, every holder of stock represented by certificates and upon request every holder of uncertificated shares shall be entitled to have a certificate signed by, or in the name of the Corporation by the chairman or vice-chairman of the board of directors, or the president or vice-president, and by the treasurer or an assistant treasurer, or the secretary or an assistant secretary of the Corporation representing the number of shares registered in certificate form. Any or all the signatures on the certificate may be a facsimile. In case any officer, transfer agent or registrar who has signed or whose facsimile signature has been placed upon a certificate shall have ceased to be such officer, transfer agent or registrar before such certificate is issued, it may be issued by the Corporation with the same effect as if he were such officer, transfer agent or registrar at the date of issue.

2. Consideration for Shares. Shares shall be issued for such consideration as shall be fixed from time to time by the board of directors. Consideration for shares shall be expressed in dollars, and shall not be less than the par value or stated value therefor, as the case may be. The par value for shares, if any, shall be stated in the Certificate of Incorporation, and the stated value for shares, if any, shall be fixed from time to time by the board of directors. Treasury shares may be disposed of by the Corporation for such consideration expressed in dollars as may be fixed from time to time by the board. Consideration for shares may consist, in whole or in part, of money, other property whether tangible, intangible or both, or in labor or services actually performed for the Corporation, but the promise of future services of a subscriber or direct purchaser of shares from the Corporation shall not constitute payment or part payment for shares.

3. Lost Certificates. The board of directors may direct a new certificate of stock or uncertificated share in place of any certificate issued by it, alleged to have been lost, stolen or destroyed if the owner makes an affidavit or affirmation of that fact and produces such evidence of loss or destruction as the board may require. The board, in its discretion, may as a condition precedent to the issuance of a new certificate require the owner to give the Corporation a bond sufficient to indemnify it against any claim that may be made against the Corporation on account of the alleged loss, theft or destruction of the certificate or the issuance of such new certificate.

4. Transfer of Shares. Shares of the Corporation shall only be transferred on its books upon the surrender to the Corporation of the share certificates duly endorsed or accompanied by proper evidence of succession, assignment or authority to transfer and such documentary stamps as may be required by law. In that event, the surrendered certificates shall be cancelled, new certificates issued to the persons entitled to them, and the transaction recorded on the books or the Corporation.

5. Registered Stockholders. The Corporation shall be entitled to treat the holder of record of shares as the holder in fact and, except as otherwise provided by the laws of Delaware, shall not be bound to recognize any equitable or other claim to or interest in the shares.

The board of directors may adopt by resolution a procedure whereby a stockholder may certify in writing to the Corporation that all or a portion of the shares registered in the name of such stockholder are held for the account of a specified person or persons. Such resolution shall set forth: (i) the classification of stockholder who may certify; (ii) the purpose or purposes for which the certification may be made; (iii) the form of certification and information to be contained therein; (iv) if the certification is with respect to a record date or closing of the stock transfer books within which the certification must be received by the Corporation; and (v) such other provisions with respect to the procedure as are deemed necessary or desirable.

Upon receipt by the Corporation of a certification complying with the procedure, the persons specified in the certification shall be deemed, for the purpose or purposes set forth in the certification, to be the holders of record of the number of shares specified in place of the stockholder making the certification.

6. Stock Ledger. An appropriate stock journal and ledger shall be kept by the secretary or such registrars or transfer agents as the directors by resolution may appoint in which all transactions in the shares of stock of the Corporation shall be recorded.

7. Location. The books, accounts and records of the Corporation may be kept at such place or places within or outside the State of Delaware as the board of directors may from time to time determine.

8. Inspection. The books, accounts and records of the Corporation shall be open for inspection by any member of the board of directors at all times, and open to inspection by the stockholders at such times, and subject to such regulations as the board of directors may prescribe, except as otherwise provided by statute.

ARTICLE VII.

Seal and Fiscal Year

1. Seal. The Corporation shall have a seal in the form impressed to the left of this paragraph of the Bylaws.

2. Fiscal Year. The fiscal year of the Corporation shall be determined by the board of directors and set forth in the minutes of the directors. Said fiscal year may be changed from time to time by the board of directors in its discretion.

ARTICLE VIII.

Dividends

Dividends shall be declared and paid out of the surplus or net profits for the fiscal year in which the dividend is declared, and/or the preceding fiscal year as often and at such times as the board of directors may determine. If the capital of the Corporation, computed in accordance with the General Corporation Law of Delaware, shall have been diminished by depreciation in the value of its property, or by losses, or otherwise, to an amount less than the aggregate amount of the capital represented by the issued and outstanding stock; the board of directors shall not declare and pay out of net profits any dividends upon any shares of its capital stock until the deficiency in the amount of capital represented by issued and outstanding stock shall have been repaired. No unclaimed dividend shall bear interest against the Corporation.

ARTICLE IX.

Amendments

Subject to repeal or change by action of the stockholders in accordance with the Certificate of Incorporation, the board of directors may amend, supplement or repeal these Bylaws or adopt new Bylaws, and all such changes shall affect and be binding upon the holders of all shares heretofore as well as hereafter authorized, subscribed for or offered.

ARTICLE X.

Miscellaneous

1. Gender. Whenever required by the context, the singular shall include the plural, the plural the singular, and one gender shall include all genders.

2. Invalid Provision. The invalidity or unenforceability of any particular provision of these Bylaws shall not affect the other provisions herein, and these Bylaws shall be construed in all respects as if such invalid or unenforceable provision was omitted.

3. Governing Law. These Bylaws shall be governed by and construed in accordance with the laws of the State of Delaware.

4. Severability. If any provision (or any part thereof) or provisions of these Bylaws shall be held to be invalid, illegal or unenforceable as applied to any circumstance for any reason whatsoever: (i) the validity, legality and enforceability of such provisions in any other circumstance and of the remaining provisions of these Bylaws (including, without limitation, each portion of any section of these Bylaws containing any such provision held to be invalid, illegal or unenforceable that is not itself held to be invalid, illegal or unenforceable) shall not in any way be affected or impaired thereby and (ii) to the fullest extent possible, the provisions of these Bylaws (including, without limitation, each such portion containing any such provision held to be invalid, illegal or unenforceable) shall be construed for the benefit of the Corporation to the fullest extent permitted by law so as to (a) give effect to the intent manifested by the provision held invalid, illegal or unenforceable, and (b) permit the Corporation to protect its directors, officers, employees and agents from personal liability in respect of their good faith service. Reference herein to laws, regulations or agencies shall be deemed to include all amendments thereof, substitutions therefor and successors thereto, as the case may be.

5. Meeting Attendance via Remote Communication Equipment.

A. Stockholder Meetings. If authorized by the board of directors in its sole discretion, and subject to such guidelines and procedures as the board of directors may adopt, stockholders entitled to vote at such meeting and proxy holders not physically present at a meeting of stockholders may, by means of remote communication:

1. participate in a meeting of stockholders; and

2. be deemed present in person and vote at a meeting of stockholders, whether such meeting is to be held at a designated place or solely by means of remote communication, provided that (A) the Corporation shall implement reasonable measures to verify that each person deemed present and permitted to vote at the meeting by means of remote communication is a stockholder or proxy holder, (B) the Corporation shall implement reasonable measures to provide such stockholders and proxy holders a reasonable opportunity to participate in the meeting and to vote on matters submitted to the stockholders, including an opportunity to read or hear the proceedings of the meeting substantially concurrently with such proceedings, and (C) if any stockholder or proxy holder votes or takes other action at the meeting by means of remote communication, a record of such votes or other action shall be maintained by the Corporation.

B. Board Meetings. Unless otherwise restricted by applicable law, the Certificate of Incorporation or these Bylaws, members of the board of directors or any committee thereof may participate in a meeting of the board of directors or any committee thereof by means of conference telephone or other communications equipment by means of which all persons participating in the meeting can hear each other. Such participation in a meeting shall constitute presence in person at the meeting, except where a person participates in the meeting for the express purpose of objecting to the transaction of any business on the ground that the meeting was not lawfully called or convened.

ARTICLE XI.

Exclusive Jurisdiction for Certain Actions

Unless the Corporation consents in writing to the selection of an alternative forum, the Court of Chancery of the State of Delaware shall, to the fullest extent permitted by law, be the sole and exclusive forum for (a) any derivative action or proceeding brought on behalf of the Corporation, (b) any action asserting a claim of breach of fiduciary duty owed by, or other wrongdoing by, any director, officer, employee or agent of the Corporation to the Corporation or the Corporation's stockholders, creditors or other constituents, (c) any action asserting a claim arising pursuant to any provision of the General Corporation Law of Delaware or the Certificate of Incorporation or these Bylaws of the Corporation, (d) any action to interpret, apply, enforce or determine the validity of the Certificate of Incorporation or these Bylaws of the Corporation or (e) any action asserting a claim governed by the internal affairs doctrine, in each case subject to said Court of Chancery of the State of Delaware having personal jurisdiction over the indispensable parties named as defendants therein; provided that if and only if the Court of Chancery of the State of Delaware dismisses any such action for lack of subject matter jurisdiction, such action may be brought in another state or federal court sitting in the State of Delaware.

Unless the Corporation consents in writing to the selection of an alternative forum, the federal district courts of the United States shall, to the fullest extent permitted by law, be the sole and exclusive forum for the resolution of any complaint asserting a cause of action arising under the Securities Act of 1933, as amended.

To the fullest extent permitted by applicable law, any person or entity purchasing or otherwise acquiring any interest in shares of capital stock of the Corporation shall be deemed to have notice of and consented to the provisions of this Article XI of these Bylaws.

EXHIBIT 19

ABEONA THERAPEUTICS INC.
POLICY ON INSIDER TRADING AND CONFIDENTIALITY

I. Purpose

The purpose of this Policy on Insider Trading and Confidentiality (the "Policy") is to provide guidelines with respect to securities of Abeona Therapeutics Inc. ("Abeona" or "Company") and the handling of confidential and material nonpublic information about the Company and the companies with which the Company does business. The Company's Board of Directors has adopted this Policy to promote compliance with federal securities laws that prohibit certain persons who are aware of material nonpublic information about a company from: (i) trading in securities of that company, or (ii) providing material nonpublic information to other persons who may trade based on that information.

II. Persons Subject to the Policy

This Policy applies to all officers of the Company and its subsidiaries, all members of the Company's Board of Directors, and all employees of the Company and its subsidiaries. The Company may also determine that other persons should be subject to this Policy, such as contractors or consultants who have access to material nonpublic information. This Policy also applies to family members, other members of a person's household and entities controlled by a person covered by this Policy, as described below.

III. Transactions Subject to the Policy

This Policy applies to transactions in the Company's securities (collectively referred to in this Policy as "Company Securities"), including the Company's common stock, options to purchase common stock, or any other type of securities that the Company may issue, including (but not limited to) preferred stock, convertible debentures and warrants, as well as derivative securities, such as restricted stock awards, and derivative securities that are not issued by the Company, such as exchange-traded put or call options or swaps relating to the Company's Securities.

IV. Administration of the Policy

The Company's Chief Financial Officer shall serve as the Compliance Officer for the purposes of this Policy, and in his absence, the Company's General Counsel or another employee designated by the Compliance Officer shall be responsible for administration of this Policy. All determinations and interpretations by the Compliance Officer shall be final and not subject to further review.

V. General Trading Policy

Generally, it is against the law to buy or sell any securities while in possession of material nonpublic information ("MNPI") relevant to that security, or to communicate such information to others who trade based on such information (known as "tipping"). In recent years, Congress has toughened the penalties for trading on or tipping MNPI and the U.S. Securities and Exchange Commission ("SEC") has aggressively brought actions against such traders and tippers.

ANY PERSON WHO ENGAGES IN INSIDER TRADING OR TIPPING CAN FACE A SUBSTANTIAL JAIL TERM (UP TO 10 YEARS) AND FINES UP TO THREE TIMES THE PROFIT GAINED (OR LOSS AVOIDED) BY THAT PERSON AND/OR HIS OR HER "TIPPEES", AS WELL AS SUBSTANTIAL CIVIL LIABILITIES.

Abeona may also be liable for the insider trading violations of an employee, if it is found that the Company failed to take appropriate steps to prevent insider trading by an employee or director.

Abeona's employees, officers, and directors **must not** engage in transactions in Company Securities if they possess MNPI as to Abeona and **must not** communicate such information to any third party except persons who have a legitimate need to know such information and understand their obligation not to trade on it.

Whether a particular item was "material" will be judged with 20-20 hindsight. **Accordingly, when in doubt as to a particular item of information, you should presume it to be material and not to have been disclosed to the public**.

More generally, all our employees, officers, and directors are reminded to use extreme care to assure that confidential information is not inadvertently disclosed to others. Be particularly careful to avoid discussing in public places, such as lobbies, trains, airports, or restaurants, any matter that might be sensitive or confidential. Meetings in which confidential information is discussed should be conducted behind closed doors. Even inadvertent "leaks" of confidential information can create problems for Abeona and our employees, officers, and directors.

AS WITH OUR OTHER EMPLOYEE POLICIES, VIOLATION OF THIS POLICY BY ANY EMPLOYEE OF ABEONA OR ANY OF OUR SUBSIDIARIES (OR BY ANY FAMILY MEMBER OF THE EMPLOYEE) IS GROUNDS FOR IMMEDIATE DISCIPLINARY ACTION, INCLUDING POSSIBLE DISMISSAL FROM EMPLOYMENT.

VI. Definition of Material Nonpublic Information

In general, information is "material" as to a security if a reasonable investor would consider the information significant in deciding whether to buy, hold, or sell the security. Examples of events or developments that should be presumed to be "material" in the context of Company Securities would be events such as the following, when they have not yet been fully disclosed to the public:

- the execution of a licensing or collaboration agreement;
- clinical results for an Abeona product candidate (whether favorable or adverse);
- any significant regulatory action, including the receipt or non-receipt of a regulatory approval;
- knowledge of a trend in Abeona's results of operations not yet fully disclosed to the public;
- gain or loss not yet disclosed to the public;
- termination of a significant agreement;
- a significant acquisition;
- major litigation;
- significant related party transactions;
- a purchase or sale of substantial assets or other significant corporate transaction;
- a change in management; or
- impending bankruptcy or the existence of severe liquidity problems.

These examples are illustrative only and are not intended to be exhaustive examples of material information.

Information that has not been disclosed to the public is generally considered to be nonpublic information. To establish that the information has been disclosed to the public, it may be necessary to demonstrate that the information has been widely disseminated. Information generally would be considered widely disseminated if it has been disclosed through newswire services, publication in a widely available newspaper, magazine or news website, the Dow Jones "broad tape," or public disclosure documents filed with the SEC that are available on the SEC's website. By contrast, information would likely not be considered widely disseminated if it is available only to the Company's employees, or if it is only available, for example, to a select group of analysts, brokers, and/or institutional investors.

Once information is widely disseminated, it is still necessary to provide the investing public with sufficient time to absorb the information. Generally, information should not be considered fully absorbed by the marketplace until after the first business day after the day on which the information is released. If, for example, the Company were to make an announcement on a Monday, you should not trade in Company Securities until the market opens on Wednesday. Depending on the particular circumstances, the Company may determine that a longer or shorter period should apply to the release of specific material nonpublic information.

VII. Policy Procedures

The Company has established additional procedures to assist the Company in the administration of this Policy, facilitate compliance with laws prohibiting insider trading while in possession of material nonpublic information, and avoid the appearance of any impropriety. These additional procedures are applicable only to those individuals described below.

A. Pre-Clearance Procedures

The persons designated by the Compliance Officer as being subject to these procedures, as well as the Family Members and Controlled Entities of such persons, may not engage in any transaction in Company Securities without first obtaining pre-clearance of the transaction from the Compliance Officer. A request for pre-clearance should be submitted to the Compliance Officer at least two business days in advance of the proposed transaction. The Compliance Officer is under no obligation to approve a transaction submitted for pre-clearance and may determine not to permit the transaction. If a person seeks pre-clearance and permission to engage in the transaction is denied, then he or she should refrain from initiating any transaction in Company Securities and should not inform any other person of the restriction.

When a request for pre-clearance is made, the requestor should carefully consider whether he or she may be aware of any material nonpublic information about the Company and should describe fully those circumstances to the Compliance Officer. The requestor should also indicate whether he or she has effected any non-exempt "opposite-way" transactions within the past six months and should be prepared to report the proposed transaction on an appropriate Form 4 or Form 5. The requestor should also be prepared to comply with SEC Rule 144 and file a Form 144, if necessary, at the time of any sale.

In this regard, directors and certain officers of the Company are subject to certain reporting requirements, trading restrictions and "short swing" profit recovery provisions under Section 16 of the Securities Exchange Act of 1934, as amended (the "Exchange Act"). In particular, each director and executive officer of Abeona must, as a general rule pursuant to Section 16, report transactions in Company Securities on a Form 4 filed with the SEC no later than 10:00 p.m. Eastern Time on the second business day following the day on which the transaction occurs (the trade date, not the settlement date). This general requirement that a Form 4 be filed within two business days of the trade date is modified in the case of purchases and sales of Company Securities made pursuant to a contract, instruction, or written plan that satisfies the affirmative defense conditions of Rule 10b5-1(c) of the Exchange Act (*i.e.*, a Rule 10b5-1 trading plan) or discretionary transactions made pursuant to an employee benefit plan, provided that the director or executive officer of Abeona does not select or has not selected the date of execution of the transaction. In these particular cases, a director or executive officer of Abeona must file a Form 4 reporting the applicable transaction before the end of the second business day following the day on which the executing broker, dealer or plan administrator, as applicable, notifies the director or executive officer of the execution of the transaction (rather than two business days after the trade date), provided that the notification date is not later than the third business day following the trade date. If the notification is provided after the third business day following the trade date, the two-day period runs from that third business day and the transaction must be reported on a Form 4 no later than the fifth business day following the trade date.

B. Quarterly Trading Restrictions

The persons designated by the Compliance Officer as subject to this restriction, as well as their Family Members or Controlled Entities, may not conduct any transactions involving the Company's Securities (other than as specified by this Policy), during a "Blackout Period" beginning 14 calendar days prior to the date of the public release of the Company's earnings results for each fiscal quarter and ending at the market open of the second business day following such release.

C. Event-Specific Trading Restriction Periods

From time to time, an event may occur that is material to the Company and is known by only a few directors, officers, or employees. So long as the event remains material and nonpublic, the persons designated by the Compliance Officer may not trade Company Securities.

In addition, the Company's financial results may be sufficiently material in a particular fiscal quarter that, in the judgment of the Compliance Officer, designated persons should refrain from trading in Company Securities even sooner than the typical Blackout Period described above. In that situation, the Compliance Officer may notify these persons that they should not trade in the Company's Securities, without disclosing the reason for the restriction.

The existence of an event-specific trading restriction period or extension of a Blackout Period may not necessarily be announced to the Company as a whole, and when not announced to the Company as a whole should not be communicated to any other person. Even if the Compliance Officer has not designated you as a person who should not trade due to an event-specific restriction, you should not trade while aware of material nonpublic information. Exceptions will not be granted during an event-specific trading restriction period.

VIII. Transactions by Family Members and Others

This Policy applies to your family members who reside with you (including a spouse, a child, a child away at college, stepchildren, grandchildren, parents, stepparents, grandparents, siblings and in-laws), anyone else who lives in your household, and any family members who do not live in your household but whose transactions in Company securities are directed by you or are subject to your influence or control, such as parents or children who consult with you before they trade in Company Securities (collectively referred to as "Family Members"). You are responsible for the transactions of these other persons and therefore should make them aware of the need to confer with you before they trade in Company Securities, and you should treat all such transactions for the purposes of this Policy and applicable securities laws as if the transactions were for your own account. This Policy does not, however, apply to personal securities transactions of Family Members where the purchase or sale decision is made by a third party not controlled by, influenced by, or related to you or your Family Members.

IX. Trading by Officer, Director, or Employee Fiduciary Accounts

This Policy applies to any entities that you influence or control, including any trusts, partnerships, or corporations (collectively referred to as "Controlled Entities"), and transactions by these Controlled Entities should be treated for the purposes of this Policy and applicable securities laws as if they were for your own account.

X. Transactions Under Company Plans

A. Restricted Stock Awards

This Policy does not apply to the vesting of restricted stock, or the exercise of a tax withholding right pursuant to which you elect to have the Company withhold shares of stock to satisfy tax withholding requirements upon the vesting of any restricted stock. The Policy does apply, however, to any market sale of restricted stock.

B. Stock Options

The foregoing trading period restrictions do not apply to exercises of stock options under our stock option and equity incentive plans. Exercises of stock options by directors and certain officers of Abeona, however, do require prior notice as described above. Sales of option shares require prior notice and remain subject to other applicable restrictions.

C. Other Similar Transactions

Any other purchase of Company Securities from the Company or sales of Company Securities to the Company are not subject to this Policy.

XI. Special and Prohibited Transactions

A. "Short Sales"

"Short sales" of Company Securities by any employee, officer, or director (*i.e.*, the sale of a security that the seller does not own) are absolutely prohibited. Short sales may evidence an expectation on the part of the seller that the securities will decline in value, and therefore have the potential to signal to the market that the seller lacks confidence in the Company's prospects In addition, Section 16(c) of the Exchange Act prohibits officers and directors from engaging in short sales.

B. Hedging Transactions

Hedging or monetization transactions can be accomplished through a number of possible mechanisms, including through the use of financial instruments such as prepaid variable forwards, equity swaps, collars, and exchange funds. Such transactions may permit a director, officer, or employee to continue to own Company Securities obtained through employee benefit plans or otherwise, but without the full risks and rewards of ownership. When that occurs, the director, officer or employee may no longer have the same objectives as the Company's other shareholders. Therefore, directors, officers and employees are prohibited from engaging in any such transactions.

XII. Rule 10b5-1 Plans

Rule 10b5-1 under the Exchange Act provides a defense from insider trading liability. To be eligible to rely on this defense, a person subject to this Policy must enter into a Rule 10b5-1 plan for transactions in Company Securities that meets certain conditions specified in the Rule (a "Rule 10b5-1 Plan"). If the plan meets the requirements of Rule 10b5-1, Company Securities may be purchased or sold without regard to certain insider trading restrictions. To comply with the Policy, a Rule 10b5-1 Plan must be approved by the Compliance Officer and meet the requirements of Rule 10b5-1 and the Company's Guidelines for Rule 10b5-1 Plans, if any, which may be obtained from the Compliance Officer. In general, a Rule 10b5-1 Plan must be entered into at a time when the person entering into the plan is not aware of material nonpublic information. Once the plan is adopted, the person must not exercise any influence over the amount of securities to be traded, the price at which they are to be traded or the date of the trade. The plan must either specify the amount, pricing, and timing of transactions in advance or delegate discretion on these matters to an independent third party.

Any Rule 10b5-1 Plan must be submitted for approval to the Compliance Officer five days prior to the entry into the Rule 10b5-1 Plan. No further pre-approval of transactions conducted pursuant to the Rule 10b5-1 Plan will be required. Please note that under Rule 10b5-1, there is generally a 90-day "cooling off" period **after** the adoption of a 10b5-1 trading plan, before the expiration of which no purchases or sales may occur.

XIII. Confidentiality

Serious problems could be caused for Abeona by unauthorized disclosure of internal information about us, whether or not for the purpose of facilitating improper trading in the stock. Abeona personnel should not discuss internal company matters or developments with anyone outside of Abeona, except as required in the performance of regular corporate duties.

This prohibition applies specifically (but not exclusively) to inquiries about us which may be made by the financial press, investment analysts or others in the financial community. It is important that all such communications on behalf of Abeona be through an appropriately designated officer under carefully controlled circumstances. Unless you are expressly authorized to the contrary, if you receive any inquiries of this nature, you should decline comment and refer the inquirer to the Compliance Officer.

XIV. Post-Termination Transactions

This Policy continues to apply to transactions in Company Securities even after termination of service to the Company. If an individual is in possession of material nonpublic information when his or her service terminates, that individual may not trade in Company Securities until that information has become public or is no longer material.

XV. Disclaimer of New Liabilities

This Policy is not intended and shall not be deemed to impose on Abeona or its employees, officers, or directors any civil, criminal or other liability that would not exist in the absence of this policy statement.

XVI. Certification

All persons subject to this Policy must certify their understanding of, and intent to comply with, this Policy.

CERTIFICATION

I hereby certify that:

1. I have read and understand the Abeona Therapeutics Inc. Policy on Insider Trading and Confidentiality (the "Policy"). I understand that the Compliance Officer is available to answer any questions I have regarding the Policy.

2. I will comply with the Policy for as long as I am subject to the Policy.

Print name: _____

Signature: _____

Date: _____

EXHIBIT 21

Subsidiaries of the Registrant

Abeona Therapeutics LLC, an Ohio company

MacroChem Corporation, a Delaware company

EXHIBIT 23.1

CONSENT OF INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM

We consent to the incorporation by reference in Registration Statement No. 333-280134 on Form S-3 and Registration Statement Nos. 333-284151, 333-279428, 333-274917, 333-272103, 333-270742, 333-267192, 333-238571, 333-221552, 333-214846, 333-204055, 333-189985, 333-169067, and 333-161642, on Form S-8 of our report dated March 16, 2026 relating to the financial statements of Abeona Therapeutics Inc. appearing in this Annual Report on Form 10-K for the year ended December 31, 2025.

/s/ DELOITTE & TOUCHE LLP

Morristown, New Jersey
March 16, 2026

EXHIBIT 31.1

PRINCIPAL EXECUTIVE OFFICER CERTIFICATION PURSUANT TO 18 U.S.C.
SECTION 1350, AS ADOPTED PURSUANT TO SECTION 302
OF THE SARBANES-OXLEY ACT OF 2002

I, Vishwas Seshadri, certify that:

1. I have reviewed this Annual Report on Form 10-K of Abeona Therapeutics Inc.;

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:

   a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

   b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

   c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

   d) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

   a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

   b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: March 17, 2026

By: */s/ Vishwas Seshadri*
Vishwas Seshadri
President and Chief Executive Officer
(Principal Executive Officer)

EXHIBIT 31.2

PRINCIPAL FINANCIAL OFFICER CERTIFICATION PURSUANT TO 18 U.S.C.
SECTION 1350, AS ADOPTED PURSUANT TO SECTION 302
OF THE SARBANES-OXLEY ACT OF 2002

I, Joseph Vazzano, certify that:

1. I have reviewed this Annual Report on Form 10-K of Abeona Therapeutics Inc.;

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:

   a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

   b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

   c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

   d) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

   a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

   b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: March 17, 2026

By: */s/ Joseph Vazzano*
Joseph Vazzano
Chief Financial Officer
(Principal Financial Officer)

EXHIBIT 32

CERTIFICATION PURSUANT TO 18 U.S.C.
SECTION 1350 AS ADOPTED PURSUANT TO SECTION 906
OF THE SARBANES-OXLEY ACT OF 2002

In connection with the Annual Report of Abeona Therapeutics Inc. (the "Company") on Form 10-K for the year ended December 31, 2025 as filed with the Securities and Exchange Commission on the date hereof (the "Report"), we, Vishwas Seshadri, President and Chief Executive Officer of the Company, and Joseph Vazzano, Chief Financial Officer of the Company, each certify, pursuant to 18 U.S.C. 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that:

(1) The Report fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934; and

(2) The information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

Date: March 17, 2026

By: */s/ Vishwas Seshadri*
Vishwas Seshadri
President and Chief Executive Officer
(Principal Executive Officer)

Date: March 17, 2026

By: */s/ Joseph Vazzano*
Joseph Vazzano
Chief Financial Officer
(Principal Financial Officer)

EXHIBIT 97

COMPENSATION RECOUPMENT POLICY OF
Abeona Therapeutics Inc.

ARTICLE A
PURPOSE AND GENERAL TERMS x

Any capitalized terms used, but not immediately defined, in this Policy have the meanings set forth in Section A-8 or Section B-1, as applicable.

Section A-1. Purpose.

The Board of Directors (the "***Board***") of Abeona Therapeutics Inc. (the "***Company***"), on recommendation of its Compensation Committee (the "***Committee***"), has adopted this Compensation Recoupment Policy (this "***Policy***") to implement a mandatory clawback policy in the event of a Restatement in compliance with the Applicable Rules, which is set forth in Article B of this Policy.

Section A-2. Administration.

Except for the powers specifically designated to the Committee in Section B-4, this Policy shall be administered in the sole discretion of the Board; *provided* that the Board may delegate its administrative responsibility to the Committee, in which case references herein to the Board shall be deemed to include the Committee. The Board shall have the discretion to interpret the Policy and make all determinations with respect to this Policy, consistent with the Applicable Rules, applicable law and this Policy, and compliance with this Policy shall not be waived by the Board in any respect.

Any interpretations and determinations made by the Board shall be final and binding on all affected individuals.

Section A-3. Effective Date; Term.

This Policy is effective as of October 2, 2023 (the "***Effective Date***") and applies to Incentive-Based Compensation that is Received by any Executive Officer on or after the Effective Date as described in Section B-3 below.

Section A-4. Amendment.

The Board may amend this Policy from time to time in its discretion, subject to any limitations under applicable law or listing standards, including the Applicable Rules. Without limiting the foregoing, the Board may amend this Policy as it deems necessary to reflect any amendment of the Applicable Rules or regulations or guidance issued under the Applicable Rules.

Section A-5. No Substitution of Rights; Non-Exhaustive Rights.

Any right of recoupment under this Policy is in addition to, and not in lieu of, any other remedies or rights that may be available to the Company pursuant to (a) the Abeona Therapeutics Inc. 2023 Equity Incentive Plan, the Abeona Therapeutics Inc. 2023 Employment Inducement Equity Incentive Plan, the Company's annual bonus plan (if any), or any other incentive plan of the Company or any of its subsidiaries or any successor plan to any of the foregoing, (b) the terms of any recoupment policy or provision in any employment agreement, compensation agreement or arrangement, or other agreement, or (c) any other legal remedies available to the Company under applicable law.

In addition to recovery of compensation as provided for in this Policy, the Company may take any and all other actions it deems necessary, appropriate, and in the Company's best interest in connection with the Board determining that this Policy should apply, including termination of the employment of, or initiating legal action against, an Executive Officer, and nothing in this Policy limits the Company's rights to take any such appropriate actions.

Section A-6. Governing Law.

This Policy and all determinations made and actions taken pursuant hereto, to the extent not otherwise governed by mandatory provisions of the Applicable Rules, shall be governed by and construed in accordance with the laws of the State of Delaware without regard to choice of law principles. If any provision of this Policy shall be held illegal or invalid for any reason, such illegality or invalidity shall not affect the remaining parts of this Policy, but this Policy shall be construed and enforced as if the illegal or invalid provision had never been included in this Policy.

Section A-7. Acknowledgment.

Each Executive Officer shall be required to sign and return to the Company the Acknowledgment Form attached hereto as Exhibit A pursuant to which such Executive Officer will agree to be bound by the terms of, and comply with, this Policy.

Section A-8. Defined Terms.

The following capitalized terms used in this Policy have the following meanings:

(a) "***Applicable Rules***" means Section 10D of the Exchange Act and Rule 10D-1promulgated thereunder and Listing Rule 5608 of the Listing Rules of Nasdaq.

(b) "***Board***" means the Board of Directors of the Company.

(c) "***Clawback Compensation***" means Incentive-Based Compensation, as determined to be subject to repayment pursuant to this Policy.

(d) "***Committee***" means the Compensation Committee of the Board, or, in the absence of such committee, a majority of independent directors serving on the Board.

(e) "***Exchange Act***" means the Securities Exchange Act of 1934, as amended.

(f) "***Regulators***" means, as applicable, the Securities and Exchange Commission and the Nasdaq Stock Market ("***Nasdaq***").

ARTICLE B
RECOUPMENT POLICY FOR
EXECUTIVE OFFICERS

Section B-1. Specific Defined Terms. For the purposes of this Policy, the following terms have the following meanings, which will be interpreted to comply with the Applicable Rules:

(a) "***Executive Officer***" means any person who is or was designated by the Board as(i)an "executive officer" of the Company according to the Applicable Rules or(ii)an "officer" in accordance with Rule 16a-1(f) promulgated under Section 16 of the Exchange Act.

(b) "***Financial Reporting Measures***" means (i) measures that are determined and presented in accordance with the accounting principles used in preparing the Company's financial statements, and any measures that are derived wholly or in part from such measures[1], (ii) the Company's stock price, and (iii) total shareholder return in respect of the Company. A "Financial Reporting Measure" need not be presented within the financial statements or included in a filing with the SEC.

[1] "Financial Reporting Measures" include, but are not limited to, the following examples of accounting-based measures and measures derived from: (i) revenues; (ii) net income; (iii) operating income; (iv) profitability of one or more reportable segments; (v) financial ratios (e.g., accounts receivable turnover and inventory turnover rates); (vi)net assets or net asset value per share (e.g., for registered investment companies and business development companies that are subject to the rule); (vii) earnings before interest, taxes, depreciation and amortization; (viii)funds from operations and adjusted funds from operations; (ix) liquidity measures (e.g., working capital, operating cash flow); (x) return measures (e.g., return on invested capital, return on assets); (xi) earnings measures (e.g., earnings per share); (xii) sales per square foot or same store sales, where sales is subject to an accounting restatement; (xiii) revenue per user, or average revenue per user, where revenue is subject to an accounting restatement; (xiv) cost per employee, where cost is subject to an accounting restatement; (xv) any of such financial reporting measures relative to a peer group, where the Company's financial reporting measure is subject to an accounting restatement; and (xvi) tax basis income.

(c) "***Incentive-Based Compensation***" means any compensation that is granted, earned, or vested, based wholly or in part upon the attainment of a Financial Reporting Measure.[2] Incentive-Based Compensation does not include, among other forms of compensation, equity awards that vest exclusively upon completion of a specified employment period, without any performance condition, and bonus awards that are discretionary or based on subjective goals or goals unrelated to Financial Reporting Measures.

(d) "***Received***" — Incentive-Based Compensation is deemed "Received" for the purposes of this Policy in the Company's fiscal period during which the Financial Reporting Measure applicable to the Incentive-Based Compensation award is attained, even if the payment or grant of the Incentive-Based Compensation occurs after the end of that period.

(e) "***Recovery Period***" means the three completed fiscal years immediately preceding the date on which the Company is required to prepare a Restatement, which date is the earlier of (i) the date the Board, a committee of the Board, or the officer or officers of the Company authorized to take such action if Board action is not required, concludes, or reasonably should have concluded, that the Company is required to prepare a Restatement or (ii) a date that a court, regulator or other legally authorized body directs the Company to prepare a Restatement.

(f) "***Restatement***" means that the Company is required to prepare an accounting restatement due to material noncompliance of the Company with any financial reporting requirement under the securities laws, including any required accounting restatement to correct an error in previously issued financial statements (i) that is material to the previously issued financial statements, or (ii) that would result in a material misstatement if the error were corrected in the current period or left uncorrected in the current period. Determination of whether noncompliance is material for the purpose of the preceding sentence will be determined consistent with the Applicable Rules.[3]

Section B-2. Recovery on a Restatement.

In the event that the Company is required to prepare a Restatement, the Company shall reasonably promptly recover from an Executive Officer the amount of any erroneously awarded Incentive-Based Compensation that is Received by such Executive Officer during the Recovery Period. The amount of erroneously Received Incentive-Based Compensation will be the excess of the Incentive-Based Compensation Received by the Executive Officer (whether in cash or shares) based on the erroneous data in the original financial statements over the Incentive-Based Compensation (whether in cash or in shares) that would have been Received by the Executive Officer had such Incentive-Based Compensation been based on the restated results, without respect to any tax liabilities incurred or paid by the Executive Officer.

Recovery of any erroneously awarded compensation under this Policy is not dependent on fraud or misconduct by any Executive Officer in connection with a Restatement.

Without limiting the foregoing, for Incentive-Based Compensation based on the Company's stock price or total shareholder return, where the amount of erroneously awarded compensation is not subject to mathematical recalculation directly from the information in the Restatement, (a) the amount shall be based on the Company's reasonable estimate of the effect of the Restatement on the stock price or total shareholder return upon which the Incentive-Based Compensation was Received and (b) the Company shall maintain documentation of the determination of that reasonable estimate and provide such estimate to the Regulators as required by the Applicable Rules.

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[2] "Incentive-Based Compensation", includes, but is not limited to, (i) non-equity incentive plan awards that are earned based wholly or in part on satisfying a Financial Reporting Measure performance goal; (ii) bonuses paid from a "bonus pool," the size of which is determined based wholly or in part on satisfying a Financial Reporting Measure performance goal; (iii) other cash awards based on satisfaction of a Financial Reporting Measure performance goal; (iv) restricted stock, restricted stock units, performance share units, stock options, and stock appreciation rights that are granted or become vested wholly or in part on satisfying a Financial Reporting Measure performance goal; and (v) proceeds received upon the sale of shares acquired through an incentive plan that were granted or vested based wholly or in part on satisfying a Financial Reporting Measure performance goal.

[3] The issuing release for the Applicable Rules suggests that determinations of materiality should be made on a facts and circumstances basis, consistent with accounting rules and consistent prior SEC guidance on level of materiality. Specifically, they point to: *Staff Accounting Bulletin No. 99, Materiality (Aug. 12, 1999) and Staff Accounting Bulletin No. 108, Considering the Effects of Prior Year Misstatements when Quantifying Misstatements in Current Year Financial Statements (Sept. 13, 2006)*.

Section B-3. Covered Executive Officers and Covered Incentive-Based Compensation.

This Policy covers all persons who are Executive Officers at any time during the Recovery Period for which Incentive-Based Compensation is Received or during the performance period applicable to such Incentive-Based Compensation. Incentive-Based Compensation shall not be recovered under this Policy to the extent Received by any person before the date the person served as an Executive Officer. Subsequent changes in an Executive Officer's employment status, including retirement or termination of employment, do not affect the Company's right to recover Incentive-Based Compensation pursuant to this Policy.

This Policy shall apply to Incentive-Based Compensation that is Received by any Executive Officer on or after the Effective Date and that results from attainment of a Financial Reporting Measure based on or derived from financial information for any fiscal period ending on or after the Effective Date. For the avoidance of doubt, this will include Incentive-Based Compensation that may have been approved, awarded, or granted to an Executive Officer on or before the Effective Date if such Incentive-Based Compensation is Received after the Effective Date.

Section B-4. Methods of Recovery; Limited Exceptions.

The Board shall determine, in its sole discretion, the method of recovering any Incentive-Based Compensation Received pursuant to this Policy, consistent with applicable law, which may include, without limitation, the methods of recovery described in Article C.

No recovery shall be required if any of the following conditions are met and the Committee (or an independent committee of the Board consistent with the Applicable Rules) determines that, on such basis, recovery would be impracticable:

(a) the direct expense paid to a third party to assist in enforcing this Article B would exceed the amount to be recovered; *provided* that prior to making a determination that it would be impracticable to recover any Incentive-Based Compensation based on the expense of enforcement, the Company shall (i) have made a reasonable attempt to recover the Incentive-Based Compensation, (ii) have documented such reasonable attempts to recover, and (iii) provide the documentation to Nasdaq;

(b) recovery would violate home country law where that law was adopted prior to November 28, 2022; *provided* that, prior to making a determination that it would be impracticable to recover any Incentive-Based Compensation based on a violation of home country law, the Company shall (i) have obtained an opinion of home country counsel, acceptable to Nasdaq, that recovery would result in such violation, and (ii) provide a copy of such opinion to Nasdaq; or

(c) recovery would likely cause an otherwise tax-qualified retirement plan, under which benefits are broadly available to employees, to fail to meet the requirements of Section 401(a)(13) or Section 411(a) of the Internal Revenue Code of 1986, as amended (the "***Code***"), and U.S. Treasury regulations promulgated thereunder.

Section B-5. Reporting; Disclosure; Monitoring.

The Company shall make all required disclosures and filings with the Regulators with respect to this Policy in accordance with the requirements of the Applicable Rules, and any other requirements applicable to the Company, including the disclosures required in connection with SEC filings.

ARTICLE C
METHODS OF RECOVERY

Section C-1. Subject to Section B-4, in the event that the Board determines that this Policy should apply, to the extent permitted by applicable law, the Company shall, as determined by the Board in its sole discretion, take any such actions as it deems necessary or appropriate to recover Clawback Compensation. The actions may include, without limitation (and as applicable):

(a) forfeit, reduce, or cancel any Clawback Compensation (whether vested or unvested)that has not been distributed or otherwise settled;
(b) seek recovery of any Clawback Compensation that was previously paid to the Executive Officer;
(c) seek recovery of any amounts realized on the vesting, exercise, settlement, sale, transfer, or other disposition of any equity-based Clawback Compensation;
(d) recoup any amount in respect of Clawback Compensation that was contributed or deferred to a plan that takes into account Clawback Compensation (excluding certain tax-qualified plans, but including deferred compensation plans, and supplemental executive retirement plans, and insurance plans to the extent otherwise permitted by applicable law, including Section 409A of the Code) and any earnings accrued on such Clawback Compensation;
(e) except as otherwise required by this Policy, determine whether Clawback Compensation should be recouped on a pre-tax or after-tax basis;
(f) offset, withhold, eliminate or cause to be forfeited any compensation that could be paid or awarded to the Executive Officer after the date of determination; and
(g) take any other remedial and recovery action permitted by law, as determined by the Board.

In addition, (x) if a breach of fiduciary duty or other violation of law has occurred, the Board may authorize legal action for such breach of fiduciary duty or other violation of law and take such other actions to enforce the obligations of the Executive Officer to the Company as the Board deems appropriate or (y) in the event that an Executive Officer fails to repay or reimburse erroneously awarded compensation that is subject to recovery, the Board may seek to compel such individual to reimburse the Company for any and all expenses reasonably incurred (including legal fees) by the Company in recovering erroneously awarded compensation under this Policy.

Section C-2. Notice. Before the Company takes action to seek recovery of compensation pursuant to this Policy against an Executive Officer, the Company may, in its discretion, take steps to provide such individual with advance written notice of such clawback; *provided* that such provision of notice shall not in any way delay the reasonably prompt recovery of any erroneously awarded Incentive-Based Compensation pursuant to this Policy.

Section C-3. No Indemnification. The Company shall not indemnify any current or former Executive Officer against the loss of erroneously awarded compensation, and shall not pay or reimburse any such person for premiums incurred or paid for any insurance policy to fund such person’s potential recovery obligations.

Exhibit A

ABEONA THERAPEUTICS INC.
COMPENSATION RECOUPMENT POLICY
ACKNOWLEDGMENT FORM

By signing below, the undersigned acknowledges and confirms that the undersigned has received and reviewed a copy of the Compensation Recoupment Policy of Abeona Therapeutics Inc. (the "***Policy***"). Capitalized terms used but not otherwise defined in this Acknowledgement Form (this "***Acknowledgement Form***") shall have the meanings ascribed to such terms in the Policy.

By signing this Acknowledgement Form, the undersigned acknowledges and agrees that the undersigned is and will continue to be subject to the Policy and that the Policy will apply both during and after the undersigned's employment with the Company. Further, by signing below, the undersigned agrees to abide by the terms of the Policy, including, without limitation, by returning any erroneously awarded Incentive-Based Compensation to the Company to the extent required by, and in a manner permitted by, the Policy.

_____
(Executive's Signature)

_____
(Executive's Printed Name)

_____
(Date)