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, 2024

Or

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

For the transition period from _____ to _____

Commission file number **001-15771**

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, 2024, was approximately $170,904,508.

The number of shares outstanding of the registrant's common stock as of March 11, 2025 was 48,533,798.

ABEONA THERAPEUTICS INC.
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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: the outcome and timing of the U.S. Food and Drug Administration's ("FDA") review of the resubmission of our Biologics License Application for pz-cel; our plans to continue development of AAV-based gene therapies designed to treat ophthalmic diseases; the FDA's potential grant of a pediatric priority voucher ("PRV") in connection with the pz-cel BLA; the achievement of or expected timing, progress and results of clinical development, clinical trials and potential regulatory approvals; our pipeline of product candidates; our belief that pz-cel could potentially benefit patients with RDEB; our belief in the adequacy of the clinical trial data from our VIITAL™ clinical trial, together with the data generated in the pz-cel program to date, to support pz-cel's regulatory approval; our dependence upon our third-party customers and vendors and their compliance with regulatory bodies; our estimates regarding expenses, future revenues, 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 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: the timing and outcome of the FDA's review of our resubmission of the Biologics License Application for pz-cel; 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 impacts of global healthcare emergencies, such as pandemics, on our business, operations, and financial condition; 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; and our ability to meet our obligations contained in license agreements to which we are party.

PART I

ITEM 1. BUSINESS

Business

Abeona Therapeutics Inc., a Delaware corporation (together with our subsidiaries, "we," "our," "Abeona" or the "Company"), is a clinical-stage biopharmaceutical company developing cell and gene therapies for life-threatening diseases. Our lead clinical program is for prademagene zamikeracel ("pz-cel"), an autologous, cell-based gene therapy currently in development for recessive dystrophic epidermolysis bullosa ("RDEB"). Pz-cel has been granted Orphan Drug and Rare Pediatric Disease ("RPD") designations by the U.S. Food and Drug Administration ("FDA") and Orphan Drug Designation by the European Medicines Agency ("EMA").

We plan to continue development of adeno-associated virus ("AAV") based gene therapies designed to treat ophthalmic diseases with high unmet need using the novel AIM™ capsids exclusively licensed from the University of North Carolina at Chapel Hill ("UNC") and developed internally through our AAV vector research programs. Abeona's novel, next-generation AAV capsids are being evaluated to improve tropism profiles for a variety of devastating diseases.

Our Mission and Strategy

Abeona is a clinical-stage biopharmaceutical company developing cell and gene therapies for life-threatening diseases. Our lead clinical program is pz-cel, autologous, COL7A1 gene-corrected epidermal sheets, an investigational product currently in development for RDEB. In November 2022, we announced positive top-line data from the VIITAL™ study evaluating the efficacy, safety and tolerability of pz-cel. The VIITAL™ study met both its co-primary efficacy endpoints demonstrating statistically significant, clinically meaningful improvements in wound healing and pain reduction in large chronic RDEB wounds. In September 2023, we submitted a Biologics License Application ("BLA") for pz-cel to the FDA. In November 2023, the FDA accepted and granted priority review for our BLA for pz-cel, and subsequently, under the Prescription Drug User Fee Act ("PDUFA"), the FDA set a target action date of May 25, 2024. In April 2024, the FDA issued a Complete Response Letter ("CRL") in response to the BLA. The CRL noted that certain additional information needed to satisfy the Chemistry Manufacturing and Controls ("CMC") requirements of the pz-cel BLA must be satisfactorily resolved before the application can be approved. The CRL did not identify any deficiencies related to the clinical efficacy or clinical safety data in the BLA, and the FDA did not request any new clinical trials or clinical data to support the approval of pz-cel. In August 2024, we completed a Type A Meeting with the FDA to discuss the forthcoming resubmission of our BLA and in October 2024, we resubmitted our BLA. The FDA notified the Company in November 2024 that the BLA was accepted for review, with an assigned PDUFA target action date of April 29, 2025.

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

Our strategy consists of:

Advancing and Commercializing our Late-Stage Clinical Cell and Gene Therapy Programs with a Focus on Life-Threatening Diseases.

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 intend to commercialize our 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 current Good Manufacturing Practice ("cGMP"), clinical-scale manufacturing capabilities for engineered cell therapies and AAV-based 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 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.

Developing Next-Generation Cell and Gene Therapy

Pz-cel 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, 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 pz-cel. Based on claims analysis, we estimate that approximately 750 moderate to severe RDEB patients in the U.S. would be pz-cel eligible patients at the time of a potential pz-cel launch (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, pz-cel was applied as a one-time surgical procedure onto RDEB wounds and has shown up to 8 years of durable wound healing and associated pain reduction even in the tough large, chronic RDEB wounds. Patients evaluated in the VIITAL™ phase 3 trial had large wounds (> 20cm^2) and, on average, had wounds that remained open for 6.2 years, and in some cases up to 21 years, prior to pz-cel 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

Standard of care in 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. The cost of wound care supplies could be as high as $996,000 per year.

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.

Program Status

Pz-cel is an investigational product comprised of autologous epidermal sheets in which a functioning COL7A1 gene is inserted into a patient's own skin cells (keratinocytes) using a retrovirus. The 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 with large and chronic RDEB wounds at Stanford University showed that pz-cel was well-tolerated and resulted in significant and durable wound healing (Siprashvili, Z., et al., 2016), with up to eight years of follow-up (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 pz-cel 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) 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. Pz-cel was shown to be well-tolerated with no serious treatment-related adverse events observed, consistent with past clinical experience. There were no deaths or instances of positive replication-competent retrovirus results, and no systemic immunologic responses were reported during the study, as well as no squamous cell carcinoma at treatment sites after application of pz-cel. Two subjects reported at least one serious adverse event unrelated to pz-cel. Four subjects reported related treatment emergent adverse events, including procedural pain, muscle spasms and pruritis. Infections unrelated to pz-cel were observed in eight patients.

In September 2023, we submitted a BLA for pz-cel to the FDA. In November 2023, the FDA accepted and granted priority review for our BLA for pz-cel, and subsequently, under the Prescription Drug User Fee Act ("PDUFA"), the FDA set a target action date of May 25, 2024. In April 2024, the FDA issued a Complete Response Letter ("CRL") in response to the BLA. The CRL noted that certain additional information needed to satisfy the Chemistry Manufacturing and Controls ("CMC") requirements of the pz-cel BLA must be satisfactorily resolved before the application can be approved. The CRL did not identify any deficiencies related to the clinical efficacy or clinical safety data in the BLA, and the FDA did not request any new clinical trials or clinical data to support the approval of pz-cel. In August 2024, we completed a Type A Meeting with the FDA to discuss our forthcoming resubmission of our BLA and in October 2024, we resubmitted our BLA. The FDA notified the Company in November 2024 that the BLA was accepted for review, with an assigned PDUFA target action date of April 29, 2025. Pz-cel has been granted Regenerative Medicine Advanced Therapy ("RMAT"), Breakthrough Therapy, Orphan Drug and RPD designations by the 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 have continued to prepare our current Good Manufacturing Practices ("cGMP") facility in Cleveland, Ohio for manufacturing commercial grade pz-cel drug product to support our planned commercial launch of pz-cel, if approved. Pz-cel study drug product for all our VIITAL™ study participants has been manufactured at our Cleveland facility. As part of our commercial planning, we continue to engage with stakeholders across the healthcare system, including private payors that cover the majority of RDEB lives, and healthcare providers to better understand market access and potential pricing for pz-cel. We are also in discussions with 5 to 7 epidermolysis bullosa centers of excellence to onboard them as pz-cel Qualified Treatment Centers ("QTC") following potential FDA approval.

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. IND-enabling efficacy and toxicology animal studies are scheduled to be completed by the end of 2025. Additionally, cGMP manufacturing of clinical grade material has been initiated at a third-party vendor in anticipation of first in human trials being initiated in 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 results 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 will have the 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 will receive 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 for CLN1 disease (infantile Batten disease) 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. On February 18, 2025, Ultragenyx reported that its BLA for its MPS IIIA product, UX111 (ABO-102), had been accepted for review by the FDA, with a PDUFA date of August 18, 2025. Under our agreements with Taysha, we are eligible to receive payments based on certain clinical, regulatory, and sales milestones and royalties on net sales.

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 efficiency for clinical trials related to our product candidates and ensure commercial demand is met in the event our therapies receive marketing approval. 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 over 16,000+ square foot manufacturing space in Cleveland, Ohio. The first phase, completed in 2018, was a 6,000 square foot state-of-the-art cGMP production facility for the manufacturing of cell and gene therapies. The facility is designed to initially manufacture clinical drug products with intent of manufacturing commercial grade cGMP drug product. The second phase, completed in 2019, was the completion of an additional 8,000 square feet of state-of-the-art laboratory space to support our expanding quality control, process development, and assay development teams. The second phase also included nearly 2,000 square feet of cGMP Inventory Control space.

On October 18, 2024, we signed a lease for 16,566 square feet of office space at 6700 Euclid Avenue, Cleveland, Ohio. The lease commenced on January 1, 2025 and the lease term matches the term for our existing 6555 Carnegie Avenue facility. The additional space at the 6700 Euclid Avenue facility will allow us to convert office space at the 6555 Carnegie Avenue facility into additional manufacturing space to increase pz-cel manufacturing capacity.

We have advanced our in-house manufacturing capabilities for pz-cel. 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 for long-term wound healing. A key component to the pz-cel 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 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 pz-cel and AAV-based vector therapies, including:

- sufficient scale to support commercial manufacturing requirements for pz-cel
- processes related to biopsy, cell collection, storage and transportation as part of manufacturing for pz-cel
- processes related to product release testing for pz-cel
- processes related to the manufacture and release testing of retroviral vector
- establishing transportation and packaging processes and materials for finished pz-cel 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
- 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. As we look to commercialize pz-cel (subject to FDA approval), we have filed our BLA to support commercial manufacturing of pz-cel from our Cleveland facility.

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 pz-cel and its use in the treatment of RDEB. Patents covering our investigational pz-cel product have been granted in the United States (U.S. Patent Nos. 12,110,504 and 12,173,314), by the European Patent Office (EP3400287B1) 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 pz-cel 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 pz-cel), 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, making its projected expiration date 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 is expected to expire on 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 (AU2015349759B2), Israel (IL252072), 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. Patents issuing from these applications would not be expected to expire before 2039.

3. CLN1 Disease (Infantile Batten Disease)

We have also licensed from UNC rights to two patent families directed to treating CLN1 disease (also known as infantile Batten disease). The first patent family is directed to optimized CLN1 genes and expression cassettes for use in treating CLN1 disease, which has applications pending in the United States, Europe, and other geographical regions. One U.S. patent in the first patent family, U.S. Patent No. 11,504,435 (the "'435 Patent"), was issued to UNC on November 22, 2022. The '435 Patent is entitled to 578 days of patent term adjustment, making its projected expiration date January 12, 2039. The second patent family is directed to treating CLN1 disease using a combination of intrathecal and intravenous administrations, and has applications pending in the United States, Europe and other geographical regions. Patents issuing from applications in the second patent family would have a 20-year expiration date of no earlier than 2040. We have entered into agreements exclusively sublicensing these two CLN1 patent families to Taysha Gene Therapies, Inc.

4. 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. 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. In October 2020, we entered into an agreement exclusively sublicensing these UNC and University of Edinburgh patent rights to Taysha Gene Therapies, Inc.

5. Multipartite AAV Delivery of Large Transgenes

We own a patent family directed to multipartite delivery of large transgenes using AAV vectors and have filed national stage applications in the United States, Europe and other geographical regions. Patents issuing from these applications would not be expected to expire before 2041.

We also own a pending U.S. application (U.S. Patent Publ. No. 2022/0090129 A1) directed to multipartite AAV delivery and its use for treating Stargardt disease.

6. ***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 would not be expected to expire before 2042.

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

   We own a pending PCT application (PCT/US2023/065877) directed to compositions and methods for treating dominant optic atrophy and X-linked retinoschisis. Patents issuing from future national stage applications of this PCT application would not be 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 assure 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 assure 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 product. 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 do not need to show the product to be effective in the pediatric population studied; rather, if the clinical trial is deemed to fairly respond to the FDA's request, the additional protection is granted. If reports of requested pediatric studies are submitted to, and accepted by, the FDA within the statutory time limits, whatever statutory or regulatory periods of exclusivity or patent protection 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 December 20, 2024 (extended from September 30, 2024 under the Continuing Appropriations and Extensions Act, 2025, signed into law by President Biden on September 26, 2024), and approved no later than September 30, 2026, unless the law is reauthorized by Congress.

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 US 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 clinical-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, 2024, we had 136 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.

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:

- 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.
- If we do not obtain the necessary U.S. or worldwide regulatory approvals to commercialize pz-cel, we will not be able to sell pz-cel.
- Even if we receive regulatory approval for pz-cel, our lead drug candidate, we may not be able to successfully manufacture or commercialize the product and the revenue that we generate from its sales, if any, may be limited.
- 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 might also experience delays in manufacturing if any of our vendors, contract laboratories or suppliers are found to be out of compliance with current Good Manufacturing Practice.
- 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.
- The widespread outbreak of an illness, communicable disease, or any other public health crisis could adversely affect our business, results of operations and financial condition.
- 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 effect our business or make us subject to liability.

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 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 such as ours 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.

Regulatory requirements governing cell and gene therapy products have evolved and may continue to change in the future. For example, the FDA has established the Office 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.

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 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, 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 and/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. For example, in September 2019, we received a clinical hold letter in connection with our phase 3 clinical trial for pz-cel stating that the FDA would not provide approval for us to begin our planned phase 3 clinical trial for pz-cel until we submitted additional data points on transport stability of pz-cel to clinical sites. Although the FDA removed the clinical hold in December 2019 and provided clearance for us to proceed with our planned phase 3 clinical trial, we may encounter similar delays in our clinical studies in the future.

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, 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 a sufficient number of 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 a sufficient number of 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.

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.

While orphan drug designation provides certain advantages, it neither shortens the development time or 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. 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.

The Complete Response Letter related to our Biologics License Application for pz-cel for the treatment of patients with recessive dystrophic epidermolysis bullosa may impair our ability to successfully commercialize pz-cel.

In April 2024, we received a CRL related to our BLA for pz-cel for the treatment of patients with RDEB. In the CRL, the FDA noted that certain additional information needed to satisfy CMC requirements must be satisfactorily resolved before the application can be approved. In August 2024, we completed a Type A Meeting with the FDA to discuss our forthcoming resubmission of our BLA and in October 2024, we resubmitted our BLA. The FDA notified the Company in November 2024 that the BLA was accepted for review, with an assigned PDUFA target action date of April 29, 2025. A delay in receiving approval of the BLA could shorten any periods during which we may have the exclusive right to commercialize our pz-cel or allow our competitors to bring products to market before we do. This may impair our ability to successfully commercialize pz-cel. If any of the foregoing were to occur, our business, financial condition, results of operations, and prospects will be materially harmed.

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 clinical trials and potentially future commercialization. Several factors could cause production interruptions, including equipment malfunctions, facility contamination, raw material shortages or contamination, natural disasters, public health emergencies such as the COVID-19 pandemic, disruption in utility services, human error, or disruptions in the operations of our suppliers. Our products 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.

There are several risks specific to the manufacturing process for pz-cel which 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 currently do not have a backup manufacturer to supply clinical trial material for pz-cel. An alternative manufacturer would need to be qualified, through regulatory filings, which could result in delays to our clinical trial timeline. The regulatory authorities also may require additional clinical trials if a new manufacturer is relied upon for commercial production. Switching manufacturers may involve substantial costs and could result in a delay in our desired clinical and commercial timelines.

Accordingly, we employ multiple steps to control our manufacturing process to assure 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 product launches or clinical trials, which could be costly to us and otherwise harm our business, financial condition, results of operations and prospects. We also may encounter problems hiring and retaining the experienced scientific, quality control and manufacturing personnel needed to operate our manufacturing process, which could result in delays in our production or difficulties in maintaining compliance with applicable regulatory requirements. Any problems in our manufacturing process or facilities could make us a less attractive collaborator for potential partners, including larger pharmaceutical companies and academic research institutions, which could limit our access to additional attractive development programs. Problems in 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 clinical trial supply demand, and eventually market demand for any product candidates for which we may receive marketing approval. Disruptions in our manufacturing process may delay or disrupt our commercialization efforts.

If we or any of our vendors, contract laboratories or suppliers are found to be out of compliance with cGMP, we may experience delays or disruptions in manufacturing while we 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 obtain regulatory approval for commercial manufacturing, we will need to continue to ensure that all of 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 expend time, money and effort in production, record keeping and quality control to assure that the product meets applicable specifications and other requirements. If we fail to comply with these requirements, we would be subject to possible regulatory action and may not be permitted to sell any products that we may develop.

We may rely on third parties to conduct aspects of our product manufacturing, and these third parties may not perform satisfactorily. We may rely on third parties to produce certain materials for our product candidates and, therefore, we can control only certain aspects 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. Poor 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 their 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 clinical trials, product approvals, and commercialization. It may also require that we conduct additional studies.

We have manufacturing agreements with third parties that provide for, among other things, production of product candidates for our current and future early-stage clinical trials. Under certain circumstances, the other party is entitled to terminate its arrangement with us. If we need to enter into alternative arrangements, it could delay our product development activities. Our reliance on third parties for certain manufacturing activities will reduce our control over these activities but will not relieve us of our responsibility to ensure compliance with all required regulations. If a third party does not successfully carry out its contractual duties, meet expected deadlines or manufacture our product candidates in accordance with regulatory requirements, or if there are disagreements between us and any such third party, we will not be able to complete, or may be delayed in completing, the preclinical studies required to support future IND submissions and the clinical trials required for approval of our product candidates. In such instances, we may need to enter into an appropriate replacement third-party relationship, which may not be readily available or on acceptable terms, which would cause additional delay or increased expense prior to the approval of our product candidates and would thereby have a material adverse effect on our business, financial condition, results of operations and prospects.

In addition, if the FDA or a comparable foreign regulatory authority does not approve our or a third party's facilities for the manufacture of our product candidates or if it withdraws any such approval in the future, we may need to find alternative manufacturing facilities, which would significantly impact our ability to develop, obtain and maintain regulatory approval for or market our product candidates, if approved. Any new manufacturers would need to either obtain or develop the necessary manufacturing know-how, and obtain the necessary equipment and materials, which may take substantial time and investment. We must also receive FDA approval for the use of any new manufacturers for commercial supply. We may not succeed in our efforts to establish manufacturing relationships or other alternative arrangements for any of our product candidates, components, and programs. For example, our product candidates may compete with other products and product candidates for access to manufacturing facilities. There are a limited number of manufacturers that operate under cGMP regulations and that are both capable of manufacturing for us and willing to do so.

The manufacture of biologic products requires significant expertise and capital investment, including the development of advanced manufacturing techniques and process controls. Manufacturers of therapeutics often encounter difficulties in production, particularly in scaling up initial production. These problems include difficulties with production costs and yields, quality control, including stability of the product candidate and quality assurance testing, shortages of qualified personnel, and compliance with strictly enforced federal, state, and foreign regulations. If we or our manufacturers were to encounter any of these difficulties and were unable to perform as agreed, our ability to provide product candidates to patients in our clinical trials and for commercial use, if approved, would be jeopardized.

Our reliance on these third parties entails risks to which we would not be subject if we manufactured the product candidates ourselves, including:

- reduced control for certain aspects of manufacturing activities;
- reduced control over the protection of our trade secrets and know-how from misappropriation or inadvertent disclosure;
- inability to negotiate manufacturing agreements with third parties under commercially reasonable terms;
  reliance on the third party for regulatory compliance and quality assurance;
- termination or nonrenewal of manufacturing and service agreements with third parties in a manner or at a time that is costly or damaging to us; and
- disruptions to the operations of our third-party manufacturers and service providers caused by conditions unrelated to our business or operations, including the bankruptcy of the manufacturer or service provider.

Any of these events could lead to clinical trial delays or failure to obtain regulatory approval or impact our ability to successfully commercialize future product candidates. Some of these events could be the basis for FDA action or action of equivalent competent authorities in foreign jurisdictions, including injunction, recall, seizure or total or partial suspension of product manufacturing. Failure to comply with ongoing regulatory requirements could cause us to suspend production or put in place costly or time-consuming remedial measures.

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, 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, 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, results of operations and prospects.

The widespread outbreak of an illness, communicable disease, or any other public health crisis could adversely affect our business, results of operations and financial condition.

We could be negatively impacted by the widespread outbreak of an illness, communicable disease, or any other public health crisis that results in economic or trade disruptions, including the disruption of global supply chains. The COVID-19 pandemic negatively impacted the economy on a global, national, and local level, disrupted global supply chains, and created volatility and disruption of financial markets. Responses from governmental authorities and companies to reduce the spread of COVID-19 affected economic activity through various containment measures including, among others, business closures, work stoppages, quarantine and work-from-home guidelines, limiting capacity at public spaces and events, vaccination requirements, or restrictions of global and regional travel. Another outbreak of an illness, a communicable disease, or any other public health crisis, and any resulting impacts, such as an extended period of global supply chain and/or economic disruption, labor shortages, or government-mandated actions in response to such public health crisis could materially affect our business, results of operations, access to sources of liquidity, and financial condition.

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 commercializing our product candidates

If we do not obtain the necessary U.S. or worldwide regulatory approvals to commercialize pz-cel, we will not be able to sell pz-cel.

If we cannot obtain regulatory approval for pz-cel, we will not be able to generate revenue from this product candidate. As a result, our ability to generate revenue from product commercialization may be further delayed. We cannot assure you that we will receive the approvals necessary to commercialize pz-cel or any other product candidate we may develop in the future. In order to obtain FDA approval of pz-cel or any other product candidate requiring FDA approval, we must successfully complete an FDA BLA review. Obtaining FDA approval of any other product candidate generally requires significant research and testing, referred to as preclinical studies, as well as human tests, referred to as clinical trials. Satisfaction of the FDA's regulatory requirements typically takes many years, depends upon the type, complexity and novelty of the product candidate and requires substantial resources for research, development and testing. We cannot predict whether our research and clinical approaches will result in products that the FDA considers safe for humans and effective for indicated uses. The FDA has substantial discretion in the product approval process and may require us to conduct additional preclinical and clinical testing or to perform post-marketing studies. The approval process may also be delayed by changes in government regulation, future legislation or administrative action or changes in FDA policy that occur prior to or during our regulatory review.

Delays in obtaining regulatory approvals may:

- delay commercialization of, and our ability to derive product revenues from, pz-cel or any other product candidate;
- impose costly procedures on us; and
- diminish any competitive advantages that we may otherwise enjoy.

Even if we comply with all FDA requests, the FDA may ultimately reject our BLA. In addition, the FDA could determine that we must test additional subjects or require that we conduct further studies with more subjects. We may never obtain regulatory approval for pz-cel, or any other future potential product candidate. Failure to obtain FDA approval of any of our product candidates will severely undermine our business by leaving us without the ability to generate additional accretive revenues. There is no guarantee that we will ever be able to develop or acquire other product candidates. In foreign jurisdictions, we must receive approval from the appropriate regulatory authorities before we can commercialize any products or product candidates outside the U.S. Foreign regulatory approval processes generally include all of the risks and uncertainties associated with the FDA approval procedures described above. We cannot assure you that we will receive the approvals necessary to commercialize any product candidate for sale outside the U.S.

Even if we receive regulatory approval for pz-cel, our lead drug candidate, we may not be able to successfully commercialize the product and the revenue that we generate from its sales, if any, may be limited.

If approved for marketing, the commercial success of pz-cel will depend upon the product's acceptance by the medical community, including physicians, patients and healthcare payors. The degree of market acceptance for our drug candidate will depend on a number of factors, including:

- actual and perceived efficacy and safety of pz-cel;
- relative convenience, dosing burden and ease of administration;
- potential or perceived advantages or disadvantages over alternative treatments;
- potential post-marketing commitments imposed by regulatory authorities, such as patient registries;
- strength of sales, marketing and distribution support;
- price of our future products, both in absolute terms and relative to alternative treatments;
- the effect of current and future healthcare laws on pz-cel; and
- availability of coverage and reimbursement from government and other third party payers.

If our drug candidate is approved but does not achieve an adequate level of acceptance by physicians, healthcare payors and patients, we may not generate sufficient revenue and we may not be able to achieve or sustain profitability. Our efforts to educate the medical community and third-party payors on the benefits of our drug candidates may require significant resources and may never be successful.

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.

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 will expand with respect to our drug candidates, if any, that receive regulatory approval for commercial sale 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 used in clinical tests or marketed to the public. 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, or 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 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.

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

Our products and product candidates may face competition from other products that are the same as or similar to ours. If the FDA or comparable foreign regulatory authorities approve biosimilar versions of our products or product candidates, or such authorities do not grant our products appropriate or anticipated periods of regulatory exclusivity, the sales of our products could be adversely affected. Moreover, even if we receive periods of regulatory exclusivity, that exclusivity 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 any 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.

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.

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 products. 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.

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

The products 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.

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.

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.

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, results of operations and prospects.

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, 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 that we were the first to make the inventions claimed in any owned or any licensed patents or pending patent applications, or that we were the first to file for patent protection of such inventions. 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, 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. In order 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 right. 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 $813.3 million through December 31, 2024. The net loss for the year ended December 31, 2024, was $63.7 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 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:

- 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.

As of December 31, 2024, our cash, cash equivalents, restricted cash and short-term investments were $98.1 million. Based upon our existing cash resources and the $4.8 million in net proceeds from our subsequent sales of common stock under stock under the ATM Agreement, we believe that we have sufficient resources to fund operations through at least the next 12 months from the date of the issuance of our consolidated financial statements.

However, our operating plan may change as a result of many factors currently unknown to us, and we may need to seek additional funds sooner than planned, through public or private equity, government or other third-party funding, marketing and distribution arrangements and other collaborations, strategic alliances and licensing arrangements or a combination of these approaches. In any event, we may require additional capital to obtain potential regulatory approval for, and to potentially commercialize, our product candidates. Even if we believe we have sufficient funds for our current or future operating plans, we may seek additional capital if market conditions are favorable or if we have specific strategic objectives.

Any additional fundraising efforts may divert our management from their day-to-day activities, which may adversely affect our ability to develop and commercialize our product candidates. In addition, we cannot guarantee that future financing will be available in sufficient amounts or on terms acceptable to us, if at all. Moreover, 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. The sale of additional equity or convertible securities would dilute all of our stockholders. The incurrence of indebtedness would result in increased fixed payment obligations, and we may be required to agree to certain restrictive covenants, such as limitations on our ability to incur additional debt, limitations on our ability to acquire, sell or license intellectual property rights and other operating restrictions that could adversely impact our ability to conduct our business. We could also be required to seek funds through arrangements with collaborative partners or otherwise at an earlier stage than otherwise would be desirable and we may be required to relinquish rights to some of our technologies or product candidates or otherwise agree to terms unfavorable to us, any of which may have a material adverse effect on our business, operating results, and prospects.

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, and results of operations.

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 complete the development of our drug candidates, to develop and obtain patent protection and regulatory approvals for our drug candidates and to manufacture and commercialize the resulting drugs. We are not expecting any significant revenues in the short-term from our products or 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. In the next couple of years, we expect limited revenues from product sales, if any, and any amounts that we receive under strategic partnerships and research or drug development collaborations that we may establish and, as a result, we may be unable to achieve or maintain profitability in the future or to achieve significant revenues in order to fund our 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.

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 pre-clinical 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.

Risks related to our common stock

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

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

- our ability to integrate operations, technology, products, and services;
- our ability to execute our business plan;
- 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 our product candidates;
- litigation or public concern about the safety of our potential products;
- 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;
- period-to-period fluctuations in our financial results; and
- whether an active trading market in our common stock develops and is maintained.

In addition, the securities markets have from time-to-time experienced significant price and volume fluctuations 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 may 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. Any future debt financing may involve covenants that, among other restrictions, 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.

Our ability to use our net operating loss carry forwards may be subject to limitation.

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 net operating loss carryforwards attributable to the period prior to the change. As a result, if we earn net taxable income, our ability to use our pre-change net operating loss carryforwards to offset U.S. federal and state taxable income may become subject to limitations, which could potentially result in increased future tax liability for us. As of December 31, 2024, we had net operating loss carryforwards aggregating $429.4 million.

Risks related to cybersecurity

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 ("NIST") cybersecurity framework that include 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 NIST. Our Information Technology Security and Risk Manager has a PhD in a scientific field and various information security certifications such as Certified Ethical Hacker ("CEH") and Holistic Information Security Practitioner ("HISP"). 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," under the heading "Risks related to cybersecurity."

ITEM 2. PROPERTIES

Our corporate headquarters are located in Cleveland, Ohio, where we currently lease approximately 62,000 square feet of manufacturing, laboratory and office space. That lease expires in December 2030. We also lease 10,400 square feet of office space located in New York, New York. That lease expires in September 2025 and has been sublet as of December 31, 2024.

In addition, we have signed a lease for approximately 16,566 square feet of office space with a commencement date of January 1, 2025. That lease expires in December 2030. This additional space will allow the Company to convert office space at the existing lease into additional manufacturing space to increase pz-cel manufacturing capacity.

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 commercialization.

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.

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.

The number of record holders of our common stock as of March 11, 2025 was 310.

Equity Compensation Plan Information

The following table sets forth, as of December 31, 2024, information about shares of common stock outstanding and available for issuance under our existing equity compensation plans.

Plan Category	Number of securities to be issued upon exercise of outstanding options, warrants and rights (a)	Weighted average exercise price of outstanding options warrants and rights (b)	Number of securities remaining available for future issuance under equity compensation plans (excluding securities reflected in column (a)) (c)
Equity compensation plans approved by security holders:			
2015 Equity Incentive Plan	176,587	$ 38.64	—
Equity compensation plans not approved by security holders:	—	$ —	—
Total equity compensation plans	176,587	$ 38.64	—

Recent Sales of Unregistered Securities

None.

Issuer Repurchases of Equity Securities

The following table provides information about purchases of equity securities that are registered pursuant to Section 12 of the Exchange Act for the three months ended December 31, 2024:

	Number of securities to Total number of shared (our units) purchased (a)	Weighted average Average price paid per share (or unit)
Shares delivered or withheld pursuant to restricted stock awards		
October 1, 2024 - October 31, 2024	251	$ 6.53
November 1, 2024 - November 30, 2024	—	$ —
December 1, 2024 - December 31, 2024	—	$ —
	251	$ 6.53

(a) Reflects shares of common stock surrendered to the Company for payment of tax withholding obligations in connection with the vesting of restricted stock.

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

Abeona is a clinical-stage biopharmaceutical company developing cell and gene therapies for life-threatening diseases. Our lead clinical program is pz-cel, investigational autologous, COL7A1 gene-corrected epidermal sheets, currently in development for recessive dystrophic epidermolysis bullosa ("RDEB"). In 2022, we announced positive data from the VIITAL™ study evaluating the efficacy, safety and tolerability of pz-cel. The VIITAL™ study met both its co-primary efficacy endpoints demonstrating statistically significant, clinically meaningful improvements in wound healing and pain reduction in large chronic RDEB wounds. In September 2023, we submitted a Biologics License Application ("BLA") for pz-cel to the U.S. Food and Drug Administration ("FDA"). In November 2023, the FDA accepted and granted priority review for our BLA for pz-cel, and subsequently, under the Prescription Drug User Fee Act ("PDUFA"), the FDA set a target action date of May 25, 2024. In April 2024, the FDA issued a Complete Response Letter ("CRL") in response to the BLA. The CRL noted that certain additional information needed to satisfy the Chemistry Manufacturing and Controls ("CMC") requirements of the pz-cel BLA must be satisfactorily resolved before the application can be approved. The CRL did not identify any deficiencies related to the clinical efficacy or clinical safety data in the BLA, and the FDA did not request any new clinical trials or clinical data to support the approval of pz-cel. In August 2024, we completed a Type A Meeting with the FDA to discuss our forthcoming resubmission of our BLA and in October 2024, we resubmitted our BLA. The FDA notified the Company in November 2024 that the BLA was accepted for review, with an assigned PDUFA target action date of April 29, 2025.

We have continued to prepare our current Good Manufacturing Practices ("cGMP") facility in Cleveland, Ohio for manufacturing commercial grade pz-cel drug product to support our planned commercial launch of pz-cel, if approved. Pz-cel study drug product for all our VIITAL™ study participants has been manufactured at our Cleveland facility. As part of our commercial planning, we continue to engage with stakeholders across the healthcare system, including public and private payors, and healthcare providers to better understand market access and potential pricing for pz-cel. We have also begun discussions with high volume treatment centers of excellence to onboard them for pz-cel application upon potential FDA approval.

Our development portfolio also features adeno-associated virus ("AAV") based gene therapies designed to treat ophthalmic diseases using the novel AIM™ capsids that we have exclusively licensed from the University of North Carolina at Chapel Hill and developed internally through our AAV vector research programs.

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

On October 18, 2024, we signed a lease for 16,566 square feet of office space at 6700 Euclid Avenue, Cleveland, Ohio. The lease commences on January 1, 2025 and the lease term matches the term for our existing 6555 Carnegie Avenue facility. The additional space at the 6700 Euclid Avenue facility will allow us to convert office space at the 6555 Carnegie Avenue facility into additional manufacturing space to increase pz-cel manufacturing capacity.

RESULTS OF OPERATIONS

Comparison of Years Ended December 31, 2024 and December 31, 2023

($ in thousands)	For the year ended December 31, 2024	2023	Change $	Change %
Revenues:				
License and other revenues	$ —	$ 3,500	$ (3,500)	(100)%
Expenses:				
Royalties	—	1,605	(1,605)	(100)%
Research and development	34,360	31,091	3,269	11%
General and administrative	29,851	19,004	10,847	57%
Gain on operating lease right-of-use assets	—	(1,065)	1,065	(100)%
Total expenses	64,211	50,635	13,576	27%
Loss from operations	(64,211)	(47,135)	(17,076)	36%
Interest income	4,246	2,117	2,129	101%
Interest expense	(4,208)	(418)	(3,790)	907%
Change in fair value of warrant and derivative liabilities	(755)	(11,695)	10,940	(94)%
Other income	1,194	2,943	(1,749)	(59)%
Net loss	$ (63,734)	$ (54,188)	$ (9,546)	18%

N/A - not applicable or not meaningful

License and other revenues

License and other revenues for the year ended December 31, 2024 was nil, as compared to $3.5 million for the same period of 2023. There was no license or other revenue in 2024 as no clinical development milestones were met in 2024. The revenue in 2023 consists of revenue resulting from achieving clinical development milestones achieved under a sublicense agreement we entered into with Taysha Gene Therapies in October 2020 relating to an investigational AAV-based gene therapy for Rett syndrome.

Royalties

Total royalty expenses were nil for the year ended December 31, 2024, as compared to $1.6 million for the same period of 2023. The royalty expense in 2023 was due to royalties owed to our licensors resulting from the milestones due from Taysha related to Rett syndrome.

Research and development

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

Total research and development spending for the year ended December 31, 2024 was $34.4 million, as compared to $31.1 million for the same period of 2023, an increase of $3.3 million. The increase in expenses was primarily due to a $4.0 million increase in salaries and $0.5 million in non-cash stock-based compensation costs due to increased headcount related to manufacturing capacity expansion preparing for the potential launch of pz-cel, partially offset by a decrease in clinical and development work costs of $1.3 million due to reduced spending on clinical trials as the majority of our clinical trials have finalized except for our long-term follow up trials.

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.

General and administrative

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), pre-commercial launch activity costs and other general operating expenses not otherwise included in research and development expenses.

Total general and administrative expenses were $29.9 million for the year ended December 31, 2024, as compared to $19.0 million for the same period of 2023, an increase of $10.9 million. The increase in expenses was primarily due to:

- increased salary and related costs of $3.8 million;
- increased pre-commercial preparation costs of $3.6 million;
- increased non-cash stock-based compensation of $1.4 million; and
- increased other costs such as recruiting and professional fees of $2.1 million.

Gain on operating lease right-of-use assets

The gain on operating lease right-of-use assets was $1.1 million for the year ended December 31, 2023. The gain on operating lease right-of-use assets for 2023 was related to the termination of our operating leases for office space that we no longer use, resulting in a gain from the difference between the carrying value of the right-of-use lease assets and the related lease liabilities. There was no such gain during the year ended December 31, 2024.

Interest income

Interest income was $4.2 million for the year ended December 31, 2024, as compared to $2.1 million in the same period of 2023. The increase resulted from higher earnings on short-term investments driven by higher interest rates and increased average short-term investment balances.

Interest expense

Interest expense was $4.2 million for the year ended December 31, 2024, as compared to $0.4 million in the same period of 2023. The increase was primarily due to the Avenue credit facility entered into by the Company in January 2024, resulting in recognized interest expense of $3.8 million.

Change in fair value of warrant and derivative liabilities

The change in fair value of warrant and derivative liabilities was a loss of $0.8 million for the year ended December 31, 2024, as compared to a loss of $11.7 million in the same period of 2023.

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 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.

Other income

Other income was $1.2 million for the year ended December 31, 2024, as compared to $2.9 million in the same period of 2023. The change was primarily a result of $2.1 million in other income related to the impact of the employee retention credit that was recorded in 2023, partially offset by a refundable job creation tax credit of $0.5 million received in 2024.

LIQUIDITY AND CAPITAL RESOURCES

Cash Flows for the Years Ended December 31, 2024 and 2023

	For the year ended December 31,	
($ in thousands)	**2024**	**2023**
Total cash, cash equivalents and restricted cash (used in) provided by:		
Operating activities	$ (56,015)	$ (37,009)
Investing activities	(39,240)	208
Financing activities	104,139	37,057
Net increase in cash, cash equivalents and restricted cash	$ 8,884	$ 256

Operating activities

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.

Net cash used in operating activities was $37.0 million for the year ended December 31, 2023, primarily comprised of our net loss of $54.2 million and increases in operating assets and liabilities of $1.8 million partially offset by net non-cash charges of $19.0 million. Non-cash charges consisted primarily of $11.7 million of the change in fair value of warrant liabilities, $4.8 million of stock-based compensation and $2.2 million of depreciation and amortization.

Investing activities

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.

Net cash provided by investing activities was $0.2 million for the year ended December 31, 2023, primarily comprised of proceeds from maturities of short-term investments of $51.9 million and proceeds from the disposal of property and equipment of $0.2 million, partially offset by purchases of short-term investments of $51.6 million and capital expenditures of $0.3 million.

Financing activities

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.

Net cash provided by financing activities was $37.1 million for the year ended December 31, 2023, primarily comprised of proceeds of $14.4 million from open market sales of common stock pursuant to the ATM Agreement (as defined below) and net proceeds of $23.0 million from our July 2023 direct placement offering of common stock.

We have historically funded our operations primarily through sales of common stock.

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, 2024, our cash resources were $98.1 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 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 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. We sold 3,659,882 shares of our common stock under the ATM Agreement and received $14.6 million of net proceeds during the year ended December 31, 2023. Subsequent to December 31, 2024 and through March 11, 2025, we sold 915,925 shares of our common stock under the ATM Agreement resulting in $4.8 million in net proceeds.

Since our inception, 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 potential commercialization efforts. We have not been profitable since inception and to date have received limited revenues from the sale of products or licenses. We expect to 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 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 minimum lease payments above do not include any related common area maintenance charges or real estate taxes.

On November 12, 2021, we entered into a Settlement Agreement with REGENXBIO to resolve all current disputes between the parties including the aforementioned AAA arbitration and New York State Supreme Court action. In accordance with the Settlement Agreement, we agreed to pay REGENXBIO a total of $30 million, payable as follows: (1) $20 million payable that was paid in 2021 after execution of the Settlement Agreement, (2) $5 million on the first anniversary of the effective date of the Settlement Agreement that was paid in 2022, and (3) $5 million upon the earlier of: (i) the third anniversary of the effective date of the Settlement Agreement or (ii) the closing of a Strategic Transaction, as defined in the Settlement Agreement. As of December 31, 2024, we have paid all amounts due under the Settlement Agreement.

In addition, we are also party to other license agreements, which 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, 2024 and, if satisfied, the timing of payment for these amounts was not reasonably estimable as of December 31, 2024. 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 potential BLA approval and sales of pz-cel 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.

Derivative Liability

We account for the fair value of the conversion right embedded within the loan agreement in accordance with the guidance in ASC 815, which requires us to bifurcate and separately account for the conversion feature as an embedded derivative contained in our loan agreement. Accordingly, we account for the conversion feature as a derivative liability in our condensed consolidated balance sheet. Derivatives are measured at their fair value on the balance sheet. In determining the appropriate fair value, we use 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 until the derivative is exercised, expired, reclassified, or otherwise settled. At September 30, 2024, the conversion feature in the Company's loan agreement no longer met the criteria of a derivative liability, and the $1.1 million derivative liability was reclassified to equity.

Leases

We account for leases pursuant to ASC 842, *Leases* ("ASC 842"). ASC 842 requires the recognition of lease assets and lease liabilities by lessees for those leases classified as operating leases. We determine if an arrangement is a lease at inception or when amended. Right-of-use lease assets represent our right to use an underlying asset for the lease term and lease liabilities represent our obligation to make lease payments arising from the lease. The classification of our leases as operating or finance leases along with the initial measurement and recognition of the associated right-of-use assets and lease liabilities is performed at the lease commencement date or when amended. The measurement of lease liabilities is based on the present value of future lease payments over the lease term. As our leases do not provide an implicit rate, we use our incremental borrowing rate based on the information available at the lease commencement date in determining the present value of future lease payments. As we have no external borrowings, the incremental borrowing rates are determined using information on indicative borrowing rates that would be available to us based on the value, currency and borrowing terms provided by financial institutions, adjusted for company and market specific factors. Although we do not expect our estimates of the incremental borrowing rates to generate material differences within a reasonable range of sensitivities, judgement is involved in selecting an appropriate rate, and the rate selected for each lease will have an impact on the value of the lease liability and corresponding right-of-use lease asset in the consolidated balance sheets.

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 or lease amendment and excludes lease incentives and initial direct costs incurred, as applicable. Rent expense for our operating leases is recognized on a straight-line basis over the lease term. We do not have any leases classified as finance leases.

Our leases do not have significant rent escalation, holidays, concessions, material residual value guarantees, material restrictive covenants or contingent rent provisions. Our 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 we have elected the practical expedient to group lease and non-lease components for all leases. We have elected the practical expedient to exclude short-term leases from our right-of-use assets and lease liabilities.

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

In October 2024, we signed a lease for 16,566 square feet of office space at 6700 Euclid Avenue, Cleveland, Ohio. Pursuant to the lease agreement, the lease 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 additional space at the 6700 Euclid Avenue facility will allow us to convert office space at the 6555 Carnegie Avenue facility into additional manufacturing space to increase pz-cel manufacturing capacity. As the lease does not commence and we do not have access to the leased space until January 1, 2025, the impact of this lease agreement is not reflected in our consolidated financial statements as of December 31, 2024.

In June 2023, we terminated one of our operating leases for office space. The termination resulted in a gain of $1.1 million for the year ended December 31, 2023, representing the difference between the carry value of the right-of-use assets and the related lease liabilities. This gain is included in gain on right-of-use lease assets in the consolidated statement of operations and comprehensive loss.

In June of 2023, we modified one of our operating leases for office space to add up to 14,032 square feet to our existing facility in Cleveland, Ohio. The lease modification resulted in the recognition of $0.4 million of additional right-of-use assets and related lease liabilities in our consolidated balance sheet during the year ended December 31, 2023.

Impairment of Long-Lived Assets

Long-Lived Assets consist of property and equipment, licensed technology, and right-of-use ("ROU") assets. We test our long-lived assets for impairment on an annual basis, or 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. We assess the recoverability of the affected long-lived assets by determining whether the carrying value of such assets can be recovered through undiscounted future operating cash flows. If the carrying amount is not recoverable, we measure the amount of any impairment by comparing the carrying value of the asset to the present value of the expected future cash flows associated with the use of the asset. The undiscounted future operating cash flows require considerable judgement and are sensitive to changes in underlying assumptions such as operating costs related to our current facilities, headcount requirements and our clinical costs. As a result, there can be no assurance that the estimates and assumptions made for purpose of our impairment determinations would prove to be an accurate prediction of the future.

Revenue Recognition

We account for revenue under ASC 606, *Revenue from Contracts with Customers*, ("ASC 606"). We recognize revenue when our customer obtains control of promised goods or services, in an amount that reflects the consideration which we expect to receive in exchange for those goods or services. To determine revenue recognition for arrangements that we determine are within the scope of ASC 606, we perform the following five steps: (i) identify the contract(s) with our 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) we satisfy a performance obligation.

Exclusive Licenses

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 therefore 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.

Sublicense and Inventory Purchase Agreements Relating to CLN1 Disease:

In August 2020, we entered into sublicense and inventory purchase agreements with Taysha Gene Therapies ("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 we had referred to as ABO-202. Under the inventory purchase agreement, we sold to Taysha certain inventory and other items related to ABO-202. We 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 us and determined that the license has significant stand-alone functionality. Furthermore, we have no ongoing activities associated with the license to support or maintain the license's utility. Based on this, we 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) $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) other royalty-based payments based on net sales. The event-based milestone payments are based on certain development and regulatory events occurring. At inception, we 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 our 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, we 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 are based on a level of sales for which the license is deemed to be the predominant item to which the royalties relate. We 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, we have not recognized any sales-based or royalty revenue resulting from this licensing arrangement.

There was no revenue recognized under this agreement during the years ended December 31, 2024 and 2023. As of December 31, 2024 and 2023, we have no contract assets or contract liabilities as a result of this transaction.

Sublicense Agreement Relating to Rett Syndrome:

In October 2020, we entered into a sublicense agreement with Taysha for a gene therapy for Rett syndrome and MECP2 gene constructs and regulation of their expression. The agreement grants Taysha worldwide exclusive rights to intellectual property developed by scientists at the University of North Carolina at Chapel Hill, the University of Edinburgh and us, and our 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.

We 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 us and determined that the license has significant stand-alone functionality. Furthermore, we have no ongoing activities associated with the license to support or maintain the license's utility. Based on this, we 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) other royalty-based payments based on net sales. The event-based milestone payments are based on certain development and regulatory events occurring. We 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. We determined that these milestone payments are not within our control or the licensee's control, such as regulatory approvals, and are not considered probable of being achieved until those approvals are received. Accordingly, we have fully constrained the $26.5 million of 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. We 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, we have not recognized any sales-based or royalty revenue resulting from this licensing arrangement.

Under this arrangement, we recognized nil and $3.5 million of revenue during the years ended December 31, 2024 and 2023, respectively, which amount related solely to variable consideration. As of December 31, 2024 and 2023, we do not have any contract assets or contract liabilities as a result of this transaction.

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. There may be instances in which payments made to our vendors will exceed the level of services provided and result in a prepayment of the expense. In accruing service fees, we estimate the time period over which services will be performed and the level of effort to be expended in each period. 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. Although we do not expect our estimates to be materially different from amounts actually incurred, our understanding of the status and timing of services performed relative to the actual status and timing of services performed may vary and may result in us reporting amounts that are too high or too low in any particular period. To date, we have not made any material adjustments to our prior estimates of accrued expenses.

Share-Based Compensation Expense

We account for share-based compensation expense in accordance with ASC 718, *Stock Based Compensation*. We have share-based compensation plans under which incentive and qualified stock options and restricted shares may be granted to employees, directors, and consultants. We measure the cost of the employee/director/consultant services received in exchange for an award of equity instruments based on the fair value for employees and directors and vesting date fair value of the award for consultants. We use the Black-Scholes option pricing model to determine the fair value of options as of the grant date and the Hull White I lattice model as of any option repricing dates. The model used to determine the fair value of options includes assumptions for expected volatility, risk-free interest rate, dividend yield and estimated expected term. Expected volatility is estimated considering the Company's own historical volatility. The risk-free interest rate is based on a treasury instrument whose term is consistent with the expected term of the stock options. The expected dividend yield is assumed to be zero as we have never paid dividends and have no current plans to pay any dividends on our common stock. Expected term is estimated using the "simplified" method, as outlined in SEC Staff Accounting Bulletin No. 107, "Share-Based Payment." We use the closing price of our common stock as quoted on Nasdaq to determine the fair value of restricted stock. We account for forfeitures as they occur, which may result in the reversal of compensation costs in subsequent periods as the forfeitures arise.

Stock option-based compensation expense recognized for the years ended December 31, 2024 and 2023 was $1.1 million and $1.4 million, respectively. Restricted stock-based compensation expense recognized for the years ended December 31, 2024 and 2023 was $5.6 million and $3.4 million, respectively.

Warrants

We have issued warrants associated with capital raises from time to time. 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*. The first step is to determine if the warrants are to be classified as either a liability or equity depending on the warrant terms. The second step is to then determine the value of the warrants. We measure the value of any liability classified warrants on their issuance date based on their fair value using the Black-Scholes pricing model. The model used to determine the fair value of these warrants utilizes certain unobservable inputs and this therefore considered a Level 3 fair value measurement. Inputs used in the model include assumptions for expected volatility, risk-free interest rate, dividend yield and estimated expected term. The liability classified 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 loss. 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. 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 year ended December 31, 2024 and 2023 resulted in a loss of $0.8 million and $11.7 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, 2024, based on criteria for effective internal control over financial reporting established in Internal Control — Integrated Framework (2013), issued by the Committee of Sponsoring Organizations of the Treadway Commission (COSO). 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, 2024, 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 15(d)-15(f) promulgated under the Exchange Act, during the fourth quarter of 2024 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, 2024, the following officers, as defined in Rule 16a-1(f) under the Exchange Act, as amended, adopted a "Rule 10b5-1 trading arrangement" as defined in Regulation S-K Item 408, as follows:

- On November 23, 2024, Don Wuchterl, a member of the Company's board of directors, adopted a Rule 10b5-1 trading arrangement providing for the sale from time to time of an aggregate of up to 40,176 shares of our common stock. The duration of the trading arrangement is until February 24, 2026, or earlier if all transactions under the trading arrangement are completed.

ITEM 9C. DISCLOSURE REGARDING FOREIGN JURISDICTIONS THAT PREVENT INSPECTIONS

Not applicable.

PART III

ITEM 10. DIRECTORS, EXECUTIVE OFFICERS AND CORPORATE GOVERNANCE

Directors and Executive Officers

Directors and Reports of Beneficial Ownership. The information required by this Item is incorporated herein by reference from the information to be contained in our 2025 Proxy Statement to be filed with the SEC within 120 days after December 31, 2024, in connection with the solicitation of proxies for our 2025 Annual Meeting of Stockholders (the "2025 Proxy Statement").

Code of Ethics. 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 waiver of a provision of the Code applicable to any executive officer or director, by posting such information on such website. We shall provide to any person without charge, upon request, a copy of the Code. Any such request must be made in writing to Abeona Therapeutics Inc., c/o Investor Relations, 6555 Carnegie Ave, 4th Floor, Cleveland, OH 44103.

Our corporate governance guidelines and the charters of the Audit Committee, Compensation Committee and Nominating and Corporate Governance Committee of the Board of Directors are available on our website at www.abeonatherapeutics.com under the heading "Investors & Media—Corporate Governance—Governance—Governance Documents." We shall 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 Abeona Therapeutics Inc., c/o Investor Relations, 6555 Carnegie Ave, 4th Floor, Cleveland, OH 44103.

ITEM 11. EXECUTIVE COMPENSATION

The information required by this Item is contained in the 2025 Proxy Statement and is incorporated herein by reference.

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

The information required by this Item is contained in the 2025 Proxy Statement and is incorporated herein by reference.

ITEM 13. CERTAIN RELATIONSHIPS AND RELATED TRANSACTIONS AND DIRECTOR INDEPENDENCE

The information required by this Item is contained in the 2025 Proxy Statement and is incorporated herein by reference.

ITEM 14. PRINCIPAL ACCOUNTING FEES AND SERVICES

The information required by this Item is contained in the 2025 Proxy Statement and 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:

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. (incorporated by reference to Exhibit 3.1 of our Form 8-K filed on July 9, 2024).
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*	2015 Equity Incentive Plan (incorporated by reference to Exhibit 4.1 to our Form S-8 filed May 11, 2015)
4.2*	2015 Equity Incentive Plan Amendment (incorporated by reference to our Definitive Proxy Statement on Schedule 14A filed on April 4, 2016)
4.3	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)
4.4	Form of Pre-Funded Warrant (incorporated by reference to Exhibit 4.1 of our Form 8-K filed on May 3, 2024)
4.5	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)
4.6	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)
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	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)
10.4	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)
10.5	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)
10.6*	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)
10.7*	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)
10.8*	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)
10.9	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)
10.10	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)

10.11+	Settlement Agreement and Mutual Release, dated November 12, 2021, between the Company and REGENXBIO Inc. (incorporated by reference to Exhibit 10.14 of our Form 10-K for the year ended December 31, 2021)
10.12	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)
10.13	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)
10.14+	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)
10.15	Retention Bonus Letter, dated June 15, 2023, to Vishwas Seschadri, Ph.D. (incorporated by reference to Exhibit 10.5 of our Form 10-Q for the quarter ended June 30, 2023)
10.16	Retention Bonus Letter, dated June 15, 2023, to Joseph Vazzano, Ph.D. (incorporated by reference to Exhibit 10.5 of our Form 10-Q for the quarter ended June 30, 2023)
10.17	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)
10.18	Securities Purchase Agreement, dated July 3, 2023 (incorporated by reference to Exhibit 10.1 of our Form 8-K filed on July 3, 2023)
10.19	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
10.20	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)
10.21	Underwriting Agreement, dated May 3, 2024 (incorporated by reference to Exhibit 1.1 of our Form 8-K filed on May 3, 2024)
16	Letter from Whitley Penn addressed to the United States Securities and Exchange Commission, dated October 17, 2023 (incorporated by reference to Exhibit 16.1 of our Form 8-K filed on October 18, 2023)
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 required to be filed as an exhibit to this report pursuant to Item 15(a)(3) of Form 10-K.
+ 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 20, 2025

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 20, 2025

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

Date: March 20, 2025

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

Date: March 20, 2025

/s/ Leila Alland
Leila Alland, Director

Date: March 20, 2025

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

Date: March 20, 2025

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

Date: March 20, 2025

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

Date: March 20, 2025

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

Date: March 20, 2025

/s/ Christine Silverstein
Christine Silverstein, Director

Date: March 20, 2025

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

Date: March 20, 2025

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

REPORT OF INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM

To the stockholders 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 (the "Company") as of December 31, 2024 and 2023, the related consolidated statements of operations and comprehensive loss, stockholders' equity and cash flows for each of the two years in the period ended December 31, 2024, 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, 2024 and 2023, and the results of its operations and its cash flows for each of the two years in the period ended December 31, 2024, 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 audit. 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.

Loan and Security Agreement and Common Stock Warrants — Refer to Notes 9 and 10 to the financial statements

Critical Audit Matter Description

As more fully described in Notes 9 and 10 to the financial statements, on January 8, 2024, the Company entered into a Loan and Security Agreement, as supplemented by a Supplement, with Avenue Venture Opportunities Fund, L.P. and Avenue Venture Opportunities Fund II, L.P. The Loan Agreement provides for senior secured term loans in an aggregate principal amount up to $50 million. 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 at any time while the Loans are outstanding, subject to certain terms and conditions, including ownership limitations. On January 8, 2024, in connection with entering into the Loan and Security Agreement, the Company issued to each of Avenue and Avenue II warrants to purchase up to $480,000 and $1,920,000 worth of shares, respectively, of Company common stock.

We identified the assessment of the accounting for the Loan and Security Agreement and related Common Stock Warrants as a critical audit matter because of the complexity in applying the accounting framework and the significant judgments made by management in the determination of the existence of embedded derivative liabilities and classification of the Common Stock Warrants related to the Loan and Security Agreement. Auditing these conclusions required a high degree of auditor judgment and an increased extent of effort.

How the Critical Audit Matter Was Addressed in the Audit

Our audit procedures related to accounting for the Loan and Security Agreement and Common Stock Warrants included the following, among others:

- We obtained and read the agreements associated with the Loan and Security Agreements, including the related Common Stock Warrant agreement, and tested the accuracy and completeness of the significant terms identified by management for purposes identifying embedded derivative liabilities and classification of the common stock warrants.
- With the assistance of professionals in our firm having expertise in the accounting treatment for debt and equity instruments, including warrants, we evaluated the Company's conclusions regarding the accounting treatment applied to the Loan and Security Agreement and Common Stock Warrants, including the identification and recognition of embedded derivatives, initial classification of embedded derivatives as a liability, and classification of Common Stock Warrants as a liability. We also evaluated the Company's subsequent reclassification of the derivative liability associated with the Conversion Right to equity on September 30, 2024 as it was considered indexed to the Company's own stock.
- Evaluated the completeness and accuracy of the disclosures related to the Loan and Security Agreement and Common Stock Warrants.

/s/ Deloitte & Touche LLP

Morristown, New Jersey
March 19, 2025

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, 2024	December 31, 2023
ASSETS		
Current assets:		
Cash and cash equivalents	$ 23,357	$ 14,473
Short-term investments	74,363	37,753
Restricted cash	338	338
Other receivables	1,652	2,444
Prepaid expenses and other current assets	1,143	729
Total current assets	100,853	55,737
Property and equipment, net	4,430	3,533
Operating lease right-of-use assets	3,552	4,455
Other assets	96	277
Total assets	$ 108,931	$ 64,002
LIABILITIES AND STOCKHOLDERS' EQUITY		
Current liabilities:		
Accounts payable	$ 3,441	$ 1,858
Accrued expenses	6,333	5,985
Current portion of long-term debt	5,926	—
Current portion of operating lease liability	823	998
Current portion payable to licensor	—	4,580
Other current liabilities	64	1
Total current liabilities	16,587	13,422
Long-term operating lease liabilities	3,262	4,402
Long-term debt	13,037	—
Warrant liabilities	32,014	31,352
Total liabilities	64,900	49,176
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, 2024 and 2023, respectively	—	—
Common stock - $0.01 par value; authorized 200,000,000 shares; 45,644,091 and 26,523,878 shares issued and outstanding as of December 31, 2024 and 2023, respectively	457	265
Additional paid-in capital	856,824	764,151
Accumulated deficit	(813,258)	(749,524)
Accumulated other comprehensive loss	8	(66)
Total stockholders' equity	44,031	14,826
Total liabilities and stockholders' equity	$ 108,931	$ 64,002

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

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

	For the years ended December 31,	
	2024	**2023**
Revenues:		
License and other revenues	$ —	$ 3,500
Expenses:		
Royalties	—	1,605
Research and development	34,360	31,091
General and administrative	29,851	19,004
Gain on operating lease right-of-use assets	—	(1,065)
Total expenses	64,211	50,635
Loss from operations	(64,211)	(47,135)
Interest income	4,246	2,117
Interest expense	(4,208)	(418)
Change in fair value of warrant and derivative liabilities	(755)	(11,695)
Other income	1,194	2,943
Net loss	$ (63,734)	$ (54,188)
Basic and diluted loss per common share	$ (1.55)	$ (2.53)
Weighted average number of common shares outstanding - basic and diluted	41,048,206	21,380,476
Other comprehensive income (loss):		
Change in unrealized gains related to available-for-sale debt securities	74	34
Foreign currency translation adjustments	—	29
Comprehensive loss	$ (63,660)	$ (54,125)

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	Accumulated	Accumulated Other Comprehensive	Total Stockholders'
	Shares	Amount	Capital	Deficit	Loss	Equity
Balance at December 31, 2022	17,719,720	$ 177	$ 722,049	$ (695,336)	$ (129)	$ 26,761
Stock-based compensation expense	—	—	4,768	—	—	4,768
Issuance of common stock in connection with restricted share awards, net of cancellations and shares settled for tax withholding settlement	1,859,869	18	(200)	—	—	(182)
Issuance of common stock, net of offering costs under open market sale agreement (ATM)	3,659,882	37	14,586	—	—	14,623
Issuance of common stock, net of offering costs under direct placement offering	3,284,407	33	22,948	—	—	22,981
Net loss	—	—	—	(54,188)	—	(54,188)
Other comprehensive income	—	—	—	—	63	63
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

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,	
	2024	**2023**
Cash flows from operating activities:		
Net loss	$ (63,734)	$ (54,188)
Adjustments to reconcile net loss to cash used in operating activities:		
Depreciation and amortization	2,004	2,288
Stock-based compensation expense	6,628	4,768
Change in fair value of warrant and derivative liabilities	755	11,695
Gain on operating lease right-of-use assets	—	(1,065)
Accretion and interest on short-term investments	276	(93)
Amortization of right-of-use lease assets	903	910
Non-cash interest	1,538	417
(Gain) loss on disposal of property and equipment	(2)	47
Change in operating assets and liabilities:		
Other receivables	792	(2,041)
Prepaid expenses and other current assets	(564)	(155)
Other assets	181	(234)
Accounts payable and accrued expenses	1,507	2,041
Lease liabilities	(1,315)	(1,196)
Change in payable to licensor	(5,000)	—
Other current liabilities	16	(203)
Net cash used in operating activities	(56,015)	(37,009)
Cash flows from investing activities:		
Capital expenditures	(2,446)	(331)
Proceeds from disposal of property and equipment	18	204
Purchases of short-term investments	(157,010)	(51,636)
Proceeds from maturities of short-term investments	120,198	51,971
Net cash (used in) provided by investing activities	(39,240)	208
Cash flows from financing activities:		
Proceeds from ATM sales of common stock, net of issuance costs	15,475	14,408
Proceeds from sales of common stock under direct placement offering, net of issuance costs	—	22,981
Payments related to net settlement of restricted share awards	(526)	(182)
Proceeds from sales of common stock, net of issuance costs	70,153	—
Proceeds from issuance of long-term debt	20,000	—
Payment of debt issuance costs	(963)	(150)
Net cash provided by financing activities	104,139	37,057
Net increase in cash, cash equivalents and restricted cash	8,884	256
Cash, cash equivalents and restricted cash at beginning of period	14,811	14,555
Cash, cash equivalents and restricted cash at end of period	$ 23,695	$ 14,811
Supplemental cash flow information:		
Cash and cash equivalents	$ 23,357	$ 14,473
Restricted cash	338	338
Total cash, cash equivalents and restricted cash	$ 23,695	$ 14,811
Supplemental non-cash flow information:		
Right-of-use asset obtained in exchange for new operating lease liabilities	$ —	$ 419
Derivative and warrant additions associated with loan and security agreement	$ 1,042	$ —
Reclassification of derivative liability to equity	$ 1,135	$ —
Changes in accrued property and equipment	$ 471	$ —
Cash paid for interest	$ 2,670	$ —
Cash paid for taxes	$ 7	$ 14

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

NOTE 1 – NATURE OF OPERATIONS AND SUMMARY OF SIGNIFICANT ACCOUNTING POLICIES

Background

Abeona Therapeutics Inc. (together with the Company's subsidiaries, "Abeona" or the "Company"), a Delaware corporation, is a clinical-stage biopharmaceutical company developing cell and gene therapies for life-threatening diseases. The Company's lead clinical program is for pz-cel, an autologous, cell-based gene therapy currently in development for recessive dystrophic epidermolysis bullosa ("RDEB"). 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 that the Company has exclusively licensed from the University of North Carolina at Chapel Hill and developed internally through its AAV vector research programs.

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 pz-cel and other product candidates, business planning, raising capital, establishing its intellectual property portfolio, acquiring or discovering product candidates, and providing 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 and anticipates such losses and negative cash flows will continue for the foreseeable future.

Since its inception, the Company has funded its operations primarily with proceeds from sales of shares of its stock. The Company has incurred recurring losses since its inception, including net losses of $63.7 million and $54.2 million for the years ended December 31, 2024 and 2023, respectively. As of December 31, 2024, the Company had an accumulated deficit of $813.3 million. To date, the Company has not generated any significant revenues and expects to continue to generate operating losses for the foreseeable future. As of the issuance date of these consolidated financial statements, the Company expects that its existing cash, cash equivalents, restricted cash and short-term investments of $98.1 million as of December 31, 2024, in addition to the $4.8 million in net proceeds from the Company's subsequent sale of common stock under the ATM Agreement, 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.

While the Company believes its capital resources are sufficient to fund the Company's on-going operations for the next 12 months from the issuance date of these consolidated financial statements, the Company's liquidity could be materially affected over this period by: (1) its ability to raise additional capital through equity offerings, debt financings, or other non-dilutive third-party funding; (2) costs associated with new or existing strategic alliances, or licensing and collaboration arrangements; (3) negative regulatory events or unanticipated costs related to pz-cel; (4) any other unanticipated material negative events or costs. One or more of these events or costs could materially affect the Company's liquidity. If the Company is unable to meet its obligations when they become due, the Company may have to delay expenditures, reduce the scope of its research and development programs, or make significant changes to its operating plan. The accompanying consolidated financial statements do not include any adjustments that might result from the outcome of this uncertainty.

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, fair value of warrant and derivative liabilities, the incremental borrowing rate related to the Company's operating leases and stock-based compensation. 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 serves as collateral for leased office space.

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.

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, 2024 and 2023, the Company had ERC receivables of $1.6 million and $2.1 million, respectively which was recorded in other receivables and as a component of other income in the consolidated statements of operations and comprehensive loss.

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.

Licensed Technology

The Company has entered into agreements to license the rights to certain technologies. The Company records the purchase price paid for the license, which represents fair value, on its consolidated balance sheet. Licensed technology is amortized over the life of the patent or the agreement. The Company maintains licensed technology on its consolidated balance sheet until either the licensed technology agreement underlying it is completed or the asset becomes impaired. When the Company determines that an asset has become impaired, as discussed below, or the Company abandons a project, the Company writes down the carrying value of the related intangible asset to its fair value and recognizes an impairment charge in the period in which the impairment occurs. The Company has fully written off the licensed technology as of December 31, 2024 and December 31, 2023.

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, 2024, 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 receivable are with customers that do not have a history of uncollectibility nor a history of significantly aged accounts receivables. As of December 31, 2024, 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 clinical-stage biopharmaceutical company developing cell and gene therapies for life-threatening diseases. Refer to Note 16 – 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.

The Company enters into licensing 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.

As part of the accounting for these arrangements, the Company must use 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. The Company uses judgment to determine whether milestones or other variable consideration, except for royalties, should be included in the transaction price as described further below. The transaction price is allocated to each performance obligation on a relative stand-alone selling price basis, for which the Company recognizes revenue as or when the performance obligations under the contract are satisfied. Amounts received prior to revenue recognition are recorded as deferred revenue.

Exclusive Licenses

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. An output method is generally used to measure progress toward complete satisfaction of a milestone. 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.

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, 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.

General and Administrative Expenses

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 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 general and administrative costs, respectively, in the consolidated financial statements. For the years ended December 31, 2024 and 2023, the Company did not recognize any uncertain tax positions, interest or penalty expense related to income taxes. It is not reasonably likely for the amounts of unrecognized tax benefits to significantly increase or decrease within the next 12 months. 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 Loss Per Share

Basic and diluted net loss per share is computed by dividing net 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 (Note 10). 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. Potential dilutive securities result from outstanding restricted stock, stock options, conversion features of loan agreements, and stock purchase warrants.

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

	For the year ended December 31,	
	2024	**2023**
Shares of common stock issuable upon exercise of stock options	176,587	179,001
Shares of common stock underlying restricted stock	3,320,811	2,448,169
Shares of common stock issuable upon exercise of conversion feature of loan agreement	614,251	—
Shares of common stock issuable upon exercise of warrants	9,987,560	9,397,879
Total	14,099,209	12,025,049

In January 2024 as part of the Loan and Security Agreement, see Note 9, the Company issued warrants to purchase $2,400,000 worth of shares of the Company's stock which have an exercise price equal to the lesser of (i) $4.75 and (ii) the price per share of the Company's net bona fide round of equity financing before September 30, 2024 (the "2024 Loan Agreement Warrants"). In connection with the underwritten common stock offering consummated on May 7, 2024, pursuant to the terms of the 2024 Loan Agreement Warrants, the exercise price was reduced to $4.07 per share and the shares issuable was calculated at 589,681 shares. 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. The Company included these shares for the year ended December 31, 2024 as shares of common stock issuable upon exercise of warrants in the table above and no shares for the year ended December 31, 2023.

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 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.

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 ("2024 Pre-Funded Warrants"). The prefunded 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 prefunded warrants were recorded at their relative fair value at issuance in the stockholders' equity section of the consolidated balance sheet and the prefunded warrants are considered outstanding shares in the basic earnings per share calculation given their nominal exercise price. On June 24, 2024 and December 2, 2024, 700,000 and 1,228,511, respectively of the 2024 Pre-Funded Warrants were exercised, leaving 4,214,125 of 2024 Pre-Funded Warrants outstanding as of December 31, 2024

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 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.

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 prefunded 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 prefunded warrants were recorded at their relative fair value at issuance in the stockholders' equity section of the consolidated balance sheet and the prefunded 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, 2024.

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 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 loss.

Recently Adopted Accounting Pronouncements

In November 2023, the Financial Accounting Standard Board ("FASB") issued Accounting Standards Update ("ASU") 2023-07, "Segment Reporting (Topic 280): *Improvements to Reportable Segment Disclosures*" ("ASU 2023-07"). ASU 2023-07 requires additional disclosures for segment reporting, including disclosure of the title and position of the Chief Operating Decision Maker and requires a public entity that has a single reportable segment to provide all the disclosures required by the amendments in ASU 2023-07, and all existing segment disclosures in Topic 280. ASU 2023-07 is effective for fiscal periods beginning after December 15, 2023, and interim periods within fiscal years beginning after December 15, 2024. The Company adopted ASU 2023-07 effective for its Annual Report on Form 10-K for the year ended December 31, 2024 and subsequent interim periods. Since ASU 2023-07 addresses only disclosures, the adoption of ASU 2023-07 did not have a significant impact on the Company's consolidated financial statements.

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 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 primarily related to the rate reconciliation and income taxes paid information. The standard is effective for annual reporting periods beginning after December 15, 2024, with early adoption permitted. The requirements of this ASU are disclosure related and will not have an impact on the Company's financial condition, results of operations, or cash flows. The Company is currently evaluating the impact of adopting this ASU on its income tax disclosures.

NOTE 3 – SHORT-TERM INVESTMENTS

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

	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

	December 31, 2023			
	Amortized Cost	Gross Unrealized Gain	Gross Unrealized Loss	Fair Value
Available-for-sale, short-term investments:				
U.S. treasury securities	$ 8,406	—	(13)	$ 8,393
U.S. federal agency securities	29,413	—	(53)	29,360
Total available-for-sale, short-term investments	$ 37,819	—	(66)	$ 37,753

As of December 31, 2024, 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, 2024, 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, 2024.

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, 2024 or 2023.

NOTE 4 – 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)	2024	2023
Laboratory equipment	5	$ 8,868	$ 6,935
Furniture, software and office equipment	3 to 5	1,113	986
Leasehold improvements	Shorter of remaining lease term or useful life	8,805	8,603
Construction-in-progress		624	—
Subtotal		19,410	16,524
Less: accumulated depreciation		(14,980)	(12,991)
Total property and equipment, net		$ 4,430	$ 3,533

Depreciation and amortization on property and equipment was $2.0 million and $2.3 million for the years ended December 31, 2024 and 2023, respectively. The Company incurred a gain on disposal of equipment of $2,000 and a loss on disposal of $47,000 during the years ended December 31, 2024 and 2023, respectively, which is reflected in other income in the consolidated statements of operations and comprehensive loss.

NOTE 5 – 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 expenses, and payables to licensor approximate their carrying amounts due to the relatively short maturity of these instruments. The estimated fair value of the Loan Agreement as of December 31, 2024, was $24.7 million. 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 measured at fair value on a recurring and non-recurring basis (in thousands):

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

Description	Fair Value at December 31, 2023	Level 1	Level 2	Level 3
Recurring Assets				
Cash equivalents				
Money market fund	$ 1,034	$ 1,034	$ —	$ —
Short-term investments				
U.S. treasury securities	8,393	8,393	—	—
U.S. federal agency securities	29,360	—	29,360	—
Total assets measured at fair value	$ 38,787	$ 9,427	$ 29,360	$ —
Liabilities				
Payable to licensor	$ 4,580	$ —	$ —	$ 4,580
Warrant liabilities	31,352	—	—	31,352
Total liabilities measured at fair value	$ 35,932	$ —	$ —	$ 35,932

Warrant Liabilities

As of December 31, 2024 and 2023, the Company had the following outstanding warrants:

	As of December 31,	
	2024	2023
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	7,609,879	7,609,879
Warrants issued as part of the 2024 Loan Agreement, expiration date January 2029, exercise price $4.07 per share	589,681	—

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 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 loss.

In January 2024, as part of the Loan and Security Agreement, see Note 9, the Company issued warrants to purchase $2,400,000 worth of shares of the Company's stock which have an exercise price 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 (the "2024 Loan Agreement Warrants"). In connection with the underwritten common stock offering consummated on May 7, 2024, pursuant to the terms of the 2024 Loan Agreement Warrants, the exercise price was reduced to $4.07 per share and the shares issuable was calculated at 589,681 shares. On September 30, 2024, per the terms of the 2023 Loan Agreement Warrants, the exercise price and the number of shares became set at $4.07 per share and 589,681 shares, respectively.

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

	As of December 31,	
	2024	2023
Beginning warrant liabilities	$ 31,352	$ 19,657
Fair value of warrants issued in connection with the Loan Agreement	220	—
Loss recognized in earnings from change in fair value	442	11,695
Ending warrant liabilities	$ 32,014	$ 31,352

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,	
	2024	2023
Common share price	$5.57	$5.01
Expected term (years)	1.96 – 4.02	2.96 – 3.84
Risk-free interest rate (%)	4.16% – 4.24%	3.84% – 3.92%
Volatility (%)	92.64% - 100.00%	100.00%
Expected dividend yield (%)	0%	0%

Derivative Liabilities

The Conversion Right embedded within the Loan Agreement (see Note 9 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 condensed consolidated statement of operations 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,	
	2024	2023
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 6 – SETTLEMENT LIABILITY

On November 12, 2021, the Company entered into a settlement agreement ("Settlement Agreement") with the Company's prior licensor REGENXBIO Inc. ("REGENXBIO") to resolve all existing disputes between the parties. In accordance with the Settlement Agreement, the Company agreed to pay REGENXBIO a total of $30.0 million, payable as follows: (1) $20.0 million paid in November 2021 after execution of the Settlement Agreement, (2) $5.0 million on the first anniversary of the effective date of the Settlement Agreement (paid in November 2022), and (3) $5.0 million upon the earlier of (i) the third anniversary of the effective date of the Settlement Agreement or (ii) the closing of a Strategic Transaction, as defined in the Settlement Agreement (paid in November 2024).

The Company recorded the payable due to REGENXBIO in the consolidated balance sheet based on the present value of the remaining payments due to REGENXBIO under the Settlement Agreement using an effective interest rate of 9.6%. The Company paid all amounts due in November 2024 and therefore there were no amounts outstanding as of December 31, 2024. The present value of the amount due as of December 31, 2023 was $4.6 million.

NOTE 7 – ACCRUED EXPENSES

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

	As of December 31,	
	2024	2023
Accrued employee compensation	$ 4,392	$ 3,688
Accrued contracted services and other	1,941	2,297
Total accrued expenses	$ 6,333	$ 5,985

NOTE 8 – LEASES

The Company leases space under operating leases for administrative, manufacturing and laboratory facilities in Cleveland, Ohio. The Company also leases office space in New York, New York, that the Company sublets. 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 additional space at the 6700 Euclid Avenue facility will allow the Company to convert office space at the 6555 Carnegie Avenue facility into additional manufacturing space to increase pz-cel manufacturing capacity. As the lease does not commence and the Company does not have access to the leased space until January 1, 2025, the impact of this lease agreement is not reflected in the consolidated financial statements of the Company as of December 31, 2024.

During 2023, the Company terminated one of its operating leases for office space. The termination resulted in a gain of $1.1 million representing the difference between the carry value of the right-of-use assets and the related lease liabilities. This gain was recorded in the year ended December 31, 2023, and is included in gain on operating lease right-of-use assets in the consolidated statement of operations and comprehensive loss.

During 2023, the Company modified one of its operating leases for office space to add up to 14,032 square feet to the Company's existing facility in Cleveland, Ohio. The lease modification resulted in the recognition of $0.4 million of additional right-of-use assets and related lease liabilities in the Company's consolidated balance sheet during the year ended December 31, 2023.

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 Company expects to receive $0.5 million in future sublease income through September 2025 from the two subleases noted above.

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

	As of December 31,	
	2024	2023
Current operating lease liability	$ 823	$ 998
Non-current operating lease liability	3,262	4,402
Total operating lease liability	$ 4,085	$ 5,400

Lease costs and rent are reflected in general and administrative expenses and research and development expenses in the consolidated statements of operations and comprehensive 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,	
	2024	2023
Operating lease cost	$ 1,288	$ 1,389
Variable lease cost	380	358
Short-term lease cost	49	63
Total operating lease costs	$ 1,717	$ 1,810

Cash paid for amounts included in the measurement of operating lease liabilities was $1.3 million and $1.2 million for the years ended December 31, 2024 and 2023, respectively.

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, 2024 were as follows (in thousands):

Future minimum lease payments and obligations	**Operating Leases**
2025	$ 853
2026	791
2027	807
2028	823
2029	859
Thereafter	834
Total undiscounted operating lease payments	4,967
Less: imputed interest	882
Present value of operating lease liabilities	$ 4,085

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 7.0% as of December 31, 2024.

The Company received $0.6 million and $0.5 million during the years ended December 31, 2024 and 2023, respectively, of sublease income which is recorded in other income on the consolidated statements of operations and comprehensive loss. Future cash receipts from the Company's sublease agreements as of December 31, 2024 are as follows (in thousands):

Future cash receipts	**Operating Subleases**
2025	$ 485
Total future cash receipts	$ 485

NOTE 9 – 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,	
	2024	2023
Loan Agreement Principal	$ 20,000	$ —
Accreted final payment fee	354	—
Unamortized debt issuance costs and discounts	(1,391)	—
Total long-term debt	18,963	—
Less: current maturities	5,926	—
Long-term debt, net of current maturities	$ 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 pz-cel in recessive dystrophic epidermolysis bullosa, 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 (the "Maturity Date"). As of December 31, 2024, the Tranche 2 is no longer available as the Company did not meet the Tranche 2 criteria.

The loan principal is repayable in equal monthly installments beginning on May 1, 2025. 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%. The stated interest rate and effective interest rate as of December 31, 2024 was 13.50% and 22.09%, respectively.

The Company may, subject to certain parameters, voluntarily prepay the Loans, in whole, at any time. If prepayment occurs on or before the one-year anniversary of the Closing Date, the Company is required to pay a prepayment fee equal to 3.00% of the principal amount of the Loans prepaid; if prepayment occurs after the one-year anniversary of the Closing Date and on or before the two-year anniversary of the Closing Date, the Company is required to pay a fee equal to 2.00% of the principal amount of the Loans; if prepayment occurs after the two-year anniversary of the Closing Date, 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, Tranche 2 Loans and Discretionary Tranche Loans is also due upon the Maturity Date 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 condensed consolidated statement of operations 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 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 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 10 below.

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

2025	$	5,926
2026		8,889
2027		5,185
Total principal	$	20,000

NOTE 10 – 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, 2024 and 2023.

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, 2024, 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, 2024 and 2023, other than the change in fair value of the warrants.

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 and on December 2, 2024 1,228,531 of the 2024 Pre-Funded Warrants were exercised, leaving 4,214,125 2024 Pre-Funded Warrants outstanding as of December 31, 2024. 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 and diluted earnings per share calculation for year ended December 31, 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 2,825,954 and 3,659,882 shares of its common stock under the ATM Agreement during the years ended December 31, 2024 and 2023, respectively, resulting in net proceeds of $15.5 million and $14.4 million during the years ended December 31, 2024 and 2023, respectively. Subsequent to December 31, 2024 and through March 1, 2025, the Company sold 915,925 shares of its common stock under the ATM Agreement resulting in $4.8 million in net proceeds.

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 commons 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 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, 2024, there were 7,609,879 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. There was no warrant activity during the year ended December 31, 2024 and 2023, 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, 2024. The 2023 Pre-Funded Warrants are classified as equity in accordance with ASC 815, *Derivatives and Hedging*, 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 year ended December 31, 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 each of Avenue and Avenue 2 (collectively, the "Warrant Holders") warrants to purchase up to $480,000 and $1,920,000 worth of shares, respectively, of Company common stock (collectively, the "January Warrants"). The Warrants expire on January 8, 2029 (the "Expiration Date") 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 and 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 the Expiration Date, 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.

NOTE 11 – STOCK-BASED COMPENSATION

The Company previously granted stock options under its 2005 Equity Incentive Plan (the "2005 Incentive Plan"), under which no further grants can be made. In addition, 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, 2024, there were 5,251,251 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, 2024, there were 584,700 shares available to be granted under the Inducement Plan.

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

	For the year ended December 31,	
	2024	2023
Research and development	$ 1,561	$ 1,085
General and administrative	5,067	3,683
Total stock-based compensation expense	$ 6,628	$ 4,768

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 recognize 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, 2024 and 2023.

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, 2024 and 2023.

	Number of Options	Weighted Average Exercise Price	Weighted Average Remaining Contractual Term (years)	Aggregate Intrinsic Value (in thousands)
Outstanding at December 31, 2022	240,770	$ 37.04	6.42	$ —
Granted	—	$ —	—	$ —
Cancelled/forfeited	(61,769)	$ 32.59	—	$ —
Exercised	—	$ —	—	$ —
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	176,587	$ 38.64	5.83	$ 6
Exercisable	163,386	$ 39.07	5.77	$ 4
Unvested	13,201	$ 33.29	6.65	$ 2

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, 2024, the total compensation cost related to non-vested option awards not yet recognized was $0.3 million with a weighted average remaining vesting period of 0.5 years.

As of December 31, 2024, there are no options outstanding under the 2005 Incentive Plan. Further information regarding options outstanding under the 2015 Incentive Plan as of December 31, 2024 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	20,088	7.0	$ 16.52	15,154	6.9	$ 17.16
25.50	47.00	104,279	5.5	33.52	99,263	5.4	33.38
54.50	58.50	52,020	6.2	56.96	48,769	6.2	56.96
164.75	183.50	200	4.1	164.75	200	4.1	164.75
		176,587			163,386		

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, 2022	816,958	$	5.35
Granted	1,958,159	$	3.99
Cancelled/forfeited	(56,398)	$	4.32
Vested	(270,550)	$	5.59
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

As of December 31, 2024, there was $11.9 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 2.1 years. The total fair value of restricted stock awards that vested was $4.7 million and $1.5 million during the years ended December 31, 2024 and 2023, respectively.

NOTE 12 – 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, 2024, the Company is subject to remaining milestone payments totaling approximately $0.2 million which is due upon FDA approval of pz-cel.

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 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. As of December 31, 2024, no milestone or royalty payments under this agreement have been made.

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. As of December 31, 2024, no milestone or royalty payments under this agreement have been made. 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.

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. As of December 31, 2024, no milestone or royalty payments under this agreement have been made. 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.

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. Under the inventory purchase agreement, the Company sold to Taysha certain inventory and other items related to ABO-202. 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) $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 is obligated to pay a portion of milestone payments and royalties on net sales received from Taysha to the UNC.. The event-based milestone payments are 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 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 has not recognized any revenue during the years ended December 31, 2024 and 2023, respectively based on event-based-milestone payments. The Company has no contract assets or liabilities as of December 31, 2024 and 2023 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 the 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 has 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 nil and $3.5 million in revenue during the years ended December 31, 2024 and 2023. The revenue recognized was related to clinical milestones achieved by our sublicensor as per the sublicense agreement noted above. As of December 31, 2024 and 2023, the Company does 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.

Additionally, pursuant to the License Agreement, Ultragenyx will reimburse the Company for certain development and transition costs actually incurred by the Company. These costs are passed through to Ultragenyx without mark-up. The Company has determined that these costs are not incurred for the purpose of satisfying any performance obligation under the License Agreement. Accordingly, the reimbursement of these costs is recognized as a reduction of research and development costs. As of December 31, 2024 and 2023, the Company does not have any contract assets or contract liabilities as a result of this transaction.

NOTE 13 – 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 ($23,000 in 2024 and $22,500 in 2023 for employees who are under age 50 and $30,500 in 2024 and $30,000 in 2023 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 $0.5 million and $0.3 million for the years ended December 31, 2024 and 2023.

NOTE 14 – INCOME TAXES

Income tax expense differs from the statutory amounts for each of the following years (in thousands):

	For the year ended December 31,	
	2024	**2023**
Income taxes at U.S. statutory rate	$ (13,384)	$ (11,379)
State tax, net of federal benefit	(679)	(242)
Research and development credit	(1,535)	(1,137)
Deferred true ups	8,032	—
Valuation allowance	5,418	8,687
Change in fair value of warrant liabilities	159	2,456
Expired tax losses and credits	2,116	1,503
Permanent differences	(127)	112
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,	
	2024	**2023**
Deferred tax assets (liabilities):		
Net operating loss carryforwards	$ 88,059	$ 78,698
General business credit carryforwards	6,000	4,752
State credits	2,780	2,780
Property, equipment and goodwill	1,002	887
Stock based compensation	2,463	11,983
Intangible assets	661	615
Accruals	107	347
Capitalized research and development	13,264	8,843
Other	70	83
Gross deferred tax assets	114,406	108,988
Valuation allowance	(114,406)	(108,988)
Net deferred taxes	$ —	$ —

As of December 31, 2024, the Company identified adjustments related primarily to the recognition of deferred tax assets for stock-based compensation. As a result, the Company has written off $8.0 million of deferred tax assets in the current period, with a corresponding adjustment to the valuation allowance. There was no impact to total tax expense in the prior periods or current period.

Net operating Loss and Other Carryforwards

As of December 31, 2024, the Company had $416.1 million of U.S. federal net operating loss carryforwards and $6.0 million of general business credit carryforwards. These carryforwards expire as follows (in thousands):

	Net operating loss carryforwards	General business credit carryforwards
2025	$ 2,370	$ 182
2026	7,160	72
2027	9,977	93
2028	6,886	141
2029	7,908	83
Thereafter	65,644	5,429
	$ 99,945	$ 6,000

On December 22, 2017, the "Tax Cuts and Jobs Act" was signed into law. The tax reform has the following effects on the Company: (1) permanently reduces the maximum corporate income tax rate from 35% to 21% effective for tax years beginning after December 31, 2017, (2) allows temporary 100% expensing for certain business assets and property placed in service after September 27, 2018 and before January 1, 2023, (3) disallows NOL carrybacks but allows for the indefinite carryforward of those NOLs which applies to losses arising in tax years beginning after December 31, 2018 and, (4) limits NOL deductions for each year equal to the lesser of the available carryover or 80% of a taxpayer's pre-NOL deduction taxable income. This applies to losses arising in tax years ending on or after December 31, 2017. As of December 31, 2024 and 2023, the Company has concluded that it is more likely than not that the Company will not realize the benefit of its deferred tax assets due to its history of losses. Accordingly, the net deferred tax assets have been fully reserved.

In accordance with Section 382 of the Internal Revenue Code of 1986, as amended, a change in equity ownership of greater than 50% within a three-year period results in an annual limitation on the Company's ability to utilize its NOL carryforwards created during the tax periods prior to the change in ownership. The Company has not completed an ownership change analysis pursuant to Section 382. Because the Company has incurred cumulative net operating losses since inception, all tax years remain open to examination by U.S. federal and state income tax authorities.

As of December 31, 2024, the Company had $316.2 million of U.S. federal net operating loss carryforwards that do not expire and can be carried forward indefinitely. Such net operating loss carryforwards can only be used to offset 80% of taxable income in any given tax year. The Company also has $13.3 million of state net operating loss carryforwards in varying amounts depending on the different state tax laws.

The Company acquired MacroChem Corporation on March 25, 2009, and Somanta Pharmaceuticals, Inc. on January 4, 2008. Both of these corporations were loss-making entities at the time of acquisition. As a result, the net operating losses related to those acquisitions may be subject to annual limitations. The Company has not performed a study to determine whether or not there is such a limitation.

Valuation Allowance

At December 31, 2024 and 2023, 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 2024, the valuation allowance increased by approximately $5.4 million. In 2023, the valuation allowance increased by approximately $8.7 million.

Unrecognized Tax Benefits

At December 31, 2024 and 2023, 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 2020 and forward, and 2019 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 15 – 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, 2024 and 2023, there was no litigation against the Company.

NOTE 16 – 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 clinical-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 clinical-stage biopharmaceutical segment are the same as those described in the summary of significant accounting policies. The CODM assesses performance for the clinical-stage biopharmaceutical segment based on net loss, which is reported on the consolidated statements of operations and comprehensive loss as consolidated net 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 not generated any product revenue. The Company expects to continue to incur significant expenses and operating losses while it seeks regulatory approval for pz-cel.

As such, the CODM uses cash forecast models in deciding how to invest into the clinical-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 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 reviewed by the CODM for the years ended December 31, 2024, and 2023:

	For the year ended December 31,	
	2024	2023
License and other revenues	$ —	$ 3,500
Research and development costs		
Salaries & related costs	15,345	11,373
Non-cash stock-based compensation	1,561	1,086
Other research and development costs (a)	17,454	18,632
Total research and development costs	34,360	31,091
General and administrative costs		
Salaries & related costs	$ 10,729	$ 6,942
Non-cash stock-based compensation	5,067	3,682
Pre-commercial preparation costs	4,818	1,224
Other general and administrative costs (b)	9,237	7,156
Total general and administrative costs	$ 29,851	$ 19,004
Other segment items (c)	(477)	7,593
Net loss	$ (63,734)	$ (54,188)

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

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

(c) Other segment items includes royalties, interest income, interest expense, change in fair value of warrant and derivative liabilities and other income.

NOTE 17 – SUBSEQUENT EVENTS

In January of 2025, 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 1,956,280 shares of the Company's common stock. Total stock compensation estimated for these awards at the time of grant was $10.3 million, with $8.7 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 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, 333-161642, and 333-125796 on Form S-8 of our report dated March 19, 2025, relating to the financial statements of Abeona Therapeutics Inc. appearing in this Annual Report on Form 10-K for the year ended December 31, 2024.

/s/ DELOITTE & TOUCH LLP

Morristown, New Jersey
March 19, 2025

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 20, 2025

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 20, 2025

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, 2024 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 20, 2025

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

Date: March 20, 2025

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.

[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)